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together for short lives

Synopsis

International digest of children's palliative care research abstracts

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Together for Short Lives is the leading UK charity for all children with life-threatening and life-limiting conditions and all those who support, love and care for them. We support families, professionals and services, including children's hospices. Our work helps to ensure that children can get the best possible care, wherever and whenever they need it.

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Too few medicines for children with cancer - reply

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JAMA Pediatr 2014; 168(6): 583-584

http://www.ncbi.nlm.nih.gov/pubmed/24886802

Parental decision-making for medically complex infants and children: An integrated literature review

Allen, K. A.
Int J Nurs Stud 2014; 51(9): 1289-1304

BACKGROUND: Many children with life-threatening conditions who would have died at birth are now surviving months to years longer than previously expected. Understanding how parents make decisions is necessary to prevent parental regret about decision-making, which can lead to psychological distress, decreased physical health, and decreased quality of life for the parents. OBJECTIVE: The aim of this integrated literature review was to describe possible factors that affect parental decision-making for medically complex children. The critical decisions included continuation or termination of a high-risk pregnancy, initiation of life-sustaining treatments such as resuscitation, complex cardiothoracic surgery, use of experimental treatments, end-of-life care, and limitation of care or withdrawal of support. DESIGN: PubMed, Cumulative Index of Nursing and Allied Health Literature, and PsycINFO were searched using the combined key terms 'parents and decision-making' to obtain English language publications from 2000 to June 2013. RESULTS: The findings from each of the 31 articles retained were recorded. The strengths of the empirical research reviewed are that decisions about initiating life support and withdrawing life support have received significant attention. Researchers have explored how many different factors impact decision-making and have used multiple different research designs and data collection methods to explore the decision-making process. These initial studies lay the foundation for future research and have provided insight into parental decision-making during times of crisis. CONCLUSIONS: Studies must begin to include both parents and providers so that researchers can evaluate how decisions are made for individual children with complex chronic conditions to understand the dynamics between parents and parent-provider relationships. The majority of studies focused on one homogenous diagnostic group of premature infants and children with complex congenital heart disease. Thus comparisons across other child illness categories cannot be made. Most studies also used cross-sectional and/or retrospective research designs, which led to researchers and clinicians having limited understanding of how factors change over time for parents.

http://www.ncbi.nlm.nih.gov/pubmed/24636443

Inclusion--in whose best interest? How pediatricians can effectively advocate for children with neurodevelopmental disabilities

Wilcox, D. W. and A. H. Hoon
Dev Med Child Neurol 2014; 56(3): 206-209

http://www.ncbi.nlm.nih.gov/pubmed/23889529

Potential brain death organ donors - challenges and prospects: A single center retrospective review

Al-Maslamani, Y., et al.
Saudi J Kidney Dis Transpl 2014; 25(3): 589-596

Organ donation after brain death (BD) is a major source for obtaining transplantable organs for patients with end-stage organ disease (ESOD). This retrospective, descriptive study was carried out on all potential BD patients admitted in different intensive care units (ICUs) of the Hamad medical Corporation (HMC), Doha, Qatar during a period from January 2011 to April 2012. Our aim was to evaluate various demographic criteria and challenges of organ donation among potential BD organ donors and plan a strategy to improve the rate of organ donation in Qatar. Various aspects of BD patients in the ICUs and their possible effects on organ donation were studied. The time intervals analyzed to determine the possible causes of delay of organ retrieval were: time of diagnosing fixed dilated pupils in the ICU, to performing the first BD test, then to the second BD test, to family approach, to organ retrieval and/or circulatory death (CD) without organ retrieval. There were a total of 116 potential BD organ donors of whom 96 (82.75%) were males and 20 (17.25%) were females. Brain hemorrhage and head injury contributed to 37 (31.9%) and 32 (27.6%) BD cases, respectively. Time interval between diagnosing fixed dilated pupil and performing the first test of BD was delayed >24 h in 79% of the cases and between the first and second BD tests was >6 h in 70.8% of the cases. This delay is not compatible with the Hamad Medical Corporation (HMC) policy for BD diagnosis and resulted in a low number of organs retrieved. BD organ donation, a potential source for organs to save patients with ESOD has several pitfalls and every effort should be made to increase the awareness of the public as well as medical personnel to optimize donation efficacy.

http://www.ncbi.nlm.nih.gov/pubmed/24821157

A charter for the rights of the dying child

Benini, F., et al.
Lancet 2014; 383(9928): 1547-1548

http://www.ncbi.nlm.nih.gov/pubmed/24792853

Obtaining informed consent in pediatric clinical trials

Benkouiten, S., et al.
J Clin Epidemiol 2014; 67(7): 840-841

http://www.ncbi.nlm.nih.gov/pubmed/24739464

Children growing up with HIV infection: The responsibility of success

Bernays, S., et al.
Lancet 2014; 383(9925): 1355-1357

http://www.ncbi.nlm.nih.gov/pubmed/24529338

Deficiencies and Missed Opportunities to Formulate Clinical Guidelines in Australia for Withholding or Withdrawing Life-Sustaining Treatment in Severely Disabled and Impaired Infants

Bhatia, N. and J. Tibballs

J Bioeth Inq 2014

This paper examines the few, but important legal and coronial cases concerning withdrawing or withholding life-sustaining treatment from severely disabled or critically impaired infants in Australia. Although sparse in number, the judgements should influence common clinical practices based on assessment of "best interests" but these have not yet been adopted. In particular, although courts have discounted assessment of "quality of life" as a legitimate component of determination of "best interests," this remains a prominent component of clinical guidelines. In addition, this paper highlights the lack of uniform clinical guidelines available to medical professionals and parents in Australia when making end-of-life decisions for severely ill infants. Thus, it is argued here that there is a need for an overarching prescriptive uniform framework or set of guidelines in end-of-life decision-making for impaired infants. This would encourage greater transparency, consistency, and some degree of objectivity in an area that often appears subjective.

http://www.ncbi.nlm.nih.gov/pubmed/25173981

The potential for neonatal organ donation in a children's hospital

Charles, E., et al.

Arch Dis Child Fetal Neonatal Ed 2014; 99(3): F225-229

OBJECTIVE: Neonatal organ donation does not occur in the UK. Unlike in other European countries, Australasia and the USA death verification/certification standards effectively prohibit use of neurological criteria for diagnosing death in infants between 37 weeks' gestation and 2 months of age and therefore donation after neurological determination of death. Neonatal donation after circulatory definition of death is also possible but is not currently undertaken. There is currently no specific information about the potential neonatal organ donation in the UK; this study provides this in one tertiary children's hospital. DESIGN: Retrospective mortality database, clinical document database and patient notes review. SETTING: Neonatal and Paediatric Intensive Care in a tertiary children's hospital. PATIENTS: Infants dying between 37 weeks' gestation and 2 months of age between 1 January 2006 and 31 October 2012. Potential assessed using current UK guidelines for older children and neonatal criteria elsewhere. RESULTS: 84 infants died with 45 (54%) identified as potential donors. 34 (40%) were identified as potential donors after circulatory definition of death and 11 (13%) were identified as being theoretical potential donors after neurological determination of death. 10 (12%) were identified as unlikely donors due to relative contraindications and 39 (46%) were definitely not potential donors. CONCLUSIONS: With around 60 paediatric organ donors in the UK annually, there does appear significant potential for donation within the neonatal population. Reconsideration of current infant brain stem death guidelines is required to allow parents the opportunity of donation after neurological determination of death, together with mandatory training in organ donation for neonatal teams, which will also facilitate donation after circulatory definition of death.

http://www.ncbi.nlm.nih.gov/pubmed/24636932

Life threatening illness in popular movies-a first descriptive analysis

Drukarczyk, L., et al.
Springerplus 2014; 3: 411

In the last two decades, public attention towards illness, dying and death has evolved. In particular, advance care planning, living wills, end-of-life care, and autonomy are increasingly discussed. How this change in public awareness has influenced the presentation of dying and death in cinema needs clarification. Over a one year period, November 2011 until October 2012, a systematic search was conducted to identify movies dealing with incurable diseases produced in 1991-2010 35 movies could be identified and were analyzed in detail and investigated the presentation of illness and death. The number of movies focusing on terminal illness, dying, and death has increased since 1991. The total number of movies that made the yearly German Federal Film Board (FFA) hit list and included a focus on terminal illness, dying, and death increased from 1991 (1 movie) to 2011 (6 movies). The gender of the main characters suffering from terminal illness was distributed equally; three movies portrayed terminally ill children. More than one third of the terminally ill characters died in hospital. The terms “palliative” or “hospice care” were not mentioned once in any films. The number of movies dealing with terminal illness continues to increase and a considerable audience has shown interest in these films. Due to a limited true-to-life performance in the films, a presentation closer to reality could be a major public educational resource.

http://www.ncbi.nlm.nih.gov/pubmed/25161864

Neonatal euthanasia: Lessons from the Groningen Protocol

Eduard Verhagen, A. A.
Semin Fetal Neonatal Med 2014; 19(5): 296-299

Decisions about neonatal end-of-life care have been studied intensely over the last 20 years in The Netherlands. Nationwide surveys were done to quantify these decisions, provide details and monitor the effect of guidelines, new regulations and other interventions. One of those interventions was the Groningen Protocol for newborn euthanasia in severely ill newborns, published in 2005. Before publication, an estimated 20 cases of euthanasia per year were performed. After publication, only two cases in five years were reported. Studies suggested that this might be partly caused by the lack of consensus about the dividing line between euthanasia and palliative care. New recommendations about paralytic medication use in dying newborns were issued to increase transparency and to improve reporting of euthanasia. New surveys will be needed to measure the effects of these interventions. This cycle of interventions and measurements seems useful for continuous improvement of end-of-life care in newborns.

http://www.ncbi.nlm.nih.gov/pubmed/25150794

Does the Belgian law legalising euthanasia for minors really address the needs of life-limited children?

Friedel, M. 2014
Int J Palliat Nurs; 20(6): 265-267

http://www.ncbi.nlm.nih.gov/pubmed/2504086

Association between Physician Orders for Life-Sustaining Treatment for Scope of Treatment and in-hospital death in Oregon

Fromme, E. K., et al.

J Am Geriatr Soc 2014; 62(7): 1246-1251

OBJECTIVES: To examine the relationship between Physician Orders for Life-Sustaining Treatment (POLST) for Scope of Treatment and setting of care at time of death. DESIGN: Cross-sectional. SETTING: Oregon in 2010 and 2011. PARTICIPANTS: People who died of natural causes. MEASUREMENTS: Oregon death records containing cause and location of death were matched with POLST orders for people with a POLST form in the Oregon POLST registry. Logistic regression was used to measure the association between POLST orders and location of death. RESULTS: Of 58,000 decedents, 17,902 (30.9%) had a POLST form in the registry. Their orders for Scope of Treatment were comfort measure only, 11,836 (66.1%); limited interventions, 4,787 (26.7%); and full treatment, 1,153 (6.4%). Comfort measures only (CMO) orders advise avoiding hospitalization unless comfort cannot be achieved in the current setting; 6.4% of participants with POLST CMO orders died in the hospital, compared with 44.2% of those with orders for full treatment and 34.2% for those with no POLST form in the registry. In the logistic regression, the odds of dying in the hospital of those with an order for limited interventions was 3.97 times as great (95% CI = 3.59-4.39) as of those with a CMO order, and the odds of those with an order for full treatment was 9.66 times as great (95% CI = 8.39-11.13). CONCLUSIONS: The association with numbers of deaths in the hospital suggests that end-of-life preferences of people who wish to avoid hospitalization as documented in POLST orders are honoured.

http://www.ncbi.nlm.nih.gov/pubmed/24913043

Real life clinic visits do not match the ideals of shared decision making

Lipstein, E. A., et al.

J Pediatr 2014; 165(1): 178-183 e171

OBJECTIVE: To use observation to understand how decisions about higher-risk treatments, such as biologics, are made in pediatric chronic conditions. METHODS: Gastroenterology and rheumatology providers who prescribe biologics were recruited. Families were recruited when they had an outpatient appointment in which treatment with biologics was likely to be discussed. Consent/assent was obtained to video the visit. Audio of the visits in which a discussion of biologics took place were transcribed and analyzed. Our coding structure was based on prior research, shared decision making (SDM) concepts, and the initial recorded visits. Coded data were analyzed using content analysis and comparison with an existing model of SDM. RESULTS: We recorded 21 visits that included discussions of biologics. In most visits, providers initiated the decision-making discussion. Detailed information was typically given about the provider's preferred option with less information about other options. There was minimal elicitation of preferences, treatment goals, or prior knowledge. Few parents or patients spontaneously stated their preferences or concerns. An implicit or explicit treatment recommendation was given in nearly all visits, although rarely requested. In approximately one-third of the visits, the treatment decision was never made explicit, yet steps were taken to implement the provider's preferred treatment. CONCLUSIONS: We observed limited use of SDM, despite previous research indicating that parents wish to collaborate in decision making. To better achieve SDM in chronic conditions, providers and families need to strive for bidirectional sharing of information and an explicit family role in decision making.

http://www.ncbi.nlm.nih.gov/pubmed/24795203

Minors' decision-making capacity to refuse life-saving and life-sustaining treatment: Legal and psychiatric perspectives

Mendelson, D. and I. Haywood

J Law Med 2014; 21(4): 762-773

Laws in Belgium and The Netherlands permit euthanasia and assisted suicide for seriously ill children who experience "constant and unbearable suffering"--they have the capacity to request death by lethal injection if they convey a "reasonable understanding of the consequences" of that request. The child's capacity to understand death is therefore a prerequisite to the implementation of the request. However, modern neuro-psychological and fMRI (functional Magnetic Resonance Imaging) studies of the relationship between the neuro-anatomical development of the brain in human beings and their emotional and experiential capacity demonstrates that both are not fully developed until the early 20s for girls and mid-20s for boys. Unlike Belgium and The Netherlands, the clinical and legal implications of the immaturity of the brain on medical decision-making of minors, in particular life and death decisions, have been implicit in the Australian courts' approach to the refusal of life-saving and life-sustaining treatment by minors. This approach is exemplified by X v Sydney Children's Hospitals Network [2013] NSWCA 320 (and a series of earlier cases).

http://www.ncbi.nlm.nih.gov/pubmed/25087358

Barriers of healthcare providers against end-of-life discussions with pediatric cancer patients

Yoshida, S., et al.

Jpn J Clin Oncol 2014; 44(8): 729-735

OBJECTIVE: End-of-life discussions with patients can be one of the most difficult and stressful tasks for the oncologist. However, little is known about the discussions that healthcare providers have with patients in such situations and the difficulties they face. The primary end points of this study were to describe the contents of end-of-life discussion in the pediatric setting and the barriers to end-of-life discussion for pediatric patients, as perceived by pediatric healthcare providers. METHODS: Participants were 10 healthcare providers. Semi-structured interviews were conducted, and the KJ method was performed to analyze the data. RESULTS: We found 23 barriers against end-of-life discussion with pediatric cancer patients. These barriers were classified as follows: healthcare provider factors, patient factors, parent factors and institutional or cultural factors. In addition to barriers found in previous studies, some unique barriers were uncovered such as, 'Lack of confidence to face the patient after the discussion', 'Uncertain responsibility for treatment decision-making' and 'No compelling reason to discuss'. Healthcare providers actively discussed the purpose of treatment and the patients' wishes and concerns; however, they were reluctant to deal with the patients' own impending death and their estimated prognosis. CONCLUSIONS: End-of-life discussion with pediatric patients differs from that with adult patients. Further studies are required to analyze pediatric cases associated with end-of-life discussion and carefully discuss its adequacy, pros and cons.

http://www.ncbi.nlm.nih.gov/pubmed/24903853

Rare diseases in children: towards better and fairer treatment

Lancet 2014; 384 (9939): 208

Practice and specialist roles defined in guide

Nurs Child Young People 2014; 26(6): 6

The RCN has published a guide to clarify the distinction between advanced practice and specialist roles in children and young people's nursing practice.

http://www.ncbi.nlm.nih.gov/pubmed/25004029

Caring for Children Who Have Severe Neurological Impairment: A Life with Grace

Hauer Julie M

Nurs Child Young People 2014; 26(7): 15

CARING FOR children and young people with severe neurological conditions is often complex and can pose challenges for professionals and families. This comprehensive text uses an evidence-based approach to explore the challenges and covers topics, ranging from treating pain to end of life care.

http://www.ncbi.nlm.nih.gov/pubmed/25200237

Death and bereavement: A whole-school approach

Adams, J.

Community Pract 2014; 87(8): 35-36

http://www.ncbi.nlm.nih.gov/pubmed/25226706

Transforming children's palliative care through the International Children's Palliative Care Network

Downing, J., et al. 2014

Int J Palliat Nurs 2014; 20(3): 109-114

http://www.ncbi.nlm.nih.gov/pubmed/24675535

Improving the practice of child death overview panels: A paediatric perspective

Allen, L., et al.
Arch Dis Child 2014; 99(3): 193-196.

OBJECTIVE: In England, every death in childhood is reviewed by a local multidisciplinary Child Death Overview Panel (CDOP) with the intention of understanding causation and implementing interventions to reduce future deaths. This study aimed to establish how well panels work from the perspective of the paediatricians involved and to ascertain whether they deliver good value and identify areas for improvement. DESIGN: A questionnaire was sent to every CDOP paediatrician in the country (n=93). Questions focused on the quality of CDOP case discussions as well as examples of effective and significant recommendations. Responses were analysed using simple quantitative and qualitative methods. RESULTS: 84/93 (90%) of the paediatricians responded. Among the respondents, 60 (71%) believe that investment in CDOPs is offering good value, 73 (87%) feel that case discussions are rigorous and consistent and over 90% believe that the correct issues are emerging from discussions. However, responders noted many areas for improvement: 40 (48%) suggested devolving the discussion of specialist deaths (eg, neonates) to hospital-based review meetings or holding themed meetings with invited specialists, 11 (13%) suggested filtering out cases where learning is unlikely before full CDOP meetings and 13 (15%) called for national integration and analysis of data. CONCLUSIONS: In this time of economic austerity it is vital that the CDOPs add value to the invested resources. Although CDOP paediatricians feel that panels are working well, there is scope for improvement through enhancing relationships with commissioning bodies, aggregate review and analysis of CDOP data at a national level and consideration of specialist and/or network review of certain categories of deaths such as cardiac surgery, oncology and neonates.

http://www.ncbi.nlm.nih.gov/pubmed/24255566

Learning from child death review in the USA, England, Australia, and New Zealand

Fraser, J., et al.
Lancet 2014; 384(9946): 894-903

Despite pronounced reductions in child mortality in industrialised countries, variations exist within and between countries. Many child deaths are preventable, and much could be done to further reduce mortality. For the family, their community, and professionals caring for them, every child's death is a tragedy. Systematic review of all child deaths is grounded in respect for the rights of children and their families, and aimed towards the prevention of future child deaths. In a Series of three papers, we discuss child death in high-income countries in the context of evolving child death review processes. This paper outlines the background to and development of child death review in the USA, England, Australia, and New Zealand. We consider the purpose, process, and outputs of child death review, and discuss how these factors can contribute to a greater understanding of children's deaths and to knowledge for the prevention of future child deaths.

http://www.ncbi.nlm.nih.gov/pubmed/25209489

Off-label use of drugs in children

Frattarelli, D. A., et al.
Pediatrics 2014; 133(3): 563-567

The passage of the Best Pharmaceuticals for Children Act and the Pediatric Research Equity Act has collectively resulted in an improvement in rational prescribing for children, including more than 500 labeling changes. However, off-label drug use remains an important public health issue for infants, children, and adolescents, because an overwhelming number of drugs still have no information in the labeling for use in pediatrics. The purpose of off-label use is to benefit the individual patient. Practitioners use their professional judgment to determine these uses. As such, the term “off-label” does not imply an improper, illegal, contraindicated, or investigational use. Therapeutic decision-making must always rely on the best available evidence and the importance of the benefit for the individual patient.

http://www.ncbi.nlm.nih.gov/pubmed/24567009

Grief reactions and impact of patient death on pediatric oncologists

Granek, L., et al.
Pediatr Blood Cancer 2015; 62(1): 134-142

BACKGROUND: To examine pediatric oncologists’ grief reactions to patient death, and the impact patient death has on their personal and professional lives. PROCEDURE: The grounded theory method was used. Data was collected between March 2012 and July 2012 at two academic centres in Canada. Twenty-one out of 34 eligible pediatric oncologists at different stages of their career were recruited and interviewed about their experiences with patient death. Inclusion criteria were: being able to speak English and having had a patient die in their care. The participants formed three groups of oncologists at different stages of career including: fellows, junior oncologists, and senior oncologists who varied in sub-specialties, gender, and ethnicities. RESULTS: Pediatric oncologists reported a range of reactions to patient death including sadness, crying, sleep loss, exhaustion, feeling physically ill, and a sense of personal loss. They also reported self-questioning, guilt, feelings of failure and helplessness. The impact of these deaths had personal consequences that ranged from irritability at home, feeling disconnected from family members and friends, and becoming more desensitized towards death, to gaining a greater and more appreciative perspective on life. Professional impacts included concern about turnover or burnout at work and improving holistic care as a result of patient deaths. CONCLUSIONS: Grief over patient death and the emotional labour involved in these losses are a robust part of the pediatric oncology workplace and have major impacts on pediatric oncologist’s personal and professional lives. Interventions that focus on how to help pediatric oncologists deal with these reactions are needed. Pediatr Blood Cancer 2015;62:134-142. (c) 2014 Wiley Periodicals, Inc.

http://www.ncbi.nlm.nih.gov/pubmed/25214471

Euthanasia: 10 myths

Hain, R. D.
Arch Dis Child 2014; 99(9): 798-799

http://www.ncbi.nlm.nih.gov/pubmed/250166

Advance care planning: Challenges and approaches for pediatricians

Heckford, E. and A. J. Beringer

J Palliat Med 2014; 17(9): 1049-1053

BACKGROUND: There is increasing recognition of the value of advance care planning for children with life-limiting conditions. It is important that we acknowledge and reflect on the challenges that this work presents in order to optimize practice. OBJECTIVE: Our aim was to review advance care planning for children with life-threatening or life-limiting conditions (LTLLCs) in our local area. METHODS: We conducted a retrospective case note review. Study subjects were from two National Health Service (NHS) Trusts in Bristol in the United Kingdom. Cases were identified from Child Death Overview Panel data. Forty-two sets of case notes were reviewed in relation to 20 children. Measurements included quantitative and qualitative review of advance care planning in relation to standards set by The Association for Children's Palliative Care (ACT). RESULTS: In 25% of cases there was no documented discussion with families about the approach to end of life (EOL). In 25% of cases there was no evidence of an advance care plan, and the content and accessibility of those that did exist was variable. Forty-five percent of families were not offered a choice with regard to location of care (LOC) in the last months of life and 50% were not offered a choice about location of death (LOD). CONCLUSIONS: We hope that acknowledgement of some of the challenges, alongside recognition of the clear benefits, of planning will help pediatricians to deliver this important area of care.

http://www.ncbi.nlm.nih.gov/pubmed/24955940

Supporting communication for children with cerebral palsy in hospital: Views of community and hospital staff

Hemsley, B., et al.

Dev Neurorehabil 2014; 17(3): 156-166

OBJECTIVE: We aimed to investigate the views of allied health and nursing staff on supporting the communication of children with cerebral palsy (CP) and complex communication needs (CCN) in hospital. METHOD: We conducted 12 focus groups with 49 community- and hospital-based allied health professionals and hospital nurses. RESULTS: Participants reported having active roles in supporting children's seating, mobility, equipment, mealtime management and psychosocial needs, but not in supporting the children's communication in hospital. Participants described several environmental barriers to supporting children's augmentative and alternative communication (AAC) in hospital, and suggested a range of strategies to ease communication difficulties at the bedside. CONCLUSION: Results indicate a potential new role for community- and hospital-based health professionals in supporting nurses to implement AAC strategies at the bedside. Supporting nursing staff to remove environmental barriers and use communication technologies might create a more communicatively accessible hospital ward for children with CP and CCN.

http://www.ncbi.nlm.nih.gov/pubmed/24102353

Why is UK performance in child and youth mortality so poor?

Johnston, B. D.
Lancet 2014; 384(9946): 837-838

http://www.ncbi.nlm.nih.gov/pubmed/24929451

Improving recruitment in pediatric clinical research: A strategy to consider

Kaguelidou, F., et al.
J Clin Epidemiol 2014; 67(7): 841-842

http://www.ncbi.nlm.nih.gov/pubmed/24739463

Withdrawal of ventilatory support outside the intensive care unit: guidance for practice

Laddie, J., et al.
Arch Dis Child 2014; 99(9): 812-816.

OBJECTIVE: To review the work of one tertiary paediatric palliative care service in facilitating planned withdrawal of ventilatory support outside the intensive care setting, with the purpose of developing local guidance for practice. METHODS: Retrospective 10-year (2003-2012) case note review of intensive care patients whose parents elected to withdraw ventilation in another setting. Demographic and clinical data revealed common themes and specific incidents relevant to local guideline development. RESULTS: 18 children (aged 2 weeks to 16 years) were considered. Three died prior to transfer. Transfer locations included home (5), hospice (8) and other (2). Primary pathologies included malignant, neurological, renal and respiratory diseases. Collaborative working was evidenced in the review including multidisciplinary team meetings with the palliative care team prior to discharge. Planning included development of symptom management plans and emergency care plans in the event of longer than anticipated survival. Transfer of children and management of extubations demonstrated the benefits of planning and recognition that unexpected events occur despite detailed planning. We identified the need for local written guidance supporting healthcare professionals planning and undertaking extubation outside the intensive care setting, addressing the following phases: (i) introduction of withdrawal, (ii) preparation pretransfer, (iii) extubation, (iv) care postextubation and (v) care postdeath. CONCLUSIONS: Planned withdrawal of ventilatory support outside the intensive care setting is challenging and resource intensive. The development of local collaborations and guidance can enable parents of children dependent on intensive care to consider a preferred place of death for their child, which may be outside the intensive care unit.

http://www.ncbi.nlm.nih.gov/pubmed/24951460

Pediatric palliative care: A national survey of French pediatric residents' knowledge, education, and clinical experience

Lefebvre, C., et al.

Arch Pediatr 2014; 21(8): 834-844

BACKGROUND: The need for educational training of healthcare professionals in palliative care is an important issue. Training and practice of pediatric residents in the field of pediatric palliative care (PPC) has never been assessed, although the organization of the medical curriculum in France is currently being revised. MATERIALS AND METHODS: This study presents a national survey of pediatric residents, using a computerized anonymous questionnaire. Four different areas were studied: epidemiological data, theoretical and practical knowledge, education, and clinical experience in PPC. RESULTS: The response rate was 39% (n=365/927). Whatever their age or regional location, 25% of residents did not know any details of the French law concerning patients' rights and the end of life. Experience with PPC starts very early since 77% of the first-year pediatric residents experienced at least one child in a palliative care and/or end-of-life situation. During their entire residency, 87% of the residents had experience with PPC and nearly all (96%) end-of-life care. Furthermore, 76% had participated in announcing palliative care (cancer, ICU, etc.) or a serious illness, and 45% had met and discussed with bereaved parents. Furthermore, while 97% of the pediatric residents received training in adult palliative care, mainly before their residency, only 60% received specific PPC training. DISCUSSION AND CONCLUSION: Ninety-six percent of all French pediatric residents encountered a PPC situation during their residency. That 77% of them had experienced PPC during their first year of residency shows the importance of early training in PPC for pediatric residents. Furthermore, this study points out that there is a significant lack in PPC training since 40% of all residents in the study received no specific PPC training. Progress in education remains insufficient in the dissemination of knowledge on the legal framework and concepts of palliative medicine: while the medical curriculum is being revised, we suggest that training in medical ethics and PPC should be introduced very early and systematically.

http://www.ncbi.nlm.nih.gov/pubmed/24993148

First International Children's Palliative Care Network Conference

Ling, J.

Int J Palliat Nurs 2014; 20(3): 149

http://www.ncbi.nlm.nih.gov/pubmed/24675542

Advance Care Planning in palliative care: A systematic literature review of the contextual factors influencing its uptake 2008-2012

Lovell, A. and P. Yates

Palliat Med 2014; 28(8): 1026-1035

BACKGROUND: Advance Care Planning is an iterative process of discussion, decision-making and documentation about end-of-life care. Advance Care Planning is highly relevant in palliative care due to intersecting clinical needs. To enhance the implementation of Advance Care Planning, the contextual factors influencing its uptake need to be better understood. AIM: To identify the contextual factors influencing the uptake of Advance Care Planning in palliative care as published between January 2008 and December 2012. METHODS: Databases were systematically searched for studies about Advance Care Planning in palliative care published between January 2008 and December 2012. This yielded 27 eligible studies, which were appraised using National Institute of Health and Care Excellence Quality Appraisal Checklists. Iterative thematic synthesis was used to group results. RESULTS: Factors associated with greater uptake included older age, a college degree, a diagnosis of cancer, greater functional impairment, being white, greater understanding of poor prognosis and receiving or working in specialist palliative care. Barriers included having non-malignant diagnoses, having dependent children, being African American, and uncertainty about Advance Care Planning and its legal status. Individuals' previous illness experiences, preferences and attitudes also influenced their participation. CONCLUSION: Factors influencing the uptake of Advance Care Planning in palliative care are complex and multifaceted reflecting the diverse and often competing needs of patients, health professionals, legislature and health systems. Large population-based studies of palliative care patients are required to develop the sound theoretical and empirical foundation needed to improve uptake of Advance Care Planning in this setting.

http://www.ncbi.nlm.nih.gov/pubmed/24821708

Use of formal advance care planning documents: A national survey of UK Paediatric Intensive Care Units

Mitchell, S., et al.

Arch Dis Child 2014; 99(4): 327-330.

OBJECTIVE: Advance Care Planning (ACP) is nationally a core element of adult and paediatric palliative care strategies. It is defined as a process of discussion between an individual, their care providers and those close to them, about future care. Formal procedures and processes can help with some of the most difficult elements of communication related to ACP. The majority of children who die do so in a Paediatric Intensive Care Unit (PICU). This survey aimed to identify and compare paediatric ACP documents that are in use within UK hospitals with a PICU. DESIGN: Email survey of lead clinicians from UK PICUs (n=28). RESULTS: 24 (86%) questionnaires were returned. 14 (58%) responded that formal ACP documents were currently in use within their hospital trust. Of the remainder, 2 (8%) detailed plans to launch local ACP documents in the near future, 1 (4%) had a 'Children and Young Persons Deterioration Management (CAYPDM) Document' and 3 (12%) listed rapid discharge and extubation pathways. 6 (25%) provided details of the document in use. They varied widely in terms of their presentation, content and intended use with some having been developed locally and others having been adopted across regions. CONCLUSIONS: There is variation around the UK in the existence of formal ACP documents for paediatric patients with palliative care needs, as well as variation in the type of document that is used. Consideration of a national policy should be informed by further review and evaluation of these documents, as well as current practice in ACP.

http://www.ncbi.nlm.nih.gov/pubmed/24336425

Pediatric palliative care

Moore, D. and J. Sheetz
Pediatr Clin North Am 2014; 61(4): 735-747

Pediatric palliative care (PPC) is a relatively new and quickly growing pediatric subspecialty. It is generally provided using a consultative model, and is available in most specialized pediatric hospitals. This article discusses PPC consultation with specific focus on the added value of PPC, elements of a PPC consultation, and challenges to and opportunities for PPC consultation. Ongoing research, current publication, expert opinion, and institutional experience were compiled for this article.

http://www.ncbi.nlm.nih.gov/pubmed/25084721

Sleep: The other life of children with cerebral palsy

Newman, C. J.
Dev Med Child Neurol 2014; 56(7): 610-611

http://www.ncbi.nlm.nih.gov/pubmed/24641776

Interdisciplinary care: Using your team

Ogelby, M. and R. D. Goldstein
Pediatr Clin North Am 2014; 61(4): 823-834

The interdisciplinary approach is a cornerstone of a well-functioning pediatric palliative care team. These teams are most often available as an inpatient consultation service, are composed of professionals representing multiple disciplines, and are used during the illness course of a child with life-threatening and chronic complex conditions, especially during challenging health care decision-making near end of life. This article reviews the current structure of the palliative care team, explores opportunities for inclusion of other vital team members, and proactively identifies the challenges that may occur when involving more providers in a child's and family's care.

http://www.ncbi.nlm.nih.gov/pubmed/25084726

Death of a child in the emergency department

O'Malley, P. J., et al.
Ann Emerg Med 2014; 64(1): e1-17

The death of a child in the emergency department (ED) is one of the most challenging problems facing ED clinicians. This revised technical report and accompanying policy statement reaffirm principles of patient- and family-centered care. Recent literature is examined regarding family presence, termination of resuscitation, bereavement responsibilities of ED clinicians, support of child fatality review efforts, and other issues inherent in caring for the patient, family, and staff when a child dies in the ED. Appendices are provided that offer an approach to bereavement activities in the ED, carrying out forensic responsibilities while providing compassionate care, communicating the news of the death of a child in the acute setting, providing a closing ritual at the time of terminating resuscitation efforts, and managing the child with a terminal condition who presents near death in the ED.

http://www.ncbi.nlm.nih.gov/pubmed/24998719

Transfusion in critically ill children: indications, risks, and challenges

Parker, R. I.

Crit Care Med 2014; 42(3): 675-690

OBJECTIVE: To provide a concise review of transfusion-related issues and practices in the pediatric patient population, with a focus on those issues of particular importance to the care of critically ill children. DATA SOURCE: Electronic search of the PubMed database using the search terms “pediatric transfusion,” “transfusion practices,” “transfusion risks,” “packed red blood cell transfusion,” “white blood cell transfusion,” “platelet transfusion,” “plasma transfusion,” and “massive transfusion” either singly or in combination. STUDY SELECTION AND DATA EXTRACTION: All identified articles published since 2000 were manually reviewed for study design, content, and support for indicated conclusions, and the bibliographies were scrutinized for pertinent references not identified in the PubMed search. Selected studies from this group were then manually reviewed for possible inclusion in this review. DATA SYNTHESIS: Well-designed studies have demonstrated the benefit of “restrictive” transfusion practices across the entire age spectrum of pediatric patients across a wide spectrum of pediatric illness. However, clinician implementation of the more restrictive transfusion practices supported by these studies is variable. Additionally, the utilization of both platelet and plasma transfusions in either a “prophylactic” or “therapeutic” setting appears to be greater than that supported by published data. CONCLUSIONS: The preponderance of prospective, randomized trials and retrospective analyses support the use of a restrictive packed RBC transfusion policy in most clinical conditions in children. Neonatal transfusions guidelines rely largely on “expert opinion” rather than experimental data. Current transfusion practices for both platelets and coagulant products (e.g., fresh-frozen plasma and recombinant-activated factor VII) are poorly aligned with recommended transfusion guidelines. As with adults, current transfusion practices in children often do not reflect implementation of our current knowledge on the need for transfusion. Greater efforts to implement current evidence-based transfusion practices are needed.

http://www.ncbi.nlm.nih.gov/pubmed/24534955

Death certification: A primer. Part II The cause of death statement

Randall, B.

S D Med 2014; 67(6): 231-233, 235

The cause of death statement is the core of the death certification process for the physician certifier. The World Health Organization defines the cause of death as the disease or injury that initiates a chain of events leading to death. This cause of death needs to be listed at the bottom of the cause of death statement with the events the cause of death initiated (mechanisms of death) listed above in a direct causal relationship (cause of death ‘A,’ initiated process ‘B,’ that in turn caused process ‘C,’ that in turn produced ‘D,’ that directly led to the death). In addition to the cause of death and its attendant mechanism(s) of death, the death certificate also includes an area for other significant conditions. This area is to be used for significant medical conditions that are not part of the chain of event leading from the cause of death. An example of an, other significant condition, would be metastatic breast carcinoma in an individual dying of a ruptured aortic aneurysm. The manner of death is restricted to either natural or unnatural (accident, homicide and suicide). Physicians, unless they are also acting as a coroner, are only allowed to certify natural deaths.

http://www.ncbi.nlm.nih.gov/pubmed/24979983

AAP releases guideline for the management of gastroesophageal reflux in children

Randel, A.

Am Fam Physician 2014; 89(5): 395-397

http://www.ncbi.nlm.nih.gov/pubmed/24695514

Lost among the trees? The autonomic nervous system and paediatrics

Rees, C. A.

Arch Dis Child 2014; 99(6): 552-562

The autonomic nervous system (ANS) has been strikingly neglected in Western medicine. Despite its profound importance for regulation, adjustment and coordination of body systems, it lacks priority in training and practice and receives scant attention in numerous major textbooks. The ANS is integral to manifestations of illness, underlying familiar physical and psychological symptoms. When ANS activity is itself dysfunctional, usual indicators of acute illness may prove deceptive. Recognising the relevance of the ANS can involve seeing the familiar through fresh eyes, challenging assumptions in clinical assessment and in approaches to practice. Its importance extends from physical and psychological well-being to parenting and safeguarding, public services and the functioning of society. Exploration of its role in conditions ranging from neurological, gastrointestinal and connective tissue disorders, diabetes and chronic fatigue syndrome, to autism, behavioural and mental health difficulties may open therapeutic avenues. The ANS offers a mechanism for so-called functional illnesses and illustrates the importance of recognising that 'stress' takes many forms, physical, psychological and environmental, desirable and otherwise. Evidence of intrauterine and post-natal programming of ANS reactivity suggests that neonatal care and safeguarding practice may offer preventive opportunity, as may greater understanding of epigenetic change of ANS activity through, for example, accidental or psychological trauma or infection. The aim of this article is to accelerate recognition of the importance of the ANS throughout paediatrics, and of the potential physical and psychological cost of neglecting it.

http://www.ncbi.nlm.nih.gov/pubmed/24573884

Interventions to reduce pediatric medication errors: a systematic review

Rinke, M. L., et al.

Pediatrics 2014; 134(2): 338-360

BACKGROUND AND OBJECTIVE: Medication errors cause appreciable morbidity and mortality in children. The objective was to determine the effectiveness of interventions to reduce pediatric medication errors, identify gaps in the literature, and perform meta-analyses on comparable studies. METHODS: Relevant studies were identified from searches of PubMed, Embase, Scopus, Web of Science, the Cochrane Library, and the Cumulative Index to Nursing Allied Health Literature and previous systematic reviews. Inclusion criteria were peer-reviewed original data in any language testing an intervention to reduce medication errors in children. Abstract and full-text article review were conducted by 2 independent authors with sequential data extraction. RESULTS: A total of 274 full-text articles were reviewed and 63 were included. Only 1% of studies were conducted at community hospitals, 11% were conducted in ambulatory populations, 10% reported preventable adverse drug events, 10% examined administering errors, 3% examined dispensing errors, and none reported cost-effectiveness data, suggesting persistent research gaps. Variation existed in the methods, definitions, outcomes, and rate denominators for all studies; and many showed an appreciable risk of bias. Although 26 studies (41%) involved computerized provider order entry, a meta-analysis was not performed because of methodologic heterogeneity. Studies of computerized provider order entry with clinical decision support compared with studies without clinical decision support reported a 36% to 87% reduction in prescribing errors; studies of preprinted order sheets revealed a 27% to 82% reduction in prescribing errors. CONCLUSIONS: Pediatric medication errors can be reduced, although our understanding of optimal interventions remains hampered. Research should focus on understudied areas, use standardized definitions and outcomes, and evaluate cost-effectiveness.

http://www.ncbi.nlm.nih.gov/pubmed/25022737

Sharing the care: The key-working experiences of professionals and the parents of life-limited children

Rodriguez, A. and N. King

Int J Palliat Nurs 2014; 20(4): 165-171

AIMS: To explore the lived experience of caring and care planning for a child with a life-limiting condition (LLC). METHOD: Using van Manen's conceptualisation of hermeneutic phenomenology, three focus groups were conducted with 21 paediatric palliative care professionals, and interviews were conducted with 20 parents of children with LLCs. FINDINGS: Parents' expectations for support were raised by the diagnosis, but the reality could disappoint, which put pressures on professionals. Current service designs with respect to key working did not always coincide with family preferences. Both parents and professionals found that the care journey required them to shift personas to respond to different contexts. CONCLUSIONS: The findings are limited by the sample characteristics, but they provide insight for current policy and practice initiatives. The key worker needs to be mindful of historical care arrangements and be prepared to step into the family 'team' arrangements.

http://www.ncbi.nlm.nih.gov/pubmed/24763324

Trends in paediatric clinical pharmacology data in US pharmaceutical labelling

Samiee-Zafarghandy, S., et al.
Arch Dis Child 2014; 99(9): 862-865

BACKGROUND: There is often a lack of safety and efficacy data in the paediatric population at the time of drug approval. Legislative efforts have promoted clinical pharmacology research in this underserved population. We sought to determine the quantity and quality of paediatric clinical pharmacology data in US drug labelling at the time of initial approval and to evaluate trends over time. MATERIALS AND METHODS: The labelling data of 213 new molecular entities approved between 2003 and 2012 were systematically reviewed. The type of paediatric pharmacology data present at the time of approval was recorded and stratified by age group. Labelling revisions were analysed for updated paediatric data. The presence of paediatric-specific black-box warnings was noted. RESULTS: Of the 213 drugs evaluated, 48 had adult-specific indications. Of the remaining 165 medicines, only 47 (28%) had paediatric study data at the time of initial labelling. The number of approved drugs with paediatric data was the greatest in 2005 (8, 44%) and was at its lowest point in 2012 (3, 11%). Only five medicines had neonatal data, with none of the anti-infective agents presenting neonatal information. Seven medications had a paediatric-specific black-box warning. Additional 16 medicines presented paediatric data during general labelling updates. CONCLUSIONS: Despite efforts to improve the quality of paediatric clinical pharmacology data, there was not a significant increase in drugs with paediatric data at the time of approval over this 10-year study period. Paediatric drug approvals and labelling revisions continue to lag behind their adult counterparts.

http://www.ncbi.nlm.nih.gov/pubmed/25063835

Hospital medicine and pediatric palliative care

Stanton, B. F.
Pediatr Clin North Am 2014; 61(4): xvii-xviii

http://www.ncbi.nlm.nih.gov/pubmed/25084729

Comanagement of medically complex children by subspecialists, generalists, and care coordinators

Stiles, A. D., et al.
Pediatrics 2014; 134(2): 203-205

http://www.ncbi.nlm.nih.gov/pubmed/25070309

The growth of palliative care

Strand, J. J., et al.
Minn Med 2014; 97(6): 39-43

Palliative care specialists focus on meeting the needs of patients with serious and/or life-threatening illnesses. These physicians have expertise in managing complex pain and non pain symptoms, providing psychosocial and spiritual support to patients and their families, and communicating about complex topics and advance care planning. The American Board of Medical Specialties has allowed 10 of its member boards to co-sponsor certification in Hospice and Palliative Medicine. Thus, physicians from specialties ranging from pediatrics to surgery now practice hospice and palliative medicine. At the core of this field, however, are physicians who trained as internists and are boarded by the American Board of Internal Medicine. This article discusses the central principles of palliative care and explores its growth in two areas: oncology and critical care medicine.

http://www.ncbi.nlm.nih.gov/pubmed/25029799

Perceptions of lethal fetal abnormality among perinatal professionals and the challenges of neonatal palliative care

Tosello, B., et al.
J Palliat Med 2014; 17(8): 924-930

BACKGROUND: After prenatal diagnosis of lethal fetal abnormality (LFA), some couples choose to continue the pregnancy rather than opt for termination of the pregnancy. This may result in the requirement for neonatal palliative care, which in France is prescribed by the Leonetti Law. These rare situations raise various questions about when and how palliative care is provided in cases of LFA. OBJECTIVE: The main goal of the study was to clarify the place given to the concept of perinatal palliative care within the antenatal information provided by perinatal professionals. This work was specifically aimed at revealing caregivers' perceptions of and attitudes toward LFA, how it is managed, and procedures for decision making and providing information. METHODS: This is a qualitative study using focus groups from two French Multidisciplinary Centers for Prenatal Diagnosis. All verbal production (individual statements, verbal exchanges, etc.) produced during the two focus groups was fully transcribed and the content analyzed. RESULTS: Content analysis revealed four main themes: (1) defining LFA; (2) the source and nature of information about LFA and how it is communicated; (3) therapeutic options and decisions in the management of LFA; and (4) palliative care (limits and criteria) in the context of LFA. CONCLUSIONS: Consistency as regards the perceived intention of care among all members of the health care team is essential to support parents facing a possible fatal outcome. Attitudes and practices at Multidisciplinary Centers for Prenatal Diagnosis need to be shaped on a national basis.

http://www.ncbi.nlm.nih.gov/pubmed/24854190

Caring for children living with life-threatening illness: A growing relationship between pediatric hospital medicine and pediatric palliative care

Ullrich, C. K. and J. Wolfe
Pediatr Clin North Am 2014; 61(4): xxi-xxiii

http://www.ncbi.nlm.nih.gov/pubmed/25084730

Quality of communication in interpreted versus noninterpreted PICU family meetings

Van Cleave, A. C., et al.
Crit Care Med 2014; 42(6): 1507-1517

OBJECTIVES: To describe the quality of physician-family communication during interpreted and noninterpreted family meetings in the PICU. DESIGN: Prospective, exploratory, descriptive observational study of noninterpreted English family meetings and interpreted Spanish family meetings in the pediatric intensive care setting. SETTING: A single, university-based, tertiary children's hospital. SUBJECTS: Participants in PICU family meetings, including medical staff, family members, ancillary staff, and interpreters. INTERVENTIONS: Thirty family meetings (21 English and nine Spanish) were audio-recorded, transcribed, de-identified, and analyzed using the qualitative method of directed content analysis. MEASUREMENTS AND MAIN RESULTS: Quality of communication was analyzed in three ways: 1) presence of elements of shared decision-making, 2) balance between physician and family speech, and 3) complexity of physician speech. Of the 11 elements of shared decision-making, only four occurred in more than half of English meetings, and only three occurred in more than half of Spanish meetings. Physicians spoke for a mean of 20.7 minutes, while families spoke for 9.3 minutes during English meetings. During Spanish meetings, physicians spoke for a mean of 14.9 minutes versus just 3.7 minutes of family speech. Physician speech complexity received a mean grade level score of 8.2 in English meetings compared to 7.2 in Spanish meetings. CONCLUSIONS: The quality of physician-family communication during PICU family meetings is poor overall. Interpreted meetings had poorer communication quality as evidenced by fewer elements of shared decision-making and greater imbalance between physician and family speech. However, physician speech may be less complex during interpreted meetings. Our data suggest that physicians can improve communication in both interpreted and noninterpreted family meetings by increasing the use of elements of shared decision-making, improving the balance between physician and family speech, and decreasing the complexity of physician speech.

http://www.ncbi.nlm.nih.gov/pubmed/24394631

Child death review five years on

Ward Platt, M.
Arch Dis Child 2014; 99(3): 187-188

http://www.ncbi.nlm.nih.gov/pubmed/24344175

Impact of pediatric exclusivity on drug labeling and demonstrations of efficacy

Wharton, G. T., et al.

Pediatrics 2014; 134(2): e512-518

BACKGROUND: Besides vaccines and otitis media medicines, most products prescribed for children have not been studied in the pediatric population. To remedy this, Congress enacted legislation in 1997, known as pediatric exclusivity (PE), which provides 6 months of additional market protection to drug sponsors in exchange for studying their products in children. METHODS: We reviewed requests for pediatric studies and subsequent labeling for drugs granted PE from 1998 through 2012. Regression analysis estimates the probability of demonstrating efficacy in PE trials. Variables include therapeutic group, year of exclusivity, product sales, initiation process, and small disease population. RESULTS: From 1998 through 2012, the US Food and Drug Administration issued 401 pediatric study requests. For 189 drugs, studies were completed and granted exclusivity. A total of 173 drugs (92%) received new pediatric labeling, with 108 (57%) receiving a new or expanded pediatric indication. Three drugs had non-efficacy trials. Efficacy was not established for 78 drugs. Oncology, cardiovascular, and endocrine drugs were less likely to demonstrate efficacy ($P < .01$) compared with gastrointestinal and pain/anesthesia drugs. Drugs studied later in the program were less likely to demonstrate efficacy ($P < .05$). Sales, initiation process, and small disease population were not significant predictors. CONCLUSIONS: Most drugs (173; 92%) granted exclusivity added pediatric information to their labeling as a result of PE, with 108 (57%) receiving a new or expanded pediatric indication. Therapeutic area and year of exclusivity influenced the likelihood of obtaining a pediatric indication. Positive and negative outcomes continue to inform the construct of future pediatric trials.

http://www.ncbi.nlm.nih.gov/pubmed/25022732

Brain injury: Younger is not better

Arch Dis Child 2014; 99(8): 782

http://www.ncbi.nlm.nih.gov/pubmed/24919711

Astroblastomas: A Surveillance, Epidemiology, and End Results (SEER)-based patterns of care analysis

Ahmed, K. A., et al.
World Neurosurg 2014; 82(1-2): e291-297

OBJECTIVE: This study sought to report patient characteristics, risk factors, and trends in management for astroblastoma patients. METHODS: A retrospective analysis was conducted utilizing the Surveillance, Epidemiology and End Results Program of the National Cancer Institute. RESULTS: Two hundred and thirty-nine patients were identified, with 206 patients receiving treatment. The median age at diagnosis was 35 years (range 0 to 84 years). Tumor location was available for 177 patients, and the majority were supratentorial (n = 144, 81.3%). The median overall survival and cause-specific survival for the cohort receiving treatment was 55 and 65 months, respectively. On univariate analysis, patients receiving surgery alone compared to only radiotherapy displayed improved overall survival (OS) and cause-specific survival (CSS) with a 5-year OS of 62.2% vs. 27.3%, P < .001, and CSS of 67.3% vs. 31.9%, P = .003. Supratentorial tumor location was associated with worse survival, with an estimated 5-year OS of 44.9% for supratentorial tumors compared to 75% for infratentorial tumors (hazard ratio 3.41 [95% CI, 1.76 to 6.62]; P < .001) and CSS of 47.5% (supratentorial) to 82% (infratentorial) (hazard ratio 3.95 [95% CI, 1.81 to 8.62]; P = .001). Age >60 years at diagnosis and treatment before 1990 were correlated with decreased survival on both the univariate and the multivariate analyses. CONCLUSIONS: To our knowledge, this is the largest report of astroblastoma patients described in the literature. Supratentorial tumor location, older age, and treatment prior to 1990 were poor prognostic factors.

http://www.ncbi.nlm.nih.gov/pubmed/24141003

Pediatric brainstem tumors. Classifications, investigations, and growth patterns

Alaqeel, A. M. and A. J. Sabbagh
Neurosciences (Riyadh) 2014; 19(2): 93-99

Brainstem gliomas occur in 10-20% of brain tumors in pediatrics. Over the past 3 decades, the treatment of brainstem gliomas has significantly progressed as a result of the gradual advancements in microsurgical techniques, sophisticated imaging technology and, most importantly, the availability of MRI. In this article, we review the current literature on brainstem gliomas and cover diagnosis, imaging, classification, and management. Surgical approaches and intraoperative modalities to tackle operable cases of brainstem gliomas will be discussed in a follow up article.

http://www.ncbi.nlm.nih.gov/pubmed/24739404

Long-term incidence and risk factors for development of spinal deformity following resection of pediatric intramedullary spinal cord tumors

Ahmed, R., et al.

J Neurosurg Pediatr 2014; 13(6): 613-621

OBJECT: Spinal deformity in pediatric patients with intramedullary spinal cord tumors (IMSCTs) may be either due to neurogenic disability or due to secondary effects of spinal decompression. It is associated with functional decline and impairment in health-related quality-of-life measures. The authors sought to identify the long-term incidence of spinal deformity in individuals who had undergone surgery for IMSCTs as pediatric patients and the risk factors and overall outcomes in this population. METHODS: Treatment records for pediatric patients (age < 21 years) who underwent surgical treatment for histology-proven primary IMSCTs between 1975 and 2010 were reviewed. All patients were evaluated in consultation with the pediatric orthopedics service. Clinical records were reviewed for baseline and follow-up imaging studies, surgical fusion treatment, and long-term skeletal and disease outcomes. RESULTS: The authors identified 55 patients (30 males and 25 females) who were treated for pediatric IMSCTs between January 1975 and January 2010. The mean duration of follow-up (+/- SEM) was 11.4 +/- 1.3 years (median 9.3 years, range 0.2-37.2 years). Preoperative skeletal deformity was diagnosed in 11 (20%) of the 55 patients, and new-onset postoperative deformity was noted in 9 (16%). Conservative management with observation or external bracing was sufficient in 8 (40%) of these 20 cases. Surgical fusion was necessary in 11 (55%). Posterior surgical fusion was sufficient in 6 (55%) of these 11 cases, while combined anterior and posterior fusion was undertaken in 5 (45%). Univariate and multivariate analysis of clinical and surgical treatment variables indicated that preoperative kyphoscoliosis ($p = 0.0032$) and laminectomy/laminoplasty at more than 4 levels ($p = 0.05$) were independently associated with development of spinal deformity that necessitated surgical fusion. Functional scores and 10-year disease survival outcomes were similar between the 2 groups. CONCLUSIONS: Long-term follow-up is essential to monitor for delayed development of spinal deformity, and regular surveillance imaging is recommended for patients with underlying deformity. The authors' extended follow-up highlights the risk factors associated with development of spinal deformity in patients treated for pediatric IMSCTs. Surgical fusion allows patients who develop progressive deformity to achieve long-term functional and survival outcomes comparable to those of patients who do not develop progressive deformity.

http://www.ncbi.nlm.nih.gov/pubmed/24702614

Long-term disease and neurological outcomes in patients with pediatric intramedullary spinal cord tumors

Ahmed, R., et al.

J Neurosurg Pediatr 2014; 13(6): 600-612

OBJECT: Radical resection is recommended as the first-line treatment for pediatric intramedullary spinal cord tumors (IMSCTs), but it is associated with morbidity, including risk of neurological decline and development of postoperative spinal deformity. The authors report long-term data on clinical and treatment determinants affecting disease survival and neurological outcomes. METHODS: Case records for pediatric patients (< 21 years of age at presentation) who underwent surgery for IMSCTs at the authors' institution between January 1975 and January 2010 were analyzed. The patients' demographic and clinical characteristics (including baseline neurological condition), the treatment they received, and their disease course were reviewed. Long-term disease survival and functional outcome measures were analyzed. RESULTS: A total of 55 patients (30 male and 25 female) were identified. The mean duration of follow-up (+/- SEM) was 11.4 +/- 1.3 years (median 9.3 years, range 0.2-37.2 years). Astrocytomas were the most common tumor subtype (29 tumors [53%]). Gross-total resection (GTR) was achieved in 21 (38%) of the 55 patients. At the most recent follow-up, 30 patients (55%) showed neurological improvement, 17 (31%) showed neurological decline, and 8 (15%) remained neurologically stable. Patients presenting with McCormick Grade I were more likely to show functional improvement by final follow-up ($p = 0.01$) than patients who presented with Grades II-V. Kaplan-Meier actuarial tumor progression-free survival rates at 5, 10, and 20 years were 61%, 54%, and 44%, respectively; the overall survival rates were 85% at 5 years, 74% at 10 years, and 64% at 20 years. On multivariate analysis, GTR ($p = 0.04$) and tumor histological grade ($p = 0.02$) were predictive of long-term survival; GTR was also associated with improved 5-year progression-free survival ($p = 0.01$). CONCLUSIONS: The prognosis for pediatric IMSCTs is favorable with sustained functional improvement expected in a significant proportion of patients on long-term follow-up. Long-term survival at 10 years (75%) and 20 years (64%) is associated with aggressive resection. Gross-total resection was also associated with improved 5-year progression-free survival (86%). Hence, the treatment benefits of GTR are sustained on extended follow-up.

http://www.ncbi.nlm.nih.gov/pubmed/24702616

Treatment of cerebral aneurysms in children: Analysis of the Kids' Inpatient Database

Alawi, A., et al.

J Neurosurg Pediatr 2014; 14(1): 23-30

OBJECT.: Endovascular coiling and surgical clipping are viable treatment options of cerebral aneurysms. Outcome data of these treatments in children are limited. The objective of this study was to determine hospital mortality and complication rates associated with surgical clipping and coil embolization of cerebral aneurysms in children, and to evaluate the trend of hospitals' use of these treatments. METHODS: The authors identified a cohort of children admitted with the diagnoses of cerebral aneurysms and aneurysmal subarachnoid hemorrhage from the Kids' Inpatient Database for the years 1998 through 2009. Hospital-associated complications and in-hospital mortality were compared between the treatment groups and stratified by aneurysmal rupture status. A multivariate regression analysis was used to identify independent variables associated with in-hospital mortality. The Cochrane-Armitage test was used to assess the trend of hospital use of these operations. RESULTS: A total of 1120 children were included in this analysis; 200 (18%) underwent aneurysmal clipping and 920 (82%) underwent endovascular coiling. Overall in-hospital mortality was higher in the surgical clipping group compared with the coil embolization group (6.09% vs 1.65%, respectively; adjusted odds ratio [OR] 2.52, 95% CI 0.97-6.53, p = 0.05). The risk of postoperative stroke or hemorrhage was similar between the two treatment groups (p = 0.86). Pulmonary complications and systemic infection were higher in the surgical clipping population (p < 0.05). The rate of US hospitals' use of endovascular coiling has significantly increased over the years included in this study (p < 0.0001). Teaching hospitals were associated with a lower risk of death (OR 0.13, 95% CI 0.03-0.46; p = 0.001). CONCLUSIONS: Although both treatments are valid, endovascular coiling was associated with fewer deaths and shorter hospital stays than clip placement. The trend of hospitals' use of coiling operations has increased in recent years.

http://www.ncbi.nlm.nih.gov/pubmed/24835049

Prognostic significance of being overweight and obese at diagnosis in children with acute lymphoblastic leukemia

Aldhafiri, F. K., et al.

J Pediatr Hematol Oncol 2014; 36(3): 234-236

This study tested the hypothesis that being overweight/obese at diagnosis of childhood ALL was related to risk of relapse. In a national cohort of 1033 patients from the UK, there was no evidence that weight status at diagnosis was related significantly to risk of relapse: log-rank test (P=0.90) with overweight and obesity as the exposure (n=917); individual (P=0.42) and stepwise (P=0.96) proportional hazards models, with BMI Z score as the exposure. The study does not support the hypothesis that being overweight/obese at diagnosis impairs prognosis in childhood ALL in the UK.

http://www.ncbi.nlm.nih.gov/pubmed/24276040

Cystic fibrosis mortality trend in Italy from 1970 to 2011

Alicandro, G., et al.

J Cyst Fibros 2014

BACKGROUND: Survival in cystic fibrosis (CF) has progressively improved and the female-gender disadvantage first described many years ago remains controversial. OBJECTIVES: To describe the mortality trend due to CF in Italy over the last decades; to verify the female-mortality disadvantage; to compare the comorbidities reported in death certificates of CF patients with those of the general population. METHODS: Mortality data were extracted from the database of underlying cause of death (1970-2011) and multiple causes of death (2003-2011) of the Italian National Institute of Statistics. Age-standardized mortality ratio (SMR) was calculated to compare the mortality between genders. The association between CF and other contributing causes of death was verified by calculating the age- and gender-adjusted proportional mortality ratio (PMR). RESULTS: During the study period, 1947 death certificates reported CF as the underlying cause of death. Mortality rate due to CF decreased in newborns and children and by the end of the 1990s also in adolescents and young adults. Adult mortality started to increase in the early 1990s. Over the whole period an excess in mortality was observed in young CF females (1-29years). The multiple causes of death database included 531 certificates with CF listed as cause of death. Pneumonia, chronic lower respiratory diseases, pulmonary heart disease and diseases of pulmonary circulation, aspergillosis, sepsis, renal failure, diabetes, malnutrition and amyloidosis were more frequently reported in CF death certificates compared to those of the general population (PMR>1). CONCLUSIONS: This mortality trend provides evidence of a consistent improvement in survival, although the excess female-mortality persists despite aggressive treatment of CF lung disease. Several extra-pulmonary conditions associated with CF contributed to the morbidity leading to death.

http://www.ncbi.nlm.nih.gov/pubmed/25151032

Prospective study of activities of daily living outcomes in children with cerebellar atrophy

Al-Maawali, A., et al.

Dev Med Child Neurol 2014; 56(5): 460-467

AIM: The aim of this study was to identify clinical and radiological predictors of activities of daily living (ADL) outcomes in children with cerebellar atrophy. METHOD: Over a period of 5 years, we evaluated 44 participants (25 males, 19 females) children with confirmed cerebellar atrophy using magnetic resonance imaging (MRI). The median age at the time of assessment 9 years; range 16 mo-18y. Participants were grouped according to whether the cerebellar atrophy was isolated or associated with other radiological abnormalities. Severity of cerebellar atrophy was graded using qualitative and quantitative scoring systems. A standardized ADL assessment was used to characterize functional outcomes. The characteristics of the participants were analysed using descriptive statistics. RESULTS: The mean age at symptom onset was 20 months (range birth-10y). The group with isolated cerebellar atrophy had better outcomes than the group with cerebellar atrophy associated with other radiological abnormalities, with a mean total ADL score difference of 8.0 points (95% confidence interval 1.8-14.2 points, p=0.01). Age at onset of cerebellar atrophy before 2 years of age, progression of cerebellar atrophy on magnetic resonance imaging, presence of seizures, and decreased size of transverse cerebellar hemisphere diameter were all associated with worse outcomes. INTERPRETATION: We present a prospective study of clinical and radiological predictors of ADL outcome in children with cerebellar atrophy. This information may be useful in the diagnosis and future management of this complex group of disorders.

http://www.ncbi.nlm.nih.gov/pubmed/24116951

Childhood acute lymphoblastic leukemia in the Middle East and neighboring countries: A prospective multi-institutional international collaborative study (CALLME1) by the Middle East Childhood Cancer Alliance (MECCA)

Al-Mulla, N. A., et al.

Pediatr Blood Cancer 2014; 61(8): 1403-1410

BACKGROUND: Little is known about childhood ALL in the Middle East. This study was undertaken by MECCA as initial efforts in collaborative data collection to provide clinical and demographic information on children with ALL in the Middle East. PROCEDURE: Clinical and laboratory data for patients with ALL between January 2008 and April 2012 were prospectively collected from institutions in 14 Middle East countries and entered into a custom-built-database during induction phase. All laboratory studies including cytogenetics were done at local institutions. RESULTS: The 1,171 voluntarily enrolled patients had a mean age of 6.1 +/- 3.9 years and 59.2% were boys. T-ALL represented 14.8% and 84.2% had B-precursor ALL. At diagnosis, 5.6% had CNS disease. The distribution of common genetic abnormalities reflected a similar percentage of hyperdiploidy (25.6%), but a lower percentage of ETV6-RUNX1 translocation (14.7%) compared to large series reported from Western populations. By clinical criteria, 47.1% were low/standard risk, 16.9% were intermediate risk, and 36% were high risk. Most patients received all their care at the same unit (96.9%). Patients had excellent induction response to chemotherapy with an overall complete remission rate of 96%. Induction toxicities were acceptable. CONCLUSIONS: This first collaborative study has established a process for prospective data collection and future multinational collaborative research in the Middle East. Despite the limitations of an incomplete population-based study, it provides the first comprehensive baseline data on clinical characteristics, laboratory evaluation, induction outcome, and toxicity. Further work is planned to uncover possible biologic differences of ALL in the region and to improve diagnosis and management.

http://www.ncbi.nlm.nih.gov/pubmed/24648275

Extraneural metastasis of an ependymoma: A rare occurrence

Alzahrani, A., et al.

Neuroradiol J 2014; 27(2): 175-178

Extraneural metastases of ependymoma are very rare, and have been reported in the lungs, lymph nodes, pleura, mediastinum, liver, diaphragmatic muscle, and bone. We describe the radiological findings of pathologically proven lung metastases from an anaplastic ependymoma. The tumour which arose in the posterior fossa was first diagnosed in 2007 when first surgical resection was performed outside our institute. Multiple operations were performed after that due to tumour relapse. Multiple lung nodules were discovered incidentally during a VP shunt survey. Biopsy from the lung nodules displayed identical histomorphology to the primary brain tumour.

http://www.ncbi.nlm.nih.gov/pubmed/24750705

Low-weight infants are at increased mortality risk after palliative or corrective cardiac surgery

Alsoufi, B., et al.
J Thorac Cardiovasc Surg 2014

BACKGROUND: Low weight is an established risk factor for mortality after congenital cardiac surgery. Given the advances in the care of neonates and infants after surgery, we sought to examine the effect of low weight on outcomes in the current era. METHODS: From 2002 to 2012, 2051 infants aged 90 days or less underwent cardiac surgery including 534 (26.0%) with single-ventricle pathology. Regression models examined the effect of low weight (</=2.5 kg; n = 274, 13.4%) on early and late outcomes. RESULTS: Overall, the incidence of prematurity, associated chromosomal/extracardiac abnormalities was higher in infants who weighed 2.5 kg or less than in those who weighed more than 2.5 kg; the incidence of single-ventricle pathology was comparable between the 2 groups. In addition, infants who weighed 2.5 kg or less underwent more palliation and had a higher proportion of STAT (Society of Thoracic Surgeons-European Association for Cardio-Thoracic Surgery) risk category 4 and 5 procedures. Adjusted regression models showed that low weight (</=2.5 kg) did not increase unplanned reoperation (odds ratio [OR], 0.90; 95% confidence interval [CI], 0.48-1.67; P = .73) or extracorporeal membrane oxygenation requirement (OR, 1.23; 95% CI, 0.68-2.22; P = .49), however it was associated with significant increase in hospital mortality (OR, 2.15; 95% CI, 1.33-3.50; P = .002). In addition, there was a significant association between low weight and increased duration of postoperative mechanical ventilation and intensive care unit and hospital stays. Adjusted hazard analysis showed that weight equal to or less than 2.5 kg was associated with diminished late survival (hazard ratio, 1.89; 95% CI, 1.39-2.55; P < .001) and that was evident in all patients subgroups (P < .001 for all). CONCLUSIONS: In a large single-center series, low weight continues to be associated with increased early mortality risk and resource utilization after palliative and corrective cardiac surgery. The hazard of death in low-weight patients continues beyond the perioperative period for at least 1 year before normalizing. Strategies to improve outcomes for this high-risk population must address perioperative care, outpatient surveillance, and management.

http://www.ncbi.nlm.nih.gov/pubmed/25238883

Double-trouble in pediatric neurology: Myotonia congenita combined with charcot-marie-tooth disease type 1a

Ardissone, A., et al.
Muscle Nerve 2014; 50(1): 145-147

http://www.ncbi.nlm.nih.gov/pubmed/24515601

Aging and risk of severe, disabling, life-threatening, and fatal events in the childhood cancer survivor study

Armstrong, G. T., et al.

J Clin Oncol 2014; 32(12): 1218-1227

PURPOSE: The first generation of childhood cancer survivors is now aging into their fourth and fifth decades of life, yet health risks across the aging spectrum are not well established. METHODS: Analyses included 14,359 5-year survivors from the Childhood Cancer Survivor Study, who were first diagnosed when they were younger than 21 years old and who received follow-up for a median of 24.5 years after diagnosis (range, 5.0 to 39.3 years) along with 4,301 of their siblings. Among the survivors, 5,604 were at least 35 years old (range, 35 to 62 years) at last follow-up. Severe, disabling, life-threatening, and fatal health conditions more than 5 years from diagnosis were classified using the Common Terminology Criteria for Adverse Events, grades 3 to 5 (National Cancer Institute). RESULTS: The cumulative incidence of a severe, disabling, life-threatening, or fatal health condition was greater among survivors than siblings (53.6%; 95% CI, 51.5 to 55.6; v 19.8%; 95% CI, 17.0 to 22.7) by age 50 years. When comparing survivors with siblings, hazard ratios (HR) were significantly increased within the age group of 5 to 19 years (HR, 6.8; 95% CI, 5.5 to 8.3), age group of 20 to 34 years (HR, 3.8; 95% CI, 3.2 to 4.5), and the >/= 35 years group (HR, 5.0; 95% CI, 4.1 to 6.1), with the HR significantly higher among those >/= 35 years versus those 20 to 34 years old (P = .03). Among survivors who reached age 35 years without a previous grade 3 or 4 condition, 25.9% experienced a subsequent grade 3 to 5 condition within 10 years, compared with 6.0% of siblings (P < .001). CONCLUSION: Elevated risk for morbidity and mortality among survivors increases further beyond the fourth decade of life, which affects the future clinical demands of this population relative to ongoing surveillance and interventions.

http://www.ncbi.nlm.nih.gov/pubmed/24638000

Left ventricular dysfunction in duchenne muscular dystrophy and genotype

Ashwath, M. L., et al.

Am J Cardiol 2014; 114(2): 284-289

Prognosis in patients with Duchenne muscular dystrophy (DMD) is guarded, and most deaths are due to cardiac or respiratory causes. It is unclear if some DMD gene mutations might be predictive of either mild or severe cardiac dysfunction. We studied 75 patients with DMD followed at our institution. Cardiac function, as assessed by yearly echocardiography, showed marked variability in left ventricular (LV) function. Some patients in their 3rd decade had no or minimal dysfunction, whereas others in their 2nd decade had very severe dysfunction. Therefore, 4 severity groups were defined ranging from no or mild LV dysfunction to severe LV dysfunction using patient age at first abnormal echocardiographic finding and degree of LV dysfunction. Genetic data were collected for all patients. Most patients had mutations from exon 1 to 20 to exon 41 to 55. The distribution of the 4 severity groups of LV dysfunction did not significantly differ between these 2 mutation groups. An analysis based on the number of exons involved (<5 vs >/=5 exons) also found no significant difference in cardiac severity. When patients having identical mutations were compared with their cardiac course, concordance was often not evident. Steroid therapy had no apparent protection for the development of cardiomyopathy. In conclusion, 75 patients with DMD showed marked variability in the severity of LV dysfunction. Neither the age of onset nor the severity of cardiomyopathy correlated with any of the mutation groups.

http://www.ncbi.nlm.nih.gov/pubmed/24878125

A guide to diagnosis and treatment of Leigh syndrome

Baertling, F., et al.

J Neurol Neurosurg Psychiatry 2014; 85(3): 257-265

Leigh syndrome is a devastating neurodegenerative disease, typically manifesting in infancy or early childhood. However, also late-onset cases have been reported. Since its first description by Denis Archibald Leigh in 1951, it has evolved from a post mortem diagnosis, strictly defined by histopathological observations, to a clinical entity with indicative laboratory and radiological findings. Hallmarks of the disease are symmetrical lesions in the basal ganglia or brain stem on MRI, and a clinical course with rapid deterioration of cognitive and motor functions. Examinations of fresh muscle tissue or cultured fibroblasts are important tools to establish a biochemical and genetic diagnosis. Numerous causative mutations in mitochondrial and nuclear genes, encoding components of the oxidative phosphorylation system have been described in the past years. Moreover, dysfunctions in pyruvate dehydrogenase complex or coenzyme Q10 metabolism may be associated with Leigh syndrome. To date, there is no cure for affected patients, and treatment options are mostly unsatisfactory. Here, we review the most important clinical aspects of Leigh syndrome, and discuss diagnostic steps as well as treatment options.

http://www.ncbi.nlm.nih.gov/pubmed/23772060

The national incidence and outcomes of gastroschisis repairs

Barrett, M. J., et al.

Ir Med J 2014; 107(3): 83-85

The birth prevalence of gastroschisis worldwide has increased over the past decades. We aim to determine the Irish national incidence of gastroschisis repairs (NIGR) over a 5 year period (2007- 2011) and clinical outcomes by a retrospective cohort review of cases admitted to all Irish paediatric surgical units. Seventy patients were identified. The NIGR per 10,000 live births was 1.96 (SD 0.51) per year. Fifty eight (82%) were antenatally detected. Twenty eight (40%) had primary repair day 1 with the remaining repaired in a median of 3(2-5.75) days. Thirty three (47%) experienced a central catheter related infection. Duration of stay was significantly correlated with decreasing gestational age ($p = 0.016$), decreasing birthweight ($p = 0.005$), increasing numbers of blood transfusions ($p < 0.001$) and co-morbidity or complication ($p < 0.001$). This study provides individual centres with patient outcomes and national data that can be provided to parents and clinical staff regarding the clinical course of gastroschisis.

http://www.ncbi.nlm.nih.gov/pubmed/24757894

Survival from teenage and young adult cancer in Northern England, 1968-2008

Basta, N. O., et al.

Pediatr Blood Cancer 2014; 61(5): 901-906

BACKGROUND: Although cancer is relatively rare in teenagers and young adults (TYAs) aged 15-24 years, it is a major cause of death in this age group. This study investigated survival trends in TYA cancer diagnosed in Northern England, 1968-2008. METHODS: Five-year survival was analyzed using Kaplan-Meier estimation for four successive time periods. Cox regression analysis was used to investigate associations with demographic factors. RESULTS: The study included 2,987 cases (1,634 males, 1,353 females). Five-year survival for all patients with cancer improved greatly from 46% in 1968-1977 to 84% in 1998-2008 ($P < 0.001$), for patients with leukemia from 2% to 71% ($P < 0.001$), lymphoma from 66% to 86% ($P < 0.001$), central nervous system tumors from 53% to 84% ($P < 0.001$), bone tumors from 29% to 72% ($P < 0.001$), germ cell tumors from 39% to 94% ($P < 0.001$), melanoma and skin cancer from 64% to 100% ($P < 0.001$), and carcinomas from 48% to 80% ($P < 0.001$). Cox analysis showed that for all patients with cancer, survival was better for females than males (HR = 0.83; 95% CI 0.74-0.94, $P < 0.001$), for patients aged 20-24 years compared with those aged 15-19 years (HR = 0.84; 95% CI 0.75-0.94, $P = 0.002$), but survival was worse for patients who resided in more deprived areas (HR = 1.06; 95% CI 1.01-1.11, $P = 0.025$). CONCLUSION: There have been large improvements in TYA cancer survival in Northern England over the last four decades. Future work should determine factors that could lead to even better survival, including possible links with delayed diagnosis.

http://www.ncbi.nlm.nih.gov/pubmed/24436167

Pediatric low-grade gliomas: How modern biology reshapes the clinical field

Bergthold, G., et al.

Biochim Biophys Acta 2014; 1845(2): 294-307

Low-grade gliomas represent the most frequent brain tumors arising during childhood. They are characterized by a broad and heterogeneous group of tumors that are currently classified by the WHO according to their morphological appearance. Here we review the clinical features of these tumors, current therapeutic strategies and the recent discovery of genomic alterations characteristic to these tumors. We further explore how these recent biological findings stand to transform the treatment for these tumors and impact the diagnostic criteria for pediatric low-grade gliomas.

http://www.ncbi.nlm.nih.gov/pubmed/24589977

Evidence of increasing mortality with longer time to diagnosis of cancer: Is there a paediatric exception?

Brasme, J. F., et al.

Eur J Cancer 2014; 50(4): 864-866

http://www.ncbi.nlm.nih.gov/pubmed/24388772

Pediatric human immunodeficiency virus infection and cancer in the highly active antiretroviral treatment (HAART) era

Chiappini, E., et al.

Cancer Lett 2014; 347(1): 38-45

Highly active antiretroviral therapy (HAART) changed the natural history of pediatric HIV infection. This review focuses on trends of HIV-associated cancers in childhood in the HAART era and analyses potential pathogenetic mechanisms. HAART reduced AIDS-defined-malignancies (ADM), but incidence of several non-ADM is increasing. HIV-associated immune activation and inflammation, promoting tumorigenesis, can only partially be reduced by HAART. In addition, HIV-infected children may undergo accelerated immune senescence that favors cancer development. How HAART affects this condition is an open question. Lastly, there is no evidence that prenatal exposure to HAART increases the risk of cancer in childhood, but long-term studies are needed.

http://www.ncbi.nlm.nih.gov/pubmed/24513180

Secondary malignancies in pediatric cancer survivors: perspectives and review of the literature

Choi, D. K., et al.

Int J Cancer 2014; 135(8): 1764-1773

With continuing improvements in the successful treatment of pediatric malignancies, long term survivors of pediatric cancers and their providers are faced with new oncologic issues regarding long-term morbidities. As pediatric cancer survivors have matured into adulthood, the development of secondary malignancies has become a significant issue for these patients. Whether a consequence of treatment for the patient's original cancer, such as chemotherapy, ionizing radiation, or hematopoietic stem cell transplantation, secondary malignancies now present patients and providers with new challenges regarding treatment, surveillance and counselling. We review the major risk factors for secondary malignancies in pediatric cancer survivors, with particular emphasis on important molecular and cytogenetic risk factors, both inherited and acquired. We conclude with a discussion of recommendations for surveillance and counselling of these patients.

http://www.ncbi.nlm.nih.gov/pubmed/24945137

Left ventricular function in long-term survivors of childhood lymphoma
Christiansen, J. R., et al.
Am J Cardiol 2014; 114(3): 483-490

Survivors of childhood lymphoma (CL) have markedly increased risk of developing heart failure. Echocardiographic studies after cardiotoxic treatment have primarily demonstrated left ventricular (LV) systolic dysfunction. In the present study, we hypothesized that longer follow-up and a more comprehensive echocardiographic examination would reveal more cardiac abnormalities. We conducted a cross-sectional study with echocardiography 20.4 +/- 8.6 years after diagnosis in 125 survivors of CL, grouped according to treatment methods, and compared with matched controls. Treatment included mediastinal radiotherapy (median 40.0 Gy) in 66 and anthracyclines (median dose 160 mg/m(2)) in 92 survivors of CL. Abnormal LV function, left-sided valve dysfunction, or both occurred in 62 patients (50%). Diastolic dysfunction occurred in 29%. Compared with control subjects, mitral annular early diastolic velocities (e') were reduced in patients (septal e' 0.09 +/- 0.03 vs 0.12 +/- 0.03 m/s, p <0.001), and the E/e' ratio was increased, particularly after mediastinal radiotherapy (10.6 +/- 6.4 vs 5.6 +/- 1.3, p <0.001). Survivors of CL had lower fractional shortening than control subjects (32 +/- 6 vs 36 +/- 7, p <0.001), but mean ejection fraction was equal and overt systolic dysfunction was infrequent. After mediastinal radiotherapy alone, global longitudinal myocardial strain was lower (p <0.05) compared with other treatment groups. Left-sided valvular dysfunction occurred in 55% of patients after mediastinal radiotherapy. In conclusion, survivors of CL had reduced LV diastolic function assessed by tissue Doppler imaging. This was more pronounced after mediastinal radiotherapy, which also frequently led to valvular disease. Systolic function was normal in most survivors of CL.

http://www.ncbi.nlm.nih.gov/pubmed/24948492

Cerebral palsy

Colver, A., et al.
Lancet 2014; 383(9924): 1240-1249

The syndrome of cerebral palsy encompasses a large group of childhood movement and posture disorders. Severity, patterns of motor involvement, and associated impairments such as those of communication, intellectual ability, and epilepsy vary widely. Overall prevalence has remained stable in the past 40 years at 2-3.5 cases per 1000 livebirths, despite changes in antenatal and perinatal care. The few studies available from developing countries suggest prevalence of comparable magnitude. Cerebral palsy is a lifelong disorder; approaches to intervention, whether at an individual or environmental level, should recognise that quality of life and social participation throughout life are what individuals with cerebral palsy seek, not improved physical function for its own sake. In the past few years, the cerebral palsy community has learned that the evidence of benefit for the numerous drugs, surgery, and therapies used over previous decades is weak. Improved understanding of the role of multiple gestation in pathogenesis, of gene environment interaction, and how to influence brain plasticity could yield significant advances in treatment of the disorder. Reduction in the prevalence of post-neonatal cerebral palsy, especially in developing countries, should be possible through improved nutrition, infection control, and accident prevention.

http://www.ncbi.nlm.nih.gov/pubmed/24268104

A comparison of pediatric, adolescent, and adult testicular germ cell malignancy

Cost, N. G., et al.

Pediatr Blood Cancer 2014; 61(3): 446-451

BACKGROUND: Testicular germ cell tumors (T-GCTs) occur from infancy to adulthood, and are the most common solid tumor in adolescent and young adult males. Traditionally, pediatric T-GCTs were perceived as more indolent than adult T-GCTs. However, there are few studies comparing these groups and none that specifically evaluate adolescents. METHODS: An institutional database of T-GCT patients was reviewed and patients were categorized into Pediatric, aged 0-12 years, Adolescent, aged 13-19 years, and Adult, older than 20 years, cohorts. Demographics, tumor characteristics, disease stage, treatment, event-free survival (EFS), and overall survival (OS) were compared between groups. RESULTS: Overall, 413 patients (20 pediatric, 39 adolescent, 354 adult) met study criteria and were followed for a median of 2.0 years (0.1-23.6). Adolescents presented with more advanced stage than children (P = 0.018) or adults (P = 0.008). There was a higher rate of events in Adolescents (13, 33.3%) than in Adults (61, 17.2%) or Children (2, 10.0%). Three-year EFS was 87.2% in the Pediatric group, 59.9% in Adolescents and 80.0% in Adults (P = 0.011). In a multivariate analysis, controlling for stage, IGCCCG risk, and histology, the hazard ratio (HR) for an event was: 1 (Reference) for Adults, HR = 0.82 (95% CI 0.19-3.46; P = 0.33) for the Pediatric group, and HR = 2.22 (95% CI 1.21-4.07; P = 0.01) for Adolescents. Five-year OS was 100% in the Pediatric group, 84.8% in Adolescents, and 92.8% in Adults (P = 0.388). CONCLUSION: Lower EFS in adolescent T-GCT patients was observed than in either children or adults. Elucidating factors associated with inferior outcomes in adolescents is an important focus of future research.

http://www.ncbi.nlm.nih.gov/pubmed/24106160

Relapsed and refractory pediatric acute myeloid leukemia: Current and emerging treatments

Davila, J., et al.

Paediatr Drugs 2014; 16(2): 151-168

Survival rates for children with acute myeloid leukemia (AML) exceed 60 % when modern, intensified chemotherapeutic regimens and enhanced supportive care measures are employed. Despite well-recognized improvements in outcomes, primary refractory or relapsed pediatric AML yields significant morbidity and mortality, and improved understanding of this obstinate population along with refined treatment protocols are urgently needed. Although a significant number of patients with refractory or relapsed disease will achieve remission, long-term survival rates remain poor, and efforts to identify therapies which will improve OS are under continuous investigation. The current fundamental goal of such investigation is the achievement of as complete a remission as possible without dose-limiting toxicities, and the progression to hematopoietic stem cell transplantation thereafter. In this review the scope of the problem of relapsed and refractory AML as well as current and emerging chemotherapy options will be discussed.

http://www.ncbi.nlm.nih.gov/pubmed/24158739

Sudden unexpected death in epilepsy: Who are the children at risk?

Donner, E. J.
Paediatr Child Health 2014; 19(7): 389

http://www.ncbi.nlm.nih.gov/pubmed/25332679

Neuromuscular disease and respiratory physiology in children: Putting lung function into perspective

Fauroux, B. and S. Khirani
Respirology 2014; 19(6): 782-791

Neuromuscular diseases represent a heterogeneous group of disorders of the muscle, nerve or neuromuscular junction. The respiratory muscles are rarely spared in neuromuscular diseases even if the type of muscle involvement, severity and time course greatly varies among the different diseases. Diagnosis of respiratory muscle weakness is crucial because of the importance of respiratory morbidity and mortality. Presently, routine respiratory evaluation is based on non-invasive volitional tests, such as the measurement of lung volumes, spirometry and the maximal static pressures, which may be difficult or impossible to obtain in some young children. Other tools or parameters are thus needed to assess the respiratory muscle weakness and its consequences in young children. The measurement of oesogastric pressures can be helpful as they allow the diagnosis and quantification of paradoxical breathing, as well as the assessment of the strength of the inspiratory and expiratory muscles by means of the oesophageal pressure during a maximal sniff and of the gastric pressure during a maximal cough. Sleep assessment should also be part of the respiratory evaluation of children with neuromuscular disease with at least the recording of nocturnal gas exchange if polysomnography is not possible or unavailable. This improvement in the assessment of respiratory muscle performance may increase our understanding of the respiratory pathophysiology of the different neuromuscular diseases, improve patient care, and guide research and innovative therapies by identifying and validating respiratory parameters.

http://www.ncbi.nlm.nih.gov/pubmed/24975704

Recent developments and current concepts in medulloblastoma

Gerber, N. U., et al.
Cancer Treat Rev 2014; 40(3): 356-365

Medulloblastoma is the most common malignant brain tumor of childhood. While prognosis has significantly improved in the last decades with multimodal therapy including surgery, radiotherapy, and chemotherapy, one third of patients still succumb to their disease. Further research is needed to find more efficient treatment strategies for prognostically unfavorable patient groups and to minimize long-term sequelae of tumor treatment. This review gives a summary of the current state of treatment concepts including an outlook on the near future. We describe recent advances in the understanding of molecular mechanisms, their potential impact on risk stratification in upcoming clinical trials, and perspectives for the clinical implementation of targeted therapies.

http://www.ncbi.nlm.nih.gov/pubmed/24389035

Estimating the prevalence of chronic conditions in children who die in England, Scotland and Wales: A data linkage cohort study

Hardelid, P., et al.
BMJ Open 2014; 4(8): e005331

OBJECTIVES: To estimate the proportion of children who die with chronic conditions and examine time trends in childhood deaths involving chronic conditions. DESIGN: Retrospective population-based death cohort study using linked death certificates and hospital discharge records. SETTING: England, Scotland and Wales. PARTICIPANTS: All resident children who died aged 1-18 years between 2001 and 2010. PRIMARY AND SECONDARY OUTCOME MEASURES: The primary outcome was the proportion of children who died with chronic conditions according to age group and type of chronic condition. The secondary outcome was trends over time in mortality rates involving chronic conditions per 100,000 children and trends in the proportion of children who died with chronic conditions. RESULTS: 65.4% of 23,438 children (95% CI 64.8%, 66.0%) died with chronic conditions, using information from death certificates. This increased to 70.7% (95% CI 70.1% to 71.3%) if hospital records up to 1 year before death were also included and was highest (74.8-79.9% depending on age group) among children aged less than 15 years. Using data from death certificates only led to underascertainment of all types of chronic conditions apart from cancer/blood conditions. Neurological/sensory conditions were most common (present in 38.5%). The rate of children dying with a chronic condition has declined since 2001, whereas the proportion of deaths affected by chronic conditions remained stable. CONCLUSIONS: The majority of children who died had a chronic condition. Neurological/sensory conditions were the most prevalent. Linkage between death certificate and hospital discharge data avoids some of the under-recording of non-cancer conditions on death certificates, and provides a low-cost, population-based method for monitoring chronic conditions in children who die.

http://www.ncbi.nlm.nih.gov/pubmed/25085264

Pulmonary presentation of relapsed acute myeloid leukemia

Hoffman, L. M., et al.

J Pediatr Hematol Oncol 2014; 36(3): 228-230

Extramedullary manifestations of acute myeloid leukemia (AML), often referred to as myeloid sarcoma (MS), occur relatively commonly in children with newly diagnosed or relapsed AML and have been associated with certain French-American-British morphologies and gene/chromosomal rearrangements. The most common locations of MS include the skin, orbit, skeleton, central nervous system, skin, and gut. Pulmonary MS is uncommon in adults and is extremely rare in children. We report the case of a 19-year-old man with French-American-British M5 AML, who before bone marrow transplant, presented with fever, hypotension, and respiratory symptoms that were ultimately attributed to pulmonary MS.

http://www.ncbi.nlm.nih.gov/pubmed/23619108

Consensus paper: Management of degenerative cerebellar disorders

Ilg, W., et al.

Cerebellum 2014; 13(2): 248-268

Treatment of motor symptoms of degenerative cerebellar ataxia remains difficult. Yet there are recent developments that are likely to lead to significant improvements in the future. Most desirable would be a causative treatment of the underlying cerebellar disease. This is currently available only for a very small subset of cerebellar ataxias with known metabolic dysfunction. However, increasing knowledge of the pathophysiology of hereditary ataxia should lead to an increasing number of medically sensible drug trials. In this paper, data from recent drug trials in patients with recessive and dominant cerebellar ataxias will be summarized. There is consensus that up to date, no medication has been proven effective. Aminopyridines and acetazolamide are the only exception, which are beneficial in patients with episodic ataxia type 2. Aminopyridines are also effective in a subset of patients presenting with downbeat nystagmus. As such, all authors agreed that the mainstays of treatment of degenerative cerebellar ataxia are currently physiotherapy, occupational therapy, and speech therapy. For many years, well-controlled rehabilitation studies in patients with cerebellar ataxia were lacking. Data of recently published studies show that coordinative training improves motor function in both adult and juvenile patients with cerebellar degeneration. Given the well-known contribution of the cerebellum to motor learning, possible mechanisms underlying improvement will be outlined. There is consensus that evidence-based guidelines for the physiotherapy of degenerative cerebellar ataxia need to be developed. Future developments in physiotherapeutical interventions will be discussed including application of non-invasive brain stimulation.

http://www.ncbi.nlm.nih.gov/pubmed/24222635

Skeletal health in Duchenne dystrophy: Bone-size and subcranial dual-energy X-ray absorptiometry analyses

King, W. M., et al.

Muscle Nerve 2014; 49(4): 512-519

INTRODUCTION: We performed subcranial and bone-size-adjusted whole body dual-energy X-ray absorptiometry (DXA) to evaluate skeletal health in Duchenne dystrophy (DMD). METHODS: Total body bone mineral density (TBBMD)-for-age, subcranial, and size-adjusted DXA analyses were performed on 22 DMD patients (5-17 years) and compared with 267 controls from a database. The skull contribution to total body bone mineral content (TBBMC) and corticosteroid effects were also examined. RESULTS: DMD boys had deficits in TBBMD-for-age (Z = -1.2), which increased with age. The skull's contribution to TBBMC decreased from 45% to 15% with growth. Z-scores for subcranial skeleton were significantly lower than TBBMC-for-area and TBBMD-for-age. CONCLUSIONS: Size-adjusted and subcranial analyses improve evaluation of whole body DXA. DMD boys have low BMD for size not commensurate with total body areal BMD-for-age. Bone fragility fractures in DMD may result from both decreased BMD and smaller bones. This information is vital to determine appropriate intervention. Muscle Nerve 49:512-519, 2014.

http://www.ncbi.nlm.nih.gov/pubmed/23893858

Childhood cancer in Africa

Kruger, M., et al.

Pediatr Blood Cancer 2014; 61(4): 587-592

The majority of children with cancer live in low- and middle-income countries (LMICs) with little or no access to cancer treatment. The purpose of the paper is to describe the current status of childhood cancer treatment in Africa, as documented in publications, dedicated websites and information collected through surveys. Successful twinning programmes, like those in Malawi and Cameroon, as well as the collaborative clinical trial approach of the Franco-African Childhood Cancer Group (GFAOP), provide good models for childhood cancer treatment. The overview will hopefully influence health-care policies to facilitate access to cancer care for all children in Africa.

http://www.ncbi.nlm.nih.gov/pubmed/24214130

Variants of cardiomyopathy and hypertension in neuroblastoma

Kwok, S. Y., et al.

J Pediatr Hematol Oncol 2014; 36(3): e158-161

Catecholamine-associated cardiomyopathies caused by neuroblastoma have rarely been reported. We are reporting 2 cases of neuroblastoma associated with hypertension and severe cardiomyopathic changes in different extremes. One case was dilated cardiomyopathy with heart failure, and the other showed echocardiographic features simulating hypertrophic obstructive cardiomyopathy. Both girls had high levels of urine catecholamines on presentation. Anthracycline group of chemotherapy was avoided. Chemotherapy and tumor resection resulted in successful normalization of blood pressure and regression of cardiomyopathic changes. Blood pressure and cardiomyopathic changes should be monitored not only at presentation, but also during the treatment for neuroblastoma.

http://www.ncbi.nlm.nih.gov/pubmed/23652880

X marks the spot: Duchenne's cardiomyopathy

Lee, J. J., et al.
Am J Med 2014; 127(7): e13-14

http://www.ncbi.nlm.nih.gov/pubmed/24726503

Niemann-Pick disease type C: A case series of Brazilian patients

Lorenzoni, P. J., et al.
Arq Neuropsiquiatr 2014; 72(3): 214-218

The aim of the study was to analyze a series of Brazilian patients with Niemann-Pick disease type C (NP-C). METHOD: Correlations between clinical findings, laboratory data, molecular findings and treatment response are presented. RESULT: The sample consisted of 5 patients aged 8 to 26 years. Vertical supranuclear gaze palsy, cerebellar ataxia, dementia, dystonia and dysarthria were present in all cases. Filipin staining showed the "classical" pattern in two patients and a "variant" pattern in three patients. Molecular analysis found mutations in the NPC1 gene in all alleles. Miglustat treatment was administered to 4 patients. CONCLUSION: Although filipin staining should be used to confirm the diagnosis, bone marrow sea-blue histiocytes often help to diagnosis of NP-C. The p.P1007A mutation seems to be correlated with the "variant" pattern in filipin staining. Miglustat treatment response seems to be correlated with the age at disease onset and disability scale score at diagnosis.

http://www.ncbi.nlm.nih.gov/pubmed/24676439

Critical issues for the proper diagnosis of Metachromatic Leukodystrophy

Lorioli, L., et al.
Gene 2014; 537(2): 348-351

Metachromatic Leukodystrophy is a lysosomal storage disorder caused by Arylsulfatase A deficiency. Diagnosis is usually performed by measurement of enzymatic activity and/or characterization of the gene mutations. Here we describe a family case in which the determination of enzyme activity alone did not allow diagnosis of the pre-symptomatic sibling of the index case. Only combination of gene sequencing with thorough biochemical analysis allowed the correct diagnosis of the sibling, who was promptly directed to treatment.

http://www.ncbi.nlm.nih.gov/pubmed/24334127

Hemolysis in pediatric patients receiving centrifugal-pump extracorporeal membrane oxygenation: Prevalence, risk factors, and outcomes

Lou, S., et al.

Crit Care Med 2014; 42(5): 1213-1220

OBJECTIVES: To explore the prevalence and risk factors for hemolysis in children receiving extracorporeal membrane oxygenation and examine the relationship between hemolysis and adverse outcomes. DESIGN: Retrospective, single-center study. SETTING: Tertiary PICU. PATIENTS: Two hundred seven children receiving extracorporeal membrane oxygenation. INTERVENTIONS: None. MEASUREMENTS AND MAIN RESULTS: Plasma-free hemoglobin was tested daily and hemolysis was diagnosed based on peak plasma-free hemoglobin as mild (< 0.5 g/L), moderate (0.5-1.0 g/L), or severe (> 1.0 g/L). Gender, age, weight, diagnosis, oxygenator type, cannulation site, mean venous inlet pressure, mean pump speed, mean flow, and visible clots in the extracorporeal membrane oxygenation circuit were entered into the ordered logistic regression model to identify risk factors of hemolysis. Complications and clinical outcomes were compared across four hemolysis groups. Of the 207 patients, 69 patients (33.3%; 95% CI, 27.0-40.2%) did not have hemolysis, 98 patients (47.3%; 95% CI, 40.4-54.4%) had mild hemolysis, 26 patients (12.5%; 95% CI, 8.4-17.9%) had moderate hemolysis, and 14 patients (6.8%; 95% CI, 3.7-11.1%) had severe hemolysis with a median peak plasma-free hemoglobin of 1.51 g/L (1.18-2.05 g/L). The independent risk factors for hemolysis during extracorporeal membrane oxygenation were use of Quadrox D (odds ratio, 7.25; 95% CI, 3.10-16.95; p < 0.001) or Lilliput (odds ratio, 37.32; 95% CI, 8.95-155.56; p < 0.001) oxygenators, mean venous inlet pressure (odds ratio, 0.95; 95% CI, 0.91-0.98; p = 0.002), and mean pump speed (odds ratio, 2.89; 95% CI, 1.36-6.14; p = 0.006). Patients with hemolysis were more likely to experience a longer extracorporeal membrane oxygenation run and require more blood products. After controlling for age, weight, pediatric index of mortality 2, and diagnosis, patients with severe hemolysis were more likely to die in the ICU (odds ratio, 5.93; 95% CI, 1.64-21.43; p = 0.007) and in hospital (odds ratio, 6.34; 95% CI, 1.71-23.54; p = 0.006). CONCLUSIONS: Hemolysis during extracorporeal membrane oxygenation with centrifugal pumps was common and associated with a number of adverse outcomes. Risk factors for hemolysis included oxygenator types, mean venous inlet pressure, and mean pump speed. Further studies are warranted comparing pump types while controlling both physical and nonphysical confounders.

http://www.ncbi.nlm.nih.gov/pubmed/24351369

HIV infection: Epidemiology, pathogenesis, treatment, and prevention

Maartens, G., et al.

Lancet 2014; 384(9939): 258-271

HIV prevalence is increasing worldwide because people on antiretroviral therapy are living longer, although new infections decreased from 3.3 million in 2002, to 2.3 million in 2012. Global AIDS-related deaths peaked at 2.3 million in 2005, and decreased to 1.6 million by 2012. An estimated 9.7 million people in low-income and middle-income countries had started antiretroviral therapy by 2012. New insights into the mechanisms of latent infection and the importance of reservoirs of infection might eventually lead to a cure. The role of immune activation in the pathogenesis of non-AIDS clinical events (major causes of morbidity and mortality in people on antiretroviral therapy) is receiving increased recognition. Breakthroughs in the prevention of HIV important to public health include male medical circumcision, antiretrovirals to prevent mother-to-child transmission, antiretroviral therapy in people with HIV to prevent transmission, and antiretrovirals for pre-exposure prophylaxis. Research into other prevention interventions, notably vaccines and vaginal microbicides, is in progress.

http://www.ncbi.nlm.nih.gov/pubmed/24907868

Stroke and nonstroke brain attacks in children

Mackay, M. T., et al.
Neurology 2014; 82(16): 1434-1440

OBJECTIVES: To determine symptoms, signs, and etiology of brain attacks in children presenting to the emergency department (ED) as a first step for developing a pediatric brain attack pathway. METHODS: Prospective observational study of children aged 1 month to 18 years with brain attacks (defined as apparently abrupt-onset focal brain dysfunction) and ongoing symptoms or signs on arrival to the ED. Exclusion criteria included epilepsy, hydrocephalus, head trauma, and isolated headache. Etiology was determined after review of clinical data, neuroimaging, and other investigations. A random-effects meta-analysis of similar adult studies was compared with the current study. RESULTS: There were 287 children (46% male) with 301 presentations over 17 months. Thirty-five percent arrived by ambulance. Median symptom duration before arrival was 6 hours (interquartile range 2-28 hours). Median time from triage to medical assessment was 22 minutes (interquartile range 6-55 minutes). Common symptoms included headache (56%), vomiting (36%), focal weakness (35%), numbness (24%), visual disturbance (23%), seizures (21%), and altered consciousness (21%). Common signs included focal weakness (31%), numbness (13%), ataxia (10%), or speech disturbance (8%). Neuroimaging included CT imaging (30%), which was abnormal in 27%, and MRI (31%), which was abnormal in 62%. The most common diagnoses included migraine (28%), seizures (15%), Bell palsy (10%), stroke (7%), and conversion disorders (6%). Relative proportions of conditions in children significantly differed from adults for stroke, migraine, seizures, and conversion disorders. CONCLUSIONS: Brain attack etiologies differ from adults, with stroke being the fourth most common diagnosis. These findings will inform development of ED clinical pathways for pediatric brain attacks.

http://www.ncbi.nlm.nih.gov/pubmed/24658929

The prenatal origins of cancer

Marshall, G. M., et al.
Nat Rev Cancer 2014; 14(4): 277-289

The concept that some childhood malignancies arise from postnatally persistent embryonal cells has a long history. Recent research has strengthened the links between driver mutations and embryonal and early postnatal development. This evidence, coupled with much greater detail on the cell of origin and the initial steps in embryonal cancer initiation, has identified important therapeutic targets and provided renewed interest in strategies for the early detection and prevention of childhood cancer.

http://www.ncbi.nlm.nih.gov/pubmed/24599217

Recent advances in understanding the etiology and pathogenesis of pediatric germ cell tumors

Mosbech, C. H., et al.

J Pediatr Hematol Oncol 2014; 36(4): 263-270

Pediatric germ cell tumors (GCTs) are rare neoplasms arising predominantly in the gonads and sacrococcygeal, mediastinal, and intracranial localizations. In this article, we review current knowledge of pathogenesis of pediatric GCTs, which differs from adult/adolescent GCTs. One distinctive feature is the absence of a progenitor stage, such as carcinoma in situ or gonadoblastoma, which are seen in adult/adolescent GCTs, except spermatocytic seminoma. The primordial germ cell (PGC) is the suggested origin of all GCTs, with variations in histology reflecting differentiation stage. Expression of pluripotency transcription factors OCT-3/4, NANOG, and AP-2gamma in germinomas/seminomas/dysgerminomas is consistent with retaining a germ cell phenotype. Teratomas, in contrast, develop through a pathway of aberrant somatic differentiation of immature germ cells, and the yolk sac tumors and choriocarcinomas result from abnormal extraembryonic differentiation. In pediatric GCTs, origin is suggested at an earlier developmental stage because of predisposing genetic factors, although responsible genes remain largely unknown. Some extragonadal GCTs have been linked to overexpression of the KIT/KITLG system, allowing for survival of aberrantly migrated ectopic PGCs. Infant gonadal/sacrococcygeal GCTs may be caused by apoptosis-related pathways, consistent with an association with polymorphisms in BAK1. Although recent advances have identified candidate pathways, further effort is needed to answer central questions of pathogenesis of these fascinating tumors.

http://www.ncbi.nlm.nih.gov/pubmed/24577549

Neuroblastoma in older children, adolescents and young adults: A report from the International Neuroblastoma Risk Group project

Mosse, Y. P., et al.

Pediatr Blood Cancer 2014; 61(4): 627-635

BACKGROUND: Neuroblastoma in older children and adolescents has a distinctive, indolent phenotype, but little is known about the clinical and biological characteristics that distinguish this rare subgroup. Our goal was to determine if an optimal age cut-off exists that defines indolent disease and if accepted prognostic factors and treatment approaches are applicable to older children. PROCEDURE: Using data from the International Neuroblastoma Risk Group, among patients >/=18 months old (n = 4,027), monthly age cut-offs were tested to determine the effect of age on survival. The prognostic effect of baseline characteristics and autologous hematopoietic cell transplant (AHCT) for advanced disease was assessed within two age cohorts; >/=5 to <10 years (n = 730) and >/=10 years (n = 200). RESULTS: Older age was prognostic of poor survival, with outcome gradually worsening with increasing age at diagnosis, without statistical evidence for an optimal age cut-off beyond 18 months. Among patients >/=5 years, factors significantly prognostic of lower event-free survival (EFS) and overall survival (OS) in multivariable analyses were INSS stage 4, MYCN amplification and unfavorable INPC histology classification. Among stage 4 patients, AHCT provided a significant EFS and OS benefit. Following relapse, patients in both older cohorts had prolonged OS compared to those >/=18 months to <5 years (P < 0.0001). CONCLUSIONS: Despite indolent disease and infrequent MYCN amplification, older children with advanced disease have poor survival, without evidence for a specific age cut-off. Our data suggest that AHCT may provide a survival benefit in older children with advanced disease. Novel therapeutic approaches are required to more effectively treat these patients.

http://www.ncbi.nlm.nih.gov/pubmed/24038992

PDE5 inhibition alleviates functional muscle ischemia in boys with Duchenne muscular dystrophy

Nelson, M. D., et al.

Neurology 2014; 82(23): 2085-2091

OBJECTIVE: To determine whether phosphodiesterase type 5 (PDE5) inhibition can alleviate exercise-induced skeletal muscle ischemia in boys with Duchenne muscular dystrophy (DMD). METHODS: In 10 boys with DMD and 10 healthy age-matched male controls, we assessed exercise-induced attenuation of reflex sympathetic vasoconstriction, i.e., functional sympatholysis, a protective mechanism that matches oxygen delivery to metabolic demand. Reflex vasoconstriction was induced by simulated orthostatic stress, measured as the decrease in forearm muscle oxygenation with near-infrared spectroscopy, and performed when the forearm muscles were rested or lightly exercised with rhythmic handgrip exercise. Then, the patients underwent an open-label, dose-escalation, crossover trial with single oral doses of tadalafil or sildenafil. RESULTS: The major new findings are 2-fold: first, sympatholysis is impaired in boys with DMD-producing functional muscle ischemia-despite contemporary background therapy with corticosteroids alone or in combination with cardioprotective medication. Second, PDE5 inhibition with standard clinical doses of either tadalafil or sildenafil alleviates this ischemia in a dose-dependent manner. Furthermore, PDE5 inhibition also normalizes the exercise-induced increase in skeletal muscle blood flow (measured by Doppler ultrasound), which is markedly blunted in boys with DMD. CONCLUSIONS: These data provide in-human proof of concept for PDE5 inhibition as a putative new therapeutic strategy for DMD. CLASSIFICATION OF EVIDENCE: This study provides Class IV evidence that in patients with DMD, PDE5 inhibition restores functional sympatholysis.

http://www.ncbi.nlm.nih.gov/pubmed/24808022

Brain tumours in children: Reducing time to diagnosis

Paul, S. P., et al.

Emerg Nurse 2014; 22(1): 32-36; quiz 37

Although the leading cause of childhood, cancer-related deaths, initial presentations of brain tumours can mimic less serious conditions, which can delay diagnosis. To reduce the time between presentation and diagnosis, the HeadSmart campaign has produced a symptom card to raise suspicion of brain tumours among healthcare professionals in all settings. This article refers to two case studies to describe how emergency department nurses can recognise the signs and symptoms of brain tumours in children. Definitive management is delivered in specialist centres, so safe and prompt transfer is a priority once children are stabilised.

http://www.ncbi.nlm.nih.gov/pubmed/24689482

Sibling concordance for clinical features of Duchenne and Becker muscular dystrophies

Pettygrove, S., et al.

Muscle Nerve 2014; 49(6): 814-821

INTRODUCTION: The correlation of markers of disease severity among brothers with Duchenne or Becker muscular dystrophy has implications for clinical guidance and clinical trials. METHODS: Sibling pairs with Duchenne or Becker muscular dystrophy (n = 60) were compared for ages when they reached clinical milestones of disease progression, including ceased ambulation, scoliosis of >/= 20 degrees , and development of cardiomyopathy. RESULTS: The median age at which younger brothers reached each milestone, compared with their older brothers ranged from 25 months younger for development of cardiomyopathy to 2 months older for ceased ambulation. For each additional month of ambulation by the older brother, the hazard of ceased ambulation by the younger brother decreased by 4%. CONCLUSIONS: The ages when siblings reach clinical milestones of disease vary widely between siblings. However, the time to ceased ambulation for older brothers predicts the time to ceased ambulation for their younger brothers.

http://www.ncbi.nlm.nih.gov/pubmed/24030636

Pediatric oncologic emergencies

Prusakowski, M. K. and D. Cannone

Emerg Med Clin North Am 2014; 32(3): 527-548

The overall prognosis for most pediatric cancers is good. Mortality for all childhood cancers combined is approximately half what it was in 1975, and the survival rates of many malignancies continue to improve. However, the incidence of childhood cancer is significant and the related emergencies that develop acutely carry significant morbidity and mortality. Emergency providers who can identify and manage oncologic emergencies can contribute significantly to an improved prognosis. Effective care of pediatric malignancies requires an age-appropriate approach to patients and compassionate understanding of family dynamics.

http://www.ncbi.nlm.nih.gov/pubmed/25060248

Weight charts of infants dying of sudden infant death in England

Scheimberg, I., et al.
Pediatr Dev Pathol 2014; 17(4): 271-277

The organ weights in cases of sudden infant death syndrome (SIDS) and undetermined deaths in previously healthy infants do not correspond to "the normal range" of organ weights in international standard charts for infants currently in use in some institutions. The aim of our study was to ascertain the organ weights of infants dying suddenly and unexpectedly in England and for whom a cause of death was not found, therefore falling under the category of SIDS or undetermined. We collated the organs weights from 2 institutions covering between them the South East and North of England including London, Yorkshire, and Derbyshire. The cases from The Royal London Hospital were autopsied between 1997 and 2013, and the cases from Sheffield Children's Hospital were autopsied between 2006 and 2013. There were 188 babies who had been born at term (62 female and 126 male) and 26 ex-premature babies (15 female and 11 male). Organs of male babies were slightly heavier than those of female babies but as there was no significant differences male and female babies were considered together. Comparison with standard charts (from 1932 and 1962) and with more recent charts confirmed the discrepancy between the older charts commonly in use with more recent measurements, including ours. The main reason for these differences is that babies in the recent charts were previously healthy babies with no long term disease and improved in the health of the population.

http://www.ncbi.nlm.nih.gov/pubmed/24856661

Patterns of child death in England and Wales

Sidebotham, P., et al.
Lancet 2014; 384(9946): 904-914

In the past century, child mortality has fallen to very low rates in all developed countries. However, rates between and within countries vary widely, and factors can be identified that could be modified to reduce the risk of future deaths. An understanding of the nature and patterns of child death and of the factors contributing to child deaths is essential to drive preventive initiatives. We discuss the epidemiology of child deaths in England and Wales. We use available data, particularly that of death registration and other available datasets, and published literature to emphasise issues relevant to reduction of child deaths in developed countries. We examine the different patterns of mortality at different ages in five broad categories of death: perinatal causes, congenital abnormalities, acquired natural causes, external causes, and unexplained deaths. For each category, we explore what is known about the main causes of death and some of the contributory factors. We then explain how this knowledge might be used to help to drive prevention initiatives.

http://www.ncbi.nlm.nih.gov/pubmed/25209490

Pretransplant comorbidities predict severity of acute graft-versus-host disease and subsequent mortality

Sorror, M. L., et al.
Blood 2014; 124(2): 287-295

Whether the hematopoietic cell transplantation comorbidity index (HCT-CI) can provide prognostic information about development of acute graft-versus-host disease (GVHD) and subsequent mortality is unknown. Five institutions contributed information on 2985 patients given human leukocyte antigen-matched grafts to address this question. Proportional hazards models were used to estimate the hazards of acute GVHD and post-GVHD mortality after adjustment for known risk variables. Higher HCT-CI scores predicted increased risk of grades 3 to 4 acute GVHD (P < .0001 and c-statistic of 0.64), and tests of interaction suggested that this association was consistent among different conditioning intensities, donor types, and stem cell sources. Probabilities of grades 3 to 4 GVHD were 13%, 18%, and 24% for HCT-CI risk groups of 0, 1 to 4, and >/=5. The HCT-CI was statistically significantly associated with mortality rates following diagnosis of grade 2 (hazard ratio [HR] = 1.24; P < .0001) or grades 3 to 4 acute GVHD (HR = 1.19; P < .0001). Patients with HCT-CI scores of >/=3 who developed grades 3 to 4 acute GVHD had a 2.63-fold higher risk of mortality than those with scores of 0 to 2 and did not develop acute GVHD. Thus, pretransplant comorbidities are associated with the development and severity of acute GVHD and with post-GVHD mortality. The HCT-CI could be useful in designing trials for GVHD prevention and could inform expectations for GVHD treatment trials.

http://www.ncbi.nlm.nih.gov/pubmed/24797298

Antenatal detection of Edwards (Trisomy 18) and Patau (Trisomy 13) syndrome: England and Wales 2005-2012

Springett, A. L. and J. K. Morris
J Med Screen 2014; 21(3): 113-119

OBJECTIVES: Pregnancies with Edwards or Patau syndrome are often detected through screening for Down's syndrome. We aimed to evaluate the impact of screening for Down's syndrome on the prevalence of live births and antenatal diagnoses of Edwards and Patau syndrome. SETTING: England and Wales, 2005 to 2012. METHODS: Data from the National Down Syndrome Cytogenetic Register, which contains information on nearly all ante- or postnatally diagnosed cases of Edwards or Patau syndrome in which a karyotype was confirmed, were analysed. RESULTS: From 2005 to 2012, 3,941 diagnoses of Edwards syndrome and 1,567 diagnoses of Patau syndrome were recorded (prevalence of 7.0 and 2.8 per 10,000 births respectively). Only 11% (95% confidence interval [CI]: 10-12) of diagnoses of Edwards syndrome and 13% (95% CI: 11-14) of Patau syndrome were live births, resulting in live birth prevalences of 0.8 (95% CI: 0.7-0.8) and 0.4 (95% CI: 0.3-0.4) per 10,000 live births respectively. About 90% of pregnancies with Edwards or Patau syndrome were diagnosed antenatally, and this proportion remained constant over time. The proportion of diagnoses detected before 15 weeks increased from 50% in 2005 to 53% in 2012 for Edwards syndrome, and from 41% in 2005 to 63% in 2012 for Patau syndrome. CONCLUSIONS: Almost 700 women per year had a pregnancy with Edwards or Patau syndrome. Over 90% of these pregnancies were detected antenatally, with the increased use of first trimester screening for Down's syndrome resulting in the reduction in the mean gestational age at diagnosis of these syndromes.

http://www.ncbi.nlm.nih.gov/pubmed/24993362

Cooperative International Neuromuscular Research Group Duchenne Natural History Study demonstrates insufficient diagnosis and treatment of cardiomyopathy in Duchenne muscular dystrophy

Spurney, C., et al.

Muscle Nerve 2014; 50(2): 250-256

INTRODUCTION: Cardiomyopathy is a common cause of morbidity and death in patients with Duchenne muscular dystrophy (DMD). METHODS: This investigation was a cross-sectional cross-sectional analysis of clinical data from the multi-institutional Cooperative International Neuromuscular Research Group (CINRG) DMD Natural History Study of 340 DMD patients aged 2-28 years. Cardiomyopathy was defined as shortening fraction (SF) <28% or ejection fraction (EF) <55%. RESULTS: Two hundred thirty-one participants reported a prior clinical echocardiogram study, and 174 had data for SF or EF. The prevalence of cardiomyopathy was 27% (47 of 174), and it was associated significantly with age and clinical stage. The association of cardiomyopathy with age and clinical stage was not changed by glucocorticoid use as a covariate ($P > 0.68$). In patients with cardiomyopathy, 57% (27 of 47) reported not taking any cardiac medications. Cardiac medications were used in 12% (15 of 127) of patients without cardiomyopathy. CONCLUSIONS: We found that echocardiograms were underutilized, and cardiomyopathy was undertreated in this DMD natural history cohort.

http://www.ncbi.nlm.nih.gov/pubmed/24395289

Childhood and adolescent cancer statistics

Ward, E., et al.

CA Cancer J Clin 2014; 64(2): 83-103

In this article, the American Cancer Society provides estimates of the number of new cancer cases and deaths for children and adolescents in the United States and summarizes the most recent and comprehensive data on cancer incidence, mortality, and survival from the National Cancer Institute, the Centers for Disease Control and Prevention, and the North American Association of Central Cancer Registries (which are reported in detail for the first time here and include high-quality data from 45 states and the District of Columbia, covering 90% of the US population). In 2014, an estimated 15,780 new cases of cancer will be diagnosed and 1960 deaths from cancer will occur among children and adolescents aged birth to 19 years. The annual incidence rate of cancer in children and adolescents is 186.6 per 1 million children aged birth to 19 years. Approximately 1 in 285 children will be diagnosed with cancer before age 20 years, and approximately 1 in 530 young adults between the ages of 20 and 39 years is a childhood cancer survivor. It is therefore likely that most pediatric and primary care practices will be involved in the diagnosis, treatment, and follow-up of young patients and survivors. In addition to cancer statistics, this article will provide an overview of risk factors, symptoms, treatment, and long-term and late effects for common pediatric cancers.

http://www.ncbi.nlm.nih.gov/pubmed/24488779

Deaths in young people aged 0-24 years in the UK compared with the EU15+ countries, 1970-2008: analysis of the WHO Mortality Database

Viner, R. M., et al.

Lancet 2014; 384(9946): 880-892

BACKGROUND: Concern is growing that mortality and health in children and young people in the UK lags behind that of similar countries. METHODS: We analysed death registry data provided to the WHO Mortality Database to compare UK mortality for children and young people aged 0-24 years with that of European Union member states (before May, 2004, excluding the UK, plus Australia, Canada, and Norway [the EU15+ countries]) from 1970 to 2008 using the WHO World Mortality Database. We grouped causes of death by Global Burden of Disease classification: communicable, nutritional, or maternal causes; non-communicable disorders; and injury. UK mortality trends were compared with quartiles of mortality in EU15+ countries. We used quasi-likelihood Poisson models to explore differences between intercepts and slopes between the UK and the EU15+ countries. FINDINGS: In 1970, UK total mortality was in the best EU15+ quartile (<25th centile) for children and young people aged 1-24 years, with UK infant mortality similar to the EU15+ median. Subsequent mortality reductions in the UK were smaller than were those in the EU15+ countries in all age groups. By 2008, total mortality for neonates, infants, and children aged 1-4 years in the UK was in the worst EU15+ quartile (>75th centile). In 2008, UK annual excess mortality compared with the EU15+ median was 1035 deaths for infants and 134 for children aged 1-9 years. Mortality from non-communicable diseases in the UK fell from being roughly equivalent to the EU15+ median in 1970 to the worst quartile in all age groups by 2008, with 446 annual excess deaths from non-communicable diseases in the UK (280 for young people aged 10-24 years) in 2008. UK mortality from injury remained in the best EU15+ quartile for the study period in all age groups. INTERPRETATION: The UK has not matched the gains made in child, adolescent, and young adult mortality by other comparable countries in the 40 years since 1970, particularly for infant deaths and mortality from non-communicable diseases, including neuropsychiatric disorders. The UK needs to identify and address amenable social determinants and health system factors that lead to poor health outcomes for infants and for children and young people with chronic disorders. FUNDING: None.

http://www.ncbi.nlm.nih.gov/pubmed/24929452

The pathological spectrum of solid CNS metastases in the pediatric population

Wiens, A. L. and E. M. Hattab

J Neurosurg Pediatr 2014; 14(2): 129-135

OBJECT: Collectively, metastatic tumors are the most common malignancy encountered in the adult central nervous system (CNS), arising most often from lung, breast, skin, and gastrointestinal tract carcinomas. Limited information is available in the literature regarding solid nonhematopoietic CNS metastases in children. The authors carried out a retrospective study of pediatric metastatic neoplasms to the CNS treated in a 30-year period to characterize their frequency, common histological subtypes, and sites of origin. METHODS: The archival pathology files were searched (1981-2011) for metastatic tumors to the CNS in patients 21 years of age and younger. Pathology material was reviewed, tumors were classified by site of origin and histological subtype, and survival was evaluated. RESULTS: The authors identified 26 patients with solid nonhematopoietic CNS metastases out of 1135 pediatric CNS tumors diagnosed from 1981 to 2011. Patients ranged in age from 1.5 to 20.3 years and were equally divided between sexes. Most CNS metastases were supratentorial (85%) and solitary (65%). The mean interval from primary malignant diagnosis to CNS metastasis was 27 months. Sites of origin included kidney/adrenal, bone/soft tissue, gonads, head and neck, lung, and liver. Mean survival after CNS involvement was 36.6 months. Overall 1-year and 5-year survival rates were 52% and 16%, respectively. CONCLUSIONS: In neuropathology practice, nonhematopoietic pediatric CNS metastases are far less common than are nonhematopoietic adult CNS metastases, accounting for approximately 2% of all pediatric CNS tumors. The most common tumors to exhibit CNS metastasis are of kidney/adrenal origin, followed by those from bone/soft tissue. As expected, prognosis is dismal, despite aggressive therapy.

http://www.ncbi.nlm.nih.gov/pubmed/24926970

Hepatic tumours in children with biliary atresia: Single-centre experience in 13 cases and review of the literature

Yoon, H. J., et al.

Clin Radiol 2014; 69(3): e113-119

AIM: To establish the risks of developing of hepatic tumours and to investigate their clinical and imaging findings in children with biliary atresia (BA) after Kasai portoenterostomy (Kasai). MATERIALS AND METHODS: Among 157 children who had undergone Kasai for BA over an 18 year period, patients who had newly developed hepatic tumours were identified. Patient demographics, clinical features, and imaging findings were retrospectively reviewed. RESULTS: Three male and 10 female patients (mean age 3.9 years) all (8%, of 157) had single hepatic tumours, which were confirmed in 10 explanted and three non-explanted livers. Ten (77%) were benign and three (23%) were malignant. Of the benign hepatic tumours, focal nodular hyperplasia (FNH; n = 6) was the most common, followed by regenerative nodules (n = 3) and adenoma (n = 1). All FNH appeared in young children <1 year of age and showed a subcapsular location, bulging contour, and lack of central scar. Malignant tumours included two hepatocellular carcinomas and one cholangiocarcinoma. CONCLUSION: Hepatic tumours developed in approximately 8% of children with BA after Kasai. Although benign tumours, including FNHs and regenerative nodules, were more common than malignant tumours, screening with alpha-foetoprotein (AFP) levels and regular imaging studies are the mainstay of malignant tumour detection.

http://www.ncbi.nlm.nih.gov/pubmed/24332171

Is the UK NICE "reference case" influencing the practice of pediatric quality-adjusted life-year measurement within economic evaluations?

Adlard, N., et al.

Value Health 2014; 17(4): 454-461

OBJECTIVES: To report findings from a systematic review, this article sought to address two related questions. First, how has the practice of UK pediatric cost-utility analyses evolved over time, in particular how are health-related outcomes assessed and valued? Second, how do the methods compare to the limited guidance available, in particular, the National Institute for Health and Care Excellence (NICE) reference case(s)? METHODS: Electronic searches of MEDLINE, Embase, and Cochrane databases were conducted for the period May 2004 to April 2012 and the Paediatric Economic Database Evaluation database for the period May 2004 to December 2010. Identified studies were screened by three independent reviewers. RESULTS: Forty-three studies were identified, 11 of which elicit utility values through primary research. A discrepancy was identified between the methods used for outcome measurement and valuation and the methods advocated within the NICE reference case. Despite NICE recommending the use of preference-based instruments designed specifically for children, most studies that were identified had used adult measures. In fact, the measurement of quality-adjusted life-years is the aspect of economic evaluation with the greatest amount of variability and the area that most digressed from the NICE reference case. CONCLUSIONS: Recommendations stemming from the review are that all studies should specify the age range of childhood and include separate statements of perspective for costs and effects as well as the reallocation of research funding away from systematic review studies toward good quality primary research measuring utilities in children.

http://www.ncbi.nlm.nih.gov/pubmed/24969007

Earlier stage 1 palliation is associated with better clinical outcomes and lower costs for neonates with hypoplastic left heart syndrome

Anderson, B. R., et al.

J Thorac Cardiovasc Surg 2014

OBJECTIVES: Our aim was to examine the effects of surgical timing on major morbidity, mortality, and total hospital reimbursement for late preterm and term infants with hypoplastic left heart syndrome (HLHS) undergoing stage 1 palliation within the first 2 weeks of life. METHODS: We conducted a retrospective cohort study of infants aged >/=35 weeks gestation, with HLHS, admitted to our institution at age </=5 days, between January 1, 2003, and January 1, 2013. Children with other cardiac abnormalities or other major comorbid conditions were excluded. Univariable and multivariable analyses were performed to determine the association between age at stage 1 palliation and major morbidity, mortality, and hospital reimbursement. RESULTS: One hundred thirty-four children met inclusion criteria. Mortality was 7.5% (n = 10). Forty-three percent (n = 58) experienced major morbidity. Median costs were $97,000, in 2013 dollars (interquartile range, $72,000-$151,000). Median age at operation was 5 days (interquartile range, 3-7 days; full range, 1-14 days). All deaths occurred in patients operated on between 4 and 8 days of life. For every day later that surgery was performed, the odds of major morbidity rose by 15.7% (95% confidence interval, 2.5%-30.7%; P = .018) and costs rose by 4.7% (95% confidence interval, 0.9%-8.2%; P < .014). CONCLUSIONS: Delay of stage 1 palliation for neonates with HLHS is associated with increased morbidity and health care costs, even within the first 2 weeks of life.

http://www.ncbi.nlm.nih.gov/pubmed/25227701

Outcome differences in patients with precursor B cell acute lymphocytic leukemia over time: A retrospective analysis

Apel, A., et al.

Isr Med Assoc J 2014; 16(4): 224-228

BACKGROUND: Acute lymphocytic leukemia (ALL) is a rare disease with a poor outcome in adults. Over the years different protocols have been developed with the aim of improving the outcome. The German study group protocols (GMALL), which are the most frequently used in our institutions, changed significantly between the periods 1989-93 and 1999-2003. OBJECTIVES: To investigate whether the change in protocols over the years resulted in an outcome difference at two hospitals in Israel. METHODS: We thoroughly reviewed the records of 153 patients from Sheba Medical Center and Soroka Medical Center, of whom 106 comprised the study group. The patients were divided into two groups according to the treatment protocol used: 40 patients with the 1989/93 protocol and 66 with the 1999/2003 protocol. Outcome was analyzed for the two groups. RESULTS: We found a significant difference in disease-free survival (DFS) between the two groups for B cell-ALL (B-ALL) patients who achieved complete remission after induction. There was no difference in overall survival. We did not find any difference in outcome for T cell-ALL patients or for CD20-positive patients. CONCLUSIONS: In our retrospective analysis, GMALL 99/2003 led to a better DFS for B-ALL patients who were in complete remission after induction. This is possibly related to the differences in medications between the protocols but may also be due to better supportive care. Despite the proven advantage of the newer protocols regarding overall survival, in our experience there was no other significant difference between the two regimens.

http://www.ncbi.nlm.nih.gov/pubmed/24834758

Effect of body mass in children with hematologic malignancies undergoing allogeneic bone marrow transplantation

Aplenc, R., et al.

Blood 2014; 123(22): 3504-3511

The rising incidence of pediatric obesity may significantly affect bone marrow transplantation (BMT) outcomes. We analyzed outcomes in 3687 children worldwide who received cyclophosphamide-based BMT regimens for leukemias between 1990 and 2007. Recipients were classified according to age-adjusted body mass index (BMI) percentiles as underweight (UW), at risk of UW (RUW), normal, overweight (OW), or obese (OB). Median age and race were similar in all groups. Sixty-one percent of OB children were from the United States/Canada. Three-year relapse-free and overall survival ranged from 48% to 52% (P = .54) and 55% to 58% (P = .81) across BMI groups. Three-year leukemia relapses were 33%, 33%, 29%, 25%, and 21% in the UW, RUW, normal, OW, and OB groups, respectively (P < .001). Corresponding cumulative incidences for transplant-related mortality (TRM) were 18%, 19%, 21%, 22%, and 28% (P < .01). Multivariate analysis demonstrated a decreased risk of relapse compared with normal BMI (relative risk [RR] = 0.73; P < .01) and a trend toward higher TRM (RR = 1.28; P = .014). BMI in children is not significantly associated with different survival after BMT for hematologic malignancies. Obese children experience less relapse posttransplant compared with children with normal BMI; however, this benefit is offset by excess in TRM.

http://www.ncbi.nlm.nih.gov/pubmed/24711663

Validation and refinement of the Disease Risk Index for allogeneic stem cell transplantation

Armand, P., et al.

Blood 2014; 123(23): 3664-3671

Because the outcome of allogeneic hematopoietic cell transplantation (HCT) is predominantly influenced by disease type and status, it is essential to be able to stratify patients undergoing HCT by disease risk. The Disease Risk Index (DRI) was developed for this purpose. In this study, we analyzed 13,131 patients reported to the Center for International Blood and Marrow Transplant Research who underwent HCT between 2008 and 2010. The DRI stratified patients into 4 groups with 2-year overall survival (OS) ranging from 64% to 24% and was the strongest prognostic factor, regardless of age, conditioning intensity, graft source, or donor type. A randomly selected training subgroup of 9849 patients was used to refine the DRI, using a multivariable regression model for OS. This refined DRI had improved prediction ability for the remaining 3282 patients compared with the original DRI or other existing schemes. This validated and refined DRI can be used as a 4- or 3-group index, depending on the size of the cohort under study, for prognostication; to facilitate the interpretation of single-center, multicenter, or registry studies; to adjust center outcome data; and to stratify patients entering clinical trials that enroll patients across disease categories.

http://www.ncbi.nlm.nih.gov/pubmed/24744269

Irinotecan and temozolomide for treatment of neuroblastoma in a patient with renal failure on hemodialysis

Armstrong, A. E., et al.

Pediatr Blood Cancer 2014; 61(5): 949-950

Renal failure is a rare complication of neuroblastoma or its therapy. To our knowledge, no reports describe treatment of children with neuroblastoma with chemotherapy in the setting of renal failure and maintenance hemodialysis. We report a 6-year-old child with high-risk neuroblastoma who developed renal failure requiring long-term hemodialysis. She was subsequently treated with 13 cycles of intravenous irinotecan 20 mg/m(2)/day and oral temozolomide 100 mg/m(2)/day for 5 days before disease progression without any dose adjustments, transfusions, febrile neutropenia or diarrhea. This case demonstrates that irinotecan and temozolomide can be safely administered in children with renal failure requiring hemodialysis.

http://www.ncbi.nlm.nih.gov/pubmed/24273036

Iron deficiency anemia as a risk factor for cerebrovascular events in early childhood: A case-control study

Azab, S. F., et al.

Ann Hematol 2014; 93(4): 571-576

In recent years, iron-deficiency anemia (IDA) has been suggested to have an association with childhood-onset ischemic stroke in otherwise healthy children, but few cases have proven it thus far. In this study, we aimed to investigate whether iron-deficiency anemia is a risk factor for cerebrovascular events and childhood-onset ischemic stroke in previously healthy children. This was a case-control study that included 21 stroke cases with patients who had previously been generally healthy, and matched with age and gender of 100 healthy control subjects. Patients were included if a diagnosis of definite stroke had been made and other known etiologies of childhood onset stroke were excluded. For all subjects, iron parameters including serum iron, ferritin, transferrin, total iron binding capacity, and transferrin saturation were assessed. We screened all case patients for prothrombotic factors including level of hemoglobin S, protein C, protein S, antithrombin III, lupus anticoagulant, factor V Leiden, and prothrombin gene mutation (G20210A). Brain magnetic resonance images (MRI), magnetic resonance angiography (MRA), and magnetic resonance venography (MRV) were performed to all case patients. All case patients have normal results regarding functional, immunological, and molecular assay for prothrombotic factors screening. Our results showed that IDA was disclosed in 57.1 % of stroke cases with no identified cause, as compared to 26 % of controls. Our study suggest that previously healthy children who developed stroke are 3.8 times more likely to have IDA than healthy children, who do not develop stroke (OR, 3.8; 95 % CI:1.3-11.2 P = 0.005). In addition, there was significant interaction between IDA and thrombocytosis among studied cases (OR, 10.5; 95 % CI, 1.0-152 P = 0.02). There were nonsignificant differences between stroke patients with IDA and those with normal iron parameters regarding stroke subtype (P > 0.05). Public health messages on the importance of early detection of iron-deficiency anemia in young children, especially in our developing countries so that it can be treated before a life-threatening complication like stroke develops

http://www.ncbi.nlm.nih.gov/pubmed/24141332

Management of infants born with severe neonatal alloimmune thrombocytopenia: The role of platelet transfusions and intravenous immunoglobulin

Bakchoul, T., et al.

Transfusion 2014; 54(3): 640-645

BACKGROUND: Neonatal alloimmune thrombocytopenia (NAIT) is a fetomaternal incompatibility most commonly induced by maternal anti-HPA-1a alloantibodies. Transfusion of immunologically compatible platelets (PLTs) to prevent cerebral hemorrhage, the most severe complication in affected newborns, is usually recommended. Such PLT concentrates, however, are often not readily available. STUDY DESIGN AND METHODS: The efficacy of random-donor PLT transfusions and intravenous immunoglobulin (IVIG) for the management of 17 neonates across four centers with unexpected, severe NAIT was evaluated. Neonates were treated with random-donor PLTs alone (n=7), random-donor PLTs with IVIG (n=8), or matched HPA-1bb PLTs (n=2). RESULTS: All but one patient (treated with random PLTs and IVIG) achieved a posttransfusion PLT count of higher than 30 x 10(9) /L after the first PLT transfusion. The PLT count remained higher than 30 x 10(9) /L for longer than 24 hours in five of seven, seven of eight, and two of four newborns who received random-donor PLTs alone, random-donor PLTs with IVIG, or matched HPA-1bb PLTs, respectively. None of the newborns developed major bleeding or intracranial hemorrhage. IVIG did not appear to improve either posttransfusion PLT counts or total PLT transfusion requirements. CONCLUSION: Transfusion of random-donor PLTs alone was effective at correcting critically low PLT counts and should be considered as first-line treatment of newborns with unexpected severe NAIT.

http://www.ncbi.nlm.nih.gov/pubmed/23869512

Determinants of gross motor function of young children with cerebral palsy: A prospective cohort study

Bartlett, D. J., et al.

Dev Med Child Neurol 2014; 56(3): 275-282

AIM: The aim of this study was to test a model of determinants of gross motor function of young children with cerebral palsy (CP). METHOD: Four hundred and twenty-nine children with CP (242 males, 187 females; mean age 3 y 2 mo, SD 11 mo) representing all levels of the Gross Motor Function Classification System (GMFCS) participated. Children in levels I to II and III to V were classified as Groups 1 and 2 respectively. Distribution of CP was quadriplegia, 44%; hemiplegia, 24%; diplegia, 23%; triplegia, 6%; and monoplegia, 2% (data not available for 1%). Impairment and motor function data were collected by reliable assessors; parents completed questionnaires on health conditions and adaptive behavior. Seven months later, parents were interviewed about family life and services received. One year after the study onset, motor function was re-evaluated. Analysis involved structural equation modeling. RESULTS: The well-fitting model explained 58% and 75% of the variance in motor function at study completion for Groups 1 and 2 respectively. Primary impairments (spasticity, quality of movement, postural stability, and distribution of involvement; beta=0.52-0.68) and secondary impairments (strength, range of motion limitations, and reduced endurance; beta=0.25-0.26) explained the most variance. Adaptive behavior was a significant determinant only for Group 2 (beta=0.21) and participation in community programs was significant only in Group 1 (beta=0.13). INTERPRETATION: Motor function is supported by optimizing body structures and function for all children and enhancing adaptive behavior for children with greater motor challenges.

http://www.ncbi.nlm.nih.gov/pubmed/24127787

Ketogenic diet in children with intractable epilepsy: what about resting energy expenditure and growth?

Bertoli, S., et al.
Dev Med Child Neurol 2014; 56(9): 806-807

http://www.ncbi.nlm.nih.gov/pubmed/24828524

Use of corticosteroids after hepatoportoenterostomy for bile drainage in infants with biliary atresia: The START randomized clinical trial

Bezerra, J. A., et al.
JAMA 2014; 311(17): 1750-1759

IMPORTANCE: Biliary atresia is the most common cause of end-stage liver disease in children. Controversy exists as to whether use of steroids after hepatoportoenterostomy improves clinical outcome. OBJECTIVE: To determine whether the addition of high-dose corticosteroids after hepatoportoenterostomy is superior to surgery alone in improving biliary drainage and survival with the native liver. DESIGN, SETTING, AND PATIENTS: The multicenter, double-blind Steroids in Biliary Atresia Randomized Trial (START) was conducted in 140 infants (mean age, 2.3 months) between September 2005 and February 2011 in the United States; follow-up ended in January 2013. INTERVENTIONS: Participants were randomized to receive intravenous methylprednisolone (4 mg/kg/d for 2 weeks) and oral prednisolone (2 mg/kg/d for 2 weeks) followed by a tapering protocol for 9 weeks (n = 70) or placebo (n = 70) initiated within 72 hours of hepatoportoenterostomy. MAIN OUTCOMES AND MEASURES: The primary end point (powered to detect a 25% absolute treatment difference) was the percentage of participants with a serum total bilirubin level of less than 1.5 mg/dL with his/her native liver at 6 months posthepatoportoenterostomy. Secondary outcomes included survival with native liver at 24 months of age and serious adverse events. RESULTS: The proportion of participants with improved bile drainage was not statistically significantly improved by steroids at 6 months posthepatoportoenterostomy (58.6% [41/70] of steroids group vs 48.6% [34/70] of placebo group; adjusted relative risk, 1.14 [95% CI, 0.83 to 1.57]; P = .43). The adjusted absolute risk difference was 8.7% (95% CI, -10.4% to 27.7%). Transplant-free survival was 58.7% in the steroids group vs 59.4% in the placebo group (adjusted hazard ratio, 1.0 [95% CI, 0.6 to 1.8]; P = .99) at 24 months of age. The percentage of participants with serious adverse events was 81.4% [57/70] of the steroids group and 80.0% [56/70] of the placebo group (P > .99); however, participants receiving steroids had an earlier time of onset of their first serious adverse event by 30 days posthepatoportoenterostomy (37.2% [95% CI, 26.9% to 50.0%] of steroids group vs 19.0% [95% CI, 11.5% to 30.4%] of placebo group; P = .008). CONCLUSIONS AND RELEVANCE: Among infants with biliary atresia who have undergone hepatoportoenterostomy, high-dose steroid therapy following surgery did not result in statistically significant treatment differences in bile drainage at 6 months, although a small clinical benefit could not be excluded. Steroid treatment was associated with earlier onset of serious adverse events in children with biliary atresia. TRIAL REGISTRATION: clinicaltrials.gov Identifier: NCT00294684.

http://www.ncbi.nlm.nih.gov/pubmed/24794368

Transplantation for children with acute myeloid leukemia: A comparison of outcomes with reduced intensity and myeloablative regimens

Bitan, M., et al.
Blood 2014; 123(10): 1615-1620

The safety and efficacy of reduced-intensity conditioning (RIC) regimens for the treatment of pediatric acute myeloid leukemia is unknown. We compared the outcome of allogeneic hematopoietic cell transplantation in children with acute myeloid leukemia using RIC regimens with those receiving myeloablative-conditioning (MAC) regimens. A total of 180 patients were evaluated (39 with RIC and 141 with MAC regimens). Results of univariate and multivariate analysis showed no significant differences in the rates of acute and chronic graft-versus-host disease, leukemia-free, and overall survival between treatment groups. The 5-year probabilities of overall survival with RIC and MAC regimens were 45% and 48%, respectively (P = .99). Moreover, relapse rates were not higher with RIC compared with MAC regimens (39% vs 39%; P = .95), and recipients of MAC regimens were not at higher risk for transplant-related mortality compared with recipients of RIC regimens (16% vs 16%; P = .73). After carefully controlled analyses, we found that in this relatively modest study population, the data supported a role for RIC regimens for acute myeloid leukemia in children undergoing allogeneic hematopoietic cell transplantation. The data also provided justification for designing a carefully controlled randomized clinical trial that examines the efficacy of regimen intensity in this population.

http://www.ncbi.nlm.nih.gov/pubmed/24435046

Safety and efficacy of aprepitant for chemotherapy-induced nausea and vomiting in pediatric patients: A prospective, observational study

Bodge, M., et al.
Pediatr Blood Cancer 2014; 61(6): 1111-1113

Pediatric patients between the ages of 12 months and 17 years with a confirmed malignancy who were scheduled to receive aprepitant as part of triple therapy antiemetic prophylaxis for a cycle of moderately- or highly emetogenic chemotherapy were eligible for enrollment. Patients were evaluated for the incidence of nausea, episodes of emesis, interference with activities of daily living (ADLs), and appetite through utilization of a patient survey. Eleven patients were enrolled for a total of 20 patient encounters, mean age 9.55 +/- 4.85 (range, 12 months-17 years). Aprepitant was well-tolerated and complete response (CR) rate was 38.9%.

http://www.ncbi.nlm.nih.gov/pubmed/24357337

Question 2: Is there any long-term benefit from injecting botulinum toxin-A into children with cerebral palsy?

Bradley, L. J. and J. S. Huntley
Arch Dis Child 2014; 99(4): 392-394

http://www.ncbi.nlm.nih.gov/pubmed/24626322

Outpatient management of vascular access devices in children receiving radiotherapy: complications and morbidity

Bratton, J., et al.

Pediatr Blood Cancer 2014; 61(3): 499-501

BACKGROUND: When treating children with cancer, long-term venous access is critical. This is especially true in the context of children receiving daily radiation therapy (RT) under general anesthesia. We have previously reported <0.1% risk of complications in complications in over 4,040 pediatric treatments under general anesthesia in our outpatient facility. Here, we present our experience with venous catheter access techniques in children receiving daily proton RT. PROCEDURE: After Institutional Review Board approval, we reviewed our center's records between September 9, 2004 and October 23, 2012 with respect to complications and morbidity of indwelling catheters in our pediatric patients. RESULTS: Vascular access device (VAD) types included: 110 patients with indwelling port-a-cath (PAC), 34 PICC line devices, and 34 central venous catheter (CVC) devices in 170 patients. Median catheter life during RT was 43 days (range 1-86 days) with a total of 7,169 total catheter days while patients received RT. A 14% PAC complication rate included negative blood return (6.3%) and infection (3.6%). Complication rates for PICC and CVC access devices were 38% and 20.5%, respectively (chi(2) P = 0.007 when compared with PAC). Most frequent complications for PICC lines were no blood return (11.7%), and infection or occlusion (8.8% each). CVC complications were breakage (8.8%) and infection (8.8%). Access device replacement rates were 3.6% (PAC), 14.7% (PICC), and 8.8% (CVC). CONCLUSIONS: In the outpatient delivery of RT to children, indwelling ports provide greater convenience, less likelihood of infection or complication, and greater durability than PICC or CVC devices.

http://www.ncbi.nlm.nih.gov/pubmed/23956113

Rasch analysis of clinical outcome measures in spinal muscular atrophy

Cano, S. J., et al.

Muscle Nerve 2014; 49(3): 422-430

INTRODUCTION: Trial design for SMA depends on meaningful rating scales to assess outcomes. In this study Rasch methodology was applied to 9 motor scales in spinal muscular atrophy (SMA). METHODS: Data from all 3 SMA types were provided by research groups for 9 commonly used scales. Rasch methodology assessed the ordering of response option thresholds, tests of fit, spread of item locations, residual correlations, and person separation index. RESULTS: Each scale had good reliability. However, several issues impacting scale validity were identified, including the extent that items defined clinically meaningful constructs and how well each scale measured performance across the SMA spectrum. CONCLUSIONS: The sensitivity and potential utility of each SMA scale as outcome measures for trials could be improved by establishing clear definitions of what is measured, reconsidering items that misfit and items whose response categories have reversed thresholds, and adding new items at the extremes of scale ranges.

http://www.ncbi.nlm.nih.gov/pubmed/23836324

Development of a quality of life instrument for children with advanced cancer:The pediatric advanced care quality of life scale (PAC-QoL)

Cataudella, D., et al.

Pediatr Blood Cancer 2014; 61(10): 1840-1845

BACKGROUND: There is currently no published, validated measures available that comprehensively capture quality of life (QoL) symptoms for children with poor-prognosis malignancies. The pediatric advanced care-quality of life scale (PAC-QoL) has been developed to address this gap. The current paper describes the first two phases in the development of this measure. PROCEDURES: The first two phases included: (1) construct and item generation, and (2) preliminary content validation. Domains of QoL relevant to this population were identified from the literature and items generated to capture each; items were then adapted to create versions sensitive to age/developmental differences. Two types of experts reviewed the draft PAC-QoL and rated items for relevance, understandability, and sensitivity of wording: bereaved parents (n = 8) and health care professionals (HCP; n = 7). Content validity was calculated using the index of content validity (CVI [Lynn. Nurs Res 1986;35:382-385]). RESULTS: One hundred and forty-one candidate items congruent with the domains identified as relevant to children with advanced malignancies were generated, and four report versions with a 5-choice response scale created. Parent mean scores for importance, understandability, and sensitivity of wording ranged from 4.29 (SD = 0.52) to 4.66 (SD = 0.50). The CVI ranged from 95% to 100%. These steps resulted in reductions of the PAC-QoL to 57-65 items, as well as a modification of the response scale to a 4-choice option with new anchors. CONCLUSIONS: The next phase of this study will be to conduct cognitive probing with the intended population to further modify and reduce candidate items prior to psychometric evaluation.

http://www.ncbi.nlm.nih.gov/pubmed/24947134

The modern Fontan operation shows no increase in mortality out to 20 years: A new paradigm

Dabal, R. J., et al.

J Thorac Cardiovasc Surg 2014

OBJECTIVE: Dating back to the first published report of the Fontan circulation in 1971, multiple studies have examined the long-term results of this standard procedure for palliation of single-ventricle heart disease in children. Although the technique has evolved over the last 4 decades to include a polytetrafluorethylene (PTFE) conduit for a large percentage of patients, the long-term outcome has not yet been established. The aim of the current study was to investigate the possibility of a late increasing risk for death after 15 years among patients with a modern Fontan operation and to evaluate late morbidity. METHODS: Between January 1, 1988, and December 31, 2011, 207 patients underwent the Fontan procedure using an internal or external PTFE conduit plus a bidirectional cavopulmonary connection. Survival and late adverse events were analyzed. Risk factors for early and late mortality were examined using hazard function methodology. RESULTS: At 1, 10, and 20 years, survival for the entire cohort was 95%, 88%, and 76%, respectively, with no deaths in the last 6 years of the study. Hazard modeling showed a 1.3% risk of death per year 24 years after the Fontan procedure, with no late increasing hazard phase. Freedom from reoperations was greater than 90% at 20 years and freedom from thrombotic complications was 98% at 20 years (with greater than 80% of patients on aspirin alone). Survival curves were superimposable for 16- to 20-mm conduits, and the freedom from any reoperation including transplantation was greater than 90% after 20 years. Multivariable risk factor analysis identified only earlier date of operation as a predictor of early and late mortality. By era of surgery, the 10-year predicated survival is 89% for patients undergoing surgery in 2000 and 94% for patients in 2010. CONCLUSIONS: Early and late survival after a Fontan operation with a PTFE conduit is excellent, with no late phase of increasing death risk after 20 years. Late functional status is good, the need for late reoperation is rare, and thrombotic complications are uncommon on a standard medical regimen including aspirin as the only anticoagulation medication.

http://www.ncbi.nlm.nih.gov/pubmed/25277471

Successful use of indwelling tunneled catheters for the management of effusions in children with advanced cancer

den Hollander, B. S., et al.

Pediatr Blood Cancer 2014; 61(6): 1007-1012

BACKGROUND: Malignant pleural effusion (MPE) and ascites (MA) negatively impact quality of life of palliative patients. Treatment options are limited. This study's purpose is to examine the experience with indwelling tunneled catheters (ITCs) for management of MPE/MA in children with advanced cancer. METHODS: Children with MPE/MA who underwent ITC insertion (2007-2012) were retrospectively reviewed. Clinical, procedural, complication and outcome details were analyzed. RESULTS: PleurX(R) ITCs (n = 12) were inserted in eight patients (5-18 years) with sarcoma (11 MPE, 1 MA), achieving symptom relief and facilitating discharge home post ITC (median 2 days). Median survival following ITC was 51 days. There were two major complications: pain (n = 1), late site infection (n = 1), and five minor complications. Drainage ceased in four patients (pleurodesis/tumor progression). At time of death, six ITCs (five patients) were still in situ. CONCLUSIONS: ITC appears to be a safe, effective treatment for MPE/MA in advanced pediatric cancer, achieving symptomatic relief and discharge home.

http://www.ncbi.nlm.nih.gov/pubmed/24376007

Utility and safety of rituximab in pediatric autoimmune and inflammatory CNS disease

Dale, R. C., et al.

Neurology 2014; 83(2): 142-150

OBJECTIVE: To assess the utility and safety of rituximab in pediatric autoimmune and inflammatory disorders of the CNS. METHODS: Multicenter retrospective study. RESULTS: A total of 144 children and adolescents (median age 8 years, range 0.7-17; 103 female) with NMDA receptor (NMDAR) encephalitis (n = 39), opsoclonus myoclonus ataxia syndrome (n = 32), neuromyelitis optica spectrum disorders (n = 20), neuropsychiatric systemic lupus erythematosus (n = 18), and other neuroinflammatory disorders (n = 35) were studied. Rituximab was given after a median duration of disease of 0.5 years (range 0.05-9.5 years). Infusion adverse events were recorded in 18/144 (12.5%), including grade 4 (anaphylaxis) in 3. Eleven patients (7.6%) had an infectious adverse event (AE), including 2 with grade 5 (death) and 2 with grade 4 (disabling) infectious AE (median follow-up of 1.65 years [range 0.1-8.5]). No patients developed progressive multifocal leukoencephalopathy. A definite, probable, or possible benefit was reported in 125 of 144 (87%) patients. A total of 17.4% of patients had a modified Rankin Scale (mRS) score of 0-2 at rituximab initiation, compared to 73.9% at outcome. The change in mRS 0-2 was greater in patients given rituximab early in their disease course compared to those treated later. CONCLUSION: While limited by the retrospective nature of this analysis, our data support an off-label use of rituximab, although the significant risk of infectious complications suggests rituximab should be restricted to disorders with significant morbidity and mortality. CLASSIFICATION OF EVIDENCE: This study provides Class IV evidence that in pediatric autoimmune and inflammatory CNS disorders, rituximab improves neurologic outcomes with a 7.6% risk of adverse infections.

http://www.ncbi.nlm.nih.gov/pubmed/24920861

Implantable cardioverter-defibrillators in children

DeWitt, E. S. and D. J. Abrams

Arch Dis Child 2014

Implantable cardioverter-defibrillators (ICD) have become an integral component in the management of children with life-threatening cardiac arrhythmias complicating a variety of different inherited and congenital cardiovascular conditions. Implantation often requires novel approaches and configurations to overcome the size and anatomic limitations posed by many children needing ICDs. While their use has undoubtedly saved many lives, ICD use may be associated with significant morbidity, so detailed case selection and individualised postimplant programming is critical.

http://www.ncbi.nlm.nih.gov/pubmed/25249497

Redefining expectations of long-term survival after the Fontan procedure: Twenty-five years of follow-up from the entire population of Australia and New Zealand

d'Udekem, Y., et al.

Circulation 2014; 130(11 Suppl 1): S32-38

BACKGROUND: The life expectancy of patients undergoing a Fontan procedure is unknown. METHODS AND RESULTS: Follow-up of all 1006 survivors of the 1089 patients who underwent a Fontan procedure in Australia and New Zealand was obtained from a binational population-based registry including all pediatric and adult cardiac centers. There were 203 atriopulmonary connections (AP; 1975-1995), 271 lateral tunnels (1988-2006), and 532 extracardiac conduits (1997-2010). The proportion with hypoplastic left heart syndrome increased from 1/173 (1%) before 1990 to 80/500 (16%) after 2000. Survival at 10 years was 89% (84%-93%) for AP and 97% (95% confidence interval [CI], 94%-99%) for lateral tunnels and extracardiac conduits. The longest survival estimate was 76% (95% CI, 67%-82%) at 25 years for AP. AP independently predicted worse survival compared with extracardiac conduits (hazard ratio, 6.2; P<0.001; 95% CI, 2.4-16.0). Freedom from failure (death, transplantation, takedown, conversion to extracardiac conduits, New York Heart Association III/IV, or protein-losing enteropathy/plastic bronchitis) 20 years after Fontan was 70% (95% CI, 63%-76%). Hypoplastic left heart syndrome was the primary predictor of Fontan failure (hazard ratio, 3.8; P<0.001; 95% CI, 2.0-7.1). Ten-year freedom from failure was 79% (95% CI, 61%-89%) for hypoplastic left heart syndrome versus 92% (95% CI, 87%-95%) for other morphologies. CONCLUSIONS: The long-term survival of the Australia and New Zealand Fontan population is excellent. Patients with an AP Fontan experience survival of 76% at 25 years. Technical modifications have further improved survival. Patients with hypoplastic left heart syndrome are at higher risk of failure. Large, comprehensive registries such as this will further improve our understanding of late outcomes after the Fontan procedure.

http://www.ncbi.nlm.nih.gov/pubmed/25200053

Performance of the timed "up & go" test in spinal muscular atrophy

Dunaway, S., et al.

Muscle Nerve 2014; 50(2): 273-277

INTRODUCTION: The timed "up & go" (TUG) test is a quick measure of balance and mobility. TUG scores correlate with clinical, functional, and strength assessment and decline linearly over time. Reliability and validity have not been tested in spinal muscular atrophy (SMA). METHODS: Fifteen ambulatory SMA participants performed TUG testing and strength, functional, and clinical assessments. Intraclass correlation coefficients quantified test-retest reliability. Convergent validity was determined using Pearson correlation coefficients. RESULTS: Test-retest reliability was excellent for all participants. TUG was associated significantly with total leg and knee flexor strength, as well as the Hammersmith Functional Motor Scale Expanded, the 10-meter walk/run, and 6-minute walk tests. TUG findings were not associated with knee extensor strength, pulmonary function, or fatigue. CONCLUSIONS: In SMA, the TUG test is easily administered, reliable, and correlates with established outcome measures. TUG testing is a potentially useful outcome measure for clinical trials and a measure of disability in ambulatory patients with SMA.

http://www.ncbi.nlm.nih.gov/pubmed/24375426

Supraglottoplasty outcomes in neurologically affected and syndromic children

Durvasula, V. S., et al.

JAMA Otolaryngol Head Neck Surg 2014; 140(8): 704-711

IMPORTANCE: Supraglottoplasty (SGP) failure is frequently attributed to coexistent medical comorbidities, but studies specifically evaluating outcomes in these populations are lacking. OBJECTIVE: To assess SGP outcomes in patients with neurologic and syndromic comorbidities and severe laryngomalacia (LM). DESIGN, SETTING, AND PARTICIPANTS: Case series with retrospective review of medical records of 54 patients with neurologic and/or syndromic comorbidity and severe LM who underwent SGP between 2004 and 2012 at a tertiary care pediatric institution. INTERVENTIONS: Patients presented with severe LM that required SGP. Supraglottoplasty failure necessitated revision SGP, tracheostomy, or gastrostomy tube insertion, or LM and obstructive sleep apnea that required assisted ventilation (continuous positive airway pressure and bilevel positive airway pressure). MAIN OUTCOMES AND MEASURES: Medical records were reviewed with a focus on patient factors, surgical timing, complications, and surgical and dysphagia outcomes. Patients were grouped based on their age at the time of SGP as infants (aged </=12 months) and children (aged >12 months). Statistical comparisons were performed with SGP outcomes of infants with LM and no comorbidities. RESULTS: Fifty-four patients met the inclusion criteria. Thirty-one (13 infants, 18 children) had a neurologic condition and 23 (15 infants, 8 children) had syndromes. The overall success rate of SGP was 67% (36 of 54) in these populations. Neurologic (P = .003) and syndromic (P < .001) comorbidities were associated with significant reduction in SGP success rates vs no comorbidities. Among SGP failures (18 of 54 [33%]), 13% (7 of 54) required tracheostomy, 9% (5 of 54) needed assisted ventilation, 7% (4 of 54) required a postoperative gastrostomy tube, and 4% (2 of 54) required revision SGP. In the neurologic comorbidities group, patients with cerebral palsy had significantly higher tracheostomy rates compared with those who had other neurologic pathologies constituting comorbidities (2 of 11 [18%] vs 0 of 20; P = .049). In infants, acute airway obstruction was the most common indication for SGP in the neurologically comorbidity and syndrome populations (success rates, 69% and 67%, respectively). In children, obstructive sleep apnea was the most common indication for SGP in the neurologic comorbidity and syndrome populations (success rates, 78% and 50%, respectively). CONCLUSIONS AND RELEVANCE: Supraglottoplasty remains useful and outcomes were better in patients with neurologic comorbidity than in patients with syndromic comorbidity.

http://www.ncbi.nlm.nih.gov/pubmed/25073682

Observational study of spinal muscular atrophy type I and implications for clinical trials

Finkel, R. S., et al.

Neurology 2014; 83(9): 810-817

OBJECTIVES: Prospective cohort study to characterize the clinical features and course of spinal muscular atrophy type I (SMA-I). METHODS: Patients were enrolled at 3 study sites and followed for up to 36 months with serial clinical, motor function, laboratory, and electrophysiologic outcome assessments. Intervention was determined by published standard of care guidelines. Palliative care options were offered. RESULTS: Thirty-four of 54 eligible subjects with SMA-I (63%) enrolled and 50% of these completed at least 12 months of follow-up. The median age at reaching the combined endpoint of death or requiring at least 16 hours/day of ventilation support was 13.5 months (interquartile range 8.1-22.0 months). Requirement for nutritional support preceded that for ventilation support. The distribution of age at reaching the combined endpoint was similar for subjects with SMA-I who had symptom onset before 3 months and after 3 months of age (p=0.58). Having 2 SMN2 copies was associated with greater morbidity and mortality than having 3 copies. Baseline electrophysiologic measures indicated substantial motor neuron loss. By comparison, subjects with SMA-II who lost sitting ability (n=10) had higher motor function, motor unit number estimate and compound motor action potential, longer survival, and later age when feeding or ventilation support was required. The mean rate of decline in The Children's Hospital of Philadelphia Infant Test for Neuromuscular Disorders motor function scale was 1.27 points/year (95% confidence interval 0.21-2.33, p=0.02). CONCLUSIONS: Infants with SMA-I can be effectively enrolled and retained in a 12-month natural history study until a majority reach the combined endpoint. These outcome data can be used for clinical trial design.

http://www.ncbi.nlm.nih.gov/pubmed/25080519

Age at referral and mortality from critical congenital heart disease

Fixler, D. E., et al.

Pediatrics 2014; 134(1): e98-105

BACKGROUND AND OBJECTIVE: Newborn pulse oximetry screening is recommended to promote early referral of neonates with critical congenital heart disease (CCHD) and reduce mortality; however, the impact of late referral on mortality is not well defined. The purpose of this population-based study was to describe the association between timing of referral to a cardiac center and mortality in 2360 liveborn neonates with CCHD. METHODS: Neonates with CCHD born before pulse oximetry screening (1996-2007) were selected from the Texas Birth Defects Registry and linked to state birth and death records. Age at referral was ascertained from date of first cardiac procedure at a cardiac center. Logistic and Cox proportional hazards regression models were used to estimate factors associated with late referral and mortality; the Kaplan-Meier method was used to estimate 3-month survival. RESULTS: Median age at referral was 1 day (25th-75th percentile: 0-6 days). Overall, 27.5% (649 of 2360) were referred after age 4 days and 7.5% (178 of 2360) had no record of referral. Neonatal mortality was 18.1% (277 of 1533) for those referred at 0 to 4 days of age, 9.0% (34 of 379) for those referred at 5 to 27 days of age, and 38.8% (69 of 178) for those with no referral. No improvement in age at referral was found across the 2 eras within 1996-2007. CONCLUSIONS: A significant proportion of neonates with CCHD experienced late or no referral to cardiac specialty centers, accounting for a significant number of the deaths. Future population-based studies are needed to determine the benefit of pulse oximetry screening on mortality and morbidity.

http://www.ncbi.nlm.nih.gov/pubmed/25080519

Safety of intranasal fentanyl in the out-of-hospital setting: a prospective observational study

Karlsen, A. P., et al.

Ann Emerg Med 2014; 63(6): 699-703

STUDY OBJECTIVE: Initial out-of-hospital analgesia is sometimes hampered by difficulties in achieving intravenous access or lack of skills in administering intravenous opioids. We study the safety profile and apparent analgesic effect of intranasal fentanyl in the out-of-hospital setting. METHODS: In this prospective observational study, we administered intranasal fentanyl in the out-of-hospital setting to adults and children older than 8 years with severe pain resulting from orthopedic conditions, abdominal pain, or acute coronary syndrome refractory to nitroglycerin spray. Patients received 1 to 3 doses of either 50 or 100 mug, and the ambulance crew recorded adverse effects and numeric rating scale (0 to 10) pain scores before and after treatment. RESULTS: Our 903 evaluable patients received a mean cumulative fentanyl dose of 114 mug (range 50 to 300 mug). There were no serious adverse effects and no use of naloxone. Thirty-six patients (4%) experienced mild adverse effects: mild hypotension, nausea, vomiting, vertigo, abdominal pain, rash, or decrease of Glasgow Coma Scale score to 14. The median reduction in pain score was 3 (interquartile range 2 to 5) after fentanyl administration. CONCLUSION: The out-of-hospital administration of intranasal fentanyl in doses of 50 to 100 mug is safe and appears effective.

http://www.ncbi.nlm.nih.gov/pubmed/24268523

Readmission to paediatric intensive care unit: frequency, causes and outcome

Khan, M. R., et al.

J Coll Physicians Surg Pak 2014; 24(3): 216-217

Readmission to intensive care units is considered to be an important quality indicator in ICU settings. This study was carried out at the paediatric intensive unit (PICU) and step down units of paediatric ward at the Aga Khan University Hospital, Karachi, Pakistan, to assess the frequency, common causes and outcome of patients readmitted in PICU within 48 hours after discharge from unit. During the study period, 1022 patients were admitted in PICU, out of which 24 (2.34%) patients required readmission. Male to female ratio was 1.2:1. The mean length of stay on paediatric floor before readmission was 24 hours. Fifteen (62%) patients were readmitted due to worsening of primary condition while 9 (38%) developed new problems. Respiratory problems accounted for 15 (62.5%) of readmissions, followed by cardiovascular 4 (16.5%) and sepsis related causes 3 (12.5%). The mortality rate of readmitted patients was 21% (5/24) in this study as compared to overall PICU mortality of 122 (11.93%).

http://www.ncbi.nlm.nih.gov/pubmed/24613123

The burden of Duchenne muscular dystrophy: an international, cross-sectional study

Landfeldt, E., et al.

Neurology 2014; 83(6): 529-536

OBJECTIVE: The objective of this study was to estimate the total cost of illness and economic burden of Duchenne muscular dystrophy (DMD). METHODS: Patients with DMD from Germany, Italy, United Kingdom, and United States were identified through Translational Research in Europe-Assessment & Treatment of Neuromuscular Diseases registries and invited to complete a questionnaire online together with a caregiver. Data on health care use, quality of life, work status, informal care, and household expenses were collected to estimate costs of DMD from the perspective of society and caregiver households. RESULTS: A total of 770 patients (173 German, 122 Italian, 191 from the United Kingdom, and 284 from the United States) completed the questionnaire. Mean per-patient annual direct cost of illness was estimated at between $23,920 and $54,270 (2012 international dollars), 7 to 16 times higher than the mean per-capita health expenditure in these countries. Indirect and informal care costs were substantial, each constituting between 18% and 43% of total costs. The total societal burden was estimated at between $80,120 and $120,910 per patient and annum, and increased markedly with disease progression. The corresponding household burden was estimated at between $58,440 and $71,900. CONCLUSIONS: We show that DMD is associated with a substantial economic burden. Our results underscore the many different costs accompanying a rare condition such as DMD and the considerable economic burden carried by affected families. Our description of the previously unknown economic context of a rare disease serves as important intelligence input to health policy evaluations of intervention programs and novel therapies, financial support schemes for patients and their families, and the design of future cost studies.

http://www.ncbi.nlm.nih.gov/pubmed/24991029

Management of Budd-Chiari: A single-center experience of 280 cases

Li, K., et al.

Hepatogastroenterology 2014; 61(130): 460-462

OBJECTIVE: Budd-Chiari syndrome (BCS) is a rare and life-threatening disorder secondary to hepatic venous outflow obstruction. How to manage this complex disease has haunted many surgeons. The aim of this study is to investigate the treatment of Budd-Chiari syndrome in our hospital. METHODS: The clinical data of 280 BCS patients were analyzed retrospectively in our hospital between July 2000 and March 2013. RESULTS: The total effective rate was 90% (252/280). The rate of mortality was 7.14% (20/280), the rate of complication was 17.14% (48/280). We carried out followup in 198 cases from 6 months to 10 years, the rate of recurrence was 6.07% (12/198). CONCLUSIONS: Treatment of BCS need to get a corrective diagnosis and classification at first, then select corrective methods of treatment based on different pathological change of IVC and main hepatic vein.

http://www.ncbi.nlm.nih.gov/pubmed/24901162

Analysis of preoperative condition and interstage mortality in Norwood and hybrid procedures for hypoplastic left heart syndrome using the Aristotle scoring system

Lloyd, D. F., et al.
Heart 2014; 100(10): 775-780

OBJECTIVE: The 'hybrid procedure', consisting of surgical banding of the pulmonary arteries with intraoperative stenting of the arterial duct, was developed as primary palliation in hypoplastic left heart syndrome (HLHS), avoiding the risks of cardiopulmonary bypass. In many centres, it is reserved for low birth weight, premature or unstable neonates; however, its role in such high risk cases of HLHS has yet to be defined. METHODS: The preoperative condition of all patients with HLHS who underwent either the hybrid or the Norwood procedure for HLHS between 2005-2011 was analysed retrospectively, using a modified comprehensive Aristotle score. We then compared operative, interstage and 1 year mortalities between the groups after Aristotle adjustment via Cox proportional hazards analyses. RESULTS: Of 138 patients with HLHS, 27 had hybrid and 111 Norwood procedures. The hybrid group had significantly higher Aristotle scores (mean 4.1 vs 1.8; p<0.001); however, there was no significant difference in mortality at any stage. At 1 year, the overall unadjusted survival among Norwood and hybrid patients was 58.6% and 51.9%, respectively, yielding an Aristotle adjusted hazard ratio for mortality among hybrid patients of 1.09 (95% CI 0.56 to 2.11, p=0.80). CONCLUSIONS: Applying a hybrid approach to high risk patients with HLHS produces a comparable early and interstage mortality risk to lower risk patients undergoing the Norwood procedure. Prospective studies are needed to establish whether the hybrid procedure is a viable alternative to the Norwood procedure in all HLHS patients in terms of both mortality and long term morbidity.

http://www.ncbi.nlm.nih.gov/pubmed/24415666

The mixed blessing: Neonatal tracheostomy

Mammel, M. C.
J Pediatr 2014; 164(6): 1255-1256

http://www.ncbi.nlm.nih.gov/pubmed/24607246

Bloodstream infections in patients with solid tumors: Epidemiology, antibiotic therapy, and outcomes in 528 episodes in a single cancer center

Marin, M., et al.

Medicine (Baltimore) 2014; 93(3): 143-149

Current information regarding bloodstream infection (BSI) in patients with solid tumors is scarce. We assessed the epidemiology, antibiotic therapy, and outcomes of BSI in these patients. We also compared patients who died with those who survived to identify risk factors associated with mortality. From January 2006 to July 2012 all episodes of BSI in patients with solid tumors at a cancer center were prospectively recorded and analyzed. A total of 528 episodes of BSI were documented in 489 patients. The most frequent neoplasms were hepatobiliary tumors (19%), followed by lung cancer (18%) and lower gastrointestinal malignancies (16%). Many patients had received corticosteroid therapy (41%), and 15% had neutropenia (<500 neutrophils/muL) at the time of BSI. The most common source of BSI was cholangitis (21%), followed by other abdominal (19.5%) and urinary tract infections (17%). Gram-negative BSI occurred in 55% of cases, mainly due to Escherichia coli (55%), Pseudomonas aeruginosa (18%), and Klebsiella pneumoniae (16%). Among gram-positive BSI (35%), viridans group streptococci were the most frequent causative organisms (22%), followed by Staphylococcus aureus (21%) and Enterococcus species (18%). We identified 61 multidrug-resistant (MDR) organisms (13%), mainly extended-spectrum beta-lactamase-producing Enterobacteriaceae (n = 20) and AmpC-producing Enterobacteriaceae (n = 13). The majority of patients with BSI caused by MDR organisms had received antibiotics (70%), and they had been previously hospitalized (61.4%) more frequently than patients with BSI caused by susceptible strains. Inadequate empirical antibiotic therapy was given to 23% of patients, with a higher proportion in those with BSI due to a MDR strain (69%). Early (<48 h) and overall (30 d) case-fatality rates were 7% and 32%, respectively. The overall case-fatality rate was higher among cases caused by MDR organisms (39.3%). The only independent risk factors for the early case-fatality rate were the endogenous source of BSI (odds ratio [OR], 3.57; 95% confidence interval [CI], 1.06-12.02), shock at presentation (OR, 3.63; 95% CI, 1.63-8.09), and corticosteroid therapy (OR, 3.245; 95% CI, 1.43-7.32). The independent risk factors for overall case-fatality rate were the presence of a chronic advanced cancer (OR, 35.39; 95% CI, 2.48-504.91), shock at presentation (OR, 25.84; 95% CI, 3.73-179.0), and corticosteroid therapy (OR, 6.98; 95% CI, 1.61-30.21).BSI in patients with solid tumors occurred mainly among those with hepatobiliary cancer, and cholangitis was the most frequent source; gram-negative bacilli were the most frequent causative agents. MDR organisms were relatively common, particularly in patients who had previously received antibiotics and had been hospitalized; these patients were frequently treated with inadequate empirical antibiotic therapy and had a poorer outcome. The case-fatality rate of patients with solid tumors and BSI was high and was associated with chronic advanced cancer, corticosteroid therapy, and shock at presentation.

http://www.ncbi.nlm.nih.gov/pubmed/24797169

Development of the Pediatric Advanced Care Quality of Life Scale (PAC-QoL): Evaluating comprehension of items and response options

Morley, T. E., et al.

Pediatr Blood Cancer 2014; 61(10): 1835-1839

BACKGROUND: Validated tools that measure quality of life (QOL) for children with poor prognosis malignancies are not available. We are developing a novel instrument, The Pediatric Advanced Care-Quality of Life Scale (PAC-QoL), in order to address this gap. Instrument development requires a phase of item reduction and assessment of item comprehension in the target population. This manuscript provides a report on this phase in the development of the PAC-QoL. PROCEDURE: Children with poor prognosis cancer and/or their parents were invited to participate in cognitive probing interviews. Participants' understanding of each item was rated from 0 (did not understand) to 4 (completely understood). To evaluate the response scale, an overall percentage of respondents' ability to accurately distinguish between the four response options was calculated. RESULTS: Four age- and reporter-specific versions of the PAC-QoL were tested with 74 participants. Mean (+/-SD) comprehension scores across versions ranged from 3.40 +/- .0.30 (child self-report) to 3.69 +/- 0.23 (parent of toddler report). The number of items deleted or modified to improve understandability ranged from 46% of all items on the parent-of-child report to 56% for the child and adolescent self-reports. Respondent's abilities to accurately distinguish between response-scale options ranged from 84% (child-report) to 98% (parent-toddler report). CONCLUSIONS: We demonstrate a high degree item understandability and response-scale separation in the current version of the PAC-QoL. The scale is ready for psychometric evaluation in its target population.

http://www.ncbi.nlm.nih.gov/pubmed/24947134

Pediatric medical complexity algorithm: A new method to stratify children by medical complexity

Simon, T. D., et al.

Pediatrics 2014; 133(6): e1647-1654

OBJECTIVES: The goal of this study was to develop an algorithm based on International Classification of Diseases, Ninth Revision, Clinical Modification (ICD-9-CM), codes for classifying children with chronic disease (CD) according to level of medical complexity and to assess the algorithm's sensitivity and specificity. METHODS: A retrospective observational study was conducted among 700 children insured by Washington State Medicaid with >/=1 Seattle Children's Hospital emergency department and/or inpatient encounter in 2010. The gold standard population included 350 children with complex chronic disease (C-CD), 100 with noncomplex chronic disease (NC-CD), and 250 without CD. An existing ICD-9-CM-based algorithm called the Chronic Disability Payment System was modified to develop a new algorithm called the Pediatric Medical Complexity Algorithm (PMCA). The sensitivity and specificity of PMCA were assessed. RESULTS: Using hospital discharge data, PMCA's sensitivity for correctly classifying children was 84% for C-CD, 41% for NC-CD, and 96% for those without CD. Using Medicaid claims data, PMCA's sensitivity was 89% for C-CD, 45% for NC-CD, and 80% for those without CD. Specificity was 90% to 92% in hospital discharge data and 85% to 91% in Medicaid claims data for all 3 groups. CONCLUSIONS: PMCA identified children with C-CD (who have accessed tertiary hospital care) with good sensitivity and good to excellent specificity when applied to hospital discharge or Medicaid claims data. PMCA may be useful for targeting resources such as care coordination to children with C-CD.

http://www.ncbi.nlm.nih.gov/pubmed/24819580

Seizure burden is independently associated with short term outcome in critically ill children

Payne, E. T., et al.

Brain 2014; 137(Pt 5): 1429-1438

Seizures are common among critically ill children, but their relationship to outcome remains unclear. We sought to quantify the relationship between electrographic seizure burden and short-term neurological outcome, while controlling for diagnosis and illness severity. Furthermore, we sought to determine whether there is a seizure burden threshold above which there is an increased probability of neurological decline. We prospectively evaluated all infants and children admitted to our paediatric and cardiac intensive care units who underwent clinically ordered continuous video-electroencephalography monitoring over a 3-year period. Seizure burden was quantified by calculating the maximum percentage of any hour that was occupied by electrographic seizures. Outcome measures included neurological decline, defined as a worsening Paediatric Cerebral Performance Category score between hospital admission and discharge, and in-hospital mortality. Two hundred and fifty-nine subjects were evaluated (51% male) with a median age of 2.2 years (interquartile range: 0.3 days-9.7 years). The median duration of continuous video-electroencephalography monitoring was 37 h (interquartile range: 21-56 h). Seizures occurred in 93 subjects (36%, 95% confidence interval = 30-42%), with 23 (9%, 95% confidence interval = 5-12%) experiencing status epilepticus. Neurological decline was observed in 174 subjects (67%), who had a mean maximum seizure burden of 15.7% per hour, compared to 1.8% per hour for those without neurological decline ($P < 0.0001$). Above a maximum seizure burden threshold of 20% per hour (12 min), both the probability and magnitude of neurological decline rose sharply ($P < 0.0001$) across all diagnostic categories. On multivariable analysis adjusting for diagnosis and illness severity, the odds of neurological decline increased by 1.13 (95% confidence interval = 1.05-1.21, $P = 0.0016$) for every 1% increase in maximum hourly seizure burden. Seizure burden was not associated with mortality (odds ratio: 1.003, 95% confidence interval: 0.99-1.02, $P = 0.613$). We conclude that in this cohort of critically ill children, increasing seizure burden was independently associated with a greater probability and magnitude of neurological decline. Our observation that a seizure burden of more than 12 min in a given hour was strongly associated with neurological decline suggests that early antiepileptic drug management is warranted in this population, and identifies this seizure burden threshold as a potential therapeutic target. These findings support the hypothesis that electrographic seizures independently contribute to brain injury and worsen outcome. Our results motivate and inform the design of future studies to determine whether more aggressive seizure treatment can improve outcome.

http://www.ncbi.nlm.nih.gov/pubmed/24595203

Pediatric patients with refractory central nervous system tumors: Experiences of a clinical trial combining bevacizumab and temsirolimus

Piha-Paul, S. A., et al.

Anticancer Res 2014; 34(4): 1939-1945

BACKGROUND: Pre-clinical findings suggest that combination treatment with bevacizumab and temsirolimus could be effective against malignant pediatric central nervous system (CNS) tumors. PATIENTS AND METHODS: Six pediatric patients were treated as part of a phase I trial with intravenous temsirolimus 25 mg on days 1, 8, 15, and bevacizumab at 5, 10, or 15 mg/kg on day 1 of each 21-day cycle until disease progression or patient withdrawal. RESULTS: The median patient age was six years (range=3-14 years). The primary diagnoses were glioblastoma multiforme (n=2), medullobalstoma (n=2), pontine glioma (n=1) and ependymoma (n=1). All patients had disease refractory to standard-of-care (2-3 prior systemic therapies). Grade 3 toxicities possibly related to drugs used occurred in two patients: anorexia, nausea, and weight loss in one, and thrombocytopenia and alanine aminotransferase elevation in another. One patient with glioblastoma multiforme achieved a partial response (51% regression) and two patients (with medulloblastoma and pontine glioma) had stable disease for four months or more (20 and 47 weeks, respectively). One other patient (with glioblastoma multiforme) showed 18% tumor regression (duration=12 weeks). CONCLUSION: The combination of bevacizumab with temsirolimus was well-tolerated and resulted in stable disease of at least four months/partial response in three out of six pediatric patients with chemorefractory CNS tumors.

http://www.ncbi.nlm.nih.gov/pubmed/24692729

Functional outcomes associated with adaptive seating interventions in children and youth with wheeled mobility needs

Ryan, S. E., et al.

Arch Phys Med Rehabil 2014; 95(5): 825-831

OBJECTIVE: To determine the parent-reported functional outcomes associated with adaptive seating devices for wheeled mobility devices used by young people aged 1 to 17 years. DESIGN: Longitudinal case series. SETTING: Homes of participating parents. PARTICIPANTS: Parents (N=70, 63 mothers, 6 fathers, 1 grandmother) who had children with adaptive seating needs. INTERVENTION: Adaptive seating system for wheeled mobility devices. MAIN OUTCOME MEASURE: Family Impact of Assistive Technology Scale for Adaptive Seating (FIATS-AS). RESULTS: All parents completed the FIATS-AS 4 times-2 times before and 2 times after their child received a new adaptive seating system. Mixed-design analysis of variance did not detect significant mean differences among the FIATS-AS scores measured at baseline and 2 and 8 months after receiving the seating system (F2,134=.22, P=.81). However, the FIATS-AS detected a significant interaction between age cohort and interview time (F4,134=4.5, P<.001, partial eta(2)=.16). Post hoc testing confirmed that 8 months after receiving the seating system was associated with a large improvement in child and family functioning for children <4 years, maintenance of functioning for children between 4 and 12 years, and a moderate decline in functioning for youth between 13 and 17 years. CONCLUSIONS: Adaptive seating interventions for wheeled mobility devices are associated with functional changes in the lives of children and their families that interact inversely with age. Future controlled longitudinal studies could provide further empirical evidence of functional changes in the lives of children and their families after the introduction and long-term use of specific adaptive seating interventions.

http://www.ncbi.nlm.nih.gov/pubmed/24035768

Treatment outcome in children and adolescents with relapsed Hodgkin lymphoma - results of the UK HD3 relapse treatment strategy

Shankar, A., et al.

Br J Haematol 2014; 165(4): 534-544

The purpose of this national retrospective study was to evaluate the outcome in children with relapsed or primary refractory Hodgkin lymphoma [HL] after a primary chemotherapy alone treatment strategy. Between 2000 and 2005, 80 children with relapsed [n = 69] or primary refractory [n = 11] HL were treated on a standardized treatment protocol of 4-6 cycles of EPIC [etoposide, prednisolone, ifosfamide and cisplatin] chemotherapy. Radiotherapy was recommended to all relapsed sites. High dose therapy with stem cell rescue [SCT] was recommended for patients with poor response. The 5-year overall survival [OS] and progression-free survival from relapse was 75.8% [64.8-83.9] and 59.9% [48.3-69.7] respectively. Duration of first remission was strongly associated with OS; risk of death was decreased by 53% [Hazard ratio (HR): 0.47, 95% confidence interval (CI): 0.19-1.18] for those with a time from end of treatment to relapse of 3-12 months (compared to <3 months) and reduced by 80% (HR 0.20, 95% CI: 0.04-0.90) for those >12 months after end of treatment. Other poor prognostic factors included advanced stage disease at relapse and B symptoms at first diagnosis. The most important factor associated with salvage failure was time to relapse. Survival outcome in children with primary refractory HL is poor.

http://www.ncbi.nlm.nih.gov/pubmed/24754633

A systematic review of interventions for children with cerebral palsy: The state of the evidence

Thomason, P. and H. K. Graham

Dev Med Child Neurol 2014; 56(4): 390-391

http://www.ncbi.nlm.nih.gov/pubmed/24628590

Perioperative factors associated with in-hospital mortality or retransplantation in pediatric heart transplant recipients

Vanderlaan, R. D., et al.

J Thorac Cardiovasc Surg 2014; 148(1): 282-289

OBJECTIVE: Despite improved long-term survival after pediatric heart transplantation, perioperative mortality has remained high. We sought to understand the factors associated with perioperative graft loss after pediatric heart transplantation. METHODS: The factors associated with primary heart transplant mortality and retransplantation before hospital discharge in 226 pediatric heart transplant recipients (1995-2010) at a single-center institution were analyzed using multivariable logistic regression models adjusted for age at surgery and year of surgery. RESULTS: A total of 26 patients died (n = 21) or underwent retransplantion (n = 5) before hospital discharge secondary to primary graft failure (n = 10), multisystem organ failure (n = 5), infection (n = 4), rejection (n = 2), and perioperative complications (n = 5). United Network for Organ Sharing status 1 (vs status 2) at transplantation was associated with an increased odds of death from noncardiac causes (odd ratio [OR], 4.7; 95% confidence level [CI], 1.2-22.3; P = .002). The factors associated with increased odds of perioperative mortality or retransplant were pre- and post-transplant extracorporeal membrane oxygenation (OR, 5.3; 95% CI, 1.5-18.7; P = .01; and OR, 25.9; 95% CI, 7.0-95.9; P < .001), longer ischemic times (OR, 1.4 per 30 minutes; 95% CI, 1.0-2.0; P = .04), reoperation after transplantation (OR, 3.5; 95% CI, 1.2-10.4; P = .02), and transplantation before 2002 (OR, 4.5; 95% CI, 1.4-14.9; P = .01), respectively. CONCLUSIONS: The use of extracorporeal membrane oxygenation (both before and after transplantation), a longer ischemic time, and reoperation were key factors associated with perioperative graft loss, with noncardiac mortality closely related to United Network for Organ Sharing status at heart transplantation. Knowledge of the perioperative risk factors and how they affect graft survival will help guide difficult decisions around eligibility, timing of primary listing, and appropriateness for retransplantation, and potentially affect long-term survival.

http://www.ncbi.nlm.nih.gov/pubmed/24755331

Treatment of multiply relapsed wilms tumor with vincristine, irinotecan, temozolomide and bevacizumab

Venkatramani, R., et al.

Pediatr Blood Cancer 2014; 61(4): 756-759

As most active chemotherapy agents against Wilms tumor are incorporated into upfront therapy, particularly for those patients with high risk for recurrence, novel regimens are needed to treat children with relapsed Wilms tumor. We describe four consecutive patients with multiply relapsed Wilms tumor who were treated with a combination of vincristine, irinotecan, temozolomide, and bevacizumab. Two had a complete response, and two had a partial response to treatment. Hematological toxicity and diarrhea were the main side effects. This regimen has activity in patients with multiply relapsed Wilms tumor without excessive toxicity, and should be evaluated further in this setting.

http://www.ncbi.nlm.nih.gov/pubmed/24115645

Changes in ventilator strategies and outcomes in preterm infants

Vendettuoli, V., et al.

Arch Dis Child Fetal Neonatal Ed 2014; 99(4): F321-324

BACKGROUND: Although life-saving, intubation and mechanical ventilation can lead to complications including bronchopulmonary dysplasia (BPD). In order to reduce the incidence of BPD, non-invasive ventilation (NIV) is increasingly used. OBJECTIVE: The aim of our study was to describe changes in ventilator strategies and outcomes between 2006 and 2010 in the Italian Neonatal Network (INN). DESIGN: Multicentre cohort study. SETTINGS: 31 tertiary level neonatal units participating in INN in 2006 and 2010. PATIENTS: 2465 preterm infants 23-30 weeks gestational age (GA) without congenital anomalies. MAIN OUTCOMES MEASURES: Death, BPD and other variables defined according to Vermont Oxford Network. Logistic regressions, adjusting for confounders and clustering for hospitals, were used. RESULTS: Similar numbers of infants were studied between 2006 and 2010 (1234 in 2006 and 1231 in 2010). The baseline risk of populations studied (GA, birth weight and Vermont Oxford Network Risk-Adjustment score) did not change. After adjusting for confounding variables, infants receiving invasive mechanical ventilation decreased (OR=0.72, 95% CI 0.58 to 0.89) while NIV increased (OR=1.75, 95% CI 1.39 to 2.21); intubation in delivery room decreased (OR=0.64, 95% CI 0.51 to 0.79). Considering outcomes, there was a significant reduction in mortality (OR=0.73, 95% CI 0.55 to 0.96) and in the combined outcome mortality or BPD (OR=0.76, 95% CI 0.62 to 0.94). CONCLUSIONS: Despite a stable baseline risk, from 2006 to 2010, we observed a lower level of invasiveness, a reduction of mechanical ventilation and an increase of NIV use, and this was accompanied by a decrease in risk-adjusted mortality and BPD.

http://www.ncbi.nlm.nih.gov/pubmed/24846520

Reliability of Circulatory and Neurologic Examination by Telemedicine in a Pediatric Intensive Care Unit

Yager, P. H., et al.

J Pediatr 2014

OBJECTIVE: To test the hypothesis that telemedicine can reliably be used for many aspects of circulatory and neurologic examinations of children admitted to a pediatric intensive care unit (PICU). STUDY DESIGN: A prospective, randomized study in a 14-bed PICU in a tertiary care, academic-affiliated institution. Eligible patients were >2 months or <19 years of age, not involved in a concurrent study, had parents/guardian able to sign an informed consent form, were not at end-of-life, and had an attending who not only deemed them medically stable, but also felt that the study would not interrupt their care. Other than the Principal Investigator, 6 pediatric intensivists and 7 pediatric critical care fellows were eligible study providers. Two physician providers were randomly assigned to perform circulatory and neurologic examinations according to the American Heart Association/Pediatric Advanced Life Support guidelines in-person and via telemedicine. Findings were recorded on a standardized data collection form and compared. RESULTS: One hundred ten data collection forms were completed. For many aspects of the circulatory and neurologic examinations, outcomes showed substantial to perfect agreement between the in-person and telemedical care providers (kappa = 0.64-1.00). However, assessments of muscle tone had a kappa = 0.23, with a kappa = 0.37 for skin color. CONCLUSIONS: Telemedicine can reliably identify normal and abnormal findings of many aspects of circulatory and neurologic examinations in PICU patients. This finding opens the door to further studies on the use of telemedicine across other disciplines.

http://www.ncbi.nlm.nih.gov/pubmed/25112695

Severe infections in children with acute leukemia undergoing intensive chemotherapy can successfully be prevented by ciprofloxacin, voriconazole, or micafungin prophylaxis

Yeh, T. C., et al.

Cancer 2014; 120(8): 1255-1262

BACKGROUND: The purpose of the current study was to prevent bloodstream infection and invasive fungal infection (IFI) by administering prophylactic antibiotic and antifungal agents during intensive chemotherapy in patients being treated for acute leukemia. METHODS: Prophylaxis treatment was administered during intensive chemotherapy in children with acute myeloid leukemia (AML) and acute lymphoblastic leukemia (ALL) from January 1, 2010 to December 31, 2012. Oral ciprofloxacin (at a dose of 300 mg/m(2) /12 hours) was administered after chemotherapy when a patient with AML or ALL became neutropenic and > 7 days of neutropenia was expected. Voriconazole (at a dose of 4 mg/kg/12 hours) was initiated at the onset of neutropenia in patients with AML and after 7 days of neutropenia in patients with ALL. Micafungin (at a dose of 2 mg/kg/day) was substituted for voriconazole when patients with ALL received vincristine. Prophylaxis treatment was discontinued when the absolute neutrophil count recovered to > 100/muL. All episodes of bloodstream infection, IFI, febrile neutropenia, and intensive care unit stays related to severe infection occurring between January 1, 2005 and December 31, 2012 were recorded. RESULTS: During the preprophylaxis period, 62 children with ALL and 24 children with AML experienced a total of 44 episodes of bloodstream infection and 22 episodes of IFI. Seven patients died of severe infection. In contrast, in the prophylaxis period, 10 episodes of bloodstream infection occurred and no IFIs were reported to occur in 51 patients with ALL and 14 patients with AML. Moreover, no patient died of severe infection. Episodes of febrile neutropenia and intensive care unit stay were significantly reduced during the prophylaxis period. CONCLUSIONS: Prophylaxis with ciprofloxacin and voriconazole or micafungin was found to reduce the rates of bloodstream infection and IFI in children with acute leukemia undergoing intensive chemotherapy.

http://www.ncbi.nlm.nih.gov/pubmed/24415457

Treatment of multiply relapsed wilms tumor with vincristine, irinotecan, temozolomide and bevacizumab

Venkatramani, R., et al.

Pediatr Blood Cancer 2014; 61(4): 756-759

As most active chemotherapy agents against Wilms tumor are incorporated into upfront therapy, particularly for those patients with high risk for recurrence, novel regimens are needed to treat children with relapsed Wilms tumor. We describe four consecutive patients with multiply relapsed Wilms tumor who were treated with a combination of vincristine, irinotecan, temozolomide, and bevacizumab. Two had a complete response, and two had a partial response to treatment. Hematological toxicity and diarrhea were the main side effects. This regimen has activity in patients with multiply relapsed Wilms tumor without excessive toxicity, and should be evaluated further in this setting.

http://www.ncbi.nlm.nih.gov/pubmed/24115645

Acute pain relief after Mantram meditation in children with neuroblastoma undergoing anti-GD2 monoclonal antibody therapy

Ahmed, M., et al.

J Pediatr Hematol Oncol 2014; 36(2): 152-155

Nonpharmacologic, mind-body interventions are used to reduce anxiety in pediatric patients. Anti-ganglioside GD2 monoclonal antibody (anti-GD2 MoAb 3F8) therapy is the standard of care for high-risk neuroblastoma and pain is its major side effect. We performed a retrospective analysis of children undergoing anti-GD2 MoAb 3F8 treatment who received guided meditation. Meditation involved concentrating on the repetition of rhythmic, melodic sounds purported to slow breathing and induce a relaxation response. A total of 71% patients completed a session at first (n=19) or second attempt (n=5). Patients received fewer analgesic doses to manage anti-GD2 MoAb 3F8-induced pain when participating in meditation (n=17, mean=-0.4 dose, P<0.01). Mantram meditation is a feasible outpatient intervention associated with reduced analgesic requirements.

http://www.ncbi.nlm.nih.gov/pubmed/24065045

Neuropathic pain in patients with sickle cell disease

Brandow, A. M., et al.

Pediatr Blood Cancer 2014; 61(3): 512-517

BACKGROUND: Despite the suggestion of a neuropathic component to sickle cell disease (SCD) pain, there are minimal data on the systematic assessment of neuropathic pain in patients with SCD. Neuropathic pain is defined as pain primarily initiated by dysfunction of the peripheral or central nervous system. PROCEDURE: In a cross-sectional study, we used the painDETECT questionnaire, a one-page validated neuropathic pain screening tool, to determine the presence of neuropathic pain in patients with SCD and to evaluate the relationship between neuropathic pain, age, and gender. We hypothesized that 20% of patients with SCD will experience neuropathic pain and that neuropathic pain will be associated with older age and female gender. The completed painDETECT questionnaire yields a total score between 0 and 38 (>/= 19 = definite neuropathic pain, 13-18 = probable neuropathic pain, </= 12 = no neuropathic pain). Scores >/= 13 were designated as having evidence of neuropathic pain. RESULTS: A total of 56 patients participated. Median age was 20.3 years and 77% were female. We found 37% of patients had evidence of neuropathic pain. Age was positively correlated with total score (r = 0.43; P = 0.001) suggesting older patients experience more neuropathic pain. Females had higher mean total scores (13 vs. 8.4; P = 0.04). Significantly more patients with neuropathic pain were taking hydroxyurea (90% vs. 59%; P = 0.015). Despite 37% of patients experiencing neuropathic pain, only 5% were taking a neuropathic pain drug. CONCLUSIONS: Neuropathic pain exists in SCD. Valid screening tools can identify patients that would benefit from existing and future neuropathic pain therapies and could determine the impact of these therapies.

http://www.ncbi.nlm.nih.gov/pubmed/24167104

Patterns of symptoms and functional impairments in children with cancer

Buckner, T. W., et al.

Pediatr Blood Cancer 2014; 61(7): 1282-1288

BACKGROUND: Children with cancer experience multiple symptoms due to their disease and as a result of treatment. The purpose of this study was to demonstrate the feasibility and potential utility of using latent profile analysis (LPA), a type of cluster analysis, in children with cancer to identify groups of patients who experience similar levels of symptom severity and impairment of physical function. PROCEDURE: We analyzed patient-reported symptom and functional data previously collected using the Pediatric Patient Reported Outcomes Measurement Information System (PROMIS). LPA was used to identify and characterize groups of patients who reported similar levels of symptom severity and functional impairment. We then used the multinomial logit model to examine demographic and disease characteristics associated with symptom/function profile membership. RESULTS: The analysis included 200 patients in treatment or in survivorship. We identified four symptom/function profiles; children currently receiving cancer treatment and those with at least one other medical problem were more likely to be members of the profile with the highest levels of symptom severity and functional impairment. Gender, age, race/ethnicity, and tumor type were not associated with profile membership. CONCLUSIONS: LPA is a cluster research methodology that provides clinically useful results in pediatric oncology patients. Future studies of children with cancer using LPA could potentially lead to development of clinical scoring systems that predict patients' risk of developing more severe symptoms and functional impairments, allowing clinicians, patients, and parents to better anticipate and prevent the multiple symptoms that occur during and after treatment for childhood cancer.

http://www.ncbi.nlm.nih.gov/pubmed/24634396

Lorazepam vs diazepam for pediatric status epilepticus: A randomized clinical trial

Chamberlain, J. M., et al.

JAMA 2014; 311(16): 1652-1660

IMPORTANCE: Benzodiazepines are considered first-line therapy for pediatric status epilepticus. Some studies suggest that lorazepam may be more effective or safer than diazepam, but lorazepam is not Food and Drug Administration approved for this indication. OBJECTIVE: To test the hypothesis that lorazepam has better efficacy and safety than diazepam for treating pediatric status epilepticus. DESIGN, SETTING, AND PARTICIPANTS: This double-blind, randomized clinical trial was conducted from March 1, 2008, to March 14, 2012. Patients aged 3 months to younger than 18 years with convulsive status epilepticus presenting to 1 of 11 US academic pediatric emergency departments were eligible. There were 273 patients; 140 randomized to diazepam and 133 to lorazepam. INTERVENTIONS: Patients received either 0.2 mg/kg of diazepam or 0.1 mg/kg of lorazepam intravenously, with half this dose repeated at 5 minutes if necessary. If status epilepticus continued at 12 minutes, fosphenytoin was administered. MAIN OUTCOMES AND MEASURES: The primary efficacy outcome was cessation of status epilepticus by 10 minutes without recurrence within 30 minutes. The primary safety outcome was the performance of assisted ventilation. Secondary outcomes included rates of seizure recurrence and sedation and times to cessation of status epilepticus and return to baseline mental status. Outcomes were measured 4 hours after study medication administration. RESULTS: Cessation of status epilepticus for 10 minutes without recurrence within 30 minutes occurred in 101 of 140 (72.1%) in the diazepam group and 97 of 133 (72.9%) in the lorazepam group, with an absolute efficacy difference of 0.8% (95% CI, -11.4% to 9.8%). Twenty-six patients in each group required assisted ventilation (16.0% given diazepam and 17.6% given lorazepam; absolute risk difference, 1.6%; 95% CI, -9.9% to 6.8%). There were no statistically significant differences in secondary outcomes except that lorazepam patients were more likely to be sedated (66.9% vs 50%, respectively; absolute risk difference, 16.9%; 95% CI, 6.1% to 27.7%). CONCLUSIONS AND RELEVANCE: Among pediatric patients with convulsive status epilepticus, treatment with lorazepam did not result in improved efficacy or safety compared with diazepam. These findings do not support the preferential use of lorazepam for this condition. TRIAL REGISTRATION: clinicaltrials.gov Identifier: NCT00621478.

http://www.ncbi.nlm.nih.gov/pubmed/24756515

Patient-controlled analgesia in the pediatric population: morphine versus hydromorphone

DiGiusto, M., et al.

J Pain Res 2014; 7: 471-475

OBJECTIVE: Patient controlled analgesia (PCA) is commonly used to provide analgesia following surgical procedures in the pediatric population. Morphine and hydromorphone remain the most commonly used opioids for PCA. Although both are effective, adverse effects may occur. When these adverse effects are unremitting or severe, opioid rotation may be required. In this study, we retrospectively evaluated PCA use, the adverse effect profile, and the frequency of opioid rotation. METHODS: This retrospective study was performed at Nationwide Children's Hospital (Columbus, OH). The hospital's electronic registry was queried for PCA use delivering either morphine or hydromorphone from January 1, 2008 to December 31, 2010. RESULTS: A total of 514 patients were identified, that met study entry criteria. Of the 514 cases, 298 (56.2%) were initially started on morphine and 225 (43.8%) were initially started on hydromorphone. There were a total of 26 (5.1%) opioid changes in the cohort of 514 patients. Of the 26 switches, 23 of 298 (7.7%) were from morphine to hydromorphone, and 3 of 225 (1.3%) were from hydromorphone to morphine (P=0.0008). Of the 17 morphine-to-hydromorphone switches with adverse effects, pruritus (64.7%), and inadequate pain control (47.1%) were the most common side effects. The most common side effect resulting in a hydromorphone-to-morphine switch was nausea (66.7%). CONCLUSION: PCA switches from morphine-to-hydromorphone (88.5%) were more common than vice-versa (11.5%). The most common reasons for morphine-to-hydromorphone switch were pruritus and inadequate pain control. These data suggest that a prospective study is necessary to determine the side effect differences between morphine and hydromorphone in pediatric PCA.

http://www.ncbi.nlm.nih.gov/pubmed/25152630

Original research: Using guided imagery to manage pain in young children with sickle cell disease

Dobson, C. E. and M. W. Byrne

Am J Nurs 2014; 114(4): 26-36; test 37, 47

BACKGROUND: Despite innovations in treatment, disease-related pain is still the primary cause of hospitalization for children with sickle cell disease. Pharmacologic pain management relieves pain temporarily, but adverse effects are increasingly a concern. Cognitive behavioral therapies, which include the use of guided imagery, have shown promise in changing pain perception and coping patterns in people with chronic illnesses. Few studies have been done in children with sickle cell disease. OBJECTIVES: The purposes of this study were to test the effects of guided imagery training on school-age children who had been diagnosed with sickle cell disease, and to describe changes in pain perception, analgesic use, self-efficacy, and imaging ability from the month before to the month after training. METHODS: A quasi-experimental interrupted time-series design was used with a purposive sample of 20 children ages six to 11 years enrolled from one sickle cell disease clinic, where they had been treated for at least one year. Children completed pain diaries daily for two months, and investigators measured baseline and end-of-treatment imaging ability and self-efficacy. RESULTS: After training in the use of guided imagery, participants reported significant increases in self-efficacy and reductions in pain intensity, and use of analgesics decreased as well. CONCLUSIONS: Guided imagery is an effective technique for managing and limiting sickle cell disease-related pain in a pediatric population.

http://www.ncbi.nlm.nih.gov/pubmed/24632887

Guideline for the prevention and treatment of anticipatory nausea and vomiting due to chemotherapy in pediatric cancer patients

Dupuis, L. L., et al.

Pediatr Blood Cancer 2014; 61(8): 1506-1512

This guideline provides an approach to the prevention and treatment of anticipatory chemotherapy-induced nausea and vomiting (CINV) in children. It was developed by an international, inter-professional panel using AGREE II methods and is based on systematic literature reviews. Evidence-based recommendations for pharmacological and non-pharmacological interventions to prevent and treat anticipatory CINV in children receiving antineoplastic agents are provided. Gaps in the evidence used to support the recommendations are identified. The contribution of this guideline to anticipatory CINV control in children requires prospective evaluation.

http://www.ncbi.nlm.nih.gov/pubmed/24753095

Comparative effectiveness of senna to prevent problematic constipation in pediatric oncology patients receiving opioids: A multicenter study of clinically detailed administrative data

Feudtner, C., et al.

J Pain Symptom Manage 2014; 48(2): 272-280

CONTEXT: Pediatric oncology patients often receive prolonged courses of opioids, which can result in constipation. OBJECTIVES: Comparing patients who received senna matched with similar patients who received other oral bowel medications, determine the subsequent risk of "problematic constipation," assessed as the occurrence of the surrogate markers of receiving an enema, escalation of oral bowel medications, and abdominal radiographic imaging. METHODS: This was a retrospective cohort study of hospitalized pediatric oncology patients less than 21 years of age in 78 children's and adult hospitals between 2006 and 2011 who were started on seven consecutive days or more of opioid therapy and were started on an oral bowel medication within the first two days of opioid therapy. Clinically detailed administrative data were used from the Pediatric Health Information System and the Premier Perspective Database. After performing propensity score matching of similar patients who started senna and who started a different oral bowel medication, Cox regression modeling was used to compare the subsequent hazard of the surrogate markers. RESULTS: The final matched sample of 586 patients averaged 11.5 years of age (range 0-20 years); 41.8% (n = 245) had blood cancer, 50.3% (n = 295) had solid tumor cancer, and 7.9% (n = 46) had brain cancer. Initiating senna therapy within two days of starting the prolonged opioid course, compared with initiating another oral bowel medication, was significantly associated with a lower hazard during the ensuing five days for receipt of an enema (hazard ratio [HR], 0.31; 95% CI, 0.11-0.91) or undergoing abdominal radiographic imaging (HR, 0.74; 95% CI, 0.55-0.98), was marginally associated with a lower hazard of oral bowel medicine escalation (HR, 0.78; 95% CI, 0.59-1.03), and overall was significantly associated with a lower hazard of the composite end point of problematic constipation (HR, 0.70; 95% CI, 0.56-0.88). CONCLUSION: Initiating senna therapy, compared with other oral bowel medications, diminishes the subsequent risk of surrogate markers of problematic constipation in this population.

http://www.ncbi.nlm.nih.gov/pubmed/24321507

Massage therapy research review

Field, T.

Complement Ther Clin Pract 2014

Moderate pressure massage has contributed to many positive effects including increased weight gain in preterm infants, reduced pain in different syndromes including fibromyalgia and rheumatoid arthritis, enhanced attentiveness, reduced depression and enhanced immune function (increased natural killer cells and natural killer cell activity).Surprisingly, these recent studies have not been reviewed, highlighting the need for the current review. When moderate and light pressure massage have been compared in laboratory studies, moderate pressure massage reduced depression, anxiety and heart rate, and it altered EEG patterns, as in a relaxation response. Moderate pressure massage has also led to increased vagal activity and decreased cortisol levels. Functional magnetic resonance imaging data have suggested that moderate pressure massage was represented in several brain regions including the amygdala, the hypothalamus and the anterior cingulate cortex, all areas involved in stress and emotion regulation. Further research is needed to identify underlying neurophysiological and biochemical mechanisms associated with moderate pressure massage.

http://www.ncbi.nlm.nih.gov/pubmed/25172313

Systematic review and meta-analysis of psychological therapies for children with chronic pain

Fisher, E., et al.

J Pediatr Psychol 2014; 39(8): 763-782

OBJECTIVES: This systematic review and meta-analysis examined the effects of psychological therapies for management of chronic pain in children. METHODS: Randomized controlled trials of psychological interventions treating children (<18 years) with chronic pain conditions including headache, abdominal, musculoskeletal, or neuropathic pain were searched for. Pain symptoms, disability, depression, anxiety, and sleep outcomes were extracted. Risk of bias was assessed and quality of the evidence was rated using GRADE. RESULTS: 35 included studies revealed that across all chronic pain conditions, psychological interventions reduced pain symptoms and disability posttreatment. Individual pain conditions were analyzed separately. Sleep outcomes were not reported in any trials. Optimal dose of treatment was explored. For headache pain, higher treatment dose led to greater reductions in pain. No effect of dosage was found for other chronic pain conditions. CONCLUSIONS: Evidence for psychological therapies treating chronic pain is promising. Recommendations for clinical practice and research are presented.

http://www.ncbi.nlm.nih.gov/pubmed/24602890

Pain management at home in children with cancer: A daily diary study

Fortier, M. A., et al.
Pediatr Blood Cancer 2014; 61(6): 1029-1033

BACKGROUND: With the transition of care of cancer patients from the hospital to the home setting, parents are largely responsible for children's pain management. Children's cancer pain is undermanaged, yet, there is little empirical data on the occurrence and management of cancer pain in the home setting. The purpose of the present study, therefore, was to employ a daily diary protocol to examine barriers to pain management of children's cancer pain by parents at home. PROCEDURE: Parent-child dyads were recruited from the Cancer Institute at a major children's hospital in Southern California. A total of 45 patient/parent pairs completed baseline data on demographic and personality characteristics, children's quality of life, and parental beliefs regarding analgesic use for children and then completed daily diaries of pain and analgesic administration for 14 consecutive days. RESULTS: Most children were reported to experience chronic pain while undergoing treatment for cancer, yet overall analgesic administration at home was low. Parents who reported misconceptions regarding analgesic use for children were less likely to administer pain medication to children. Children who were less shy, more social, or had lower quality of life were more likely to receive analgesics. CONCLUSIONS: A significant proportion of children receiving outpatient treatment for cancer were rated as experiencing chronic pain and pain was not optimally managed in the home setting. Further understanding and addressing barriers to children's cancer pain management in the home setting will aid in alleviating unnecessary pain in this vulnerable patient population.

http://www.ncbi.nlm.nih.gov/pubmed/24376073

Procedural sedation and analgesia in children

Golzari, S. E., et al.
N Engl J Med 2014; 371(1): 90-91

http://www.ncbi.nlm.nih.gov/pubmed/24988583

Viscous lidocaine treatment for painful oral infections in children: Disappointingly dismissive of pediatric pain

Hoffman, R. J.
Ann Emerg Med 2014; 64(1): 96-97

http://www.ncbi.nlm.nih.gov/pubmed/24951416

Seizure control following palliative resective surgery for intractable epilepsy-a pilot study

Ilyas, M., et al.

Pediatr Neurol 2014; 51(3): 330-335

BACKGROUND: Patients with intractable epilepsy who have bilateral epileptic foci may not qualify for curative epilepsy surgery. In some cases palliative resection may be undertaken with a goal to decrease seizure frequency and improve quality of life. Here we present data on the outcome of palliative epilepsy surgery in children. METHODS: We reviewed medical charts of children who underwent palliative resection for intractable epilepsy during the years 1999-2013 at Children's Hospital of Michigan. The palliative intent of resection was declared preoperatively. Outcome was assessed in terms of seizure reduction. RESULTS: There were 18 patients (11 males, median age of surgery was 3.5 years [range 0.5-16 years]). The median duration of follow-up after surgery was 12.5 months (range 6-60 months). Hemispherectomy was the most commonly performed palliative resection (nine patients), followed by lobectomy (six patients), multilobar resection (one patient), and tuberectomy (two patients). Reduction in seizure frequency was observed in 11 patients, with eight patients achieving seizure freedom on antiepileptic drugs and three with >50% reduction in seizure frequency. Transient improvement in seizure frequency occurred in two patients, whereas there was no benefit in five patients. CONCLUSIONS: Beneficial effects of epilepsy surgery may be realized in carefully selected situations wherein the most epileptogenic focus is resected to reduce seizure burden and improve quality of life.

http://www.ncbi.nlm.nih.gov/pubmed/25160538

Safety of intranasal fentanyl in the out-of-hospital setting: A prospective observational study

Karlsen, A. P., et al.

Ann Emerg Med 2014; 63(6): 699-703

STUDY OBJECTIVE: Initial out-of-hospital analgesia is sometimes hampered by difficulties in achieving intravenous access or lack of skills in administering intravenous opioids. We study the safety profile and apparent analgesic effect of intranasal fentanyl in the out-of-hospital setting. METHODS: In this prospective observational study, we administered intranasal fentanyl in the out-of-hospital setting to adults and children older than 8 years with severe pain resulting from orthopedic conditions, abdominal pain, or acute coronary syndrome refractory to nitroglycerin spray. Patients received 1 to 3 doses of either 50 or 100 mug, and the ambulance crew recorded adverse effects and numeric rating scale (0 to 10) pain scores before and after treatment. RESULTS: Our 903 evaluable patients received a mean cumulative fentanyl dose of 114 mug (range 50 to 300 mug). There were no serious adverse effects and no use of naloxone. Thirty-six patients (4%) experienced mild adverse effects: mild hypotension, nausea, vomiting, vertigo, abdominal pain, rash, or decrease of Glasgow Coma Scale score to 14. The median reduction in pain score was 3 (interquartile range 2 to 5) after fentanyl administration. CONCLUSION: The out-of-hospital administration of intranasal fentanyl in doses of 50 to 100 mug is safe and appears effective.

http://www.ncbi.nlm.nih.gov/pubmed/24268523

Childrens' pain, a complex entity to be explored

Knauer, D.

Rev Med Suisse 2014; 10(436): 1401-1402, 1404-1405

From unrecognized babies' pain, to new discoveries made on early emotional memory and sensory capacities, chronic pain in childhood remains a complex field still to be explored. Besides, the discovery of early fetal sensorial receptions is an opening to new understanding of the origin of psychological chronic pain from early childhood to adolescence. From the silent babies suffering of emotional regulation disorder to adolescents' chronic recurrent pains, a common point is the expression of a psychic disease through the body. Different therapeutic actions are described, because without treatment chronic or recurrent pains may lead to a real risk of intellectual, affective and drive impoverishment for the suffering child.

http://www.ncbi.nlm.nih.gov/pubmed/25055474

Sedation, sleep promotion, and delirium screening practices in the care of mechanically ventilated children: A wake-up call for the pediatric critical care community

Kudchadkar, S. R., et al.

Crit Care Med 2014; 42(7): 1592-1600

OBJECTIVES: To examine pediatric intensivist sedation management, sleep promotion, and delirium screening practices for intubated and mechanically ventilated children. DESIGN: An international, online survey of questions regarding sedative and analgesic medication choices and availability, sedation protocols, sleep optimization, and delirium recognition and treatment. SETTING: Member societies of the World Federation of Pediatric Intensive and Critical Care Societies were asked to send the survey to their mailing lists; responses were collected from July 2012 to January 2013. SUBJECTS: Pediatric critical care providers. INTERVENTIONS: Survey. MEASUREMENTS AND MAIN RESULTS: The survey was completed by 341 respondents, the majority of whom were from North America (70%). Twenty-seven percent of respondents reported having written sedation protocols. Most respondents worked in PICUs with sedation scoring systems (70%), although only 42% of those with access to scoring systems reported routine daily use for goal-directed sedation management. The State Behavioral Scale was the most commonly used scoring system in North America (22%), with the COMFORT score more prevalent in all other countries (39%). The most commonly used sedation regimen for intubated children was a combination of opioid and benzodiazepine (72%). Most intensivists chose fentanyl as their first-line opioid (66%) and midazolam as their first-line benzodiazepine (86%) and prefer to administer these medications as continuous infusions. Propofol and dexmedetomidine were the most commonly restricted medications in PICUs internationally. Use of earplugs, eye masks, noise reduction, and lighting optimization for sleep promotion was uncommon. Delirium screening was not practiced in 71% of respondent's PICUs, and only 2% reported routine screening at least twice a day. CONCLUSIONS: The results highlight the heterogeneity in sedation practices among intensivists who care for critically ill children as well as a paucity of sleep promotion and delirium screening in PICUs worldwide.

http://www.ncbi.nlm.nih.gov/pubmed/24717461

Assessment and treatment of pain in children and adolescents

Lalloo, C. and J. N. Stinson

Best Pract Res Clin Rheumatol 2014; 28(2): 315-330

Pain is one of the most common and distressing symptoms experienced by children and adolescents with juvenile idiopathic arthritis. Pain is known to negatively affect all aspects of health-related quality of life, including physical, emotional, social, and role functioning. The valid and reliable assessment of pain is the first critical step to developing an effective plan for pain management. This chapter will address the following key questions: (1) What is the prevalence and impact of pain in children and adolescents with arthritis? (2) Why is it important for clinicians to assess the multidimensional nature of pain and what are the practical issues that should be considered? (3) What tools are available to help clinicians to assess pain? (4) How can Internet and mobile technologies be used to improve the assessment of pain? (5) What are the recommended strategies for clinically managing pain, including pharmacological, physical, and psychological approaches?

http://www.ncbi.nlm.nih.gov/pubmed/24974065

The case for medical marijuana in epilepsy

Maa, E. and P. Figi

Epilepsia 2014; 55(6): 783-786

Charlotte, a little girl with SCN1A-confirmed Dravet syndrome, was recently featured in a special that aired on CNN. Through exhaustive personal research and assistance from a Colorado-based medical marijuana group (Realm of Caring), Charlotte's mother started adjunctive therapy with a high concentration cannabidiol/Delta(9) -tetrahydrocannabinol (CBD:THC) strain of cannabis, now known as Charlotte's Web. This extract, slowly titrated over weeks and given in conjunction with her existing antiepileptic drug regimen, reduced Charlotte's seizure frequency from nearly 50 convulsive seizures per day to now 2-3 nocturnal convulsions per month. This effect has persisted for the last 20 months, and Charlotte has been successfully weaned from her other antiepileptic drugs. We briefly review some of the history, preclinical and clinical data, and controversies surrounding the use of medical marijuana for the treatment of epilepsy, and make a case that the desire to isolate and treat with pharmaceutical grade compounds from cannabis (specifically CBD) may be inferior to therapy with whole plant extracts. Much more needs to be learned about the mechanisms of antiepileptic activity of the phytocannabinoids and other constituents of Cannabis sativa.

http://www.ncbi.nlm.nih.gov/pubmed/24854149

Pallidotomy for medically refractory status dystonicus in childhood

Marras, C. E., et al.

Dev Med Child Neurol 2014; 56(7): 649-656

AIM: Status dystonicus is a rare and potentially fatal condition of continuous and generalized muscle contraction that can complicate dystonia. As status dystonicus is usually refractory to traditional pharmacological therapy, alternative and invasive strategies have been developed, but so far there are no guidelines on status dystonicus management. Pallidotomy has shown good results in status dystonicus treatment. METHOD: We report indications, surgical strategy, and outcome of bilateral pallidotomy in four pediatric patients (four males; mean age at surgery 11y 5mo) with secondary dystonia, who developed refractory status dystonicus. Pallidotomy was performed in the area corresponding to the mid portion of the globus pallidus internus. RESULTS: This procedure allowed patients to recover the pre-status dystonicus condition, controlling dystonic postures and movements of trunk and limbs. Moreover oromandibular dystonia, which is resistant to conservative approaches and deep brain stimulation, was significantly reduced. No postoperative complications were registered. INTERPRETATION: Our study suggests pallidotomy as a feasible treatment in patients with secondary dystonia complicated by status dystonicus.

http://www.ncbi.nlm.nih.gov/pubmed/24697701

Analysis of complications in 430 consecutive pediatric patients treated with intrathecal baclofen therapy: 14-year experience

Motta, F. and C. E. Antonello

J Neurosurg Pediatr 2014; 13(3): 301-306

OBJECT: This single-center study investigated adverse events that occurred in children and adolescent patients treated with intrathecal baclofen (ITB) therapy for spasticity and/or dystonia. METHODS: In a 14-year period, 430 consecutive patients with a mean age of 13.3 +/- 5.9 years received ITB over a mean follow-up period of 8.6 +/- 3.8 years (range 12 months to 14 years). Eighty-nine percent of these patients had cerebral palsy. Major complications, defined as those that required a surgical intervention, were infections, CSF leaks, and device problems related to the catheter or pump. Assessing infections, the authors compared the 2 groups of patients implanted with an ITB system by either the subcutaneous or subfascial technique. The temporal distribution of events related to the catheter was also considered. RESULTS: At least 1 complication was present in 25% of the patients: 9.3% experienced an infection, 4.9% a CSF leak, 15.1% a problem with the catheter, and 1% a problem related to the pump. Five percent of the assessed patients suffered more than 1 complication. The rate of infections was significantly lower ($p < 0.001$) in patients with the pump placed subfascially compared with those with the pump placed subcutaneously. A higher rate of infection was found after pump replacement compared with the first pump implantation (10.6% vs 6%, respectively). Catheter problems were the most common complication and occurred more frequently during the 1st year after the implant. CONCLUSIONS: While ITB is an effective treatment to manage spasticity of different origins, adverse events may occur and need to be managed. The surgical procedure should be meticulous and different techniques may have a diverse impact on the infection rate, which is the most critical complication. Despite the adverse events that occurred in this study, the majority of patients were satisfied with the treatment received.

http://www.ncbi.nlm.nih.gov/pubmed/24404968

Understanding pain - animated film for children with chronic pain celebrates its world premiere

Netz, H.
Kinderkrankenschwester 2014; 33(6): 237

http://www.ncbi.nlm.nih.gov/pubmed/25055450

Assessment of pain in children with brain injury: Moving to best practice

Nissen, S. and C. Dunford
Br J Nurs 2014; 23(17): 930-934

Nurses are guided to use pain tools for assessing pain. Appropriate tools exist for all ages of children, as well as accounting for diverse communicative abilities and impairments such as brain injury. Use of pain tools, and good documentation of pain management, is part of providing best practice, high-quality care. Clinical audit, based on compliance with the Royal College of Nursing guideline for pain assessment, measured current and changing practice at a 70-bed national specialist centre for children with brain injury. Compliance was initially poor. Changes in practice were supported by evidence-based measures, including a written guideline, classroom teaching, visits to practice areas, sharing of audit results, reminders and a special interest group. Over 3 years, the audits showed an increase of child-specific pain tools available in children's care files from 9% to 83%; assessment of pain using a pain tool, when indicated, increased from 0 to 30%. Documentation of interventions to relieve pain increased from 51% to 80% and reassessment of pain following an intervention increased from 15% to 63%. This article will resonate with any organisation trying to embed systematic pain assessment into routine practice.

http://www.ncbi.nlm.nih.gov/pubmed/25251174

Pediatric pain management: A review

O'Donnell, F. T. and K. R. Rosen
Mo Med 2014; 111(3): 231-237

Although the occurrence of pain in hospitalized children is common, assessment and treatment of pain presents unique challenges to practitioners who care for pediatric patients. Knowledge of drug mechanisms as well as metabolic differences in infants and children compared with adults is necessary for the successful treatment of acute and chronic pain syndromes. Recent reports of adverse events in children receiving both opioid and non-opioid analgesics have prompted re-examination of some long standing pain medication regimens and prescribing practices. We review advances in diagnosis and management of pain in pediatric populations.

http://www.ncbi.nlm.nih.gov/pubmed/25011346

Presentation and management of chronic pain

Rajapakse, D., et al.
Arch Dis Child 2014; 99(5): 474-480

Chronic pain is an important clinical problem affecting significant numbers of children and their families. The severity and impact of chronic pain on everyday function is shaped by the complex interaction of biological, psychological and social factors that determine the experience of pain for each individual, rather than a straightforward reflection of the severity of disease or extent of tissue damage. In this article we present the research findings that strongly support a biopsychosocial concept of chronic pain, describe the current best evidence for management strategies and suggest a common general pathway for all types of chronic pain. The principles of management of some of the most important or frequently encountered chronic pain problems in paediatric practice; neuropathic pain, complex regional pain syndrome (CRPS), musculoskeletal pain, abdominal pain and headache are also described.

http://www.ncbi.nlm.nih.gov/pubmed/24554056

Effect of pain in pediatric inherited neuropathies

Ramchandren, S., et al.
Neurology 2014; 82(9): 793-797

OBJECTIVE: Assess the prevalence and impact of pain in children with Charcot-Marie-Tooth (CMT) disease. METHODS: In this prospective cross-sectional study on children with CMT disease seen at study sites of the Inherited Neuropathy Consortium, we collected standardized assessments of pain (Wong-Baker FACES Pain Rating Scale) from 176 patients (140 children aged 8-18 years, and 36 children aged 2-7 years through parent proxies), along with standardized clinical assessments and quality-of-life (QOL) outcomes. We then developed a series of multivariate regression models to determine whether standardized measures of neuropathy severity, functional impact, or structural changes to the feet explained the observed pain scores. RESULTS: The mean score on the Wong-Baker FACES Pain Rating Scale was 2 (range 0-5). Increased pain strongly correlated with worse QOL scores but not with more severe neuropathy. Independent determinants of increased pain in children with CMT disease included measures of ankle inflexibility. CONCLUSION: Pain is present in children with CMT disease and negatively affects QOL. Pain scores do not positively correlate with neuropathy severity but do correlate in limited univariate analyses with measures of ankle inflexibility. Further studies to elucidate the mechanisms of pain may help identify treatments that can reduce pain and improve QOL in patients with CMT disease.

http://www.ncbi.nlm.nih.gov/pubmed/24477108

Delirium in the pediatric patient: On the growing awareness of its clinical interdisciplinary importance

Schieveld, J. N. and N. J. Janssen
JAMA Pediatr 2014; 168(7): 595-596

http://www.ncbi.nlm.nih.gov/pubmed/24797545

A review of rapid-onset opioids for breakthrough pain in patients with cancer

Simon, S. M. and L. S. Schwartzberg
J Opioid Manag 2014; 10(3): 207-215

Pain management in patients with cancer remains suboptimal. Breakthrough pain (BTP) is characterized by abrupt onset of severe pain in a background of otherwise stable managed pain and presents a substantial burden to patients, as it disrupts activities and quality of life. Rapid-onset opioids (ROOs), with an appropriate onset and duration of effect, provide new options for effective and well-tolerated management of BTP. All currently available ROOs are various formulations of transmucosal immediate-release fentanyl (TIRF) and, although they were originally developed and approved for use in children before painful procedures, are only approved for use in opioid-tolerant adult patients with cancer and BTP. The formulation options include oral lozenge, buccal tablet, buccal film, sublingual tablet, nasal spray, and a sublingual spray; each has practical considerations that vary with the product and route of administration. All have the common advantage of rapid entry into the systemic circulation via transmucosal absorption, avoiding hepatic and intestinal first-pass metabolism and allowing a rapid onset of action that rivals intravenous injections. Rapid onset and short duration of action allow good patient control of analgesia. The pharmacokinetic and analgesic properties of ROOs may allow reduction of the total opioid burden and associated adverse effects, while still providing effective pain relief. The shared TIRF risk evaluation and mitigation strategy program implemented in March 2012 has simplified enrollment and administration of these products to help mitigate the risks of abuse and misuse and to help ensure safe use in patients with cancer suffering from BTP.

http://www.ncbi.nlm.nih.gov/pubmed/24944071

Charting the territory: Symptoms and functional assessment in children with progressive, non-curable conditions

Steele, R., et al.
Arch Dis Child 2014; 99(8): 754-762

BACKGROUND: Children with progressive, non-curable genetic, metabolic, or neurological conditions require specialised care to enhance their quality of life. Prevention and relief of physical symptoms for these children needs to begin at diagnosis, yet, little is known about their patterns of symptoms and functional abilities. AIM: To describe these children's symptoms, as well as how the children's condition affects them physically. DESIGN: Cross-sectional, baseline results from an observational, longitudinal study, Charting the Territory, that followed 275 children and their families. SETTING/PARTICIPANTS: Seven tertiary care children's hospitals in Canada, 2 in the USA. Families were eligible based on the child's condition. A total of 275 children from 258 families participated. RESULTS: The 3 most common symptoms in these children were pain, sleep problems, and feeding difficulties; on average, they had 3.2 symptoms of concern. There was a pattern of under-reporting of children's symptoms for clinicians compared with parents. Regardless of use of associated medications, pain, feeding and constipation symptoms were often frequent and distressing. Children with a G/J tube had a higher total number of symptoms, and respiratory problems, pain, feeding difficulties and constipation were more likely to occur. They also tended to have frequent and distressing symptoms, and to need extensive mobility modifications which, in turn, were associated with higher numbers of symptoms. CONCLUSIONS: These children experience multiple symptoms that have been previously documented individually, but not collectively. Effective interventions are needed to reduce their symptom burden. Future longitudinal analyses will examine which disease-modifying interventions improve, or do not improve, symptom burden.

http://www.ncbi.nlm.nih.gov/pubmed/24833792

iCanCope with Pain: User-centred design of a web- and mobile-based self-management program for youth with chronic pain based on identified health care needs

Stinson, J. N., et al.

Pain Res Manag 2014; 19(5): 257-265

BACKGROUND: While there are emerging web-based self-management programs for children and adolescents with chronic pain, there is currently not an integrated web- and smartphone-based app that specifically addresses the needs of adolescents with chronic pain. OBJECTIVES: To conduct a needs assessment to inform the development of an online chronic pain self-management program for adolescents, called iCanCope with Pain. METHODS: A purposive sample of adolescents (n=23; 14 to 18 years of age) was recruited from two pediatric chronic pain clinics in Ontario. Interdisciplinary health care providers were also recruited from these sites. Three focus groups were conducted with adolescents (n=16) and one with pediatric health care providers (n=7). Individual adolescent interviews were also conducted (n=7). RESULTS: Qualitative analysis uncovered four major themes: pain impact; barriers to care; pain management strategies; and transition to adult care. Pain impacted social, emotional, physical and role functioning, as well as future goals. Barriers to care were revealed at the health care system, patient and societal levels. Pain management strategies included support systems, and pharmacological, physical and psychological approaches. Transition subthemes were: disconnect between pediatric and adult systems; skills development; parental role; and fearanxiety. Based on these identified needs, the iCanCope with Pain architecture will include the core theory-based functionalities of: symptom self-monitoring; personalized goal setting; pain coping skills training; peer-based social support; and chronic pain education. CONCLUSIONS: The proposed iCanCope with Pain program aims to address the self-management needs of adolescents with chronic pain by improving access to disease information, strategies to manage symptoms and social support.

http://www.ncbi.nlm.nih.gov/pubmed/25000507

Management of the acute painful crisis in sickle cell disease - a re-evaluation of the use of opioids in adult patients

Telfer, P., et al.

Br J Haematol 2014; 166(2): 157-164

Management of the acute painful crisis (APC) of sickle cell disease (SCD) remains unsatisfactory despite advances in the understanding and management of acute pain in other clinical settings. One reason for this is an unsophisticated approach to the use of opioid analgesics for pain management. This applies to haematologists who are responsible for developing acute sickle pain management protocols for their patients, and to health care staff in the acute care setting. The objective of this article is to evaluate the evidence for use of opioids in APC management. We have highlighted the possibilities for improving management by using alternatives to morphine, and intranasal (IN) or transmucosal routes of administration for rapid onset of analgesia in the emergency department (ED). We suggest how experience gained in managing acute sickle pain in children could be extrapolated to adolescents and young adults. We have also questioned whether patients given strong opioids in the acute setting are being safely monitored and what resources are required to ensure efficacy, safety and patient satisfaction. We also identify aspects of care where there are significant differences of opinion, which require further study by randomized controlled trial.

http://www.ncbi.nlm.nih.gov/pubmed/24750050

Neuroblastoma and pediatric delirium: A case series

Traube, C., et al.
Pediatr Blood Cancer 2014; 61(6): 1121-1123

Delirium occurs frequently in critically ill children, and children with neuroblastoma may be at particular risk. Early diagnosis and treatment may improve short- and long-term outcomes. In this case series, we present four critically ill children with neuroblastoma who were diagnosed with delirium in the post-operative period. In all four patients, the diagnosis of delirium facilitated targeted intervention and improvement. Heightened awareness by pediatric oncologists, surgeons, and intensivists may lead to earlier diagnosis and improvement in clinical outcomes.

http://www.ncbi.nlm.nih.gov/pubmed/24376154

Treatment of tetralogy of Fallot hypoxic spell with intranasal fentanyl

Tsze, D. S., et al.
Pediatrics 2014; 134(1): e266-269

We present the case of a 3-month-old girl who had unrepaired Tetralogy of Fallot who presented to the emergency department with an acute hypoxic episode. The patient was hyperpneic and cyanotic, with an initial oxygen saturation of 56%. She did not respond to knee-to-chest positioning. A single dose of intranasal fentanyl was administered with subsequent resolution of her symptoms and improvement of her oxygen saturation to 78% within 10 minutes. To our knowledge, this is the first report of the successful treatment of a hypoxic episode of Tetralogy of Fallot using intranasal fentanyl.

http://www.ncbi.nlm.nih.gov/pubmed/24936003

Mobile devices as adjunctive pain management tools

Wiederhold, B. K., et al.
Cyberpsychol Behav Soc Netw 2014; 17(6): 385-389

Approximately 108 million people in North America and Europe suffer from chronic pain. Virtual reality (VR) is a promising method for pain management in a clinical setting due to the distracting properties of an immersive virtual environment. In this study, we demonstrated the potential use of mobile phones as a means of delivering an easily accessible, immersive experience. Thirty-one patients tested VR pain distraction. Objective measurements of heart rate correlated to decreased anxiety, while, subjectively, patients also reported reduced levels of discomfort. The positive results of this study indicate that mobile phones can provide an immersive experience sufficient to deliver pain management distraction. Because mobile devices are widely available, the potential for developing pain management programs that are accessible has become a realistic possibility.

http://www.ncbi.nlm.nih.gov/pubmed/24892202

The development of social strengths in children with cerebral palsy

Adolfsson, M.
Dev Med Child Neurol 2014; 56(4): 300-301

http://www.ncbi.nlm.nih.gov/pubmed/21117883

Implementing motivational interviewing in a pediatric hospital

Apodaca, T. R., et al.
Mo Med 2014; 111(3): 212-216

Motivational Interviewing is a collaborative style of communication designed to strengthen a person's own motivation and commitment to change. We report on our ongoing efforts to implement motivational interviewing to address health behavior change among several patient populations in our pediatric hospital, including sexual risk reduction among adolescents, increased self-care for patients with spina bifida, increased adherence for adolescents with Type 1 diabetes, and facilitation with transition from pediatric to adult care among gastroenterology patients.

http://www.ncbi.nlm.nih.gov/pubmed/25011343

Keeping hope possible: A grounded theory study of the hope experience of parental caregivers who have children in treatment for cancer

Bally, J. M., et al.
Cancer Nurs 2014; 37(5): 363-372

BACKGROUND: Hope has been found to support parents as they care for their child with a life-limiting or life-threatening illness. However, very little research focuses on the nursing care of parents of pediatric oncology patients, and therefore, nurses may have difficulty in understanding and supporting parental well-being. OBJECTIVE: The purpose of this qualitative study was to gain an understanding of the experience of hope for parents who care for their child in treatment for cancer. METHODS: Using purposive theoretical sampling, 16 parents participated in this study. Thirty-three open-ended, face-to-face interviews were conducted, and 14 parent journals were collected. Analysis of the data was conducted using Charmaz's constructivist grounded theory approach. RESULTS: A developing, substantive grounded theory was constructed. Parental hope was described as an essential, powerful, deliberate, life-sustaining, dynamic, cyclical process that was anchored in time; was calming and strengthening; and provided inner guidance through the challenging experience of preparing for the worst and hoping for the best. Parents' main concern was "fearing the loss of hope," which was ameliorated by the basic social process of "keeping hope possible" through accepting reality, establishing control, restructuring hope, and pu rposive positive thinking. CONCLUSIONS: Parents journeyed through numerous transitions related to the treatment of cancer that caused feelings of uncertainty, anxiety, stress, and loss of control. Hope was identified as vital to parents. IMPLICATIONS FOR PRACTICE: To minimize these adverse experiences, nurses can support parents' ability to keep hope possible and thus to optimize their well-being by understanding, assessing, and supporting parental hope.

http://www.ncbi.nlm.nih.gov/pubmed/24145252

Dyadic Coping of Parents After the Death of a Child

Bergstraesser, E., et al.
Death Stud 2014

This article explores the grief process of parents following the death of a child due to a life-limiting illness, putting particular focus on dyadic coping. Participants included 46 married parents (23 couples). A mixed methods design was used with in-depth interviews and standardized questionnaires. All parents were interviewed separately. Aspects of common dyadic coping (e.g., sharing emotions, or maintaining bonds to the child) helped them work through their grief as a couple but also individually. We conclude that dyadic coping plays an important role in grief work and adjustment to bereavement.

http://www.ncbi.nlm.nih.gov/pubmed/25204680

Parents bereaved by infant death: Sex differences and moderation in PTSD, attachment, coping and social support

Christiansen, D. M., et al.
Gen Hosp Psychiatry 2014

OBJECTIVE: Parents bereaved by infant death experience a wide range of symptomatology, including posttraumatic stress disorder (PTSD) that may persist for years after the loss. Little research has been conducted on PTSD in fathers who have lost an infant. Mothers report most symptoms to a greater extent than fathers, but not much is known about other sex differences following infant death. METHOD: The present cross-sectional study examined sex differences in PTSD and sex differences in the relationship between PTSD severity and related variables. Subjects were 361 mothers and 273 fathers who had lost an infant either late in pregnancy, during birth or in the first year of life. Participants filled out questionnaires between 1.2 months and 18 years after the loss (M=3.4 years). RESULTS: Mothers reported significantly more PTSD symptoms, attachment anxiety, emotion-focused coping and feeling let down, but significantly lower levels of attachment avoidance than fathers. Attachment anxiety, attachment avoidance and emotion-focused coping were significantly more strongly associated with PTSD severity in mothers than fathers, but only when examined alone. When all variables and time since the loss were examined together, there were no longer any significant moderation effects of sex. CONCLUSIONS: Persistent posttraumatic symptomatology exists in both mothers and fathers long after the loss. There are several sex differences in severity and correlates of PTSD, and a few moderation effects were identified for attachment and emotion-focused coping. Overall, more similarities than differences were found between mothers and fathers in the associations between PTSD and covariates.

http://www.ncbi.nlm.nih.gov/pubmed/25218784

Exploring the Perceived Met and Unmet Need of Life-Limited Children, Young People and Families

Coad, J., et al.
J Pediatr Nurs 2014

This article presents an original study commissioned by the UK charity, Together for Short Lives which explored children and young people up to 25years of age with life-threatening/limiting conditions and their families. Using Appreciative Inquiry and framework analysis, qualitative work sought to explore perceived met and unmet needs of services and care. Fifty-one families were interviewed from one UK area, 18 of which were children/young people up to 25years old. Findings indicated that children and their families felt medical/nursing needs were well met but provision was needed for broader financial, social and emotional support alongside more responsive specialist therapies.

http://www.ncbi.nlm.nih.gov/pubmed/25301028

Griefwork online: Perinatal loss, lifecourse disruption and online support

Davidson, D. and G. Letherby
Hum Fertil (Camb) 2014; 17(3): 214-217

The Internet provides new opportunities for accessing and giving support following perinatal loss and in this article we report on a project concerned to explore the use of social networking and online networks following such an experience. Perinatal loss can be defined and perceived as biographical disruption yet this type of loss sometimes lacks social recognition. Our ethnographic study reveals that not only do mothers, and sometimes fathers and grandmothers, seek support on the Internet but they also engage in griefwork (the work the bereaved do with others).

http://www.ncbi.nlm.nih.gov/pubmed/25122092

Cultural influences in pediatric cancer from diagnosis to cure/end of life

Gray, W. N., et al.
J Pediatr Oncol Nurs 2014; 31(5): 252-271

OBJECTIVE: To review the literature on cultural factors influencing clinical care and family management of pediatric cancer. METHODS: A literature review including 72 articles related to cultural issues in pediatric cancer was conducted. Information was organized around several clinically driven themes. RESULTS: Cultural factors influenced many aspects of the cancer experience including illness representations, reaction to diagnosis, illness disclosure patterns, complementary and alternative medicine use, management of medical procedures, coping strategies, and end of life issues. CONCLUSION: Increased awareness of cultural factors is needed to improve clinical care and reduce health disparities. Specific strategies to approach cultural differences are provided to enhance patient and family care from diagnosis to cure/end of life.

http://www.ncbi.nlm.nih.gov/pubmed/25299000

De-escalation of therapy for pediatric medulloblastoma: Trade-offs between quality of life and survival

Henrich, N., et al.

Pediatr Blood Cancer 2014; 61(7): 1300-1304

BACKGROUND: Treatment intensity for pediatric medulloblastoma may vary depending on the type of medulloblastoma. In some cases, the dose of radiation may be reduced or eliminated. Correspondingly, there may be trade-offs between quality of life and survival. In this study, focus groups were conducted with parents and clinicians to explore their opinions about these trade-offs as well as the alignment/misalignment between parents and clinicians regarding the trade-offs. METHODS: One hour semi-structured focus groups were conducted with parents of children with medulloblastoma and health care providers who were involved in the care of these children. RESULTS: Parents and providers showed differences in which factors they believe have the greatest impact on quality of life for children with medulloblastoma and their families. For parents, the most important factor is social functioning and their child's ability to make friends and have a social life. In contrast, providers thought that parents cared most about their child's cognitive functioning and ability to attend and perform in school. CONCLUSION: Understanding parents' perspectives on quality of life is important in terms of providing support services that target the areas that the parents prioritize. The types of functioning that are most strongly correlated with quality of life from the parents' perspective may be the ones that should be targeted to protect during treatment. Pediatr Blood Cancer 2014;61:1300-1304. (c) 2014 Wiley Periodicals, Inc.

http://www.ncbi.nlm.nih.gov/pubmed/24616367

Primary pediatric palliative care: Psychological and social support for children and families

Hirsh, C. D. and S. Friebert

Pediatr Rev 2014; 35(9): 390-395

http://www.ncbi.nlm.nih.gov/pubmed/25183774

Parent perspectives on family-based psychosocial interventions in pediatric cancer: A mixed-methods approach

Hocking, M. C., et al.

Support Care Cancer 2014; 22(5): 1287-1294

Family-based interventions in pediatric cancer face challenges associated with integrating psychosocial care into a period of intensive treatment and escalating stress. Little research has sought input from parents on the role of interventions delivered shortly after diagnosis. This mixed-methods study obtained parents' perspectives on the potential role of family-based interventions. Twenty-five parents provided feedback on the structure and timing of psychosocial interventions via focus groups and a questionnaire. Qualitative analyses resulted in three themes that were illustrative of a traumatic stress framework: (1) tension between focusing on child with cancer and addressing other family needs, (2) factors influencing parents' perception of a shared experience with other parents, and (3) the importance of matching interventions to the trajectory of parent adjustment. Quantitative data indicated that parents preferred intervention within 6 months of diagnosis, with almost half favoring within 2 months of diagnosis, and the majority wanted interventions targeted to parents only. Qualitative themes highlight the importance of using a traumatic stress framework to inform the development of family-based interventions for those affected by pediatric cancer.

http://www.ncbi.nlm.nih.gov/pubmed/24337762

Predictors of psychological functioning in children with cancer: Disposition and cumulative life stressors

Howard Sharp, K. M., et al.

Psychooncology 2014

OBJECTIVE: This study examined psychological functioning in children with a history of cancer and a matched sample of healthy peers, while exploring the roles of disposition and stressful life events. METHOD: Participants were 255 children with a history of cancer and 101 demographically matched children (8-17 years). Children completed measures of depression, anxiety, and posttraumatic stress symptoms (PTSS); history of stressful life events; and dispositional factors, including optimism and a five-factor personality measure. RESULTS: Children with cancer did not differ from peers with regard to depression and PTSS, but reported significantly lower anxiety. In hierarchical regressions, children's depression, anxiety, and PTSS scores were largely predicted by dispositional variables and, to a lesser extent, stressful life events, after controlling for demographics and health status. CONCLUSION: Children's psychological functioning is predicted primarily by disposition, and secondarily by history of stressful life events, with health status (i.e., cancer versus control) accounting for minimal, and often non-significant variance in children's functioning. These findings further support that children with cancer are generally resilient, with factors predictive of their adjustment difficulties mirroring those of children without history of serious illness. Copyright (c) 2014 John Wiley & Sons, Ltd.

http://www.ncbi.nlm.nih.gov/pubmed/25132111

On the Child's Own Initiative: Parents Communicate With Their Dying Child About Death

Jalmsell, L., et al.
Death Stud 2014

Open and honest communication has been identified as an important factor in providing good palliative care. However, there is no easy solution to if, when and how parents and a dying child should communicate about death. This paper reports how bereaved parents communicated about death with their child, dying from a malignancy. Communication was often initiated by the child and included communication through narratives such as fairy-tales and movies and talking more directly about death itself. Parents also reported that their child prepared for death by giving instructions about his or her grave or funeral and giving away toys.

http://www.ncbi.nlm.nih.gov/pubmed/25153166

Children's experiences of cystic fibrosis: a systematic review of qualitative studies

Jamieson, N., et al.
Pediatrics 2014; 133(6): e1683-1697

BACKGROUND AND OBJECTIVE: Cystic fibrosis (CF) is a common life-shortening genetic disease and is associated with poor psychosocial and quality of life outcomes. The objective of this study was to describe the experiences and perspectives of children and adolescents with CF to direct care toward areas that patients regard as important. METHODS: MEDLINE, Embase, PsycINFO, and Cumulative Index to Nursing and Allied Health Literature were searched from inception to April 2013. We used thematic synthesis to analyze the findings. RESULTS: Forty-three articles involving 729 participants aged from 4 to 21 years across 10 countries were included. We identified 6 themes: gaining resilience (accelerated maturity and taking responsibility, acceptance of prognosis, regaining control, redefining normality, social support), lifestyle restriction (limited independence, social isolation, falling behind, physical incapacity), resentment of chronic treatment (disempowerment in health management, unrelenting and exhausting therapy, inescapable illness), temporal limitations (taking risks, setting achievable goals, valuing time), emotional vulnerability (being a burden, heightened self-consciousness, financial strain, losing ground, overwhelmed by transition), and transplant expectations and uncertainty (confirmation of disease severity, consequential timeliness, hope and optimism). CONCLUSIONS: Adolescents and children with CF report a sense of vulnerability, loss of independence and opportunities, isolation, and disempowerment. This reinforces the importance of the current model of multidisciplinary patient-centered care that promotes shared decision-making, control and self-efficacy in treatment management, educational and vocational opportunities, and physical and social functioning, which can lead to optimal treatment, health, and quality of life outcomes.

http://www.ncbi.nlm.nih.gov/pubmed/24843053

Extended family support for parents faced with life-support decisions for extremely premature infants

Kavanaugh, K., et al.

Neonatal Netw 2014; 33(5): 255-262

PURPOSE: To outline parents' descriptions of extended family involvement and support surrounding decision making for their extremely preterm infant. DESIGN: Collective case study design in a prospective, descriptive, longitudinal research. Seventy-five digitally recorded interviews were done with parents before and after the birth. SAMPLE: Fifty-four parents (40 mothers, 14 fathers). MAIN OUTCOME VARIABLE: Categories of family involvement and support in the parents' decision making. RESULTS: Most parents did not seek advice from family members for life-support decisions made prenatally. Instead, parents made the decision as a couple with their physician without seeking family input. Family members provided certain types of support: emotional support, advice and information, prayer, and instrumental help such as child care. Most parents described at least one way their family supported them. For postnatal and end-of-life decisions, parents were more likely to seek advice from extended family in addition to the other forms of support.

http://www.ncbi.nlm.nih.gov/pubmed/25161133

Social competence in children with brain disorders: A meta-analytic review

Kok, T. B., et al.

Neuropsychol Rev 2014; 24(2): 219-235

Social competence, i.e. appropriate or effective social functioning, is an important determinant of quality of life. Social competence consists of social skills, social performance and social adjustment. The current paper reviews social skills, in particular emotion recognition performance and its relationship with social adjustment in children with brain disorders. In this review, normal development and the neuro-anatomical correlates of emotion recognition in both healthy children and adults and in various groups of children with brain disorders, will be discussed. A systematic literature search conducted on PubMed, yielded nine papers. Emotion recognition tasks were categorized on the basis of task design and emotional categories to ensure optimal comparison across studies before an explorative meta-analysis was conducted. This meta-analytic review suggests that children with brain disorders show impaired emotion recognition, with the recognition of sad and fearful expressions being most impaired. Performance did not seem to be related to derivative measures of social adjustment. Despite the limited number of studies on a variety of brain disorders and control groups, outcomes were quite consistent across analyses and corresponded largely with the existing literature on development of emotion recognition in typically developing children. More longitudinal prospective studies on emotion recognition are needed to gain insight into recovery and subsequent development of children with distinct brain disorders. This will aid development, selection and implementation of interventions for improvement of social competence and quality of life in children with a brain disorder.

http://www.ncbi.nlm.nih.gov/pubmed/24648014

Attachment Style Dimensions Can Affect Prolonged Grief Risk in Caregivers of Terminally Ill Patients With Cancer

Lai, C., et al.

Am J Hosp Palliat Care 2014

OBJECTIVE: The aim of the present study was to evaluate the predictive role of attachment dimensions on the risk of prolonged grief. Sixty caregivers of 51 terminally ill patients with cancer who had been admitted in a hospice were selected. METHODS: Caregivers were interviewed using Attachment Scale Questionnaire, Hamilton Depression Rating Scale, Hamilton Anxiety Rating Scale, and Prolonged Grief Disorder 12 (PG-12). RESULTS: The consort caregivers showed higher PG-12 level compared to the sibling caregivers. Anxiety, depression, need for approval, and preoccupation with relationships levels were significantly correlated with PG-12 scores. CONCLUSION: Female gender, high levels of depression, and preoccupation with relationships significantly predicted higher levels of prolonged grief risk.

http://www.ncbi.nlm.nih.gov/pubmed/25155029

Parent-reported cognition of children with cancer and its potential clinical usefulness

Lai, J. S., et al.

Qual Life Res 2014; 23(4): 1049-1058

PURPOSE: Cognitive dysfunction is a common concern for children with brain tumors (BTs) or those receiving central nervous system (CNS) toxic cancer treatments. Perceived cognitive function (PCF) is an economical screening that may be used to trigger full, formal cognitive testing. We assessed the potential clinical utility of PCF by comparing parent-reported scores for children with cancer with scores from the general US population. METHODS: Children (n = 515; mean age = 13.5 years; 57.0 % male) and one of their parents were recruited from pediatric oncology clinics. Most children (53.3 %) had a diagnosis of CNS tumor with an average time since diagnosis of 5.6 years. PCF was evaluated using the pediatric PCF item bank (pedsPCF), which was developed and normed on a sample drawn from the US general pediatric population. Children also completed computer-based neuropsychological tests. We tested relationships between PCF and clinical variables. Differential item functioning (DIF) was used to evaluate measurement bias between the samples. RESULTS: No item showed DIF, supporting the use of pedsPCF in the cancer sample. PedsPCF differentiated children with (vs. without) a BT, $p < 0.01$, and groups defined by years since diagnosis, $p < 0.01$. It significantly ($p < 0.05$) correlated with computerized neuropsychological tests in 40 of 60 comparisons. Children with BTs were rated as having worse pedsPCF scores than the norm, regardless of years since diagnosis. CONCLUSIONS: PCF significantly differentiated cancer survivors with various clinical characteristics. It is brief and easy to implement. PCF should be considered for routine care of pediatric cancer survivors.

http://www.ncbi.nlm.nih.gov/pubmed/24197478

Meeting the needs of siblings of children with life-limiting illnesses

Lane, C. and J. Mason

Nurs Child Young People 2014; 26(3): 16-20

Siblings of children with life-threatening or life-limiting illnesses can face a number of challenges, yet this is a group that is often unacknowledged as needing specific support. It is essential that the needs of siblings are recognised and addressed as part of a family-centred approach. This article discusses the experiences and challenges faced by siblings in such families and what children's nurses can do to help. In particular, it outlines a group intervention offered by a community children's palliative care service.

http://www.ncbi.nlm.nih.gov/pubmed/24708334

Pediatric advance directives: Parents' knowledge, experience, and preferences

Liberman, D. B., et al.

Pediatrics 2014; 134(2): e436-443

OBJECTIVES: To explore parents' and caregivers' experience, knowledge, and preferences regarding advance directives (ADs) for children who have chronic illness. METHODS: We conducted a prospective, cross-sectional survey of parents and caregivers of children who have chronic illness. During ambulatory medical visits, participants were asked about previous AD experience and knowledge, future preferences regarding AD discussions, their child's past and current health status, and family demographics. RESULTS: Among 307 participants surveyed, previous AD experience was low, with 117 (38.1%) having heard of an AD, 54 (17.6%) having discussed one, and 77 (25.1%) having known someone who had an AD. Furthermore, 27 (8.8%) participants had an AD or living will of their own, and 8 (2.6%) reported that their chronically ill child had an AD. Previous AD knowledge was significantly more likely among parents and caregivers who had a college degree than those who did not have a high school diploma, yet significantly less likely among primarily Spanish-speaking parents and caregivers than those primarily English-speaking. Interest in creating an AD for the child was reported by 151 (49.2%) participants, and was significantly more likely among families who had more frequent emergency department visits over the previous year. CONCLUSIONS: The limited AD experience and knowledge of parents and caregivers of children who have chronic illness and their interest in creating an AD suggest an unmet need among families of children who have chronic illness, and an opportunity to enhance communication between families and medical teams regarding ADs and end-of-life care.

http://www.ncbi.nlm.nih.gov/pubmed/25002672

A relational understanding of sibling experiences of children with rare life-limiting conditions: Findings from a qualitative study

Malcolm, C., et al.

J Child Health Care 2014; 18(3): 230-240

Mucopolysaccharidoses (MPS) and Batten disease are rare life-limiting conditions (LLCs) characterised by progressive and permanent physical and cognitive decline. The impact of such conditions on families, and notably on siblings, has not yet been described or documented. This paper presents data from a UK-wide study that sought to understand the family experience of supporting a child with the rare degenerative LLCs of MPS and Batten disease. The aim of this paper is to report sibling experiences related to these rare degenerative and progressive conditions, in order to inform the future development of supportive interventions. Eight siblings of children with MPS (n = 7) and Batten Disease (n = 1) participated in semi-structured qualitative interviews. A card sort technique was utilised to support and engage the children. Siblings are clearly impacted emotionally, pragmatically and relationally by the ill health of another child in the family. The data indicate four key themes which demonstrate impacts on siblings: perceptions of the condition and its symptoms, impact on daily life, emotional consequences and ways of coping. Siblings often had considerable knowledge of the condition and took on important roles in symptom management. However, these experiences were in the context of managing relationships within the family (often protecting parents from an awareness of how much they knew) and relationships at school (including distraction from learning and being bullied by peers). The data highlight how sibling experiences are generated through a combination of negative disability discourses and support through peers and family members. The data indicate how these features shift as a consequence of witnessing the advancement of their brother's or sister's condition and the emotional sequelae of disease progression. Exploration of siblings' experiences of living with such rare progressive and degenerative LLCs suggest the focus of interventions to support this group should address their emotional health and ways to overcome isolation and build connections with other siblings who share their unique experiences. Critically, the data suggest that sibling support should be cognisant of the trajectory of the illness as well as the family, school and peer relational contexts that siblings inhabit.

http://www.ncbi.nlm.nih.gov/pubmed/23754839

Who listens to parents and is anything done?

Mander, R.

Pract Midwife 2014; 17(7): 24-25

The Listening to parents survey, which took place in early 2014, sought the views of parents bereaved by stillbirth or early neonatal death. The findings showed the marked variation in care provided. The low response rate may call into question the authority of the findings Research into perinatal loss seems to be unlikely to be operationalised. More qualitative research may make such implementation more feasible, hopefully making optimal care more certain for grieving parents.

http://www.ncbi.nlm.nih.gov/pubmed/25109072

Complicated grief and depression in young adults: Personality and relationship quality

Mash, H. B., et al.

J Nerv Ment Dis 2014; 202(7): 539-543

Young adults experience problematic responses to loss more often than is commonly recognized. Few empirical studies have examined the contribution of intrapersonal and interpersonal characteristics to grief and depression in bereaved young adults. This study investigated the association of dependency and quality of the relationship with the deceased (i.e., depth and conflict) with complicated grief (CG) and depression. Participants were 157 young adults aged 17 to 29 years who experienced loss of a family member or close friend within the past 3 years (mean = 1.74 years). Participants completed the Inventory of Complicated Grief, Beck Depression Inventory, Depth and Conflict subscales of the Quality of Relationships Inventory, and the Dependency subscale of the Depressive Experiences Questionnaire. Relationships among dependency and interpersonal depth and conflict and CG and depression were examined through analyses of covariance. Sixteen percent of participants met criteria for CG and 34% had mild to severe depression. Dependency and depth were independently related to CG and dependency was related to depression, but the pattern of associations was somewhat different for each outcome. Greater depth was associated with CG, at both high and low levels of dependency. High levels of dependency were related to more depressive symptoms. Interpretation of the findings is limited by the relatively small sample size and cross-sectional design. CG and depression are related but distinct responses to loss. Although dependency is associated with both CG and depression after loss, relationships between the bereaved and deceased that are characterized by high levels of depth are particularly related to the development of CG symptoms.

http://www.ncbi.nlm.nih.gov/pubmed/24921421

The role of relationship biography in advance care planning

Moorman, S. M., et al.

J Aging Health 2014; 26(6): 969-992

OBJECTIVE: We examine the ways that romantic relationship biographies are related to whether, how, and with whom individuals complete advance care planning (ACP), preparations for end-of-life medical care. METHOD: Data are from an Internet survey of 2,144 adults aged 18 to 64, all of whom were either married to or cohabiting with an opposite-sex partner. RESULTS: Cohabitors were less likely than married people to complete ACP. Relationship quality was an important influence on ACP, but did not account for the differences between married and cohabiting persons. Differences were largely explained by the age composition of the groups. DISCUSSION: Couples who foresee a long and stable future together are those most likely to engage in end-of-life planning, a preventative health behavior with long-term consequences for well-being.

http://www.ncbi.nlm.nih.gov/pubmed/24891562

A prospective study of anxiety, depression, and behavioral changes in the first year after a diagnosis of childhood acute lymphoblastic leukemia: A report from the Children's Oncology Group

Myers, R. M., et al.

Cancer 2014; 120(9): 1417-1425

BACKGROUND: The authors prospectively assessed anxiety, depression, and behavior in children with standard-risk acute lymphoblastic leukemia (SR-ALL) during the first year of therapy and identified associated risk factors. METHODS: A cohort study was performed of 159 children (aged 2 years-9.99 years) with SR-ALL who were enrolled on Children's Oncology Group protocol AALL0331 at 31 sites. Parents completed the Behavior Assessment System for Children, the General Functioning Scale of the Family Assessment Device, and the Coping Health Inventory for Parents at approximately 1, 6, and 12 months after diagnosis. RESULTS: Overall, mean scores for anxiety, depression, aggression, and hyperactivity were similar to population norms. However, more children scored in the at-risk/clinical range for depression than the expected 15% at 1 month (21.7%; P= .022), 6 months (28.6%; P< .001), and 12 months (21.1%; P= .032). For anxiety, more children scored in the at-risk/clinical range at 1 month (25.2% vs 15%; P= .001), but then reverted to expected levels. On adjusted analysis, unhealthy family functioning was found to be predictive of anxiety (odds ratio [OR], 2.24; P= .033) and depression (OR, 2.40; P= .008). Hispanic ethnicity was associated with anxiety (OR, 3.35; P= .009). Worse physical functioning (P= .049), unmarried parents (P= .017), and less reliance on social support (P= .004) were found to be associated with depression. Emotional distress at 1 month predicted anxiety (OR, 7.11; P= .002) and depression (OR, 3.31; P= .023) at 12 months. CONCLUSIONS: Anxiety is a significant problem in a subpopulation of patients with SR-ALL immediately after diagnosis, whereas depression remains a significant problem for at least 1 year. Children of Hispanic ethnicity or those with unhealthy family functioning may be particularly vulnerable. These data suggest that clinicians should screen for anxiety and depression throughout the first year of therapy.

http://www.ncbi.nlm.nih.gov/pubmed/24473774

Factor structure of quality of life in adolescents

Oles, M.

Psychol Rep 2014; 114(3): 927-946

The goal was to present the factor structure of subjective quality of life in adolescents, investigated by means of four questionnaires: the Youth Quality of Life-Research Version (YQOL-R), the Quality of Life Profile-Adolescent Version (QOLP-AV), the KIDSCREEN-52 Questionnaire, and the Quality of Life Questionnaire for Children and Adolescents (QLQ-CA). Two exploratory factor analyses conducted on the results obtained from two samples of adolescents: healthy, N = 252 (144 girls, 108 boys), and chronically ill, suffering from several illnesses, N = 189 (118 girls, 71 boys). Both factor analyses revealed four-factor solutions, each explaining about 60% of the total variance. The factor structure for the healthy group approximately reproduced the structures of the four questionnaires: Developmental quality of life (23%), Health and Well-being (16%), Relational quality of life (14%), and Ego strength (8%). The factor structure for the chronically ill group was similar for three factors: Developmental quality of life (22%), Harmony between the self and the environment (14%), and Coping and Support (12%), but different for another one: Health-related quality of life (10%). The discussion focuses on the specific nature of four aspects of quality of life observed in the healthy sample and their similarities to and differences from the factors in the chronic patients' sample.

http://www.ncbi.nlm.nih.gov/pubmed/25074312

Palliative Care in Neonatal Intensive Care, Effects on Parent Stress and Satisfaction: A Feasibility Study

Petteys, A. R., et al.

Am J Hosp Palliat Care 2014

CONTEXT: Approximately 1 in 10 infants require neonatal intensive care unit (NICU) hospitalization, which causes parental stress. Palliative care (PC) provides an opportunity to alleviate suffering and stress. OBJECTIVES: This study examines the effects of PC on NICU parent stress and satisfaction. METHODS: A prospective cohort design compares stress and satisfaction among families receiving or not receiving PC. RESULTS: No significant differences in stress scores were found (P = .27-1.00). Palliative care parents (100%) were more likely to report being "extremely satisfied" with care than usual-care parents (50%). CONCLUSION: This study supports the feasibility of evaluating NICU PC services. Infants referred for PC typically have higher morbidity/mortality; therefore, higher parental stress scores may be expected. Stress levels were similar in both cohorts, thus PC did not increase stress and may decrease PC parent stress.

http://www.ncbi.nlm.nih.gov/pubmed/25228642

Resilience and psychosocial outcomes in parents of children with cancer

Rosenberg, A. R., et al.

Pediatr Blood Cancer 2014; 61(3): 552-557

BACKGROUND: The psychosocial function of parents of children with cancer can impact the well-being of the entire family. Resilience resources are likely related to psychosocial outcomes and may be amenable to intervention. We hypothesized that parents with lower resources would report worse outcomes. METHODS: In the "Understanding Resilience in Parents of Children with Cancer" study, comprehensive surveys were mailed to consecutive, English-speaking parents of children with cancer who were treated at Seattle Children's Hospital and completed therapy between January 1, 2009 and December 31, 2010. Resilience resources were measured by the Connor-Davidson Resilience Scale; outcome measures included psychological distress, health-related behaviors, social and family function, and perceived communication with the medical team. RESULTS: Ninety-six parents (86% of contactable) completed the survey. Compared to population norms, enrolled parents had lower resilience resources, higher psychological distress, and more commonly reported binge drinking. Conversely, they reported higher social support and family adaptability (P < 0.001-0.006). Lower resilience resources were associated with higher distress, lower social support, and lower family function (P < 0.001-0.007). Parents in the lowest quartile of resilience resources had higher odds of frequent sleep difficulties (OR 5.19, 95% CI 1.74,15.45), lower health satisfaction (OR 5.71, 95% CI 2.05,15.92), and decreased ability to express worries to the medical team (OR 4.00, 95% CI 1.43,11.18). CONCLUSIONS: Parents of children with cancer are at risk for poor psychosocial outcomes and those with low resilience resources may be at greater risk. Interventions directed at promoting resilience resources may provide a novel and complimentary approach toward improving outcomes for families facing pediatric cancer.

http://www.ncbi.nlm.nih.gov/pubmed/24249426

The psychological skeleton in the closet: Mortality after a sibling's suicide

Rostila, M., et al.

Soc Psychiatry Psychiatr Epidemio 2014;I 49(6): 919-927

PURPOSE: To study the association between loss of an adult sibling due to suicide and mortality from various causes up to 18 years after bereavement. METHODS: We conducted a follow-up study between 1981 and 2002, based on register data representing the total population of Swedes aged 25-64 years (n = 1,748,069). RESULTS: An elevated mortality rate from all causes was found among men (RR 1.26; 95 % CI: 1.14-1.40) and women (1.27; 1.11-1.45) who had experienced a sibling's suicide. The standardized rate ratio of suicide of bereaved to non-bereaved persons was 2.46 (1.86-3.24) among men and 3.25 (2.28-4.65) among women. We also found some indications of an interrelation between sibling suicide and subsequent deaths from external causes other than suicide in men (1.77; 1.34-2.34) and deaths from cardiovascular disease in women (1.37; 0.99-1.91). An elevated all-cause mortality rate was found after the first year of bereavement in men, while bereaved women experienced higher mortality rates during the first 2 years and after 5 years of bereavement. CONCLUSIONS: Our study provides support for adverse health effects among survivors associated with sibling loss due to suicide. Sibling suicides were primarily associated with suicide in bereaved survivors, although there was an increased mortality rate from discordant causes, which strengthens the possibility that the observed associations might not be entirely due to shared genetic causes.

http://www.ncbi.nlm.nih.gov/pubmed/24126558

Caregiver coping, mental health and child problem behaviours in cystic fibrosis: A cross-sectional study

Sheehan, J., et al.

Int J Behav Med 2014; 21(2): 211-220

BACKGROUND: In children with cystic fibrosis (CF) sleep, eating/mealtime, physiotherapy adherence and internalising problems are common. Caregivers also often report elevated depression, anxiety and stress symptoms. PURPOSE: To identify, through principal components analysis (PCA), coping strategies used by Australian caregivers of children with CF and to assess the relationship between the derived coping components, caregiver mental health symptoms and child treatment related and non-treatment related problem behaviours. METHOD: One hundred and two caregivers of children aged 3 to 8 years from three CF clinic sites in Australia, completed self-report questionnaires about their coping and mental health and reported on their child's sleep, eating/mealtime, treatment adherence and internalising and externalising behaviours. RESULTS: Two caregiver coping components were derived from the PCA: labelled 'proactive' and 'avoidant' coping. 'Avoidant' coping correlated moderately with caregiver depression (0.52), anxiety (0.57) and stress (0.55). For each unit increase in caregiver use of avoidant coping strategies, the odds of frequent child eating/mealtime behaviour problems increased by 1.3 (adjusted 95 % CI 1.0 to 1.6, p = .03) as did the odds of children experiencing borderline/clinical internalising behaviour problems (adjusted 95 % CI 1.1 to 1.7, p = .01). Proactive coping strategies were not associated with reduced odds of any child problem behaviours. CONCLUSIONS: Avoidant coping strategies correlated with caregiver mental health and child problem behaviours. Intervening with caregiver coping may be a way to improve both caregiver mental health and child problem behaviours in pre-school and early school age children with CF.

http://www.ncbi.nlm.nih.gov/pubmed/23325547

Giving hope to families in palliative care and implications for practice

Smith, H.

Nurs Child Young People 2014; 26(5): 21-25

Caring for a dying child and the family is one of the greatest nursing challenges. The way in which care is delivered will shape the experience they are about to face. Hope plays a crucial role in helping people cope, and healthcare professionals can foster appropriate hopes ethically, while maintaining open and honest communication. If palliative care is discussed with clients and families from the time of diagnosis, they can face realistic decisions better and not feel that they are 'giving up'. They need to know that everything possible is being done to improve the quality of the time left to them.

http://www.ncbi.nlm.nih.gov/pubmed/24914668

Knowledge, attitudes, and practices related to pet contact by immunocompromised children with cancer and immunocompetent children with diabetes

Stull, J. W., et al.

J Pediatr 2014; 165(2): 348-355 e342

OBJECTIVE: To compare knowledge, attitudes, and risks related to pet contact in households with and without immunocompromised children. STUDY DESIGN: A questionnaire was distributed to parents of children diagnosed with cancer (immunocompromised; n=80) or diabetes (immunocompetent; n=251) receiving care at the Children's Hospital of Eastern Ontario. Information was collected on knowledge of pets as sources of disease, concerns regarding pet-derived pathogens, and pet ownership practices. Data were analyzed with multivariable logistic regression. RESULTS: The questionnaire was completed by 65% (214 of 331) of the individuals to whom it was given. Pet ownership was common; 45% of respondents had a household pet when their child was diagnosed, and many (households with a child with diabetes, 49%; households with a child with cancer, 20%) acquired a new pet after diagnosis. Most households that obtained a new pet had acquired a pet considered high risk for infectious disease based on species/age (diabetes, 73%; cancer, 77%). Parents of children with cancer were more likely than parents of children with diabetes to recall being asked by a physician/staff member if they owned a pet (OR, 5.9) or to recall receiving zoonotic disease information (OR, 5.3), yet these interactions were reported uncommonly (diabetes, </=13%; cancer, </=48%). Greater knowledge of pet-associated pathogens was associated with recalled receipt of previous education on this topic (OR, 3.9). Pet exposure outside the home was reported frequently for children in non-pet-owning households (diabetes, 48%; cancer, 25%). CONCLUSION: Improved zoonotic disease education is needed for pet-owning and non-pet-owning households with immunocompromised children, with ongoing provision of information while the children are at increased risk of disease. Additional efforts from pediatric and veterinary healthcare professionals are required.

http://www.ncbi.nlm.nih.gov/pubmed/24928703

What parents want from doctors in end-of-life decision-making for children

Sullivan, J., et al.

Arch Dis Child 2014; 99(3): 216-220

OBJECTIVE: End-of-life decision-making is difficult for everyone involved, as many studies have shown. Within this complexity, there has been little information on how parents see the role of doctors in end-of-life decision-making for children. This study aimed to examine parents' views and experiences of end-of-life decision-making. DESIGN: A qualitative method with a semistructured interview design was used. SETTING: Parent participants were living in the community. PARTICIPANTS: Twenty-five bereaved parents. MAIN OUTCOMES: Parents reported varying roles taken by doctors: being the provider of information without opinion; giving information and advice as to the decision that should be taken; and seemingly being the decision maker for the child. The majority of parents found their child's doctor enabled them to be the ultimate decision maker for their child, which was what they very clearly wanted to be, and consequently enabled them to exercise their parental autonomy. Parents found it problematic when doctors took over decision-making. A less frequently reported, yet significant role for doctors was to affirm decisions after they had been made by parents. Other important aspects of the doctor's role were to provide follow-up support and referral. CONCLUSIONS: Understanding the role that doctors take in end-of-life decisions, and the subsequent impact of that role from the perspective of parents can form the basis of better informed clinical practice.

http://www.ncbi.nlm.nih.gov/pubmed/24644205

Support requirements of parents caring for a child with disability and complex health needs

Whiting, M.

Nurs Child Young People 2014; 26(4): 24-27

AIM: To investigate the experiences of parents of children with complex health needs in relation to the help and support they receive when caring for their child. METHOD: A series of in-depth semi-structured interviews undertaken with the parents of 34 children (33 families) with a disability or a complex health need. Families were categorised into one of three subgroups: children with a disability, children with a life-limiting or life-threatening illness, or children with technology dependence. FINDINGS: In relation to parental experience of the need for help and support, two major categories were identified, namely 'people', and 'processes and resources', as well as a series of subcategories. Respite care was identified as the greatest unmet need. CONCLUSION: Parents identified a range of helping behaviours among key professional staff involved in support provision. The greatest area of unmet needs is for respite care.

http://www.ncbi.nlm.nih.gov/pubmed/24805033

Children with disability and complex health needs: The impact on family life

Whiting, M.

Nurs Child Young People 2014; 26(3): 26-30

AIM: To identify consistency and differences in parental perceptions of impact, need for support and 'sense making' in children with a disability, children with a life-threatening or life-limiting illness and children who are technology dependent. METHOD: A series of in-depth semi-structured interviews were undertaken with parents from 33 families that included one or more child with disabilities. The data arising from these interviews were subjected to a systematic comparative analysis based on three discrete subgroups of children: those with a disability, those with a life-limiting or life-threatening illness, and those with a technology dependence. FINDINGS: There were major areas of consistency in parental experience of impact as related in three categories that emerged from the data: time, multiple roles and the disabled family. CONCLUSION: There are many effects of childhood disability on the family. In large part, the effects cannot simply be defined or described in the context of a particular 'medical' diagnosis or prognosis. Many elements of impact do not appear to be related to whether or not a child has a life-threatening or life-limiting illness or by whether or not a child is dependent on specific medical devices or ongoing nursing care.

http://www.ncbi.nlm.nih.gov/pubmed/24708336

What it means to be the parent of a child with a disability or complex health need

Whiting, M.

Nurs Child Young People 2014; 26(5): 26-29

AIM: To explore how parents of children with disabilities and complex health needs make sense of the circumstances in which they find themselves. METHOD: A series of in-depth semi-structured interviews were undertaken with the parents of 34 children (from 33 families) with a disability or a complex health need. The families were nominated by health professionals to one of three study subgroups: children with a disability, children with a life-limiting or life-threatening illness, or children with technology dependence. Interviews were recorded and transcribed. FINDINGS: Analysis of parents' responses related to sense-making revealed two main categories: 'diagnosis' and 'personal, cultural and personality factors'. These two categories alongside the two other main study themes, 'impact' and 'need for help and support' reported in the previous two articles in this series - linked into a final study theme: 'battleground'. CONCLUSION: For the parents of children with complex health needs and disabilities, the sense that they make of their situation plays a pivotal role in determining how parents experience the impact of disability and the need for help and support.

http://www.ncbi.nlm.nih.gov/pubmed/24914669

Parental attitudes toward newborn screening for Duchenne/Becker muscular dystrophy and spinal muscular atrophy

Wood, M. F., et al.

Muscle Nerve 2014; 49(6): 822-828

INTRODUCTION: Disease inclusion in the newborn screening (NBS) panel should consider the opinions of those most affected by the outcome of screening. We assessed the level and factors that affect parent attitudes regarding NBS panel inclusion of Duchenne muscular dystrophy (DMD), Becker muscular dystrophy (BMD), and spinal muscular atrophy (SMA). METHODS: The attitudes toward NBS for DMD, BMD, and SMA were surveyed and compared for 2 categories of parents, those with children affected with DMD, BMD, or SMA and expectant parents unselected for known family medical history. RESULTS: The level of support for NBS for DMD, BMD, and SMA was 95.9% among parents of children with DMD, BMD, or SMA and 92.6% among expectant parents. CONCLUSIONS: There was strong support for NBS for DMD, BMD, and SMA in both groups of parents. Given advances in diagnostics and promising therapeutic approaches, discussion of inclusion in NBS should continue.

http://www.ncbi.nlm.nih.gov/pubmed/24307279

Pediatric oncology and palliative care

Barfield, R.
N C Med J 2014; 75(4): 276-277

http://www.ncbi.nlm.nih.gov/pubmed/25046095

Making it possible - interventions for children with cerebral palsy

Beckung, E.
Dev Med Child Neurol 2014; 56(5): 418-419

http://www.ncbi.nlm.nih.gov/pubmed/24635881

Young adults as users of adult healthcare: Experiences of young adults with complex or life-limiting conditions

Beresford, B. and L. Stuttard
Clin Med 2014; 14(4): 404-408

Awareness is growing that young adults may have distinctive experiences of adult healthcare and that their needs may differ from those of other adult users. In addition, the role of adult health teams in supporting positive transitions from paediatrics is increasingly under discussion. This paper contributes to these debates. It reports a qualitative study of the experiences of young adults - all with complex chronic health conditions - as users of adult health services. Key findings from the study are reported, including an exploration of factors that help to explain interviewees' experiences. Study findings are discussed in the context of existing evidence from young adults in adult healthcare settings and theories of 'young adulthood'. Implications for training and practice are considered, and priorities for future research are identified.

http://www.ncbi.nlm.nih.gov/pubmed/25099843

Life interrupted: Caring for young adult patients in pediatric settings

Boles, J.
Pediatr Nurs 2014; 40(4): 201-203

http://www.ncbi.nlm.nih.gov/pubmed/25269362

Transition issues for children with diffuse cortical malformations, multifocal postnatal lesions, (infectious and traumatic) and Lennox-Gastaut and similar syndromes

Camfield, P. R., et al.
Epilepsia 2014; 55 Suppl 3: 24-28

Patients with epilepsy may have diffuse, serious brain disorders including genetically determined, multilobar malformations, traumatic brain injury, encephalitis and meningitis, and the many causes of Lennox-Gastaut syndrome. Transition to adult care needs to consider concomitant intellectual disability, refractory epilepsy, underlying cause, and other nonneurologic but significant problems, especially for genetic etiologies. Adult epilepsy care coupled with dedicated primary/family care is essential. A multidisciplinary setting may be optimal to address the many issues of clinical care, decision making, custody, and ongoing supervision.

http://www.ncbi.nlm.nih.gov/pubmed/25209082

Reported Availability and Gaps of Pediatric Palliative Care in Low- and Middle-Income Countries: A Systematic Review of Published Data

Caruso Brown, A. E., et al.
J Palliat Med 2014

Abstract Background: The majority of young people in need of palliative care live in low- and middle-income countries, where curative treatment is less available. Objective: We systematically reviewed published data describing palliative care services available to young people with life-limiting conditions in low- and middle-income countries and assessed core elements with respect to availability, gaps, and under-reported aspects. Methods: PubMed, CINAHL, EMBASE (1980-2013), and secondary bibliographies were searched for publications that included patients younger than 25 years with life-limiting conditions and described palliative care programs in low- and middle-income countries. A data extraction checklist considered 15 items across seven domains: access, education/capacity building, health system support, pain management, symptom management, end-of-life care, and bereavement. Data were aggregated by program and country. Results: Of 1572 records, 238 met criteria for full-text review; 34 qualified for inclusion, representing 30 programs in 21 countries. The median checklist score was 7 (range, 1-14) of 10 reported (range, 3-14). The most pervasive gaps were in national health system support (unavailable in 7 of 17 countries with programs reporting), specialized education (unavailable in 7 of 19 countries with programs reporting), and comprehensive opioid access (unavailable in 14 of 21 countries with programs reporting). Underreported elements included specified practices for pain management and end-of-life support. Conclusion: Comprehensive pediatric palliative care provision is possible even in markedly impoverished settings. Improved national health system support, specialized training and opioid access are key targets for research and advocacy. Application of a checklist methodology can promote awareness of gaps to guide program evaluation, reporting, and strengthening.

http://www.ncbi.nlm.nih.gov/pubmed/25225748

Assessment of the need for palliative care for children in South Africa

Connor, S., et al.
Int J Palliat Nurs 2014; 20(3): 130-134

UNICEF and the International Children's Palliative Care Network undertook a joint analysis in three sub-Saharan countries-Zimbabwe, South Africa, and Kenya-to estimate the palliative care need among their children and to explore these countries' capacities to deliver children's palliative care (CPC). This report concerns the findings from South Africa. The study adopted a cross-sectional mixed-methods approach using both quantitative and qualitative data obtained from primary and secondary sources. CPC need was estimated using prevalence and mortality statistics. The response to the need and existing gaps were analysed using data obtained from a literature review, interviews with key persons, and survey data from service providers.The findings show very limited CPC service coverage for children in the public sector. In addition, services are mainly localised, with minimal reach. Less than 5% of the children needing care in South Africa are receiving it, with those receiving it being closer to the end of life. Barriers to the delivery of CPC include fear of opioid use, lack of education on CPC, lack of integration into the primary care system, lack of policies on CPC, and lack of community and health professional awareness of CPC needs and services. Estimating the need for CPC is a critical step in meeting the needs of children with life-threatening conditions and provides a sound platform to advocate for closure of the unacceptably wide gaps in coverage.

http://www.ncbi.nlm.nih.gov/pubmed/24675539

Transition from paediatric to adult care for patients with sickle cell disease

de Montalembert, M. and C. Guitton
Br J Haematol 2014; 164(5): 630-635

Advances achieved over the last three decades have transformed sickle cell disease (SCD) from a fatal childhood disease to a long-term chronic condition. Consequently, patients must transition from paediatric to adult care. The transition is a high-risk period associated with increases in hospital admissions and death. The factors underlying this increased risk include not only characteristics of the disease itself, with the accumulation of disabilities and progression of organ damage, but also psychological factors and a frequent paucity of adult-care resources for SCD. Leaving the familiar paediatric team causes marked anxiety in many patients. The transition of care coincides with the many other transitions that characterize the emotional, social and academic development of adolescents. The shift from protection by parents and physicians to independent self-management may be difficult. Finally, young adults may have limited access to health insurance. In recent years, many medical groups have suggested the development of transitioning programmes combining transition schedules, printed and web-based materials, and, in some cases, transition-dedicated physicians, nurses and psychologists. Transition must begin early, involve both the paediatric and the adult team, direct appropriate attention to the parents and occur over a period of several years. Evaluations of these programmes are urgently needed.

http://www.ncbi.nlm.nih.gov/pubmed/24345037

Children's palliative care: Considerations for a physical therapeutic environment

Downing, J., et al.
J Palliat Care 2014; 30(2): 116-124

Supportive and palliative care of children with metabolic and neurological diseases

Hauer, J. M. and J. Wolfe
Curr Opin Support Palliat Care 2014; 8(3): 296-302

PURPOSE OF REVIEW: To review the role of pediatric palliative care (PPC) for children with metabolic and neurological diseases. RECENT FINDINGS: There is a growing body of literature in PPC, though it remains limited for children with metabolic and neurological diseases. Evidence indicates the benefit of PPC. Utilization of PPC programmes can facilitate communication, ensure that families are better informed, improve certainty with decisions, enhance positive emotions, result in fewer invasive interventions at the end of life, and have an impact on location of death. Barriers to utilization of PPC include concern about taking away hope and uncertainty about prognosis. Challenging areas for children with metabolic and neurological diseases include the identification of distressing symptoms and prognostic uncertainty. This article aims to review literature relevant to this group of children, as well as provide a framework when considering specific palliative care needs. SUMMARY: PPC for children with metabolic and neurological diseases can lessen a child's physical discomfort and enhance parental certainty with decision-making. These areas along with other needs throughout the illness trajectory and bereavement are being increasingly met by the growing availability of PPC programmes.

http://www.ncbi.nlm.nih.gov/pubmed/25058989

Self-reported transition readiness among young adults with sickle cell disease

Sobota, A., et al.
J Pediatr Hematol Oncol 36(5): 389-394

BACKGROUND: A growing body of literature addresses the need for transition programs for young adults with sickle cell disease (SCD); however, studies assessing transition readiness are limited and there are few validated instruments to use. OBJECTIVE: To conduct a pilot study to assess transition readiness of patients with SCD in our transition program and to evaluate a SCD-specific assessment tool that measures 5 knowledge skill sets (medical, educational/vocational, health benefits, social, and independent living), and 3 psychological assessments (feelings, stress, and self-efficacy). RESULTS: Of the 47 SCD patients between the ages of 18 and 22 seen in our facility, 33 completed the assessment tool. The majority of patients reported good medical knowledge of SCD and said they were motivated to pursue a career despite the burden of living with the disease. We identified knowledge gaps in the area of independent living and health benefits skills sets. A majority of patients reported being worried that their SCD would prevent them from doing things in their life; however, few respondents said they were worried or anxious about their transition to adult care. CONCLUSIONS: Adolescents beginning a transition intervention program reported a high level of knowledge of their disease and demonstrated a positive attitude toward transition with good self-efficacy.

http://www.ncbi.nlm.nih.gov/pubmed/24517960

Preparing adolescents with chronic disease for transition to adult care: A technology program

Huang, J. S., et al.

Pediatrics 2015; 133(6): e1639-1646

BACKGROUND: Adolescents with chronic disease (ACD) must develop independent disease self-management and learn to communicate effectively with their health care team to transition from pediatric to adult-oriented health care systems. Disease-specific interventions have been implemented to aid specific ACD groups through transition. A generic approach might be effective and cost-saving. METHODS: Eighty-one ACD, aged 12 to 20 years, were recruited for a randomized clinical trial evaluating an 8-month transition intervention (MD2Me). MD2Me recipients received a 2-month intensive Web-based and text-delivered disease management and skill-based intervention followed by a 6-month review period. MD2Me recipients also had access to a texting algorithm for disease assessment and health care team contact. The intervention was applicable to adolescents with diverse chronic illnesses. Controls received mailed materials on general health topics. Disease management, health-related self-efficacy, and health assessments were performed at baseline and at 2 and 8 months. Frequency of patient-initiated communications was recorded over the study period. Outcomes were analyzed according to assigned treatment group over time. RESULTS: MD2Me recipients demonstrated significant improvements in performance of disease management tasks, health-related self-efficacy, and patient-initiated communications compared with controls. CONCLUSIONS: Outcomes in ACD improved significantly among recipients of a generic, technology-based intervention. Technology can deliver transition interventions to adolescents with diverse chronic illnesses, and a generic approach offers a cost-effective means of positively influencing transition outcomes. Further research is needed to determine whether improved short-term outcomes translate into an improved transition for ACD.

http://www.ncbi.nlm.nih.gov/pubmed/24843066

Bereavement support used by mothers in Ireland following the death of their child from a life-limiting condition

Jennings, V. and H. Nicholl

Int J Palliat Nurs 2014; 20(4): 173-178

BACKGROUND: Children's palliative care is a rapidly developing specialism internationally. Bereavement support is an integral component of children's palliative care but to date little research has investigated the bereavement support that mothers in Ireland use following the death of their child. OBJECTIVE: The aim of this study was to explore mothers' experiences of bereavement support in Ireland following the death of their child from a life-limiting condition. METHOD: A descriptive qualitative design was used. The study sample was ten mothers who had been bereaved in the previous 5 years. All mothers were recruited to the study by a gatekeeper from a voluntary organisation. Data were obtained through unstructured single interviews and analysed using conventional content analysis. RESULTS: The findings indicate that the mothers relied on a combination of informal and formal bereavement support. In addition to depending on others to provide support, the mothers described their ability to self-support. CONCLUSIONS: The findings show that mothers in Ireland use a variety of sources of support following the death of their child from a life-limiting condition. Health professionals involved in caring for families and children with a life-limiting condition should have an understanding of these sources.

http://www.ncbi.nlm.nih.gov/pubmed/24763325

End-of-life care for hospitalized children

Johnson, L. M., et al.
Pediatr Clin North Am 2014; 61(4): 835-854

High-quality palliative care is the standard for children with life-threatening illness, especially when a cure is not possible. This review outlines a model for clinical practice that integrates clinical, psychosocial, and ethical concerns at the end of life (EOL) into a standard operating procedure specifically focused on inpatient deaths. Palliative care for children at EOL in the hospital setting should encompass the personal, cultural, and spiritual needs of the child and family members and aim to minimize suffering and increase support for all who are involved, including hospital staff.

http://www.ncbi.nlm.nih.gov/pubmed/25084727

Home visiting programs for HIV-affected families: A comparison of service quality between volunteer-driven and paraprofessional models

Kidman, R., et al.
Vulnerable Child Youth Stud 2014; 9(4): 305-317

Home visiting is a popular component of programs for HIV-affected children in sub-Saharan Africa, but its implementation varies widely. While some home visitors are lay volunteers, other programs invest in more highly trained paraprofessional staff. This paper describes a study investigating whether additional investment in paraprofessional staffing translated into higher quality service delivery in one program context. Beneficiary children and caregivers at sites in KwaZulu-Natal, South Africa were interviewed after 2 years of program enrollment and asked to report about their experiences with home visiting. Analysis focused on intervention exposure, including visit intensity, duration and the kinds of emotional, informational and tangible support provided. Few beneficiaries reported receiving home visits in program models primarily driven by lay volunteers; when visits did occur, they were shorter and more infrequent. Paraprofessional-driven programs not only provided significantly more home visits, but also provided greater interaction with the child, communication on a larger variety of topics, and more tangible support to caregivers. These results suggest that programs that invest in compensation and extensive training for home visitors are better able to serve and retain beneficiaries, and they support a move toward establishing a professional workforce of home visitors to support vulnerable children and families in South Africa.

http://www.ncbi.nlm.nih.gov/pubmed/25379052

Perinatal palliative care: A developing specialty

Kiman, R. and L. Doumic
Int J Palliat Nurs 2014; 20(3): 143-148

Neonates and babies have the highest death rate in the paediatric population. Perinatal palliative care aims to enhance the quality of life of babies with a life-limiting condition and their families. However, very little data is available on perinatal palliative care and its impact on babies and families along their journey. End-of-life decision-making for babies with an adverse prognosis also remains ethically challenging. This paper provides an overview of perinatal palliative care and its development, and then considers some of the issues affecting this field by looking at single national, institutional, and patient case studies.

http://www.ncbi.nlm.nih.gov/pubmed/24675541

Adolescents and young adults with life-threatening illness: Special considerations, transitions in care, and the role of pediatric palliative care

Linebarger, J. S., et al.
Pediatr Clin North Am 2014; 61(4): 785-796

This article will cover the special considerations, challenges, and opportunities presented by caring for adolescents and young adults with life-threatening illnesses when the possibility of transition to an adult care setting arises.

http://www.ncbi.nlm.nih.gov/pubmed/25084724

Innovative approach to providing 24/7 palliative care for children

Maynard, L. and D. Lynn
Nurs Child Young People 2014; 26(6): 27-34

This study outlines an innovative, English hospice-based service that provides 24/7 care for children with life-limiting conditions and their families. Operational objectives were: symptom management; open access to families and professionals; choice in place of care and of death; and collaboration to develop shared pathways and management plans. Service standards were audited through questionnaires completed by professionals and families. Findings demonstrated that the nursing team filled a critical gap and met its pre-set standards. Keys to success were: having the right level and mix of specialist and advanced skills; funded on-call arrangements; anticipatory planning; symptom management plans; and clinical supervision. Further recommendations were to develop a multi-agency workforce strategy, and to increase capacity in the children's sector to undertake academic research measuring the impacts of interventions.

http://www.ncbi.nlm.nih.gov/pubmed/25004048

School Nurses and Care Coordination for Children With Complex Needs: An Integrative Review

McClanahan, R. and P. C. Weismuller
J Sch Nurs 2014

Health care for students with chronic needs can be complex and specialized, resulting in fragmentation, duplication, and inefficiencies. Students who miss school due to chronic conditions lose valuable educational exposure that contributes to academic success. As health-related disabilities increase in prevalence so does the need for the coordination of care within the school and between the school and service providing agencies. This integrative literature review provides a synthesis of published evidence identifying and describing the core concepts associated with the role of school nurses in providing care coordination/case management to students with complex needs. Six core essentials of nurse-provided care coordination were identified: collaboration, communication, care planning and the nursing process, continuous coordination, clinical expertise, and complementary components. Recommendations for improving care coordination were elucidated in the review. Analysis of the literature can help assure application of best practice methods for the coordination of care for students in the school setting.

http://www.ncbi.nlm.nih.gov/pubmed/25266887

Comparison of emergency care delivered to children and young adults with complex chronic conditions between pediatric and general emergency departments

Murtagh Kurowski, E., et al.
Acad Emerg Med 2014; 21(7): 778-784

OBJECTIVES: Increasing attention is being paid to medically complex children and young adults, such as those with complex chronic conditions, because they are high consumers of inpatient hospital days and resources. However, little is known about where these children and young adults with complex chronic conditions seek emergency care and if the type of emergency department (ED) influences the likelihood of admission. The authors sought to generate nationwide estimates for ED use by children and young adults with complex chronic conditions and to evaluate if being of the age for transition to adult care significantly affects the site of care and likelihood of hospital admission. METHODS: This was a cross-sectional study using discharge data from the 2008 Nationwide Emergency Department Sample (NEDS), Healthcare Cost and Utilization Project (HCUP), Agency for Healthcare Research and Quality to evaluate visits to either pediatric or general EDs by pediatric-aged patients (17 years old or younger) and transition-aged patients (18 to 24 years old) with at least one complex chronic condition. The main outcome measures were hospital admission, ED charges for treat-and-release visits, and total charges for admitted patients. RESULTS: In 2008, 69% of visits by pediatric-aged and 92% of visits by transition-aged patients with multiple complex chronic conditions occurred in general EDs. Not surprisingly, pediatric age was the strongest predictor of seeking care in a pediatric ED (odds ratio [OR] = 15.86; 95% confidence interval [CI] = 12.3 to 20.5). Technology dependence (OR = 1.56; 95% CI =1.2 to 2.0) and presence of multiple complex chronic conditions (OR = 1.39; 95% CI = 1.2 to 1.6) were also associated with higher odds of seeking care in a pediatric ED. When controlling for patient and hospital characteristics, type of ED was not a significant predictor of admission (p = 0.87) or total charges (p = 0.26) in either age group. CONCLUSIONS: Overall, this study shows that, despite their complexity, the vast majority of children and young adults with multiple complex chronic conditions are cared for in general EDs. When controlling for patient and hospital characteristics, the admission rate and total charges for hospitalized patients did not differ between pediatric and general EDs. This result highlights the need for increased attention to the care that these medically complex children and young adults receive outside of pediatric-specialty centers. These results also emphasize that any future performance metrics developed to evaluate the quality of emergency care for children and young adults with complex chronic conditions must be applicable to both pediatric and general ED settings.

http://www.ncbi.nlm.nih.gov/pubmed/25039935

Transitions to and from the acute inpatient care setting for children with life-threatening illness

Nageswaran, S., et al.
Pediatr Clin North Am 2014; 61(4): 761-783

Children with life-threatening illnesses (LTIs) are hospitalized more often and spend more days in the hospital than children without LTIs. Hospitalizations may be associated with changes in health status of children with LTIs and thus alter their care needs significantly. Transitional care is particularly relevant for this population. Pediatric palliative care clinicians and teams are well-positioned to improve transitional care of children with LTIs by facilitating communication between clinicians and educating clinicians about issues related to children with LTIs.

http://www.ncbi.nlm.nih.gov/pubmed/25084723

The continuum of care for individuals with lifelong disabilities: Role of the physical therapist

Orlin, M. N., et al.
Phys Ther 2014; 94(7): 1043-1053

Many individuals with lifelong disabilities (LLDs) of childhood onset are living longer, participating in adult roles, and seeking comprehensive health care services, including physical therapy, with greater frequency than in the past. Individuals with LLDs have the same goals of health and wellness as those without disabilities. Aging with a chronic LLD is not yet well understood; however, impairments such as pain, fatigue, and osteoporosis often present earlier than in adults who are aging typically. People with LLDs, especially those living with developmental disabilities such as cerebral palsy, myelomeningocele, Down syndrome, and intellectual disabilities, frequently have complex and multiple body system impairments and functional limitations that can: (1) be the cause of numerous and varied secondary conditions, (2) limit overall earning power, (3) diminish insurance coverage, and (4) create unique challenges for accessing health care. Collaboration between adult and pediatric practitioners is encouraged to facilitate smooth transitions to health practitioners, including physical therapists. A collaborative client-centered emphasis to support the transition to adult-oriented facilities and promote strategies to increase accessibility should become standard parts of examination, goal setting, and intervention. This perspective article identifies barriers individuals with selected LLDs experience in accessing health care, including physical therapy. Strategies are suggested, including establishment of niche practices, physical accessibility improvement, and inclusion of more specific curriculum content in professional (entry-level) doctorate physical therapy schools.

http://www.ncbi.nlm.nih.gov/pubmed/24557656

Family-focused children's end of life care in hospital and at home

Parker, H., et al.
Nurs Child Young People 2014; 26(6): 35-39

An increasing number of children and young people require end of life care, and providing them and their families with optimum support at this time is crucial. This article describes how nurses working with children and families in home, hospital and community settings used the principles of practice development methodology to develop end of life care provision and follow-up bereavement support. It outlines the 'ways of knowing' that informed developments and how parents' priorities were kept central to the process. Finally, it discusses how the approach taken to practice development reflected the value of compassion in nursing practice.

http://www.ncbi.nlm.nih.gov/pubmed/25004049

Characteristics of a pediatric hospice palliative care program over 15 years

Siden, H., et al.
Pediatrics 2014; 134(3): e765-772

OBJECTIVES: Pediatric palliative care has seen the adoption of several service provision models, yet there is minimal literature describing them. Canuck Place Children's Hospice (CPCH) is North America's first freestanding pediatric hospice. This study describes the characteristics of and services delivered to all children on the CPCH program from 1996 to 2010. METHODS: A retrospective review of all patient medical records CPCH was conducted. Analyses examined trends and correlations between 40 selected data points: linear regression modeling was used to assess trends over time; t tests were used to examine significant associations between independent means; and the Kaplan-Meier method was used to measure survival probabilities. RESULTS: The study cohort included 649 children. The majority of diagnoses belonged to cancers (30%), and diseases of the neuromuscular (20%), and central nervous systems (18%). The majority of deaths occurred among the cancer (45%), central nervous system (15%), and metabolic disease groups (14%). By study end date, 24% of children were still alive, 61% died, and 15% transitioned to adult services (more than half of whom were cognitively competent). On average, 1024 days were spent on the CPCH program (median = 301). The majority of inpatient hospice discharges were for respite (82%); only 7% were for end-of-life care. Location of death was shared between CPCH (61%), hospital (22%), and home (16%). CONCLUSIONS: Diagnostic groups largely determine the nature and magnitude of services used, and our involvement with pediatric life-threatening conditions is increasing. Reviews of pediatric palliative programs can help evaluate the services needed by the population served.

http://www.ncbi.nlm.nih.gov/pubmed/25157003

Self-reported transition readiness among young adults with sickle cell disease

Sobota, A., et al.
J Pediatr Hematol Oncol 36(5): 389-394

BACKGROUND: A growing body of literature addresses the need for transition programs for young adults with sickle cell disease (SCD); however, studies assessing transition readiness are limited and there are few validated instruments to use. OBJECTIVE: To conduct a pilot study to assess transition readiness of patients with SCD in our transition program and to evaluate a SCD-specific assessment tool that measures 5 knowledge skill sets (medical, educational/vocational, health benefits, social, and independent living), and 3 psychological assessments (feelings, stress, and self-efficacy). RESULTS: Of the 47 SCD patients between the ages of 18 and 22 seen in our facility, 33 completed the assessment tool. The majority of patients reported good medical knowledge of SCD and said they were motivated to pursue a career despite the burden of living with the disease. We identified knowledge gaps in the area of independent living and health benefits skills sets. A majority of patients reported being worried that their SCD would prevent them from doing things in their life; however, few respondents said they were worried or anxious about their transition to adult care. CONCLUSIONS: Adolescents beginning a transition intervention program reported a high level of knowledge of their disease and demonstrated a positive attitude toward transition with good self-efficacy.

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