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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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East Anglia's Children's Hospices (EACH) – New library and information service for children's hospices

For staff working in children's hospices it's not always easy to access the most up-to-date resources to support professional and evidence based practice. However, the good news is that a new online library service is now available to all children's hospices via the EACH library service.

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For information on membership, please contact: Sue Langley, Library and Information Services Manager **sue.langley@each.org.uk**; 01223 815103

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Elective non-therapeutic intensive care and the four principles of medical ethics

Baumann, A., et al.
J Med Ethics 2013; 39 (3): 139-142

The chronic worldwide lack of organs for transplantation and the continuing improvement of strategies for in situ organ preservation have led to renewed interest in elective non-therapeutic ventilation of potential organ donors. Two types of situation may be eligible for elective intensive care: patients definitely evolving towards brain death and patients suitable as controlled non-heart beating organ donors after life-supporting therapies have been assessed as futile and withdrawn. Assessment of the ethical acceptability and the risks of these strategies is essential. We here offer such an ethical assessment using the four principles of medical ethics of Beauchamp and Childress applying them in their broadest sense so as to include patients and their families, their caregivers, other potential recipients of intensive care, and indeed society as a whole. The main ethical problems emerging are the definition of beneficence for the potential organ donor, the dilemma between the duty to respect a dying patient's autonomy and the duty not to harm him/her, and the possible psychological and social harm for families, caregivers other potential recipients of therapeutic intensive care, and society more generally. Caution is expressed about the ethical acceptability of elective non-therapeutic ventilation, along with some proposals for precautionary measures to be taken if it is to be implemented.

http://www.ncbi.nlm.nih.gov/pubmed/23355225

Can a patient designate his doctor as his proxy decision maker?

Black, P. G., et al.
Pediatrics 2013; 131 (5): 986-990

Most lawyers and bioethicists recommend that patients enact a durable power of attorney for health care designating somebody as their proxy decision maker should they become unable to make decisions. Most people choose family members as their agent. But what if a patient wants his or her doctor to be his or her proxy decision maker? Can the doctor be both physician and surrogate decision maker? Or should those roles necessarily be kept separate? We present a case in which those issues arose, and sought comments from Sabrina Derrington, a pediatric palliative care physician; Arthur Derse, an emergency department physician and lawyer; and Phil Black, a pulmonologist.

http://www.ncbi.nlm.nih.gov/pubmed/23629617

The culture of dysthanasia: attempting CPR in terminally ill children

Clark, J. D. and D. M. Dudzinski
Pediatrics 2013; 131 (3): 572-580

Both dying children and their families are treated with disrespect when the presumption of consent to cardiopulmonary resuscitation (CPR) applies to all hospitalized children, regardless of prognosis and the likely efficacy of CPR. This "opt-out" approach to CPR fails to appreciate the nuances of the special parent-child relationship and the moral and emotional complexity of enlisting parents in decisions to withhold CPR from their children. The therapeutic goal of CPR is not merely to resume spontaneous circulation, but rather it is to provide circulation to vital organs to allow for treatment of the underlying proximal and distal etiologies of cardiopulmonary arrest. When the treating providers agree that attempting CPR is highly unlikely to achieve the therapeutic goal or will merely prolong dying, we should not burden parents with the decision to forgo CPR. Rather, physicians should carry the primary professional and moral responsibility for the decision and use a model of informed assent from parents, allowing for respectful disagreement. As emphasized in the palliative care literature, we recommend a directive and collaborative goal-oriented approach to conversations about limiting resuscitation, in which physicians provide explicit recommendations that are in alignment with the goals and hopes of the family and emphasize the therapeutic indications for CPR. Through this approach, we hope to help parents understand that "doing everything" for their dying child means providing medical therapies that ameliorate suffering and foster the intimacy of the parent-child relationship in the final days of a child's life, making the dying process more humane.

http://www.ncbi.nlm.nih.gov/pubmed/23382437

Modes of death in pediatrics: differences in the ethical approach in neonatal and pediatric patients

Fontana, M. S., et al.
J Pediatr 2013; 162 (6): 1107-1111

OBJECTIVE: To compare end-of-life decisions for neonatal and pediatric patients. STUDY DESIGN: This study involved a chart review of all pediatric deaths occurring over a 2-year period at a large maternal-child university hospital. Modes of death were compared. RESULTS: Of the 220 deaths analyzed, 145 occurred in intensive care units (ICUs), including 77 in the neonatal ICU (NICU) and 68 in the pediatric ICU (PICU). Only 6% of deaths were preceded by cardiopulmonary resuscitation. Dying while on the respirator was the most common mode of death in the PICU (51%) and the least common in the NICU (5%; P<.05). Unstable physiology at time of death was much more common in the PICU (82% vs 47%; P<.05). Withdrawal of life-sustaining interventions (LSI) in stable patients for quality of life reasons was the most common cause of death in the NICU (53% vs 16%; P<.05). Seventy-five children died outside of an ICU because LSI were withheld; neonates died mainly of extreme prematurity, and older children died mainly from terminal illness. CONCLUSION: The majority of pediatric deaths occur in ICUs. Modes of death in the NICU and the PICU are strikingly different. A greater proportion of deaths in the NICU occur in infants with stable physiology who might not have died had LSI not been withdrawn. Most deaths outside of ICUs are attributable to withholding of LSI. A significant proportion of neonates in whom LSI are withheld have a possibility of intact survival, unlike older patients.

http://www.ncbi.nlm.nih.gov/pubmed/23312685

Determining what is in the best interests of a critically ill child

Griffith, R.
Br J Nurs 2013; 22 (2): 112-113

Controversy over the use of end-of-life pathways with terminally ill patients has now extended to their use with critically ill children and babies (Boyd and Murray, 2012). At the centre of the present controversy is the claim that parents feel coerced into agreeing that it is in their child's best interests to be placed on the pathway (Ford Rojas, 2012). In this article, Richard Griffith reviews the development of the best interests test adopted by the courts when dealing with cases of seriously ill children and babies, and argues that the test should inform the decision on when continued care and treatment become futile and the use of an end-of-life care pathway should be considered.

http://www.ncbi.nlm.nih.gov/pubmed/23587896

Clinical decision making in hypotonia and gross motor delay: a case report of type 1 spinal muscular atrophy in an infant

Malerba, K. H. and J. S. Tecklin
Phys Ther 2013; 93 (6): 833-841

BACKGROUND AND PURPOSE: Children often are referred for physical therapy with the diagnosis of hypotonia when the definitive cause of hypotonia is unknown. The purpose of this case report is to describe the clinical decision-making process using the Hypothesis-Oriented Algorithm for Clinicians II (HOAC II) for an infant with hypotonia and gross motor delay. CASE DESCRIPTION: The patient was a 5-month-old infant who had been evaluated by a neurologist and then referred for physical therapy by his pediatrician. Physical therapist evaluation results and clinical observations of marked hypotonia, significant gross motor delay, tongue fasciculations, feeding difficulties, and respiratory abnormalities prompted necessary referral to specialists. Recognition of developmental, neurologic, and respiratory abnormalities facilitated clinical decision making for determining the appropriate physical therapy plan of care. OUTCOMES: During the brief episode of physical therapy care, the patient was referred to a feeding specialist and diagnosed with pharyngeal-phase dysphasia and mild aspiration. Continued global weakness, signs and symptoms of type 1 spinal muscular atrophy (SMA), and concerns about increased work of breathing and respiratory compromise were discussed with the referring physician. After inconclusive laboratory testing for metabolic etiologies of hypotonia, a genetics consult was recommended and confirmed the diagnosis of type 1 SMA at 9 months of age. DISCUSSION: Physical therapists use clinical decision making to determine whether to treat patients or to refer them to other medical professionals. Accurate and timely referral to appropriate specialists may assist families in obtaining a diagnosis for their child and guide necessary interventions. In the case of type 1 SMA, early diagnosis may affect outcomes and survival rate in this pediatric population.

http://www.ncbi.nlm.nih.gov/pubmed/23431212

End-of-life care decisions in the PICU: roles professionals play

Michelson, K. N., et al.
Pediatr Crit Care Med 2013; 14 (1): e34-44

OBJECTIVE: Describe the roles and respective responsibilities of PICU healthcare professionals in end-of-life care decisions faced by PICU parents. DESIGN: Retrospective qualitative study. SETTING: University-based tertiary care children's hospital. PARTICIPANTS: Eighteen parents of children who died in the pediatric ICU and 48 PICU healthcare professionals (physicians, nurses, social workers, child-life specialists, chaplains, and case managers). INTERVENTIONS: In depth, semi-structured focus groups and one-on-one interviews designed to explore experiences in end-of-life care decision making. MEASUREMENTS AND MAIN RESULTS: We identified end-of-life care decisions that parents face based on descriptions by parents and healthcare professionals. Participants described medical and nonmedical decisions addressed toward the end of a child's life. From the descriptions, we identified seven roles healthcare professionals play in end-of-life care decisions. The family supporter addresses emotional, spiritual, environmental, relational, and informational family needs in a nondirective way. The family advocate helps families articulate their views and needs to healthcare professionals. The information giver provides parents with medical information, identifies decisions or describes available options, and clarifies parents' understanding. The general care coordinator helps facilitate interactions among healthcare professionals in the PICU, among healthcare professionals from different subspecialty teams, and between healthcare professionals and parents. The decision maker makes or directly influences the defined plan of action. The end-of-life care coordinator organizes and executes functions occurring directly before, during, and after dying/death. The point person develops a unique trusting relationship with parents. CONCLUSIONS: Our results describe a framework for healthcare professionals' roles in parental end-of-life care decision making in the pediatric ICU that includes directive, value-neutral, and organizational roles. More research is needed to validate these roles. Actively ensuring attention to these roles during the decision-making process could improve parents' experiences at the end of a child's life.

http://www.ncbi.nlm.nih.gov/pubmed/23249788

Parental explicit heuristics in decision-making for children with life-threatening illnesses

Renjilian, C. B., et al.
Pediatrics 2013; 131 (2): e566-572

OBJECTIVE: To identify and illustrate common explicit heuristics (decision-making aids or shortcuts expressed verbally as terse rules of thumb, aphorisms, maxims, or mantras and intended to convey a compelling truth or guiding principle) used by parents of children with life-threatening illnesses when confronting and making medical decisions. METHODS: Prospective cross-sectional observational study of 69 parents of 46 children who participated in the Decision-making in Pediatric Palliative Care Study between 2006 and 2008 at the Children's Hospital of Philadelphia. Parents were guided individually through a semistructured in-depth interview about their experiences and thoughts regarding making medical decisions on behalf of their ill children, and the transcribed interviews were qualitatively analyzed. RESULTS: All parents in our study employed explicit heuristics in interviews about decision-making for their children, with the number of identified explicit heuristics used by an individual parent ranging from tens to hundreds. The heuristics served 5 general functions: (1) to depict or facilitate understanding of a complex situation; (2) to clarify, organize, and focus pertinent information and values; (3) to serve as a decision-making compass; (4) to communicate with others about a complex topic; and (5) to justify a choice. CONCLUSIONS: Explicit heuristics played an important role in decision-making and communication about decision-making in our population of parents. Recognizing explicit heuristics in parent interactions and understanding their content and functions can aid clinicians in their efforts to partner with parents in the decision-making process.

http://www.ncbi.nlm.nih.gov/pubmed/23319524

Twelve-year review of neonatal deaths in the delivery room in a perinatal tertiary centre

Tudehope, D., et al.

J Paediatr Child Health 2013; 49 (1): E40-45

AIM: To describe decisions made for babies who died in the delivery room as a result of clinical practice of non-resuscitation or unsuccessful resuscitation. METHODS: A retrospective study was conducted of neonatal deaths (NNDs) >/= 400 g and/or >/=20 weeks' gestation born at Mater Mothers' Hospitals 1998-2009 who were not admitted to a neonatal nursery. Deaths were divided into not resuscitated and unsuccessful resuscitation and subdivided by cause of death as extremely preterm, congenital abnormality or 'other'. RESULTS: Of all 539 NNDs, 217 (40.3%) were not admitted to a neonatal nursery, comprising 174 (80.2%) not resuscitated and 41 (18.9%) unsuccessful resuscitation, while in a further two newborn infants resuscitation was not required. Only 13 of 123 (10.6%) extremely preterm infants who died in the delivery room had resuscitation attempted. Of 77 infants who died from congenital abnormalities in the delivery room 18 (23.3%) had resuscitation attempted. Fifteen babies with other diagnoses died in the delivery room; five with severe intrapartum asphyxia without resuscitation and a further 10 (8 preterm) with Apgar scores of 0-1 at 1 min and </=3 at 5 min who did not respond to extensive resuscitation. CONCLUSIONS: A large proportion of NNDs occurred outside the neonatal nurseries involving end-of-life decision-making. Review of the circumstances of these NNDs in the subcategories of extreme prematurity, congenital abnormalities and 'other' raises different management dilemmas with the potential for clinical practice improvement in compassionate care and transparency in decision-making.

http://www.ncbi.nlm.nih.gov/pubmed/23198828

What neonatal intensive care nurses need to know about neonatal palliative care

Ahern, K.

Adv Neonatal Care 2013; 13 (2): 108-114

The purpose of this study was to identify and prioritize topics for a professional development program in neonatal palliative care. A total of 276 nurses and midwives who work in an Australian neonatal intensive care unit (NICU) and 26 international healthcare professionals working in NICU and palliative care served as participants. A Delphi technique was used, consisting of a series of rounds of data collection via interview and questionnaire, to identify and consolidate opinions of nurses and other healthcare professionals who work in neonatal intensive care units. The main outcome measures were: (1) Topics to be included in a professional development program for nurses working in neonatal intensive care units and (2) the preferred format of the program. Twenty-three high-priority topics were identified, which included preparing families when death is imminent, how to provide emotional support to grieving parents, advocating for a dying baby, and assessing and managing pain in a dying baby. Care of a dying infant requires the same skill set as caring for older terminally ill children internationally. A combination of face-to-face lectures and interactive workshops using case studies and audiovisual examples is the preferred format.

http://www.ncbi.nlm.nih.gov/pubmed/23532030

End-of-life care in pediatrics: ethics, controversies, and optimizing the quality of death

Basu, R. K.

Pediatr Clin North Am 2013; 60 (3): 725-739

Hospitalized children constitute most annual pediatric deaths in the United States. The details of "how-to" provide end-of-life (EOL) care are not consistently taught to staff and therefore the actual delivery of EOL care is often inconsistent and invariably negatively associated with the long-term mental health of both the patient's family and care providers. This review describes the pertinent aspects of end-of-life care in pediatrics. Finally, a framework to optimize the quality of death is described, which underscores the importance of synchrony between the care team and the family at the end of a child's life.

http://www.ncbi.nlm.nih.gov/pubmed/23639665

Pediatric palliative care-when quality of life becomes the main focus of treatment

Bergstraesser, E.

Eur J Pediatr 2013; 172 (2): 139-150

Pediatric palliative care (PPC) focuses on children and adolescents with life-limiting diseases. It may be initiated at various points of the disease trajectory, if possible early enough to support living with the best possible quality of life despite a limited lifespan. From birth to adolescence, children with a broad spectrum of diseases may benefit from PPC. Since 50% of deaths in childhood occur within the first year of life, PPC is just as relevant to neonatology. Causes of death in the neonate and young infant are due to perinatal conditions such as preterm birth and congenital disorders and syndromes; in older children, external causes, such as traumatic injuries, outweigh disease-related causes of death. PPC may last from a few hours or days for neonates to many years for children with complex chronic conditions. For neonates, PPC often has the character of end-of-life (EOL) care followed by bereavement care for the family. For older children, PPC can clearly be differentiated from EOL care; its indications include progress or deterioration of disease, marked instability of the child's condition, increase in the need for technical or medical support, increase in suffering, or failure of treatment. If a child's need for palliative care is established, useless and potentially harmful treatments may be withheld and informed choices can be made about treatment, care, and the remaining life of the child. Conclusion This review aims to provide knowledge for clinicians who care for children and adolescents at risk of dying from their disease. PPC can improve the child's remaining lifetime by focusing on quality of life and goals that are defined by the child and his or her family.

http://www.ncbi.nlm.nih.gov/myncbi/collections/43681103/

The needs of professionals in the palliative care of children and adolescents

Bergstraesser, E., et al.

Eur J Pediatr 2013; 172 (1): 111-118

The main objectives of this qualitative study were to describe the perceptions and needs of pediatric health care professionals (HCPs) taking care of children with palliative care needs and to develop a concept for the first Center of Competence for Pediatric Palliative Care (PPC) in Switzerland. Within two parts of the study, 76 HCPs were interviewed. The main interview topics were: (1) definition of and attitude toward PPC; (2) current provision of PPC; (3) the support needs of HCPs in the provision of PPC; and (4) the role of specialized PPC teams. HCPs expressed openness to PPC and reported distinctive needs for support in the care of these patients. The main tasks of specialized PPC teams in Switzerland would encompass the coaching of attending teams, coordination of care, symptom control, and direct support of affected families during and beyond the illness of their child. CONCLUSION: This study indicates the need for specialized PPC in Switzerland both inside and outside of centers providing top quality medical care (Spitzenmedizin). Specialized PPC teams could have a significant impact on the care of children and families with PPC needs. Whether hospices are an option in Switzerland remains unanswered; however, a place to meet other families with similar destinies was emphasized.

http://www.ncbi.nlm.nih.gov/pubmed/23207735

Oral health perceptions of paediatric palliative care nursing staff

Couch, E., et al.
Int J Palliat Nurs 2013; 19 (1): 9-15

Systematic oral care reduces oral complications among children in paediatric palliative care (PPC), yet little is known about the oral health perceptions of PPC nursing staff. This qualitative cross-sectional study used semi-structured interviews based on phenomenography to explore PPC nursing staff's perceptions of oral health and the relationship of oral care to comfort and quality of life. A purposive sample of nine nursing staff employed at a California PPC facility participated. Five themes emerged from the analysis of the interviews: signs of oral health, reasons for oral care, adaptation of oral care on a case-by-case basis, barriers to providing oral care, and facilitators of improving oral care. The perceived importance of oral health was the underlining similarity between the themes. A need for further research in the area of oral PPC is indicated. Collaboration with dental professionals may be needed to create oral PPC guidelines that fit the complex needs of children with life-limiting illnesses.

http://www.ncbi.nlm.nih.gov/pubmed/23354428

The thirteenth international childhood acute lymphoblastic leukemia workshop report: La Jolla, CA, USA, December 7-9, 2011

Hunger, S. P., et al.
Pediatr Blood Cancer 2013; 60 (2): 344-348.

http://www.ncbi.nlm.nih.gov/pubmed/23024117

The use of palliative chemotherapy in pediatric oncology patients: a national survey of pediatric oncologists

Kang, T. I., et al.

Pediatr Blood Cancer 2013; 60 (1): 88-94

BACKGROUND: Many children continue receiving chemotherapy after there is no realistic hope for cure. One factor that influences parental decisions to pursue medical therapies is physician preference. To date, no studies have described pediatric oncologists' perspectives and practices regarding palliative chemotherapy (PC). PROCEDURE: We surveyed via email pediatric oncologists practicing in the U.S who are members of the Children's Oncology Group to achieve the following objectives: (1) Describe pediatric oncologists treatment considerations regarding the use of PC. (2) Assess treatment considerations that influenced pediatric oncologists' therapy recommendations for their most recent patient receiving PC. There were 422 participants (40.8%) who completed the survey. RESULTS: The most important factors considered by pediatric oncologists when prescribing PC were the toxicity of the chemotherapy (4.90 mean SD = 0.36 utilizing 5 point scale with 1 = not important to 5 = very important), the preferences of the family (4.57; SD = 0.60), and the potential to decrease symptoms arising from tumor burden (4.42; SD = 0.65). These treatment considerations were not as important when PC was prescribed for their most recent patient. Similarly, the chief aims in prescribing PC were not achieved for recent patients receiving PC. For their most recent patient who received PC, 40.8% believe this treatment was primarily for parental wishes. CONCLUSION: According to 80.2% of pediatric oncologists completing the survey, some patients receive chemotherapy beyond medical benefit and 40.8% of these oncologists have prescribed PC for the purpose of parental wishes to a recent patient. The chief aims in prescribing palliative chemotherapy were not achieved for recent patients.

http://www.ncbi.nlm.nih.gov/pubmed/23024072

Moving toward quality palliative cancer care: parent and clinician perspectives on gaps between what matters and what is accessible

Kassam, A., et al.

J Clin Oncol 2013; 31 (7): 910-915

PURPOSE: The National Consensus Project (NCP) published a set of standards for quality palliative care delivery. A key step before applying these guidelines to pediatric oncology is to evaluate how much families and clinicians value these standards. We aimed to determine which elements of palliative care are considered important according to bereaved parents and pediatric oncology clinicians and to determine accessibility of these elements. METHODS: We administered questionnaires to 75 bereaved parents (response rate, 54%) and 48 pediatric oncology clinicians (response rate, 91%) at a large teaching hospital. Outcome measures included importance ratings and accessibility of core elements of palliative care delivery. RESULTS: Fifteen of 20 core elements were highly valued by both parents and clinicians (defined as > 60% of parents and clinicians reporting the item as important). Compared with clinicians, parents gave higher ratings to receiving cancer-directed therapy during the last month of life ($P < .01$) and involvement of a spiritual mentor ($P = .03$). Of the valued elements, only three were accessible more than 60% of the time according to clinicians and parents. Valued elements least likely to be accessible included a direct admission policy to hospital, sibling support, and parent preparation for medical aspects surrounding death. CONCLUSION: Parents and clinicians highly value a majority of palliative care elements described in the NCP framework. Children with advanced cancer may not be receiving key elements of palliative care despite parents and clinicians recognizing them as important. Evaluation of barriers to provision of quality palliative care and strategies for overcoming them are critical.

http://www.ncbi.nlm.nih.gov/pubmed/23024072

SMA-EUROPE workshop report: Opportunities and challenges in developing clinical trials for spinal muscular atrophy in Europe

Kayadjanian, N., et al.

Orphanet J Rare Dis 2013; 8: 44

Spinal muscular atrophy (SMA) is the most common lethal recessive disease in childhood, and there is currently no effective treatment to halt disease progression. The translation of scientific advances into effective therapies is hampered by major roadblocks in clinical trials, including the complex regulatory environment in Europe, variations in standards of care, patient ascertainment and enrolment, a narrow therapeutic window and a lack of biomarkers of efficacy. In this context, SMA-Europe organized its first international workshop in July 2012 in Rome, gathering 34 scientists, clinicians and representatives of patient organizations to establish recommendations for improving clinical trials for SMA.

http://www.ncbi.nlm.nih.gov/pubmed/23514578

Babies are still dying of SIDS

Matthews, R. and A. Moore

Am J Nurs 2013; 113 (2): 59-64

OVERVIEW: The following account of a devastating, preventable death helped change licensing regulations and brought about a safer sleep environment for all infants in licensed child-care facilities in Arkansas. Co-author Andrea Moore responded to her own personal and family tragedy with a determination to save lives by reducing risk and promoting public awareness of sudden infant death syndrome (SIDS). She teamed up with co-author Rebecca Matthews, a community health nurse, and Rebecca's husband, pediatrician David Matthews, president of the Arkansas chapter of the American Academy of Pediatrics, to initiate a four-year public health intervention. This article provides information on SIDS, suggests ways that nurses can help educate caregivers and contribute to the prevention of SIDS, and details the progress of the team's educational initiative.

http://www.ncbi.nlm.nih.gov/pubmed/23358085

Clinical update: recognising brain tumours early in children

Paul, S. P., et al.

Community Pract 2013; 86 (4): 42-45

Brain tumour accounts for a quarter of all childhood cancers and is the leading cause of cancer related deaths in children. Initial symptoms can be misleading and is often misinterpreted as being caused by a less serious childhood illness. Available statistics show that it takes almost three times longer for the brain tumour in children to get diagnosed in the United Kingdom in comparison to other developed countries. Head Smart campaign was launched in the UK in 2011 with an aim to decrease the time from the onset of symptoms to diagnosis; initial results have been highly encouraging. Community practitioners play an important role in not only identifying symptoms (by following Head Smart symptom card) and selecting patients for reassurance, review or early referral but also by providing valuable support to the family post diagnosis in the community.

http://www.ncbi.nlm.nih.gov/pubmed/23646820

Not just little adults: palliative care physician attitudes toward pediatric patients

Rapoport, A., et al.

J Palliat Med 2013; 16 (6): 675-679

BACKGROUND: Palliative care physicians are increasingly being asked to provide end-of-life (EOL) care for children. Yet very little is known about physicians' level of comfort and willingness to do so. OBJECTIVES: This study assessed the attitudes of palliative care physicians toward providing care for pediatric patients and to describe the supports they desire in order to do so. METHODS: An online questionnaire was e-mailed to all physicians in the Division of Palliative Care at the University of Toronto. The questionnaire explored perceptions, attitudes, and level of comfort caring for pediatric patients. Results are reported using frequencies, ratios, and other descriptive analyses. RESULTS: Forty-four physicians of the 74 (59%) surveyed responded. On average, physicians cared for fewer than one child per each year of practice. Although the majority of respondents perceived their pediatric training to be inadequate, 70% were willing to provide care to children. Respondents felt at ease applying their knowledge and skills in some aspects of pediatric care (e.g., principles of pain and symptom management, communication about EOL issues) but less so in others (e.g., medication dosing, ethical issues unique to pediatrics). All respondents welcomed opportunities for additional training, but a third felt it was not essential. In particular, the most frequently expressed need was for mentorship by pediatric palliative care specialists. CONCLUSIONS: Palliative physicians tend to be willing to care for children, but perceive their level of training to be insufficient. Although additional training is endorsed, physicians favored real-time support and mentorship from a pediatric expert.

http://www.ncbi.nlm.nih.gov/pubmed/23445249

Repeating blood cultures in neutropenic children with persistent fevers when the initial blood culture is negative

Rosenblum, J., et al.

Pediatr Blood Cancer 2013; 60 (6): 923-927

BACKGROUND: Febrile neutropenia is a common reason for the hospitalization of pediatric oncology patients. The initiation of antibiotics and the overall decline in rates of bacteremia, would predict a low yield of detection of bacteremia in repeated blood cultures. Despite little evidence supporting the utility of serial cultures, repeat culturing with fever persists. PROCEDURE: To determine the rate of follow-up blood culture growth when the initial blood culture showed no bacterial growth and patient risk factors for this occurrence, we reviewed the records of oncology patients admitted to the Children's Hospital at Montefiore Pediatric Hematology/Oncology service for febrile neutropenia from 2004 to 2009. RESULTS: We identified 457 febrile neutropenia episodes in 137 patients. The initial blood culture was positive in 84 episodes (18.4%). In 220 episodes comprising 105 patients, the initial blood culture was negative and a subsequent culture was obtained. In 24 episodes (10.9%), bacterial growth was detected in the repeat culture. Risk factors included a previous history of bacteremia and hospitalization for more than 48 hours prior to onset of fever. CONCLUSIONS: In patients with febrile neutropenia, bacteremia is detected nearly twice as frequently in initial blood cultures than in repeat blood cultures obtained when the initial blood culture is negative. Despite an initial negative blood culture, bacteremia can be detected in more than 10% of episodes when a repeat blood culture is obtained. The risk more than doubles for patients with a previous history of bacteremia or hospitalized for more than 48 hours prior to the onset of fever.

http://www.ncbi.nlm.nih.gov/pubmed/23047811

Oral health assessment and mouth care for children and young people receiving palliative care. Part two

Sargeant, S. and C. Chamley

Nurs Child Young People 2013; 25 (3): 30-33

This is the second part of a two-part article on oral health assessment and mouth care for children and young people receiving palliative care. This article covers basic oral hygiene and management of oral health problems: oral candidiasis, coated tongue/dirty mouth, dry mouth, hypersalivation, ulceration, painful mouth, stomatitis and mucositis. The article also covers treating patients who are immunocompromised and the need to educate families and carers in the basic principles of oral care, including the importance of preventing cross-infection. Part one outlined oral assessment and discussed the adaptation of the Nottingham Oral Health Assessment Tool (Freer 2000).

http://www.ncbi.nlm.nih.gov/pubmed/23691903

Palliative care for children with cancer

Waldman, E. and J. Wolfe

Nat Rev Clin Oncol 2013; 10 (2): 100-107

Over the past two decades, paediatric palliative care has emerged as both a primary approach and as its own medical subspecialty, the overall aim of which is to ease suffering for children with life-threatening illness and their families through a concurrent model of care. However, most discussions have been focused on the transition to palliative care when no realistic hope for cure exists. We believe that, because the course of cancer is so unpredictable, this idea is misleading. Indeed, palliative care is increasingly being recognized as being about not just how to cope with the process of dying, but also about how to engage in living when faced with a life-threatening illness. This article will examine our current understanding of several areas of palliative care, with the ultimate message that palliative care is simply a novel term for the total care of a child and family, an approach that should be applied consistently and concurrently regardless of disease status. By improving familiarity with palliative care and building relationships with palliative care specialists, the paediatric oncology clinician will ensure that the best care possible for children and families is provided, regardless of outcome.

http://www.ncbi.nlm.nih.gov/pubmed/23337915

Neonatal palliative care

Walsh, H. and E. Molloy

Ir Med J 2013; 106 (2): 60-61.

http://www.ncbi.nlm.nih.gov/pubmed/23472393

Mortality risks in new-onset childhood epilepsy

Berg, A. T., et al.

Pediatrics 2013; 132 (1): 124-131

OBJECTIVES: Estimate the causes and risk of death, specifically seizure related, in children followed from onset of epilepsy and to contrast the risk of seizure-related death with other common causes of death in the population. METHODS: Mortality experiences from 4 pediatric cohorts of newly diagnosed patients were combined. Causes of death were classified as seizure related (including sudden unexpected death [SUDEP]), natural causes, nonnatural causes, and unknown. RESULTS: Of 2239 subjects followed up for >30 000 person-years, 79 died. Ten subjects with lethal neurometabolic conditions were ultimately excluded. The overall death rate (per 100 000 person-years) was 228; 743 in complicated epilepsy (with associated neurodisability or underlying brain condition) and 36 in uncomplicated epilepsy. Thirteen deaths were seizure-related (10 SUDEP, 3 other), accounting for 19% of all deaths. Seizure-related death rates were 43 overall, 122 for complicated epilepsy, and 14 for uncomplicated epilepsy. Death rates from other natural causes were 159, 561, and 9, respectively. Of 48 deaths from other natural causes, 37 were due to pneumonia or other respiratory complications. CONCLUSIONS: Most excess death in young people with epilepsy is not seizure-related. Mortality is significantly higher compared with the general population in children with complicated epilepsy but not uncomplicated epilepsy. The SUDEP rate was similar to or higher than sudden infant death syndrome rates. In uncomplicated epilepsy, sudden and seizure-related death rates were similar to or higher than rates for other common causes of death in young people (eg, accidents, suicides, homicides). Relating the risk of death in epilepsy to familiar risks may facilitate discussions of seizure-related mortality with patients and families.

http://www.ncbi.nlm.nih.gov/pubmed/23753097

Trends in mortality from leukemia in Europe: an update to 2009 and a projection to 2012

Bertuccio, P., et al.

Int J Cancer 2013; 132 (2): 427-436

We considered trends in mortality from leukemia in Europe over the period 1970-2009 using data from the World Health Organization. We computed age-standardized (world population) mortality rates, at all ages and in selected age groups, in 11 selected European countries, the European Union (EU) and, for comparative purposes, in the USA and Japan. For the EU, we also provided projections of the mortality to 2012. Over the period considered, mortality from leukemia steadily declined in most European countries in children and young adults, as well as in western and southern Europe at middle-age (45-69 years); in central/eastern Europe, reductions at ages 45-69 started since the mid-late 1990s. In the EU, annual percent changes were -3.7% in males and -3.8% in females at age 0-14, -2% in both sexes at age 15-44, and -0.6% in males and -1% in females at middle-age and overall. No decline was observed at age 70 or more. Between 1997 and 2007, overall EU rates decreased from 5.4 to 4.8/100,000 males and from 3.4 to 2.9/100,000 females. Declines were from 6.2 to 5.5/100,000 males and from 3.7 to 3.2/100,000 females in the USA and from 3.9 to 3.5/100,000 males and from 2.5 to 2.0/100,000 females in Japan. Projected overall rates in the EU at 2012 are 4.3/100,000 males (-11% compared to 2007) and 2.6/100,000 females (-12%).

http://www.ncbi.nlm.nih.gov/pubmed/22553155

A systematic review of treatment outcomes in pediatric patients with intracranial ependymomas

Cage, T. A., et al.

J Neurosurg Pediatr 2013; 11 (6): 673-681

OBJECT: Ependymoma is the third most common primary brain tumor in children. Tumors are classified according to the WHO pathological grading system. Prior studies have shown high levels of variability in patient outcomes within and across pathological grades. The authors reviewed the results from the published literature on intracranial ependymomas in children to describe clinical outcomes as they relate to treatment modality, associated mortality, and associated progression-free survival (PFS). METHODS: A search of English language peer-reviewed articles describing patients 18 years of age or younger with intracranial ependymomas yielded data on 182 patients. These patients had undergone treatment for ependymoma with 1 of 5 modalities: 1) gross-total resection (GTR), 2) GTR as well as external beam radiation therapy (EBRT), 3) subtotal resection (STR), 4) STR as well as EBRT, or 5) radiosurgery. Mortality and outcome data were analyzed for time to tumor progression in patients treated with 1 of these 5 treatment modalities. RESULTS: Of these 182 patients, 69% had supratentorial ependymomas and 31% presented with infratentorial lesions. Regardless of tumor location or pathological grade, STR was associated with the highest rates of mortality. In contrast, GTR was associated with the lowest rates of mortality, the best overall survival, and the longest PFS. Children with WHO Grade II ependymomas had lower mortality rates when treated more aggressively with GTR. However, patients with WHO Grade III tumors had slightly better survival outcomes after a less aggressive surgical debulking (STR+EBRT) when compared with GTR. CONCLUSIONS: Mortality, PFS, and overall survival vary in pediatric patients with intracranial ependymomas. Pathological classification, tumor location, and method of treatment play a role in outcomes. In this study, GTR was associated with the best overall and PFS rates. Patients with WHO Grade II tumors had better overall survival after GTR+EBRT and better PFS after GTR alone. Patients with WHO Grade III tumors had better overall survival after STR+EBRT. Patients with infratentorial tumors had improved overall survival compared with those with supratentorial tumors. Progression-free survival was best in those patients with infratentorial tumors following STR+EBRT. Consideration of all of these factors is important when counseling families on treatment options.

http://www.ncbi.nlm.nih.gov/pubmed/23540528

Congenital myotonic dystrophy: Canadian population-based surveillance study

Campbell, C., et al.

J Pediatr 2013; 163 (1): 120-125 e121-123

OBJECTIVES: To determine the incidence and neonatal morbidity and mortality of congenital myotonic dystrophy (CDM) in Canada. STUDY DESIGN: The study has 2 phases. A 5-year prospective monthly surveillance of incident cases of CDM conducted via the Canadian Pediatric Surveillance Program, from March 1, 2005-February 28, 2010, and a 5-year cohort study of eligible incident cases, which is ongoing and not the subject of this report. RESULTS: A total of 121 cases were reported, with 38 confirmed as CDM. The incidence of CDM in Canada is 2.1/100,000 (1/47,619) live births. The cases were reported from 8 provinces and 1 territory. The highest reported incidence was Ontario with 15, followed by British Columbia with 7, and Quebec with 6. External validation of cases was performed. The trinucleotide repeat level varied from 550-3100. Twenty-two (58%) of the children were the index cases for their families. Seventeen children are currently enrolled in the ongoing cohort study. CONCLUSION: Surveillance and prospective examination of CDM at a population level is important, as the impact of this rare disease is systemic, chronic, and associated with significant morbidity and mortality throughout childhood.

http://www.ncbi.nlm.nih.gov/pubmed/23415617

Polysomnographic findings in Rett syndrome: a case-control study

Carotenuto, M., et al.
Sleep Breath 2013; 17 (1): 93-98

PURPOSE: Rett syndrome is a severe neurodevelopmental disorder mainly affecting females and usually linked to mutations in the methyl-CpG-binding protein 2 gene, with an estimated prevalence of 1 in 10,000 live female births. Clinical features which usually become more apparent over time include breathing dysfunction, seizures, spasticity, peripheral vasomotor disturbance, scoliosis, growth retardation, and hypotrophic feet, with a great variety of presentations. The clear immaturity in brainstem mechanisms is expressed by the presence of early sleep disorders such as nocturnal awakenings, bruxism, and difficulty falling asleep, and no conclusive findings were derived from the few polysomnographic studies about the sleep macrostructural aspects. The aim of this study is to analyze the sleep macrostructural parameters, the nocturnal respiratory characteristic, and the presence of periodic limb movements in a sample of children affected by Rett syndrome. MATERIALS: Thirteen Rett subjects underwent a polysomnographic study, and the findings were compared with those obtained by a group of 40 healthy children. RESULTS: The Rett group shows a great impairment in sleep macrostructural and respiratory parameters, with a higher percentage of pathological periodic limb movements than the controls. CONCLUSIONS: This study may be considered a report about the ventilatory impairment during sleep in Rett syndrome and the first approach to the macrostructural aspects of sleep supported by the PSG data that could be considered mandatory for a better comprehension of this very complex syndrome.

http://www.ncbi.nlm.nih.gov/pubmed/22392651

Risk management protocol for gastrostomy and jejunostomy insertion in ventilator dependent infants

Chatwin, M., et al.
Neuromuscul Disord 2013; 23 (4): 289-297

Gastrostomy, gastrojejunostomy and anti-reflux surgery in infants and children who are chronically ventilator dependent are associated with significant risk of morbidity and mortality. We report outcomes of 22 high risk children who underwent these procedures at our centre. Pre-operative investigations included: overnight oxygen and carbon dioxide monitoring and subsequent optimisation of ventilatory support, echocardiography, video fluoroscopy, and assessment of gastroesophageal reflux. We carried out 24 procedures under general anaesthesia. Twenty-one children used ventilatory support pre-operatively. Median age of first surgical procedure was 18 months (range 3-180). Supplementary feeding was commenced in 20 children prior to procedure, median age 9 months (1-31). Median PICU length of stay was 1 (1-8) days. No children died in the post-operative period. Extubation was possible within 24h in 87% of cases. Complications included; atelectasis (n=2), ileus (n=2), abdominal distension (n=4) and loose stools (n=1). We conclude that, in this high risk cohort of ventilator dependent children with predominantly neuromuscular disorders, with careful assessment, operative intervention can be carried out under general anaesthesia, with the child being extubated early back onto their routine ventilatory support and aggressive airway clearance. Additionally this protocol can minimise post-operative complications and is associated with a good outcome in the majority.

http://www.ncbi.nlm.nih.gov/pubmed/23465657

Evaluation and treatment of the newborn with epidermolysis bullosa

Gonzalez, M. E.

Semin Perinatol 2013; 37 (1): 32-39

Epidermolysis bullosa (EB) is a heterogeneous group of inherited skin diseases characterized by increased skin fragility and variable degrees of extracutaneous involvement. The clinical spectrum ranges from localized skin disease to a life-threatening and disabling disease with extensive extracutaneous involvement. All four major types of EB, namely EB simplex, Junctional EB, Dystrophic EB and Kindler syndrome, can present with blistering and erosions at birth and cannot be distinguished clinically in the newborn period. The extensive differential diagnosis of blistering and erosions in the neonate must be considered and common etiologies ruled out. The diagnosis of EB can be confirmed via a skin biopsy for immunoflourescence mapping. This review discusses the four major subtypes of EB and their associated extracutaneous features. The evaluation of a newborn suspected of having EB, including diagnosis and management, is also reviewed.

http://www.ncbi.nlm.nih.gov/pubmed/22392651

Survival of patients with spinal muscular atrophy type 1

Gregoretti, C., et al.

Pediatrics 2013; 131 (5): e1509-1514

BACKGROUND: Spinal muscular atrophy type 1 (SMA1) is a progressive disease and is usually fatal in the first year of life. METHODS: A retrospective chart review was performed of SMA1 patients and their outcomes according to the following choices: letting nature take its course (NT); tracheostomy and invasive mechanical ventilation (TV); continuous noninvasive respiratory muscle aid (NRA), including noninvasive ventilation; and mechanically assisted cough. RESULTS: Of 194 consecutively referred patients enrolled in this study (103 males, 91 females), NT, TV, and NRA were chosen for 121 (62.3%), 42 (21.7%), and 31 (16%) patients, respectively. Survival at ages 24 and 48 months was higher in TV than NRA users: 95% (95% confidence interval: 81.8%-98.8%) and 67.7% (95% confidence interval: 46.7%-82%) at age 24 months ($P < .001$) and 89.43% and 45% at age 48 months in the TV and NRA groups, respectively ($P < .001$). The choice of TV decreased from 50% (1992-1998) to 12.7% (2005-2010) ($P < .005$) with a nonstatistically significant increase for NT from 50% to 65%. The choice of NRA increased from 8.1% (1999-2004) to 22.7% (2005-2010) ($P < .001$). CONCLUSIONS: Long-term survival outcome is determined by the choice of the treatment. NRA and TV can prolong survival, with NRA showing a lower survival probability at ages 24 and 48 months.

http://www.ncbi.nlm.nih.gov/pubmed/23610208

Respiratory and sleep disorders in female children with atypical Rett syndrome caused by mutations in the CDKL5 gene

Hagebeuk, E. E., et al.

Dev Med Child Neurol 2013; 55 (5): 480-484

AIM: In female children with drug-resistant seizures and developmental delay from birth, atypical Rett syndrome caused by mutations in the CDKL5 gene should be considered. Several clinical features resemble classic Rett syndrome. Respiratory and sleep abnormalities are frequently present in Rett syndrome, whereas little is known in patients with CDKL5 mutations. METHOD: In four genetically confirmed female patients with CDKL5 mutations (age range 2-15 y), the presence of breathing and sleep abnormalities was evaluated using the validated Sleep Disturbance Scale for Children and polysomnography (PSG). RESULTS: The Sleep Disturbance Scale for Children indicated disorders of initiating and maintaining sleep, daytime somnolence, and sleep breathing disorders. In one patient, PSG showed central apnoeas during sleep: her total apnoea-hypopnoea index (AHI) was 4.9, of which the central AHI was 3.4/h. When awake, central apnoeas were present in two of the four female children (central AHI 28/h and 41/h respectively), all preceded by hyperventilation. PSG showed low rapid eye movement (REM) sleep (9.7-18.3%), frequent awakenings, and low sleep efficiency (range 59-78%). INTERPRETATION: Episodic hyperventilation followed by central apnoeas was present while awake in two of four patients. This may indicate failure of brainstem respiratory centres. In addition, low REM sleep, frequent arousals (not caused by apnoeas/seizures), and low sleep efficiency were present. Similar to Rett syndrome, in patients with CDKL5 mutations PSG seems warranted to evaluate breathing and sleep disturbances.

http://www.ncbi.nlm.nih.gov/pubmed/23151060

Central venous catheters and catheter locks in children with cancer: a prospective randomized trial of taurolidine versus heparin

Handrup, M. M., et al.

Pediatr Blood Cancer 2013; 60 (8): 1292-1298

BACKGROUND: To determine if the catheter lock taurolidine can reduce the number of catheter-related bloodstream infections (CRBSI) in pediatric cancer patients with tunneled central venous catheters (CVC). PROCEDURE: During a study period of 34 months, 129 newly placed tunneled CVCs in 112 patients were randomly assigned to standard lock with heparin solution or experimental lock with a taurolidine solution (ClinicalTrials.gov Identifier NCT00735813). RESULTS: Sixty-five CVCs were included in the standard group and 64 CVCs in the experimental group. The groups were comparable regarding patients' characteristics. A total number of 72 bloodstream infections of which 33 were CRBSIs were observed during 39,127 CVC-days. A lower rate of CRBSI (0.4 per 1,000 CVC-days) was observed in the experimental arm compared with the standard arm (1.4 per 1,000 CVC-days, incidence rate ratio (IRR) = 0.26; 95% confidence interval (CI) 0.09-0.61; P = 0.001). A lower rate of total bloodstream infections (1.2 per 1,000 CVC-days) was also observed in the experimental arm compared with the standard arm (2.5 per 1,000 CVC-days, IRR = 0.49; 95% CI 0.29-0.82; P = 0.004). Median interval from catheter insertion until first CRBSI was significantly lower in the standard group (156 days, range 12-602) compared with the experimental group (300 days, range 12-1,176; P = 0.02). Premature removal of the CVC due to infection and overall CVC survival were similar in the two study groups. CONCLUSION: Locking of long-term tunneled CVC with taurolidine significantly reduces catheter-related bloodstream infections in children with cancer.

http://www.ncbi.nlm.nih.gov/pubmed/23417891

Cooling for newborns with hypoxic ischaemic encephalopathy

Jacobs, S. E., et al.

Cochrane Database Syst Rev 2013; 1: CD003311

BACKGROUND: Newborn animal studies and pilot studies in humans suggest that mild hypothermia following peripartum hypoxia-ischaemia in newborn infants may reduce neurological sequelae without adverse effects. OBJECTIVES: To determine the effect of therapeutic hypothermia in encephalopathic asphyxiated newborn infants on mortality, long-term neurodevelopmental disability and clinically important side effects. SEARCH METHODS: We used the standard search strategy of the Cochrane Neonatal Review Group as outlined in The Cochrane Library (Issue 2, 2007). Randomised controlled trials evaluating therapeutic hypothermia in term and late preterm newborns with hypoxic ischaemic encephalopathy were identified by searching the Oxford Database of Perinatal Trials, the Cochrane Central Register of Controlled Trials (CENTRAL, The Cochrane Library, 2007, Issue 2), MEDLINE (1966 to June 2007), previous reviews including cross-references, abstracts, conferences, symposia proceedings, expert informants and journal handsearching. We updated this search in May 2012. SELECTION CRITERIA: We included randomised controlled trials comparing the use of therapeutic hypothermia with standard care in encephalopathic term or late preterm infants with evidence of peripartum asphyxia and without recognisable major congenital anomalies. The primary outcome measure was death or long-term major neurodevelopmental disability. Other outcomes included adverse effects of cooling and 'early' indicators of neurodevelopmental outcome. DATA COLLECTION AND ANALYSIS: Four review authors independently selected, assessed the quality of and extracted data from the included studies. Study authors were contacted for further information. Meta-analyses were performed using risk ratios (RR) and risk differences (RD) for dichotomous data, and weighted mean difference for continuous data with 95% confidence intervals (CI). MAIN RESULTS: We included 11 randomised controlled trials in this updated review, comprising 1505 term and late preterm infants with moderate/severe encephalopathy and evidence of intrapartum asphyxia. Therapeutic hypothermia resulted in a statistically significant and clinically important reduction in the combined outcome of mortality or major neurodevelopmental disability to 18 months of age (typical RR 0.75 (95% CI 0.68 to 0.83); typical RD -0.15, 95% CI -0.20 to -0.10); number needed to treat for an additional beneficial outcome (NNTB) 7 (95% CI 5 to 10) (8 studies, 1344 infants). Cooling also resulted in statistically significant reductions in mortality (typical RR 0.75 (95% CI 0.64 to 0.88), typical RD -0.09 (95% CI -0.13 to -0.04); NNTB 11 (95% CI 8 to 25) (11 studies, 1468 infants) and in neurodevelopmental disability in survivors (typical RR 0.77 (95% CI 0.63 to 0.94), typical RD -0.13 (95% CI -0.19 to -0.07); NNTB 8 (95% CI 5 to 14) (8 studies, 917 infants). Some adverse effects of hypothermia included an increase sinus bradycardia and a significant increase in thrombocytopenia. AUTHORS' CONCLUSIONS: There is evidence from the 11 randomised controlled trials included in this systematic review (N = 1505 infants) that therapeutic hypothermia is beneficial in term and late preterm newborns with hypoxic ischaemic encephalopathy. Cooling reduces mortality without increasing major disability in survivors. The benefits of cooling on survival and neurodevelopment outweigh the short-term adverse effects. Hypothermia should be instituted in term and late preterm infants with moderate-to-severe hypoxic ischaemic encephalopathy if identified before six hours of age. Further trials to determine the appropriate techniques of cooling, including refinement of patient selection, duration of cooling and method of providing therapeutic hypothermia, will refine our understanding of this intervention.

http://www.ncbi.nlm.nih.gov/pubmed/23440789

Future of clinical genomics in pediatric oncology

Janeway, K. A., et al.
J Clin Oncol 2013; 31 (15): 1893-1903

The somatic genomic alterations in pediatric cancers to some extent overlap with those seen in adult cancers, but the exact distribution throughout the genome and the types and frequency of alterations differ. The ultimate goal of genomic research in children, as with adults, is translation to the clinic to achieve more accurate diagnosis, more precise risk stratification, and more effective, less toxic therapy. The genomic features of pediatric malignancies and pediatric-specific issues in clinical investigation may make translating genomic discoveries to the clinic more difficult. However, through large-scale molecular profiling of pediatric tumors, continued coordinated efforts to evaluate novel therapies in the pediatric population, thoughtful phase II and III trial design, and continued drug development, genomically based therapies will become more common in the pediatric oncology clinic in the future.

http://www.ncbi.nlm.nih.gov/pubmed/23589558

Risk factors for urolithiasis in gastrostomy tube fed children: a case-control study

Johnson, E. K., et al.
Pediatrics 2013; 132 (1): e167-174

BACKGROUND AND OBJECTIVE: Pediatric patients who are fed primarily via gastrostomy tube (G-tube) may be at increased risk for urolithiasis, but no studies have specifically examined risk factors for stones in this population. We aimed to determine clinical differences between G-tube fed (GTF) patients with and without stones, in hopes of identifying modifiable factors associated with increased risk of urolithiasis. METHODS: We conducted a retrospective case-control study, matching GTF patients with urolithiasis (cases) to GTF children without urolithiasis (controls) based on age (+/-1 year) and gender. Bivariate comparisons and matched logistic regression modeling were used to determine the unadjusted and adjusted associations between relevant clinical factors and urolithiasis. RESULTS: Forty-one cases and 80 matched controls (mean age 12.0 +/- 6.5 years) were included. On bivariate analysis, factors associated with stone formation included: white race, urinary tract infection (UTI), topiramate administration, vitamin D use, malabsorption, dehydration, 2-year duration with G-tube, and whether goal free water intake was documented in the patient chart. On regression analysis, the following factors remained significant: topiramate administration (odds ratio [OR]: 6.58 [95% confidence interval (CI): 1.76-24.59]), UTI (OR: 7.70 [95% CI: 1.59-37.17]), and <2 years with a G-tube (OR: 8.78 [95% CI: 1.27-52.50]). CONCLUSIONS: Our findings provide a preliminary risk profile for the development of urolithiasis in GTF children. Important associations identified include UTI, topiramate administration, and shorter G-tube duration, which may reflect subclinical chronic dehydration. Of these, topiramate use represents the most promising target for risk reduction.

http://www.ncbi.nlm.nih.gov/pubmed/23753093

Childhood, adolescents, and young adults (</=25 y) colorectal cancer: study of Anatolian Society of Medical Oncology

Kaplan, M. A., et al.

J Pediatr Hematol Oncol 2013; 35 (2): 83-89

PURPOSE: To evaluate the clinicopathologic characteristics and treatment outcomes of young patients with colorectal cancer (CRC). METHODS: Between May 2003 and June 2010, 76 patients were found eligible for this retrospective study. Age, sex, presenting symptoms, patients with acute presentation, family history, presence of polyps, histologic features, localization and stage of the tumor, treatment outcomes, time and site of recurrence, sites of metastasis, and survival outcomes were recorded from the patient files. RESULTS: Seventy-six patients (55.3% male) with a median age of 23 years were evaluated. Patients were evaluated in 2 groups as follows: child-adolescent (0 to 19 y, n=20) and young adult (20 to 25 y, n=56). Sex and symptoms (abdominal pain and rectal bleeding) were significantly differed between the groups and acute presentation was close to statistical significance. Overall survival significantly increased in patients undergoing curative surgery (P<0.001). Other parameters affecting the survival was stage of disease (P=0.004). Response to palliative chemotherapy in metastatic patients (P=0.042) and postoperative adjuvant chemotherapy had a statistically significant survival advantage (P=0.028). CONCLUSIONS: Diagnosis of CRC should not be excluded solely on the basis of age. CRC features in young-adult patients are more similar to adults compared with that of child-adolescent patients according to the symptoms and presentation. In patients with CRC in this age group, curative surgery, adjuvant chemotherapy, and palliative chemotherapy provide survival advantage.

http://www.ncbi.nlm.nih.gov/pubmed/23337551

Prognostic factors in adolescents and young adults (AYA) with high risk soft tissue sarcoma (STS) treated by adjuvant chemotherapy: a study based on pooled European Organisation for Research and Treatment of Cancer (EORTC) clinical trials 62771 and 62931

Kasper, B., et al.

Eur J Cancer 2013; 49 (2): 449-456

BACKGROUND: We conducted a retrospective study, pooling data from two clinical trials in high risk soft tissue sarcoma (STS) patients, with the objective of comparing two different age groups: 15-29 years (adolescents and young adults (AYA) population) and >/= 30 years. The aim was to determine prognostic factors for the AYA population. METHODS: Patients selected for analysis were treated in two randomised trials of adjuvant chemotherapy in STS (European Organisation for Research and Treatment of Cancer (EORTC) 62771 and 62931). A total of 793 patients were included with a median follow-up (FU) of 8.74 years (AYA population: n=161, median FU 9.46 years; patients >/= 30 years: n=632, median FU 8.62 years). Study endpoints were overall survival (OS) and relapse-free survival (RFS). The variables of the multivariate analysis were gender, subtype and grade, tumour size and localisation (limb versus other), absence or presence of local recurrence and treatment (control arm versus adjuvant chemotherapy). RESULTS: Patients' characteristics were globally similar with two exceptions, histological subtype (p=0.0043) and tumour size (p<.0001). The commonest sarcoma subtype in the AYA population was synovial sarcoma (29%), whereas leiomyosarcoma (18%), malignant fibrous histiocytoma (MFH, presently being termed undifferentiated pleomorphic sarcoma (UPS), 16%) and liposarcoma (15%) were more frequent in patients >/= 30 years. For OS, independent favourable prognostic factors were low grade and small tumour size for both groups; radical resection and MFH or liposarcoma subtype were favourable factors for patients >/= 30 years only. For RFS, favourable prognostic factors were small tumour size and low grade for both groups; tumour location in the extremities was a favourable factor for the AYA population only, whereas radical resection and adjuvant chemotherapy treatment were favourable factors for patients >/= 30y ears only. CONCLUSIONS: Significant differences could be found concerning prognostic factors between the AYA population and older patients. Interestingly, adjuvant chemotherapy was associated with improved RFS only in patients >/= 30 years. The results may have further implications for the treatment of STS patients in different age groups, as well as the design of future clinical trials.

http://www.ncbi.nlm.nih.gov/pubmed/22975215

Clinical spectrum of early onset epileptic encephalopathies caused by KCNQ2 mutation

Kato, M., et al.

Epilepsia 2013; 54 (7): 1282-1287

PURPOSE: KCNQ2 mutations have been found in patients with benign familial neonatal seizures, myokymia, or early onset epileptic encephalopathy (EOEE). In this study, we aimed to delineate the clinical spectrum of EOEE associated with KCNQ2 mutation. METHODS: A total of 239 patients with EOEE, including 51 cases with Ohtahara syndrome and 104 cases with West syndrome, were analyzed by high-resolution melting (HRM) analysis or whole-exome sequencing. Detailed clinical information including electroencephalography (EEG) and brain magnetic resonance imaging (MRI) were collected from patients with KCNQ2 mutation. KEY FINDINGS: A total of nine de novo and one inherited mutations were identified (two mutations occurred recurrently). The initial seizures, which were mainly tonic seizures, occurred in the early neonatal period in all 12 patients. A suppression-burst pattern on EEG was found in most. Only three patients showed hypsarrhythmia on EEG; eight patients became seizure free when treated with carbamazepine, zonisamide, phenytoin, topiramate, or valproic acid. Although the seizures were relatively well controlled, moderate-to-profound intellectual disability was found in all except one patient who died at 3 months. SIGNIFICANCE: De novo KCNQ2 mutations are involved in EOEE, most of which cases were diagnosed as Ohtahara syndrome. These cases showed distinct features with early neonatal onset, tonic seizures, a suppression-burst EEG pattern, infrequent evolution to West syndrome, and good response to sodium channel blockers, but poor developmental prognosis. Genetic testing for KCNQ2 should be considered for patients with EOEE.

http://www.ncbi.nlm.nih.gov/pubmed/23621294

Cardiac rhabdomyomas associated with tuberous sclerosis complex in 11 children: presentation to outcome

Kocabas, A., et al.

Pediatr Hematol Oncol 2013; 30 (2): 71-79

Cardiac rhabdomyomas (CRs) are the most common heart tumors in children and closely associated with tuberous sclerosis complex (TSC). This study was performed to assess the presentation type, clinical course, treatment modalities, and outcome of the patients with rhabdomyoma, associated with TSC. We reviewed our patients with cardiac rhabdomyomas (CRs), who had received a diagnosis of TSC previously or during the follow-up period between June 1996 and January 2012, retrospectively. Thirty-two patients with TSC were evaluated and among them 11 patients (34%) were associated with CRs. Five patients (45%) had multiple tumors and consequently a total of 29 CRs were analyzed in our study. The median follow-up period was 2 years (range: 1 week-15 years). Clinical presentation was cardiac murmur in three patients, cyanosis in two patients and arrhythmia in one patient. Five patients were asymptomatic at the diagnosis and CRs were detected during routine cardiac evaluation for TSC. Cardiac tumors were diagnosed prenatally in two patients. Spontaneous regression rate was 31% and we experienced a complete regression of a tumor with an echogenic bordered tissue defect and septal thinning in a patient. Three patients had hemodynamically significant tumor obstruction; two of them underwent surgery. The other patient, who had multiple CRs, was treated medically with everolimus because of high-risk potential of surgery. Although surgical resection is the preferred treatment in most of the patients with hemodynamic instability, we need novel alternative medical therapies in some critically ill patients who cannot be operated due to various reasons.

http://www.ncbi.nlm.nih.gov/pubmed/23151153

Gene and cell-mediated therapies for muscular dystrophy

Konieczny, P., et al.
Muscle Nerve 2013; 47 (5): 649-663

Duchenne muscular dystrophy (DMD) is a devastating muscle disorder that affects 1 in 3,500 boys. Despite years of research and considerable progress in understanding the molecular mechanism of the disease and advancement of therapeutic approaches, there is no cure for DMD. The current treatment options are limited to physiotherapy and corticosteroids, and although they provide a substantial improvement in affected children, they only slow the course of the disorder. On a more optimistic note, more recent approaches either significantly alleviate or eliminate muscular dystrophy in murine and canine models of DMD and importantly, many of them are being tested in early phase human clinical trials. This review summarizes advancements that have been made in viral and nonviral gene therapy as well as stem cell therapy for DMD with a focus on the replacement and repair of the affected dystrophin gene.

http://www.ncbi.nlm.nih.gov/pubmed/23553671

Trends in survival among children with Down syndrome in 10 regions of the United States

Kucik, J. E., et al.
Pediatrics 2013; 131 (1): e27-36

OBJECTIVE: This study examined changes in survival among children with Down syndrome (DS) by race/ethnicity in 10 regions of the United States. A retrospective cohort study was conducted on 16,506 infants with DS delivered during 1983-2003 and identified by 10 US birth defects monitoring programs. Kaplan-Meier survival probabilities were estimated by select demographic and clinical characteristics. Adjusted hazard ratios (aHR) were estimated for maternal and infant characteristics by using Cox proportional hazard models. RESULTS: The overall 1-month and 1-, 5-, and 20-year survival probabilities were 98%, 93%, 91%, and 88%, respectively. Over the study period, neonatal survival did not improve appreciably, but survival at all other ages improved modestly. Infants of very low birth weight had 24 times the risk of dying in the neonatal period compared with infants of normal birth weight (aHR 23.8; 95% confidence interval [CI] 18.4-30.7). Presence of a heart defect increased the risk of death in the postneonatal period nearly fivefold (aHR 4.6; 95% CI 3.9-5.4) and continued to be one of the most significant predictors of mortality through to age 20. The postneonatal aHR among non-Hispanic blacks was 1.4 (95% CI 1.2-1.8) compared with non-Hispanic whites and remained elevated by age 10 (2.0; 95% CI 1.0-4.0). CONCLUSIONS: The survival of children born with DS has improved and racial disparities in infant survival have narrowed. However, compared with non-Hispanic white children, non-Hispanic black children have lower survival beyond infancy. Congenital heart defects are a significant risk factor for mortality through age twenty.

http://www.ncbi.nlm.nih.gov/pubmed/23248222

Vagus nerve stimulation vs. corpus callosotomy in the treatment of Lennox-Gastaut syndrome: a meta-analysis

Lancman, G., et al.

Seizure 2013; 22 (1): 3-8

PURPOSE: Lennox-Gastaut syndrome (LGS) is an epileptogenic disorder that arises in childhood and is typically characterized by multiple seizure types, slow spike-and-wave complexes on EEG and cognitive impairment. If medical treatment fails, patients can proceed to one of two palliative surgeries, vagus nerve stimulation (VNS) or corpus callosotomy (CC). Their relative seizure control rates in LGS have not been well studied. The purpose of this paper is to compare seizure reduction rates between VNS and CC in LGS using meta-analyses of published data. METHODS: A systematic search of Pubmed, Ovidsp, and Cochrane was performed to find articles that met the following criteria: (1) prospective or retrospective study, (2) at least one patient diagnosed with Lennox-Gastaut syndrome, and (3) well-defined measure of seizure frequency reduction. Seizure reduction rates were divided into seizure subtypes, as well as total seizures, and categorized as 100%, >75%, and >50%. Patient groups were compared using chi-square tests for categorical variables and t-test for continuous measures. Pooled proportions with 95% confidence interval (95% CI) of seizure outcomes were estimated for total seizures and seizure subtypes using random effects methods. RESULTS: 17 VNS and 9 CC studies met the criteria for inclusion. CC had a significantly better outcome than VNS for >50% atonic seizure reduction (80.0% [67.0-90.0%] vs. 54.1% [32.1-75.4%], $p<0.05$) and for >75% atonic seizure reduction (70.0% [48.05-87.0%] vs. 26.3% [5.8-54.7%], $p<0.05$). All other seizure types, as well as total number of seizures, showed no statistically significant difference between VNS and CC. CONCLUSIONS: CC may be more beneficial for LGS patients whose predominant disabling seizure type is atonic. For all other seizure types, VNS offers comparable rates to CC.

http://www.ncbi.nlm.nih.gov/pubmed/23068970

Glucocorticoid treatment for the prevention of scoliosis in children with Duchenne muscular dystrophy: long-term follow-up

Lebel, D. E., et al.

J Bone Joint Surg Am 2013; 95 (12): 1057-1061

BACKGROUND: Duchenne muscular dystrophy, a progressive muscle disorder that occurs in males, causes a gradual decline in muscle strength. This progressive decline is associated with the development of scoliosis. Previous studies have shown that the use of glucocorticoids slows the progression of scoliosis, but it is unknown if the spine remains straight in the long term. We examined if glucocorticoid treatment has a long-term effect on the prevalence of scoliosis. METHODS: Fifty-four boys who had been diagnosed with Duchenne muscular dystrophy while they were still walking were enrolled in a non-randomized comparative study of the glucocorticoid deflazacort. The families of thirty boys elected for them to use glucocorticoid treatment and the families of twenty-four boys elected for them not to have this treatment. The boys were matched for important baseline characteristics including age and pulmonary function. Every four to six months, they were examined for the development of scoliosis, and the duration of follow-up for surviving patients was fifteen years. Because surgery was recommended for spinal curves measuring >20 degrees on sitting posteroanterior radiographs, a curve of this magnitude was used as the definition for a patient developing scoliosis. RESULTS: Five boys (21%) in the non-treatment group and one boy (3%) in the glucocorticoid treatment group died. At the most recent follow-up, of the boys who survived, six (20%) in the glucocorticoid treatment group and twenty-two (92%) in the non-treatment group developed scoliosis and underwent spinal surgery. After fifteen years of follow-up, the survivorship analysis (avoiding surgery) was 78% (95% confidence interval, 57% to 89%) in the treatment group and 8.3% (95% confidence interval, 0.8% to 28%) in the non-treatment group. Significance (p = 5.8 x 10(-7)) was calculated with log-rank and chi-square tests. None of the patients in the glucocorticoid group developed scoliosis after ten years of deflazacort treatment. CONCLUSION: The long-term use of the glucocorticoid results in a substantial decreased need for spinal surgery to treat scoliosis.

http://www.ncbi.nlm.nih.gov/pubmed/23783200

Treatment of children with acute myeloid leukaemia who relapsed after allogeneic haematopoietic stem cell transplantation

Lee, J. W., et al.

Br J Haematol 2013; 160 (1): 80-86

Despite improvements in diagnosis and treatment, 30-40% of children with acute myeloid leukaemia (AML) experience relapse. For those who relapse after allogeneic haematopoietic stem cell transplantation (allo-HSCT), the prognosis is particularly poor, with limited reported literature on these patients. We reviewed the clinical course of 49 children with AML (28 males, 21 females) who received allo-HSCT between 1993 and 2011, and who had subsequently relapsed. Study endpoints included (i) complete remission (CR) rate after intensive chemotherapy, and prognostic factors for CR, (ii) disease-free survival (DFS) and overall survival (OS) for patients who achieved CR and (iii) OS for recipients of intensive chemotherapy and prognostic factors for OS. Of the 36 patients who received intensive chemotherapy after post-HSCT relapse, 26 (72%) achieved CR. For patients who achieved CR, 5-year DFS and OS were 32.6 +/- 10.2% and 44.4 +/- 11.1%, respectively. For all recipients of intensive chemotherapy, 5-year OS was 31.6 +/- 8.7%. Cumulative incidence of treatment-related death was 14.4%. All three recipients of second HSCT died. Amongst prognostic factors predicting improved survival, only disease status at HSCT (early first CR vs. others) proved significant in multivariate study (Hazard Ratio 2.42, 95% Confidence Interval 1.02-5.74, P = 0.045). Treatment with curative intent was able to salvage a minor but important subset of children with AML who relapsed post-allogeneic transplant.

http://www.ncbi.nlm.nih.gov/pubmed/23106148

Epilepsy in four genetically determined syndromes of intellectual disability

Leung, H. T. and H. Ring

J Intellect Disabil Res 2013; 57 (1): 3-20

BACKGROUND: Epilepsy occurs with increased frequency in people with an intellectual disability (ID) compared to the rest of the population. A variety of research has in recent years shed light on genetic and biochemical aetiologies of epilepsy and, often in a different literature, on syndromes of ID. The aims of this annotation are to review developments in understanding of the pathophysiology of several ID syndromes in which epilepsy is a frequent co-occurrence and to relate these observations to recent advances in understanding of how these pathophysiological disturbances may lead to epilepsy. METHOD: The ID syndromes selected for review were fragile X (FXS), Rett (RTT) and Angelman syndromes (AS) and tuberous sclerosis complex (TSC). Epilepsy is a significant aspect of these syndromes and relevant research into the genetic and biochemical pathophysiology of these four ID syndromes may be informative in establishing the association between epilepsy and ID. Employing a structured approach the authors initially searched the PubMed database for large case series describing the characteristics of epilepsy as manifested in these ID syndromes. The criteria for inclusion of the case series in the review were a sample size of greater than 50 and the description of several of the characteristic features of epilepsy, namely prevalence of seizures, age of seizure onset, seizure frequency, seizure semiology, severity and treatment. Following this, studies of the genetic and biochemical pathophysiology of these four ID syndromes were reviewed and the potential relevance of this research in understanding the association with epilepsy highlighted. Findings were considered in a focused manner in terms of effects on excitatory and inhibitory neurotransmitter systems and on glial function. RESULTS: Diverse genetic pathologies underlying several ID syndromes can lead to alterations in the functioning of the glutamatergic and GABAergic neurotransmitter systems. The mechanisms involved include transcriptional regulation in RTT, translational regulation in FXS and TSC, and UBE3A-mediated proteolysis in AS. Expression or functioning of receptor subunits, uptake sites and enzymes involved in neurotransmitter metabolism are often affected by these changes, and may lead to modifications in network excitability and neuronal plasticity that may contribute to epileptogenesis and ID. Dysfunction in astrocytes may also contribute to epileptogenesis and ID in FXS, RTT and TSC with potential mechanisms including failure of astrocytic support functions, glial inflammation and homeostatic disturbances that affect the excitability and architecture of neuronal networks. CONCLUSIONS: The annotation highlights research describing disturbances in excitatory and inhibitory neurotransmitter systems, neuronal ion channel and glial functions that provide possible explanations for the co-occurrence of seizures within several ID syndromes, in some cases suggesting possible avenues for research into novel therapeutic targets. Phenotypic overlaps between syndromes may also relate to roles for the implicated genes in different disturbances in linked biochemical pathways.

http://www.ncbi.nlm.nih.gov/pubmed/22142420

An epidemiological study of factors associated with preterm infant in-hospital mortality

Lutomski, J., et al.

Ir Med J 2013; 106 (1): 9-12

Nationally representative in-hospital mortality rates among preterm infants are essentially unknown in Ireland. We examined preterm infants born in hospital and admitted to intensive care unit (ICU) between 2005 and 2008. Unadjusted incidence rates and risk ratios were derived. Overall, 6,599 preterm infants were admitted to ICU of whom 256 (3.9%) died prior to hospital discharge. Infants with a birthweight less than 1,000 g were 18.1 (95% CI 12.1-27.1) times more likely to die in hospital. Mortality was high among preterm infants diagnosed with Grade 3/4 intra-ventricular haemorrhage (43.6 deaths per 100 cases; 95% CI 31.0-56.7). Congenital anomaly diagnosis was associated with a five-fold increased risk (RR 5.1; 95% CI 4.0-6.6) of in-hospital mortality. Our population-based study provides reliable estimates of in-hospital mortality among preterm infants admitted to Irish ICUs.

http://www.ncbi.nlm.nih.gov/pubmed/23472368

The cooperative international neuromuscular research group Duchenne natural history study--a longitudinal investigation in the era of glucocorticoid therapy: design of protocol and the methods used

McDonald, C. M., et al.

Muscle Nerve 2013; 48 (1): 32-54

Contemporary natural history data in Duchenne muscular dystrophy (DMD) is needed to assess care recommendations and aid in planning future trials. METHODS: The Cooperative International Neuromuscular Research Group (CINRG) DMD Natural History Study (DMD-NHS) enrolled 340 individuals, aged 2-28 years, with DMD in a longitudinal, observational study at 20 centers. Assessments obtained every 3 months for 1 year, at 18 months, and annually thereafter included: clinical history; anthropometrics; goniometry; manual muscle testing; quantitative muscle strength; timed function tests; pulmonary function; and patient-reported outcomes/health-related quality-of-life instruments. RESULTS: Glucocorticoid (GC) use at baseline was 62% present, 14% past, and 24% GC-naive. In those >/=6 years of age, 16% lost ambulation over the first 12 months (mean age 10.8 years). CONCLUSIONS: Detailed information on the study methodology of the CINRG DMD-NHS lays the groundwork for future analyses of prospective longitudinal natural history data. These data will assist investigators in designing clinical trials of novel therapeutics.

http://www.ncbi.nlm.nih.gov/pubmed/23677550

Mortality risk among children initially treated with dialysis for end-stage kidney disease, 1990-2010

Mitsnefes, M. M., et al.

JAMA 2013; 309 (18): 1921-1929

IMPORTANCE: Most children with end-stage kidney disease (ESKD) are treated with dialysis prior to transplant. It is not known whether their outcomes have changed in recent years. OBJECTIVE: To determine if all-cause, cardiovascular, and infection-related mortality rates for children and adolescents beginning dialysis improved between 1990 and 2010. DESIGN, SETTING, AND PARTICIPANTS: Retrospective cohort study of patients younger than 21 years initially treated with dialysis for ESKD, recorded in the United States Renal Data System between 1990 and 2010. Children with a prior kidney transplant were excluded. We used Cox proportional hazard models to estimate the hazard ratios (HRs) for mortality associated with a 5-year increment in year of ESKD treatment initiation. Primary analyses censored observation at kidney transplant. MAIN OUTCOMES AND MEASURES: All-cause, cardiovascular, and infection-related mortality. RESULTS: A total of 3450 children younger than 5 years and 19,951 children 5 years or older started dialysis from 1990-2010. Of those younger than 5 years, 705 died during dialysis treatment (98.8/1000 person-years); mortality rates were 112.2 and 83.4 per 1000 person-years in those initiating dialysis in 1990-1994 and 2005-2010, respectively. Of those 5 years and older at treatment initiation, 2270 died during dialysis treatment (38.6/1000 person-years). Their mortality rates were 44.6 and 25.9 per 1000 person-years in those initiating dialysis in 1990-1994 and 2005-2010, respectively. Each 5-year increment in calendar year of dialysis initiation was associated with an adjusted HR of 0.80 (95% CI, 0.75-0.85) among children younger than 5 years at initiation and an HR of 0.88 (95% CI, 0.85-0.92) among those 5 years and older. RESULTS: A total of 23,401 children and adolescents who initiated ESKD treatment with dialysis at younger than 21 years between 1990 and 2010 were identified. Crude mortality rates during dialysis treatment were higher among children younger than 5 years at the start of dialysis compared with those who were 5 years and older. Mortality rates for both children and adolescents being treated for ESKD with dialysis decreased significantly between 1990 and 2010. CONCLUSIONS AND RELEVANCE: In the United States, there was a substantial decrease in mortality rates over time among children and adolescents initiating ESKD treatment with dialysis between 1990 and 2010. Further research is needed to determine the specific factors responsible for this decrease.

http://www.ncbi.nlm.nih.gov/pubmed/23645144

Fontan circulation: success or failure?

Mondesert, B., et al.
Can J Cardiol 2013; 29 (7): 811-820

Fontan surgery represents a milestone in the evolution of congenital heart disease management. It achieved the seemingly improbable (ie, restoration of a noncyanotic state by entirely bypassing the subpulmonary ventricle). In so doing, it has allowed a generation of children who may have otherwise succumbed to their severe congenital heart defect to survive to adulthood. Perfect univentricular physiology is, however, an elusive goal. The Fontan circulation inherently represents a hemodynamic compromise that results in a catalog of potential multiorgan complications. In this review, we explore current knowledge regarding pathophysiology of the failing Fontan, its varied clinical manifestations, and potential therapeutic options. Failure of the Fontan circulation is broadly divided into 3 overlapping categories: ventricular dysfunction, systemic complications of Fontan physiology, and chronic Fontan failure. As long as the Fontan operation continues to serve as the paradigm of care for patients with univentricular hearts, efforts must be directed toward supporting this dynamic circulation that progressively declines in efficiency with age. Continued research in therapies is needed for univentricular dysfunction and systemic complications of Fontan palliation, including potential uses of mechanical support as a bridge to transplantation or as a neosubpulmonary ventricle. Fontan patients remain a major challenge to the medical and surgical community as a whole. Multicentre and multidisciplinary efforts to improve the density and depth of experiences might lead to a better appreciation for, and management of, Fontan failure and its ramifications.

http://www.ncbi.nlm.nih.gov/pubmed/23474138

Falls and spinal muscular atrophy: exploring cause and prevention

Montes, J., et al.
Muscle Nerve 2013; 47 (1): 118-123

INTRODUCTION: Falls can cause injury and may compromise function in spinal muscular atrophy (SMA) patients. Weakness and gait variability are associated with falls in other neurological disorders, and fatigue is well documented in SMA. The relationship of weakness, fatigue, and gait variability to falls has never been investigated. METHODS: Seven ambulatory patients with SMA completed a falls history questionnaire, 6MWT, gait analysis, and strength testing. Pearson correlation coefficients were used to examine associations between these variables. RESULTS: All 7 subjects reported falls in the previous year. Stride-length variability was significantly associated with falls, unlike strength, fatigue, or other gait variables. CONCLUSIONS: Stride-length variability was the key variable associated with falls. Preventive strategies to avoid falls should be incorporated into patient management plans. Gait analysis provides actionable information not revealed by standard assessments and should be included in clinical trials designed to address the prevention of falls in the SMA population.

http://www.ncbi.nlm.nih.gov/pubmed/23042039

Effect of home mechanical in-exsufflation on hospitalisation and life-style in neuromuscular disease: a pilot study

Moran, F. C., et al.

J Paediatr Child Health 2013; 49 (3): 233-237

AIM: Mechanical in-exsufflation (MI-E) augments the weakened cough of patients with neuromuscular disease (NMD), clearing secretions and overcoming atelectasis. Little has been published on the impact of MI-E alone on rates of hospitalisation and quality of life (QOL). The aim of this study was to assess the impact of home MI-E on hospital admissions and life-style in children with NMD. METHODS: A retrospective chart review was performed on children using MI-E, including data on the number of admissions to hospital, length of stay and hours of ventilation. A parental survey was used to gather information on the impact of MI-E on life-style for the child and family. RESULTS: Ten children with NMD (seven spinal muscular atrophy, two Duchenne muscular dystrophy and one centronuclear myopathy) using MI-E at home were identified. MI-E use commenced at mean age of 8.5 years (range 1.1-16.9) with 1.4 years of use (range 0.3-3.8). MI-E pressures ranged from +/-30 to 40 cmH2 O with no complications reported. There was a significant reduction in hospital days at 6 (P = 0.036) and 12 (P = 0.028) months following commencement of home MI-E compared with the same period preceding MI-E use. The survey highlighted positive benefits of MI-E use, in particular the ability to treat many pulmonary exacerbations at home. CONCLUSIONS: Home MI-E use by children with NMD can reduce hospitalisation and benefit families by maintaining their child at home.

http://www.ncbi.nlm.nih.gov/pubmed/23438093

Constipation in children with neurofibromatosis type 1

Pedersen, C. E., et al.

J Pediatr Gastroenterol Nutr 2013; 56 (2): 229-232

BACKGROUND AND OBJECTIVES: Neurofibromatosis type 1 (NF1) is a hereditary, heterogenic, and multiorganic disease. The NF1 phenotype shows great variability in expressivity and often includes symptoms from the central and peripheral nervous systems. Bowel symptoms have been reported, but gastrointestinal function in NF1 remains to be described in detail. In this first systematic study of bowel function in children with NF1, we aimed to investigate symptoms of constipation and test the hypotheses that children with NF1 have abnormally large rectum and prolonged colonic transit time (CTT). METHODS: A total of 20 children with NF1 (age 8.2 +/- 2.4 years) were evaluated with medical history; clinical examination; digital rectal examination; bowel and dietary diaries; Rome III criteria; measurement of rectal diameter by transabdominal ultrasound; and radiographic estimation of CTT. The control group for assessment of rectal diameter comprised 23 healthy children (mean age 9.1 +/- 2.7 years). RESULTS: A total of 6 children with NF1 (30%) were constipated according to Rome III criteria. Average rectal diameter was significantly larger than for healthy children (32.9 +/- 7.2 mm vs 21.4 +/- 5.9 mm, P < 0.0001). Median CTT in NF1 children was 53 hours (range 26-101). Compared with existing normative data, CTT was prolonged (>84 hours) in 3 (19%). CONCLUSIONS: Symptoms of constipation were surprisingly common in children with NF1. Correspondingly, rectal diameters were abnormally large and a higher proportion than expected had prolonged CTT. The underlying pathophysiology remains obscure, but we hypothesise that abnormalities of the enteric nervous system or disturbed cellular growth could be present.

http://www.ncbi.nlm.nih.gov/pubmed/22847463

Growth and bone health in pediatric intestinal failure patients receiving long-term parenteral nutrition

Pichler, J., et al.

Am J Clin Nutr 2013; 97 (6): 1260-1269

BACKGROUND: Children with chronic intestinal failure (IF) treated with long-term parenteral nutrition (PN) may present with low bone mineral density (BMD). The cause may reflect small body size or suboptimal bone mineralization. OBJECTIVE: We assessed growth and bone health in children with severe IF. DESIGN: Height, weight, and fracture history were recorded. The lumbar spine bone mass was measured in 45 consecutive patients (24 male subjects) aged 5-17 y receiving PN for a median of 5 y. BMD and bone mineral apparent density (BMAD) [ie, adjusted-for-height SD scores (SDSs)] were calculated. RESULTS: Diagnoses were short bowel syndrome in 12 patients (27%), intestinal enteropathy in 20 patients (44%), and motility disorder in 13 patients (29%). Mean (+/-SD) weight, height, and body mass index SDSs were -0.8 +/- 1.3, -1.80 +/- 1.5, and 0.4 +/- 1.3, respectively. The height SDS was less than -2 in 23 children (50%). Patients with enteropathy or intestinal mucosal inflammation (associated with dysmotility or short bowel) were significantly shorter than patients without enteropathy (P = 0.007). The BMD SDS was -1.7 +/- 1.6, and the BMAD SDS was -1.4 +/- 1.5, independent of primary diagnosis or mucosal inflammation. Nineteen patients (42%) had low BMD (SDS less than -2.0), and 14 patients (31%) had low BMAD. In 25 patients studied at 1-2-y intervals, the BMD SDS fell significantly with time, whereas BMAD declined less, which suggested that a poor bone mineral accretion reflected poor growth. A total of 11 of 37 patients (24%) had nonpathologic fractures (P = 0.3 compared with the general population). CONCLUSIONS: Approximately 50% of children were short, and one-third of children had low BMD and BMAD. Children with enteropathy or intestinal mucosal inflammation are at greatest risk of growth failure. Close nutritional monitoring and bespoke PN should maximize the potential for growth and bone mass.

http://www.ncbi.nlm.nih.gov/pubmed/23576042

Adolescents with acute lymphoblastic leukemia treated at pediatric versus adult hospitals

Pole, J. D., et al.

Ann Oncol 2013; 24 (3): 801-806

BACKGROUND: The objective was to compare 5-year overall survival (OS) between adolescent and young adult (AYA) patients (age 15-19) with acute lymphoblastic leukemia (ALL) treated at a pediatric versus an adult center. PATIENTS AND METHODS: This was a population-based analysis using administrative data of Ontario ALL AYA patients diagnosed between 1986-2009. We calculated predicted survival proportions (PSPs) and 95% confidence intervals (CI). We also surveyed sites to determine whether pediatric or adult-based protocols were used in each period. RESULTS: Overall, 290 patients between 15-19 years of age were diagnosed with ALL during the study period; 144 patients (49.7%) were treated at an adult center. When adjusted for gender, age, income quintile and time period, AYA patients treated at a pediatric center did not have a significantly different PSP (0.65, 95% CI: 0.56-0.75) in comparison to those treated at an adult center (0.62, 95% CI 0.52-0.73; P = 0.87). Most AYA patients treated at adult centers received pediatric protocols in the recent periods. CONCLUSIONS: Using population-based data, AYA ALL patients had similar outcomes whether treated at a pediatric or an adult center. Early introduction of aggressive treatment protocols in adult centers may have negated differences in outcomes among AYA patients by site of care.

http://www.ncbi.nlm.nih.gov/pubmed/23108950

Infantile-onset Alexander disease: a genetically proven case with mild clinical course in a 6-year-old Indian boy

Ramesh, K., et al.

J Child Neurol 2013; 28 (3): 396-398

Alexander disease is an autosomal dominant leukoencephalopathy characterized by developmental delay, macrocephaly, and characteristic neuroimaging abnormalities predominantly involving frontal lobes. We report a 6-year-old Indian boy with infantile-onset Alexander disease, who has an unusually mild clinical course and a de novo p.Leu359Val mutation in the glial fibrillary acidic protein gene.

http://www.ncbi.nlm.nih.gov/pubmed/22566711

Congenital central hypoventilation syndrome and sudden infant death syndrome: disorders of autonomic regulation

Rand, C. M., et al.

Semin Pediatr Neurol 2013; 20 (1): 44-55

Long considered a rare and unique disorder of respiratory control, congenital central hypoventilation syndrome has recently been further distinguished as a disorder of autonomic regulation. Similarly, more recent evidence suggests that sudden infant death syndrome is also a disorder of autonomic regulation. Congenital central hypoventilation syndrome typically presents in the newborn period with alveolar hypoventilation, symptoms of autonomic dysregulation and, in a subset of cases, Hirschsprung disease or tumors of neural crest origin or both. Genetic investigation identified PHOX2B, a crucial gene during early autonomic development, as disease defining for congenital central hypoventilation syndrome. Although sudden infant death syndrome is most likely defined by complex multifactorial genetic and environmental interactions, it is also thought to result from central deficits in the control of breathing and autonomic regulation. The purpose of this article is to review the current understanding of these autonomic disorders and discuss the influence of this information on clinical practice and future research directions.

http://www.ncbi.nlm.nih.gov/pubmed/23465774

Global challenges in pediatric oncology

Rodriguez-Galindo, C., et al.
Curr Opin Pediatr 2013; 25 (1): 3-15

PURPOSE OF REVIEW: Reduction of child mortality is one of the Millennium Development Goals; as low-income and middle-income countries (LMICs) advance toward the achievement of this goal, initiatives aimed at reducing the burden of noncommunicable diseases, including childhood cancer, need to be developed. RECENT FINDINGS: Approximately 200 000 children and adolescents are diagnosed with cancer every year worldwide; of those, 80% live in LMICs, which account for 90% of the deaths. Lack of quality population-based cancer registries in LMICs limits our knowledge of the epidemiology of pediatric cancer; however, available information showing variations in incidence may indicate unique interactions between environmental and genetic factors that could provide clues to cause. Outcome of children with cancer in LMICs is dictated by late presentation and underdiagnosis, high abandonment rates, high prevalence of malnutrition and other comorbidities, suboptimal supportive and palliative care, and limited access to curative therapies. Initiatives integrating program building with education of healthcare providers and research have proven to be successful in the development of regional capacity. Intensity-graduated treatments adjusted to the local capacity have been developed. SUMMARY: Childhood cancer burden is shifted toward LMICs; global initiatives directed at pediatric cancer care and control are urgently needed. International partnerships facilitating stepwise processes that build capacity while incorporating epidemiology and health services research and implementing intensity-graduated treatments have been shown to be effective.

http://www.ncbi.nlm.nih.gov/pubmed/23295716

All-cause mortality and cardiovascular outcomes with prophylactic steroid therapy in Duchenne muscular dystrophy

Schram, G., et al.
J Am Coll Cardiol 2013; 61 (9): 948-954

OBJECTIVES: This study sought to determine the impact of steroid therapy on cardiomyopathy and mortality in patients with Duchenne muscular dystrophy (DMD). BACKGROUND: DMD is a debilitating X-linked disease that afflicts as many as 1 in 3,500 boys. Although steroids slow musculoskeletal impairment, the effects on cardiac function and mortality remain unknown. METHODS: We conducted a cohort study on patients with DMD treated with renin-angiotensin-aldosterone system antagonists with or without steroid therapy. RESULTS: Eighty-six patients, 9.1 +/- 3.5 years of age, were followed for 11.3 +/- 4.1 years. Seven of 63 patients (11%) receiving steroid therapy died compared with 10 of 23 (43%) not receiving steroid therapy (p = 0.0010). Overall survival rates at 5, 10, and 15 years of follow-up were 100%, 98.0%, and 78.6%, respectively, for patients receiving steroid therapy versus 100%, 72.1%, and 27.9%, respectively, for patients not receiving steroid therapy (log-rank p = 0.0005). In multivariate propensity-adjusted analyses, steroid use was associated with a 76% lower mortality rate (hazard ratio: 0.24; 95% confidence interval: 0.07 to 0.91; p = 0.0351). The mortality reduction was driven by fewer heart failure-related deaths (0% vs. 22%, p = 0.0010). In multivariate analyses, steroids were associated with a 62% lower rate of new-onset cardiomyopathy (hazard ratio: 0.38; 95% confidence interval: 0.16 to 0.90; p = 0.0270). Annual rates of decline in left ventricular ejection fraction (-0.43% vs. -1.09%, p = 0.0101) and shortening fraction (-0.32% vs. -0.65%, p = 0.0025) were less steep in steroid-treated patients. Consistently, the increase in left ventricular end-diastolic dimension was of lesser magnitude (+0.47 vs. +0.92 mm per year, p = 0.0105). CONCLUSIONS: In patients with DMD, steroid therapy is associated with a substantial reduction in all-cause mortality and new-onset and progressive cardiomyopathy.

http://www.ncbi.nlm.nih.gov/pubmed/23352781

Early hematopoietic stem cell transplant is associated with favorable outcomes in children with MDS

Smith, A. R., et al.

Pediatr Blood Cancer 2013; 60 (4): 705-710

BACKGROUND: Although hematopoietic stem cell transplantation (HSCT) is the treatment of choice for childhood myelodysplastic syndrome (MDS), there is no consensus regarding patient or disease characteristics that predict outcomes. PROCEDURE: We reviewed 37 consecutive pediatric MDS patients who received myeloablative HSCT between 1990 and 2010 at a single center. RESULTS: Twenty had primary MDS and 17 had secondary MDS. Diagnostic cytogenetics included monosomy 7 (n = 21), trisomy 8 (n = 7) or normal/other (n = 8). According to the modified WHO MDS classification, thirty had refractory cytopenia and seven had refractory anemia with excess blasts. IPSS scores were: low risk (n = 1), intermediate-1 (n = 15), and intermediate-2 (n = 21). OS and DFS at 10 years in the entire cohort was 53% and 45%. Relapse at 10 years was 26% and 1 year TRM was 25%. In multivariate analysis, factors associated with improved 3 years DFS were not receiving pre-HSCT chemotherapy (RR = 0.30, 95% CI 0.10-0.88; P = 0.03) and a shorter interval (<140 days) from time of diagnosis to transplant (RR = 0.27, 95% CI 0.09-0.80; P = 0.02). Three years DFS in patients who did not receive pre-HSCT chemotherapy and those who had a shorter interval to transplant (n = 16) was 80%. CONCLUSION: These results suggest that children with MDS should be referred for allogeneic HSCT soon after diagnosis and that pre-HSCT chemotherapy does not appear to improve outcomes.

http://www.ncbi.nlm.nih.gov/pubmed/23152304

Eosinophilic esophagitis in children and adolescents: epidemiology, clinical presentation and seasonal variation

Sorser, S. A., et al.

J Gastroenterol 2013; 48 (1): 81-85

BACKGROUND: Eosinophilic esophagitis (EoE) is defined by infiltration of eosinophils in the esophageal mucosa (>20 eosinophils/hpf). The epidemiology and seasonal variation have not been well studied in children and adolescents. METHODS: Review of all esophageal biopsies performed from January 2001 to December 2006 on patients younger than 21 year of age, focusing on demographics, onset and duration of presenting symptoms, history of allergies and endoscopic findings. RESULTS: A total of 753 upper endoscopies were performed, 44 of which showed histologic evidence of EoE (5.8 %). Fifty percent of all EoE endoscopies were grossly normal. Onset of symptoms was 23 % in the spring, 29 % in the summer, 23 % in the fall and 25 % in the winter. More cases (36 %) were diagnosed in the fall. Time between onset of symptoms and diagnosis was 115 +/- 145 days (mean +/- SD). The most common presenting symptoms were vomiting (61 %), dysphagia (39 %), abdominal pain (34 %), feeding disorders (14 %), heartburn (14 %), food impaction (7 %), vague chest pain (5 %) and diarrhea (5 %). Children presenting with vomiting and feeding disorders were younger ($p < 0.02$), whereas children presenting with heartburn and dysphagia were older ($p < 0.02$). CONCLUSIONS: The incidence of EoE did not increase between 2001 and 2006. Onset of symptoms did not vary by season, indicating that allergens triggering EoE are present all year around. Vomiting and feeding disorders are seen in young children, while dysphagia and heartburn are seen in older children. As endoscopic findings were normal in 50 % of cases, an esophageal biopsy should be performed in all patients with suspected EoE.

http://www.ncbi.nlm.nih.gov/pubmed/22618806

Successful liver transplantation in an infant with stage 4S(M) neuroblastoma

Steele, M., et al.

Pediatr Blood Cancer 2013; 60 (3): 515-517

We report a 2.5-month-old infant with bilateral adrenal neuroblastoma, stage 4S(M), with liver metastases and chemotherapy-induced veno-occlusive disease leading to cirrhosis requiring liver transplantation. Despite unknown tumour histology and MYCN-amplification status, we proceeded with liver transplant. This decision was based on clinical suspicion that our patient was MYCN-negative due to significant tumour regression, and was supported by evidence indicating that MYCN-amplification is rare in infants with favourable-stage neuroblastoma. This is the second case report of neuroblastoma requiring liver transplantation; however, in the previously reported case, the diagnosis of neuroblastoma was not established until after transplantation. We discuss this unique case to justify the potential use of life-saving liver transplants in infants with neuroblastoma.

http://www.ncbi.nlm.nih.gov/pubmed/23152322

Hodgkin lymphoma in children: experience in a tertiary care centre in India

Trehan, A., et al.

J Pediatr Hematol Oncol 2013; 35 (3): 174-179

BACKGROUND: In developing countries Hodgkin lymphoma (HL) has been seen to have a high male to female ratio, younger age at presentation, a high proportion of patients in advanced stage of disease, constitutional symptoms, and predominance of mixed cellularity histologic type. The results of treatment appear to be comparable to the results attained in developed nations. METHODS: Children with HL who were diagnosed and treated at our center between 1990 and 2006 were retrospectively analyzed. RESULTS: A total of 206 children with a mean age of 7.9+/-2.6 (range, 3 to 16) years were treated for HL. Among them, 52% presented with advanced-stage (stages III and IV) disease, 54% had B symptoms, and 69.6% had mixed cellularity type of HL. Multiagent chemotherapy was the mainstay of treatment. The 5-year overall survival and event-free survival rates were 92.7% and 77.75%, respectively. Children with early-stage disease and absence of B symptoms had a better overall survival of 97.7% each, as compared with 87.2% and 88.2% in those with late-stage disease and B symptoms, respectively. CONCLUSIONS: Even though developing countries have a different epidemiological profile, the outcome is good. Chemotherapy alone has shown excellent results in children with HL.

http://www.ncbi.nlm.nih.gov/pubmed/23073046

Osteosarcoma after bone marrow transplantation

Ueki, H., et al.

J Pediatr Hematol Oncol 2013; 35 (2): 134-138

Three children treated with bone marrow transplantation for acute lymphoblastic leukemia, Diamond-Blackfan anemia, and congenital amegakaryocytic thrombocytopenia developed secondary osteosarcoma in the left tibia at the age of 13, 13, and 9 years, respectively, at 51, 117, and 106 months after transplantation, respectively. Through treatment with chemotherapy and surgery, all 3 patients are alive without disease. We surveyed the literature and reviewed 10 cases of osteosarcoma after hematopoietic stem cell transplantation (SCT), including our 3 cases. Eight of the patients had received myeloablative total body irradiation before SCT. The mean interval from SCT to the onset of osteosarcoma was 6 years and 4 months, and the mean age at the onset of osteosarcoma was 14 years and 5 months. The primary site of the post-SCT osteosarcoma was the tibia in 6 of 10 cases, in contrast to de novo osteosarcoma, in which the most common site is the femur. At least 7 of the 10 patients are alive without disease. Osteosarcoma should be one of the items for surveillance in the follow-up of patients who undergo SCT.

http://www.ncbi.nlm.nih.gov/pubmed/22995925

Deaths in childhood from cystic fibrosis: 10-year analysis from two London specialist centres

Urquhart, D. S., et al.

Arch Dis Child 2013; 98 (2): 123-127

INTRODUCTION: Death in childhood from cystic fibrosis (CF) is now an uncommon event in the U.K. We wished to assess the circumstances surrounding deaths (and lung transplantation) in the modern era of CF care. METHODS: A retrospective review was carried out pooling data from two large paediatric specialist CF units in London for the 10-year period 2000-2009 inclusive. RESULTS: There were 11 deaths and eight children who had a lung transplant out of 1022 children cared for in this period. Median age of death was 14.2 years and transplant 13.0 years, with a female preponderance (82% deaths and 75% transplants). Apart from one child (forced expiratory volume in 1 s (FEV1) 69%), lung function indicated severe lung disease (median FEV1 33%, range 12%-69%). Values 5 years prior to death were not predictive (median FEV1 62%, range 32%-96%), and those 1 year prior were similar to the last recorded levels. Almost all (10/11) died in hospital and 5/11 (45%) were ventilated. Respiratory failure was the commonest mode of death (64%). Only four children (36%) were receiving palliative care, and in six cases (55%) care was withdrawn. CONCLUSIONS: The number of deaths in children with CF was small but often unpredictable, so active management was continued until late in the majority, reflected by the fact that almost all were in hospital, and more than half were ventilated. If death from respiratory failure is anticipated following a steady decline, palliative care should be instituted well in advance, with attention to appropriate end of life care.

http://www.ncbi.nlm.nih.gov/pubmed/23264431

Proton pump inhibitors in pediatrics : mechanism of action, pharmacokinetics, pharmacogenetics, and pharmacodynamics

Ward, R. M. and G. L. Kearns

Paediatr Drugs 2013; 15 (2): 119-131

Proton pump inhibitors (PPIs) have become some of the most frequently prescribed medications for treatment of adults and children. Their effectiveness for treatment of peptic conditions in the pediatric population, including gastric ulcers, gastroesophageal reflux disease (GERD), and Helicobacter pylori infections has been established for children older than 1 year. Studies of the preverbal population of neonates and infants have identified doses that inhibit acid production, but the effectiveness of PPIs in the treatment of GERD has not been established except for the recent approval of esomeprazole treatment of erosive esophagitis in infants. Reasons that have been proposed for this are complex, ranging from GERD not occurring in this population to a lack of histologic identification of esophagitis related to GERD to questions about the validity of symptom scoring systems to identify esophagitis when it occurs in infants. The effectiveness of PPIs relates to their structures, which must undergo acidic activation within the parietal cell to allow the PPI to be ionized and form covalent disulfide bonds with cysteines of the H(+)-K(+)-adenosine triphosphatase (H(+)-K(+)-ATPase). Once the PPI binds to the proton pump, the pump is inactivated. Some PPIs, such as omeprazole and rabeprazole bind to cysteines that are exposed, and their binding can be reversed. After irreversible chemical inhibition of the proton pump, such as occurs with pantoprazole, the recovery of the protein of the pump has a half-life of around 50 h. Cytochrome P450 (CYP) 2C19 and to a lesser degree CYP3A4 clear the PPIs metabolically. These enzymes are immature at birth and reach adult levels of activity by 5-6 months after birth. This parallels studies of the maturation of CYP2C19 to adult levels by roughly the same age after birth. Specific single nucleotide polymorphisms of CYP2C19 reduce clearance proportionally and increase exposure and prolong proton pump inhibition. Prolonged treatment of pediatric patients with PPIs has not caused cancer or significant abnormalities.

http://www.ncbi.nlm.nih.gov/pubmed/23512128

The impact of complementary and alternative medicine on hip development in children with cerebral palsy

Willoughby, K., et al.

Dev Med Child Neurol 2013; 55 (5): 472-479

AIM: This study aimed to evaluate the effect of complementary and alternative medicine (CAM) approaches on long-term surgical requirements, and clinical and radiographic outcomes for children with cerebral palsy and hip displacement. METHOD: Twenty-three children with cerebral palsy and early hip displacement who were offered preventive hip surgery and whose parents declined in favour of CAM approaches were followed (13 males, 10 females; mean age 13 y 9 mo [SD 3 y 1 mo]; mean length of follow-up 10 y 2 mo [SD 2 y 11 mo]; 17 with spastic quadriplegia, two with spastic triplegia, and four with spastic diplegia; three with gross motor function classified at Gross Motor Function Classification System [GMFCS] level II, four at level III, six at level IV, and 10 at level V). Principal outcome measures were progression of hip displacement (measured by migration percentage: the percentage of the femoral head sitting outside of the acetabulum), eventual need for reconstructive or salvage surgery, and long-term hip morphology (classified by the Melbourne Cerebral Palsy Hip Classification Scale). The results were compared with a previously reported cohort of 46 children who had surgery when recommended (31 males, 15 females; mean age 13 y 11 mo [SD 1 y 6 mo]; mean length of follow-up 10 y 10 mo; 10 with diplegia and 36 with quadriplegia; three at GMFCS level II, 11 at level III, 20 at level IV, and 12 at level V). RESULTS: Outcomes for 23 children who had pursued CAM were analysed (mean length of follow-up 10 y 2 mo). Hip displacement progressed in one or both hips in all non-ambulant children (GMFCS level IV or V). Of the 20 children with documented progressive hip displacement, eight developed pain and deformity requiring salvage surgery. An additional 11 children with progressive hip displacement had late reconstructive surgery when symptoms first started. There was strong evidence of a relationship between GMFCS and both progressive hip displacement (chi(2) =17.78; p=0.001) and final Melbourne Cerebral Palsy Hip Classification Scale grade (odds ratio 12.5; p=0.012; 95% confidence interval 1.7-90.4). There was also evidence of those children who pursued CAM requiring more complex surgery than the group who had surgery when recommended (odds ratio 2.5; p=0.002; 95% confidence interval 1.4-4.5). INTERPRETATION: CAM therapy did not appear to influence the progression of hip displacement in children with cerebral palsy. Most children required major reconstructive surgery or salvage surgery despite pursuing CAM.

http://www.ncbi.nlm.nih.gov/pubmed/23432349

Predicting pharmacoresistance in pediatric epilepsy (epidemiology)

Wirrell, E. C.

Epilepsia 2013; 54 Suppl 2: 19-22

Approximately 20% of children with epilepsy will be pharmacoresistant. The impact of intractable epilepsy extends far beyond just the seizures to result in intellectual disability, psychiatric comorbidity, physical injury, sudden unexpected death in epilepsy (SUDEP), and poor quality of life. Various predictors of pharmacoresistance have been identified; however, accurate prediction is still challenging. Population-based epidemiologic studies show that the majority of children who develop pharmacoresistance do so relatively early in the course of their epilepsy. However, approximately one third of children who initially appear pharmacoresistant in the first few years after epilepsy onset will ultimately achieve seizure freedom without surgery. The most significant predictor that early pharmacoresistance will not remit is the presence of a neuroimaging abnormality. Such children should be strongly considered for surgical evaluation.

http://www.ncbi.nlm.nih.gov/pubmed/23646966

Dysphagia in Duchenne muscular dystrophy assessed by validated questionnaire

Archer, S. K., et al.

Int J Lang Commun Disord 2013; 48 (2): 240-246

BACKGROUND: Duchenne muscular dystrophy (DMD) leads to progressive muscular weakness and death, most typically from respiratory complications. Dysphagia is common in DMD; however, the most appropriate swallowing assessments have not been universally agreed and the symptoms of dysphagia remain under-reported. AIMS: To investigate symptoms of dysphagia in DMD and to determine the potential of the validated Sydney Swallow Questionnaire (SSQ) to diagnose dysphagia in this patient group. METHODS & PROCEDURES: Three participant groups completed the SSQ and the results were compared: nine DMD participants with dysphagia, six DMD participants without dysphagia and 12 healthy controls. OUTCOMES & RESULTS: The questionnaire scores for dysphagic DMD participants were significantly higher than for non-dysphagic DMD participants (p = 0.039) and for healthy controls (p </= 0.001). The diagnostic ability of the questionnaire was good for detecting dysphagia in participants with DMD (receiver operating characteristic (ROC) area under the curve = 0.89, p = 0.013), with a cut-off score of 224.5 (13.2%) giving a sensitivity of 0.78 and a specificity of 0.83 for determining dysphagia. Dysphagic participants rated time to eat a meal, swallowing hard food, swallowing thick liquids and needing to cough up or spit during meals with the highest severity of all questionnaire items. Results of the questionnaire by item are presented to inform the clinician of the symptoms of dysphagia in DMD. CONCLUSIONS & IMPLICATIONS: DMD leads to pervasive symptoms of dysphagia. The simple SSQ is a clinically informative assessment tool for patients with DMD.

http://www.ncbi.nlm.nih.gov/pubmed/23472962

The cooperative international neuromuscular research group Duchenne natural history study: glucocorticoid treatment preserves clinically meaningful functional milestones and reduces rate of disease progression as measured by manual muscle testing and other commonly used clinical trial outcome measures

Henricson, E. K., et al.

Muscle Nerve 2013; 48 (1): 55-67

Introduction: Glucocorticoid (GC) therapy in Duchenne muscular dystrophy (DMD) has altered disease progression, necessitating contemporary natural history studies. METHODS: The Cooperative Neuromuscular Research Group (CINRG) DMD Natural History Study (DMD-NHS) enrolled 340 DMD males, ages 2-28 years. A comprehensive battery of measures was obtained. RESULTS: A novel composite functional “milestone” scale scale showed clinically meaningful mobility and upper limb abilities were significantly preserved in GC-treated adolescents/young adults. Manual muscle test (MMT)-based calculations of global strength showed that those patients <10 years of age treated with steroids declined by 0.4 +/- 0.39 MMT unit/year, compared with -0.4 +/- 0.39 MMT unit/year in historical steroid-naive subjects. Pulmonary function tests (PFTs) were relatively preserved in steroid-treated adolescents. The linearity and magnitude of decline in measures were affected by maturational changes and functional status. CONCLUSIONS: In DMD, long-term use of GCs showed reduced strength loss and preserved functional capabilities and PFTs compared with previous natural history studies performed prior to the widespread use of GC therapy.

http://www.ncbi.nlm.nih.gov/pubmed/23649481

Fatigue and daytime sleepiness scale in myotonic dystrophy type 1

Hermans, M. C., et al.

Muscle Nerve 2013; 47 (1): 89-95

INTRODUCTION: Fatigue and excessive daytime sleepiness are frequent complaints in myotonic dystrophy type 1 (DM1) that often overlap. We aimed to construct a combined fatigue and daytime sleepiness rating scale for DM1 using the Rasch measurement model. METHODS: Questionnaires, including the Epworth sleepiness scale, fatigue severity scale, and daytime sleepiness scale, were completed by 354 patients. Data were subjected to Rasch analyses and tested for required measurement issues such as appropriate response categories, absence of item bias, local independence, and unidimensionality. RESULTS: The initial 22 items did not meet Rasch model expectations. After rescoring and removing misfitting items, the final 12-item scale showed good model fit and unidimensionality. High internal consistency (person separation index = 0.80) and validity were demonstrated. CONCLUSIONS: The Rasch-built Fatigue and Daytime Sleepiness Scale, developed specifically for DM1 patients, provides interval measures on a single continuum. Its use is suggested for future clinical trials and therapeutic follow-up.

http://www.ncbi.nlm.nih.gov/pubmed/23042586

Variability of "optimal" cut points for mild, moderate, and severe pain: neglected problems when comparing groups

Hirschfeld, G. and B. Zernikow

Pain 2013; 154 (1): 154-159

Defining cut points for mild, moderate, and severe pain intensity on the basis of differences in functional interference has an intuitive appeal. The statistical procedure to derive them proposed in 1995 by Serlin et al. has been widely used. Contrasting cut points between populations have been interpreted as meaningful differences between different chronic pain populations. We explore the variability associated with optimally defined cut points in a large sample of chronic pain patients and in homogeneous subsamples. Ratings of maximal pain intensity (0-10 numeric rating scale, NRS) and pain-related disability were collected in a sample of 2249 children with chronic pain managed in a tertiary pain clinic. First, the "optimal" cut points for the whole sample were determined. Second, the variability of these cut points was quantified by the bootstrap technique. Third, this variability was also assessed in homogeneous subsamples of 650 children with constant pain, 430 children with chronic daily headache, and 295 children with musculoskeletal pain. Our study revealed 3 main findings: (1) The optimal cut points for mild, moderate, and severe pain in the whole sample were 4 and 8 (0-10 NRS). (2) The variability of these cut points within the whole sample was very high, identifying the optimal cut points in only 40% of the time. (3) Similarly large variability was also found in subsamples of patients with a homogeneous pain etiology. Optimal cut points are strongly influenced by random fluctuations within a sample. Differences in optimal cut points between study groups may be explained by chance variation; no other substantial explanation is required. Future studies that aim to interpret differences between groups need to include measures of variability for optimal cut points.

http://www.ncbi.nlm.nih.gov/pubmed/23182623

Severe fatigue and reduced quality of life in children with hereditary motor and sensory neuropathy 1A

Jagersma, E., et al.

J Child Neurol 2013; 28 (4): 429-434

Severe fatigue and low quality of life are reported by a majority of adult patients with hereditary motor and sensory neuropathy 1A. In children with hereditary motor and sensory neuropathy 1A, the prevalence and impact of fatigue have not been studied yet. In this questionnaire survey, 55 Dutch children (response rate 77%) with genetically confirmed hereditary motor and sensory neuropathy 1A participated (mean age 15 years [standard deviation 2.1]). Prevalence of severe fatigue (based on a cut-off score of the Checklist Individual Strength) was 24%, in contrast to 14% in a Dutch school-based population ($P < .05$). Almost all quality-of-life scores (measured with the Child Health Questionnaire-Child Form 87) were significantly worse than population norms ($P < .05$). Fatigue severity was associated significantly ($P < .01$) with all quality-of-life scores ($-0.4 < r < -0.7$). In conclusion, severe fatigue and diminished quality of life are more frequent among children with hereditary motor and sensory neuropathy 1A compared to healthy peers. The strong association between fatigue severity and quality of life suggests a negative impact of fatigue on quality of life in these children.

http://www.ncbi.nlm.nih.gov/pubmed/22752492

Outcomes in children with hemorrhagic stroke

Lo, W. D., et al.

JAMA Neurol 2013; 70 (1): 66-71

OBJECTIVES: To determine if a specific intracerebral hemorrhage ratio predicts poor outcome; whether predictors of outcome in adults, specifically hemorrhage location, ventricular involvement, or initial Glasgow Coma Scale score, predict outcome in childhood hemorrhagic stroke; and whether the cause of hemorrhagic stroke predicts outcome. DESIGN: Retrospective case study. SETTING A single tertiary care pediatric hospital. PARTICIPANTS: Fifty-nine cases who had nontraumatic hemorrhages. MAIN OUTCOME MEASURES: We examined whether hemorrhage volume, location, initial Glasgow Coma Scale score, or associated diagnoses predicted outcomes. We contacted survivors and parents and assessed outcomes using measures of neurological function, quality of life, and caregiver stress. RESULTS: Twenty died of the hemorrhage or associated illnesses, and we obtained follow-up on 19 survivors. Most survivors had mild to moderate neurological deficits, but many reported impaired school or physical functioning. Increasing hemorrhage volume predicted poorer neurological outcomes and poorer quality-of-life ratings among survivors. Subjects who had intracranial vascular anomalies had the best outcomes of the group. Associated diagnoses strongly predicted scores on the parent- and child-rated quality-of-life measures. In contrast to what has been reported in adult studies, initial Glasgow Coma Scale score, primary location of the hemorrhage, and ventricular hemorrhage did not significantly predict outcomes, although ventricular hemorrhage was associated with trends toward poorer outcomes. CONCLUSIONS: The mortality of hemorrhagic stroke in children is lower than that in adults. Childhood survivors tend to have mild to moderate physical deficits, but they may have significant impairment in other domains such as school functioning.

http://www.ncbi.nlm.nih.gov/pubmed/23108798

Comparing children's self-report instruments for health-related quality of life using the International Classification of Functioning, Disability and Health for Children and Youth (ICF-CY)

Petersson, C., et al.

Health Qual Life Outcomes 2013; 11: 75

Children with chronic conditions often experience a long treatment which can be complex and negatively impacts the child's well-being. In planning treatment and interventions for children with chronic conditions, it is important to measure health-related quality of life (HrQoL). HrQoL instruments are considered to be a patient-reported outcome measure (PROM) and should be used in routine practice. Purpose: The aim of this study was to compare the content dimensions of HrQoL instruments for children's self-reports using the framework of ICF-CY. Method: The sample consist of six instruments for health-related quality of life for children 5 to 18 years of age, which was used in the Swedish national quality registries for children and adolescents with chronic conditions. The following instruments were included: CHQ-CF, DCGM-37, EQ-5D-Y, KIDSCREEN-52, Kid-KINDL and PedsQL 4.0. The framework of the ICF-CY was used as the basis for the comparison. Results: There were 290 meaningful concepts identified and linked to 88 categories in the classification ICF-CY with 29 categories of the component body functions, 48 categories of the component activities and participation and 11 categories of the component environmental factors. No concept were linked to the component body structures. The comparison revealed that the items in the HrQoL instruments corresponded primarily with the domains of activities and less with environmental factors. Conclusions: In conclusion, the results confirm that ICF-CY provide a good framework for content comparisons that evaluate similarities and differences to ICF-CY categories. The results of this study revealed the need for greater consensus of content across different HrQoL instruments. To obtain a detailed description of children's HrQoL, DCGM-37 and KIDSCREEN-52 may be appropriate instruments to use that can increase the understanding of young patients' needs.

http://www.ncbi.nlm.nih.gov/pubmed/23642162

Oral health assessment and mouth care for children and young people receiving palliative care. Part one

Sargeant, S. and C. Chamley

Nurs Child Young People 2013; 25 (2): 29-34

This is the first part of two articles exploring oral health problems and treatments for children receiving palliative care, successful management of which can improve considerably the quality of life for this group of children and young people. Part one includes an adapted oral health assessment tool for use in children and young people with complex and palliative healthcare needs that has the potential to help nurses identify and monitor oral health problems and prevent or minimise oral problems from developing. Part two--to be published next month--focuses on basic oral hygiene and the management of specific oral health problems.

http://www.ncbi.nlm.nih.gov/pubmed/23586181

Prognostic tests in term neonates with hypoxic-ischemic encephalopathy: a systematic review

van Laerhoven, H., et al.

Pediatrics 2013; 131 (1): 88-98

BACKGROUND AND OBJECTIVE: Hypoxic-ischemic encephalopathy (HIE) after perinatal asphyxia in term neonates causes long-term neurologic sequelae or death. A reliable evidence-based prognosis is essential. The study goal was to investigate the prognostic value of currently used clinical tests in neonatal patients with perinatal asphyxia and HIE. METHODS: Searches were made on MEDLINE, Embase, Central, and CINAHL for studies occurring between January 1980 and November 2011. Studies were included if they (1) evaluated outcome in term infants with perinatal asphyxia and HIE, (2) evaluated prognostic tests, and (3) reported outcome at a minimal follow-up age of 18 months. Study selection, assessment of methodologic quality, and data extraction were performed by 3 independent reviewers. Pooled sensitivities and specificities of investigated tests were calculated when possible. RESULTS: Of the 259 relevant studies, 29 were included describing 13 prognostic tests conducted 1631 times in 1306 term neonates. A considerable heterogeneity was noted in test performance, cut-off values, and outcome measures. The most promising tests were amplitude-integrated electroencephalography (sensitivity 0.93, [95% confidence interval 0.78-0.98]; specificity 0.90 [0.60-0.98]), EEG (sensitivity 0.92 [0.66-0.99]; specificity 0.83 [0.64-0.93]), and visual evoked potentials (sensitivity 0.90 [0.74-0.97]; specificity 0.92 [0.68-0.98]). In imaging, diffusion weighted MRI performed best on specificity (0.89 [0.62-0.98]) and T1/T2-weighted MRI performed best on sensitivity (0.98 [0.80-1.00]). Magnetic resonance spectroscopy demonstrated a sensitivity of 0.75 (0.26-0.96) with poor specificity (0.58 [0.23-0.87]). CONCLUSIONS: This evidence suggests an important role for amplitude-integrated electroencephalography, EEG, visual evoked potentials, and diffusion weighted and conventional MRI. Given the heterogeneity in the tests' performance and outcomes studied, well-designed large prospective studies are needed.

http://www.ncbi.nlm.nih.gov/pubmed/23248219

Current practice and recent advances in pediatric pain management

Chiaretti, A., et al.

Eur Rev Med Pharmacol Sci 2013; 17 Suppl 1: 112-126

BACKGROUND: Differently from the adult patients, in pediatric age it is more difficult to assess and treat efficaciously the pain and often this symptom is undertreated or not treated. In children, selection of appropriate pain assessment tools should consider age, cognitive level and the presence of eventual disability, type of pain and the situation in which it is occurring. Improved understanding of developmental neurobiology and paediatric analgesic drugs pharmacokinetics should facilitate a better management of childhood pain. AIM: The objective of this review is to discuss current practice and recent advances in pediatric pain management. METHODS: Using PubMed we conducted an extensive literature review on pediatric pain assessment and commonly used analgesic agents from January 2000 to January 2012. CONCLUSIONS: A multimodal analgesic regimen provides better pain control and functional outcome in children. Cooperation and communication between the anaesthesiologist, surgeon, and paediatrician are essential for successful anaesthesia and pain management.

http://www.ncbi.nlm.nih.gov/pubmed/23436673

Pain assessment in neonates and infants in the post-operative period following cardiac surgery

Cury, M. R., et al.

Postgrad Med J 2013; 89 (1048): 63-67

PURPOSE: We aimed to test the convergent validity of the COMFORT scale and the Cardiac Analgesic Assessment Scale (CAAS) and to evaluate changes in physiological parameters over time in response to a painful procedure in neonates and infants following cardiac surgery. METHODS: From October 2006 to May 2008, 16 children were prospectively evaluated over 1-3 days after cardiac surgery while they remained on mechanical ventilation and received infusions of sedatives and analgesics. Pain was assessed by the COMFORT scale and CAAS before and during endotracheal tube suctioning. Heart rate, systemic systolic blood pressure, pulmonary artery pressure, oxygen saturation and pupil size were recorded at the same times. RESULTS: During endotracheal suctioning on the first day, there was a significant increase in COMFORT and CAAS scores, systemic systolic blood pressure tended to decrease, pulmonary artery pressure significantly increased and there was a significant reduction in oxygen saturation. Heart rate and pupil size did not change significantly during the painful procedure throughout the study. COMFORT scores significantly correlated with CAAS scores on all days. Nevertheless, agreement for the detection of pain between both scales was weak (kappa<0.5). The COMFORT scale detected more patients with pain. CONCLUSIONS: There was poor agreement between the COMFORT scale and CAAS for detection of pain in neonates and infants who had undergone cardiac surgery. A reduction in systemic systolic blood pressure and a rise in pulmonary artery pressure were observed during painful stimulation on the first post-operative day. For this population, a pain scale scoring physiological parameters according to their variation to higher and lower values should be developed.

http://www.ncbi.nlm.nih.gov/pubmed/23086223

The Wong-Baker pain FACES scale measures pain, not fear

Garra, G., et al.
Pediatr Emerg Care 2013; 29 (1): 17-20

OBJECTIVE: The Wong-Baker FACES pain rating scale (WBS) is preferred by parents and patients for reporting pain severity. However, it is speculated that the "no hurt" and "hurts worst" anchors confound pain measurement with nonnociceptive states. The objective of our study was to determine if fear confounds reporting of pain severity on the WBS. We hypothesized that the WBS would correlate with a psychometrically different pain severity scale (the visual analog scale [VAS]) and not correlate with a fear measure, the Child Medical Fear Scale (CMFS). METHODS: This was a prospective observational study of children 7 to 12 years presenting to a university-based suburban pediatric ED with acute pain. Patients rated pain severity on the WBS ordinal scale and a 100-mm unhatched VAS with marked end points of "no pain" and "worse pain ever." Patients also completed a 26-item CMFS. Correlations between the WBS and VAS with the CMFS total score were assessed with Spearman correlation and exploratory factor analysis. RESULTS: All 3 scales were completed in 197 children. Correlation between the severity scales (WBS-VAS) was moderate: 0.63 (95% confidence interval [CI], 0.54-0.71). However, correlations between the WBS-CMFS and VAS-CMFS were poor: -0.02 (95% CI, -0.16 to -0.12) and 0.01 (95% CI, -0.13 to 0.15), respectively. Correlations did not differ by sex, grade, pain location, or cause of pain (traumatic vs atraumatic). Exploratory factor analysis demonstrated excellent loadings within 2 factors: pain and fear. CONCLUSIONS: The WBS demonstrates moderate correlation with another measure of pain (VAS) and is not mistaken for fear among school-aged patients presenting to the ED with pain.

http://www.ncbi.nlm.nih.gov/pubmed/23283256

Drugs for chronic pain in children: a commentary on clinical practice and the absence of evidence

Gregoire, M. C. and G. A. Finley
Pain Res Manag 2013; 18 (1): 47-50

Pediatric chronic pain is widespread, under-recognized and undertreated. Best management usually involves a multimodal approach coordinated by a multidisciplinary team. The present commentary specifically discusses common pharmacological approaches to chronic pain in children, identifies gaps in knowledge and suggests several research directions that would benefit future clinical care.

http://www.ncbi.nlm.nih.gov/pubmed/23457686

Is acute dystonia an emergency? Sometimes, it really is!

Kanburoglu, M. K., et al.
Pediatr Emerg Care 2013; 29 (3): 380-382

Most cases of acute dystonia are mild and easy to manage; nevertheless, some of them can be fatal because of the involvement of certain muscle groups such as the laryngeal muscles, thus requiring urgent intervention. In the literature, approach to life-threatening acute dystonia has not been investigated thoroughly, although the diagnosis is a challenge, and treatment should be offered immediately. Herein the management of life-threatening acute dystonia is discussed via 2 case reports.

http://www.ncbi.nlm.nih.gov/pubmed/23462398

Respiratory management strategies for Duchenne muscular dystrophy: practice variation amongst Canadian sub-specialists

Katz, S. L., et al.

Pediatr Pulmonol 2013; 48 (1): 59-66

PURPOSE: Respiratory management of Duchenne muscular dystrophy (DMD) is not well studied and may vary across centers and practitioners. Our objective was to describe and compare the respiratory management practices of Canadian Pediatric Respirologists and Neuromuscular specialists for children with DMD. METHODS: A web-based survey was sent to all 56 practicing Canadian Pediatric Respirologists and to all 24 members of the Canadian Pediatric Neuromuscular Group (CPNG) who follow children with neuromuscular diseases. The survey included 28 questions about timing and indications for respiratory consultation, sleep disordered breathing (SDB) assessments, and treatments. RESULTS: Thirty eight (68%) pediatric respirologists and 17 (71%) CPNG members responded. Respirologists provide initial consultation after a patient's first admission to hospital with respiratory complications (14/38, 37%) and when symptoms of SDB are present (14/38, 37%). Half of the CPNG members request initial Respirology consultation at the time of DMD diagnosis. Both groups request routine pulmonary function tests. Ninety-six percent of respirologists use maximal inspiratory (MIP) and expiratory pressures (MEP) to assess respiratory muscle strength, whereas 82% of CPNG members additionally use peak cough flow. Assessment for SDB is requested by both groups when pulmonary function is abnormal or patients are symptomatic. Respirologists favor polysomnography, whereas CPNG members use overnight pulse oximetry. Nocturnal non-invasive ventilation and lung volume recruitment (LVR) are used in a minority of patients. CONCLUSIONS: Respirologists and CPNG members provide similar respiratory management of DMD patients, but differ in timing of consultation and choice of tests for pulmonary function and SDB. Canadian practices differ from the American Thoracic Society and Centre for Disease Control guidelines.

http://www.ncbi.nlm.nih.gov/pubmed/22451223

The effects of music therapy on vital signs, feeding, and sleep in premature infants

Loewy, J., et al.

Pediatrics 2013; 131 (5): 902-918

OBJECTIVES: Recorded music risks overstimulation in NICUs. The live elements of music such as rhythm, breath, and parent-preferred lullabies may affect physiologic function (eg, heart and respiratory rates, O2 saturation levels, and activity levels) and developmental function (eg, sleep, feeding behavior, and weight gain) in premature infants. METHODS: A randomized clinical multisite trial of 272 premature infants aged >/=32 weeks with respiratory distress syndrome, clinical sepsis, and/or SGA (small for gestational age) served as their own controls in 11 NICUs. Infants received 3 interventions per week within a 2-week period, when data of physiologic and developmental domains were collected before, during, and after the interventions or no interventions and daily during a 2-week period. RESULTS: Three live music interventions showed changes in heart rate interactive with time. Lower heart rates occurred during the lullaby ($P < .001$) and rhythm intervention ($P = .04$). Sucking behavior showed differences with rhythm sound interventions ($P = .03$). Entrained breath sounds rendered lower heart rates after the intervention ($P = .04$) and differences in sleep patterns ($P < .001$). Caloric intake ($P = .01$) and sucking behavior ($P = .02$) were higher with parent-preferred lullabies. Music decreased parental stress perception ($P < .001$). CONCLUSIONS: The informed, intentional therapeutic use of live sound and parent-preferred lullabies applied by a certified music therapist can influence cardiac and respiratory function. Entrained with a premature infant's observed vital signs, sound and lullaby may improve feeding behaviors and sucking patterns and may increase prolonged periods of quiet-alert states. Parent-preferred lullabies, sung live, can enhance bonding, thus decreasing the stress parents associate with premature infant care.

http://www.ncbi.nlm.nih.gov/pubmed/23589814

Comfort First: an evaluation of a procedural pain management programme for children with cancer

McCarthy, M., et al.
Psychooncology 2013; 22 (4): 775-782

BACKGROUND: The Comfort First Program (CFP) provides children and their caregivers with early procedural pain management intervention to reduce procedural pain and distress. This study evaluated whether the CFP was meeting its goals and effectively implementing the Royal Australasian College of Physicians paediatric pain management guidelines. METHODS: The study was conducted as a single-site cross-sectional audit. One hundred and thirty-five patients (mean age 7.7 years) receiving treatment at the Royal Children's Hospital, Melbourne, Children's Cancer Centre Day Oncology Unit were observed. Procedural aspects related to the treatment room, carer and staff behaviour, child distress and use of pharmacologic and nonpharmacologic interventions were recorded using an audit tool developed for the study. RESULTS: The procedure room was regularly quiet and prepared before the child entered. Median procedure duration was 8 min. Median procedure wait time was 54 min. At least one carer was typically present during procedures. Comfort First (CF) clinicians were more likely to be present in procedures with a significantly distressed child. Carers, nurses and CF clinicians generally displayed comfort-promoting behaviour. Topical anaesthetic was regularly utilised. Nonpharmacologic supports were frequently used, particularly distraction. Patients under 8 years of age were significantly more likely to receive nonpharmacologic supports and have a carer and CF clinician present. Age was a significant predictor of distress, with higher distress rates in younger children. CONCLUSIONS: The CFP was found to be effectively implementing procedural pain guidelines. Regular audit is recommended to ensure adherence to pain management standards.

http://www.ncbi.nlm.nih.gov/pubmed/22416039

Pain in cognitively impaired children: a focus for general pediatricians

Massaro, M., et al.
Eur J Pediatr 2013; 172 (1): 9-14

Pain in children with cognitive impairment and cerebral palsy is a particularly relevant issue due to its high prevalence and impact on quality of life. We review available evidence about prevalence of pain, causes and specific treatment, recognition and use of specific pain scales, physiology, and consequences of pain in this subset of patients. CONCLUSIONS: Pain is very common and is a critical determinant of quality of life in children with cognitive impairment and cerebral palsy. The diseases and associated complications that frequently expose these patients to pain can be treated and pain prevented. For patients with communication difficulties, appropriate, effective, validated tools are available and should be used to diagnose pain in itself, to >choose analgesic treatment and to determine effectiveness of these therapies. The level of awareness of pediatricians towards this issue seems to be quite low.

http://www.ncbi.nlm.nih.gov/pubmed/22426858

Pain in young people aged 13 to 17 years with cerebral palsy: cross-sectional, multicentre European study

Parkinson, K. N., et al.

Arch Dis Child 2013; 98 (6): 434-440

OBJECTIVE: To determine the prevalence and associations of self- and parent-reported pain in young people with cerebral palsy (CP). DESIGN AND SETTING: Cross-sectional questionnaire survey conducted at home visits in nine regions in seven European countries. Participants were 13 to 17-year-olds (n=667) drawn from population CP registers in eight regions and from multiple sources in one region. 429 could self-report; parent-reports were obtained for 657. Data were collected on: severity, frequency, site and circumstances of pain in previous week; severity of pain associated with therapy in previous year. RESULTS: The estimated population prevalence of any pain in previous week was 74% (95% CI 69% to 79%) for self-reported pain and 77% (95% CI 73% to 81%) for parent-reported pain. 40% experienced leg pains, 34% reported headaches and 45% of those who received physiotherapy experienced pain during therapy. Girls reported more pain than boys (OR=2.1, 95% CI 1.5 to 3.0) and young people reported more pain if they had emotional difficulties (comparing highest and lowest quartiles: OR=3.1, 95% CI 1.7 to 5.6). Parents reported more pain in children with emotional difficulties (OR=4.2, 95% CI 2.7 to 6.6), or with more impaired walking ability. CONCLUSIONS: Pain in young people with CP is highly prevalent. Because pain causes immediate distress and is associated with lower subjective well-being and reduced participation, clinicians should routinely assess pain. Clinical interventions to reduce pain should be implemented and evaluated. The efficacy of medical and therapeutic interventions causing pain should be re-examined to establish if their benefit justifies the pain and fear of pain that accompany them.

http://www.ncbi.nlm.nih.gov/pubmed/22426858

Symptom clusters in children

Rodgers, C. C., et al.

Curr Opin Support Palliat Care 2013; 7 (1): 67-72

PURPOSE OF REVIEW: Researchers have focused on identifying and describing symptom experiences among children with various diseases but symptoms can have a synergistic and/or an antecedent effect that must be evaluated. This review reports the current knowledge of symptoms among various pediatric diseases and highlights symptom cluster research. RECENT FINDINGS: Symptoms of depression and anxiety are the most prevalent variables studied across pediatric disease studies followed by pain, fatigue, and quality of life. Although previous pediatric symptom research provides a foundation for understanding the complexities of these symptoms, there is limited evidence on symptom cluster research in pediatrics. Pain and fatigue are the most common symptoms analyzed for correlations, and relationships among symptoms that have been evaluated in children with juvenile idiopathic arthritis, HIV, cancer, cardiac disease requiring an implantable cardioverter defibrillator, and at end of life. Pain and fatigue have been associated with sleep disturbances, anxiety, depression, anorexia, and nausea/vomiting. SUMMARY: Pediatric oncology researchers are leading the way with symptom cluster studies; however, this work remains in the early stages. There is great potential to advance the state of the science with cluster analysis. Future research work should focus on evaluating symptoms and their interactions.

http://www.ncbi.nlm.nih.gov/pubmed/23108342

Efficacy and safety of transdermal buprenorphine in the management of children with cancer-related pain

Ruggiero, A., et al.

Pediatr Blood Cancer 2013; 60 (3): 433-437

BACKGROUND: The current study investigated the efficacy, safety, tolerability, and compliance of a transdermal buprenorphine delivery system for the management of chronic cancer pain in the pediatric population. PROCEDURE: Sixteen pediatric patients with moderate to severe cancer-related pain not satisfactorily controlled with previous non-opioid therapies were enrolled. Transdermal buprenorphine was administered following a 72 hour schedule and rescue medication (tramadol) was allowed for breakthrough pain. Pain intensity was assessed using the Wong-Baker faces pain rating scale (WBS) and other parameters related to the global quality of life were evaluated. Children's evaluations of efficacy, compliance, and tolerability were recorded using numerical scales. Adverse events were monitored during the study and the medications needed to control opioid-related nausea and constipation were recorded. RESULTS: Eleven patients (68.75%) responded to transdermal buprenorphine after 2 weeks of treatment. Pain intensity measured with WBS decreased from 6.25 at baseline to 1.38 at Day +60 ($P < 0.001$). All outcome measures of global quality of life (quality of sleep, alimentation, play and activity, speech, and crying) significantly improved over the 60-day study period. Children's evaluations of compliance and tolerability of the drug were always positive over the entire period of treatment. No severe adverse events were recorded. Opioid-related nausea was well controlled with medication on request, and the need for laxative therapy was greater at the end of the second month of treatment. CONCLUSIONS: Transdermal buprenorphine was found to represent an efficient, safe and well tolerated approach to the management of children's chronic cancer pain.

http://www.ncbi.nlm.nih.gov/pubmed/23034996

Pain tolerance in children and adolescents: sex differences and psychosocial influences on pain threshold and endurance

Schmitz, A. K., et al.

Eur J Pain 2013; 17 (1): 124-131

BACKGROUND: Laboratory studies with children and adolescents revealed inconsistent findings regarding sex differences in pain tolerance, although lower pain tolerance is commonly reported for adult women. Besides biological mechanisms, several socio-cognitive variables are discussed which may influence pain tolerance in regard to sex differences. The purpose of the present study was to investigate the pain tolerance of children and adolescents using the cold pressor task (CPT) and to analyse influences of pain-coping and pain-related self-efficacy. METHODS: About 1021 children and adolescents aged 9-17 participated in the study. Pain tolerance was defined as the length of time a participant's hand remains under water during the CPT. Two phases of pain tolerance were differentiated: the time until pain is reported (pain threshold) and the time from the threshold until the pain increases to a level resulting in the hand being withdrawn (pain endurance). Pain-coping and pain-related self-efficacy were assessed by self-report questionnaires. RESULTS: We revealed an obvious effect of sex on pain threshold, which increased with age, a small effect on pain tolerance and no significant effect on endurance. Independent of sex, pain endurance was influenced by pain-related self-efficacy and positive self-instruction. CONCLUSIONS: Our results support the assumption that female and male adolescents develop in different directions regarding their pain tolerance when reaching puberty. This seems mainly attributable to a decrease of pain threshold in girls. In contrast, boys and girls are able to endure pain to an equal extent influenced, however, by self-efficacy and coping variables.

http://www.ncbi.nlm.nih.gov/pubmed/22715044

Day pediatric hospice - necessity and demand. Position of the "Working Group of Inpatient Pediatric Hospice Administration" – [Article in German]

Kinderkrankenschwester 2013; 32 (3): 96.

http://www.ncbi.nlm.nih.gov/pubmed/23600109

Transition to adult care for young people with long-term conditions

Begley, T.

Br J Nurs 2013; 22 (9): 506, 508-511

This study aimed to clarify how the transition from child to adult healthcare is managed in young people with two long-term conditions in the Republic of Ireland.A postal survey using an adapted questionnaire (Flume et al, 2001) with closed and open questions was sent to all physicians and nurse specialists (n=132) caring for children with cystic fibrosis (CF) and insulin dependent diabetes mellitus (IDDM)in the Republic of Ireland. Data was analysed using SPSS 16.0 for Windows and the open-ended qualitative questions were analysed using content analysis. Results showed that transition management varied depending on the service, location and resources available. Comparisons were made between CF and IDDM data. Age was found to be the criterion most commonly used to determine when to transition in all services. Healthcare professionals said transition management needed to be improved. Some services managed transition following recommended guidelines.However, the management of transition nationally needs to be more streamlined.This includes a devised cohesive strategy between adult and young people's services.

http://www.ncbi.nlm.nih.gov/pubmed/23752622

Evaluation of services for children with complex needs: mapping service provision in one NHS Trust

Brooks, F., et al.

Prim Health Care Res Dev 2013; 14 (1): 52-62

AIM: The aim of this paper is to identify and descriptively map the key characteristics of the model of service delivery in operation, and to explore the user, carer and professional experience of service provision. This included an exploration of congruity and mismatch between the different stakeholder groups. BACKGROUND: In the United Kingdom (UK), 15% of the children under five years of age and 20% of the 5 to 15-year age group are reported to have a complex long-term condition, with the likelihood of having a condition increasing according to socio-economic circumstances. An increasing number of young people with complex needs are now surviving into late adolescence and early adulthood. However, service provision for children with complex needs is an area that, nationally, has been underdeveloped. METHODS: An exploratory single-site case study was undertaken across one Primary Care Trust in the UK. Documentary and policy review were undertaken along with in-depth qualitative exploration. Eighteen in-depth interviews were undertaken with relevant stakeholders and professionals across the multidisciplinary teams. Families with children between 12 months and 16 years of age who have continuing complex care needs were invited to take part in an interview to give their views about the care they receive. Interviews focused on the family experience and understanding of the child's condition, transition between secondary and primary care, effectiveness of admission and discharge planning and the overall contribution of different professionals. Professionals were also asked about their experiences of delivering care. Findings This study highlighted issues of communication between professionals and with parents and children as a major factor in determining the quality of service provision. Key aspects relating to the model of service provision, namely, paucity of communication, interagency collaboration and the parent as health worker, are highlighted. CONCLUSIONS: Parents experienced both health and social service communication challenges when seeking care for their child. These challenges can be located within a general systems theory and hierarchy approaches to understand the complexity of service provision.

http://www.ncbi.nlm.nih.gov/pubmed/22784821

**Characteristics influencing location of death for children with life-limiting illness

Chang, E., et al.

Arch Dis Child 2013; 98 (6): 419-424

OBJECTIVE: To determine whether demographic and diagnostic characteristics were associated with location of death in a series of children with life-limiting illnesses. DESIGN: A population-level case series was carried out by reviewing mortality records. Sociodemographic characteristics, diagnosis and referral to paediatric palliative care (PPC) were analysed for association with location of death. SETTING: New Zealand PARTICIPANTS: Children and young people aged 28 days-18 years who died from a life-limiting illness between 2006 and 2009 inclusive. MAIN OUTCOME MEASURES: Location of death-home, hospital, other. RESULTS: Of 494 deaths, 53.6% (256/494) died in hospital and 41.9% (203/494) died at home. Asian (OR=2.66, 95% CI 1.17 to 6.04) and Pacific children (OR=2.22, 95% CI 1.15 to 4.29) had an increased risk of death in hospital compared with European children, while children with cancer (adjusted OR=0.48, 95% CI 0.3 to 0.75) and children referred to the PPC service (adjusted OR=0.60, 95% CI 0.38 to 0.96) had a decreased risk. Population-attributable risk for referral to the PPC service was 28.2% (95% CI 11.25 to 47.75). CONCLUSIONS: Most children in New Zealand with a life-limiting illness die in hospital with a significant influence resulting from ethnic background, diagnosis and referral to the PPC service. These findings have implications for resourcing PPC services and end-of-life care.

http://www.ncbi.nlm.nih.gov/pubmed/23599439

Views of childhood cancer survivors and their families on the provision and format of a treatment summary

Firth, E. R., et al.

J Pediatr Hematol Oncol 2013; 35 (3): 193-196

INTRODUCTION: Summaries of diagnosis, treatments, and their potential late adverse health effects (treatment summaries) are increasingly being provided routinely to survivors of childhood cancer. There is relatively little research into opinions of service users on the provision and format of treatment summaries. METHODS: Semistructured interviews were conducted with 24 survivors of childhood cancer, and 25 parents of 20 of these survivors (n=49) were asked to explore these issues. Survivors were aged 4 to 22 years, with a range of previous oncological diagnoses. The mean (range) interval since treatment completion was 4.5 (0.5 to 13) years. RESULTS: Twelve survivors (50%) and 16 parents (64%) fully supported the use of treatment summaries as: a memory aid, a reference tool for planned and emergency medical situations, life and financial planning, and to promote ownership of health. Four survivors felt they did not want, or did not need, a reminder of their medical past. Most participants (63% of survivors) desired a verbal explanation plus a written A4 paper treatment summary. However, other formats (wallet sized, audio guides) were suggested to address practical and individual needs raised by participants. CONCLUSIONS: Most service users would benefit from treatment summaries, although notably not all. The format and timing of the provision need to be carefully considered.

http://www.ncbi.nlm.nih.gov/pubmed/22983417

Does referral to specialist paediatric palliative care services reduce hospital admissions in oncology patients at the end of life?

Fraser, L. K., et al.

Br J Cancer 2013; 108 (6): 1273-1279

BACKGROUND: Despite advances in the treatment of childhood cancer, some children continue to die from their disease. This study aimed to assess the impact of specialist paediatric palliative care services (SPPCSs) on the number of hospital admissions in children who subsequently died from cancer in Yorkshire, UK. METHODS: An extract of patients aged 0-19 years from the Yorkshire Specialist Register of Cancer in Children and Young People (YSRCCYP) diagnosed from 1990 to 2009 were linked to inpatient hospital episodes data and a SPPCS database. Deaths were included if they occurred before 31 August 2011. Differences in hospital admission patterns were assessed using negative binomial regression and presented as incidence rate ratios (IRRs). RESULTS: Of 2508 children on the YSRCCYP, 657 (26%) had died by the censoring date. A total of 211 children had been referred to the local SPPCS, of whom 182 (86%) had subsequently died. Referral to SPPCS was associated with a significant reduction in the rate of planned hospital admissions (IRR=0.60, 95% CI 0.43-0.85). Central nervous system tumours showed significant decreases for all planned and emergency admissions compared with all other diagnostic groups. CONCLUSION: Referral to SPPCS significantly reduced the number of planned hospital admissions for children and young people with cancer before their death, which are often integral to paediatric oncology treatment regimens. Overall, our findings show that SPPCS have a role in reducing hospital admissions during end of life care of paediatric cancer patients with potential personal, social and economic benefits.

http://www.ncbi.nlm.nih.gov/pubmed/23449361

A review of pediatric palliative care service utilization in children with a progressive neuromuscular disease who died on a palliative care program

Ho, C. and L. Straatman

J Child Neurol 2013; 28 (1): 40-44

Recent studies and consensus statements have expressed the need to involve palliative care services in the care of children with progressive neuromuscular diseases (PMD), yet there have been no reviews of the utilization of palliative care services by children who died on a palliative care program. We conducted a retrospective chart review of all children who had a PMD who died on a single-center palliative care program. Twenty cases were identified. Services utilized by these patients included respite care, transition services, pain and symptom management, and end-of-life care. Prominent symptoms in the last 24 hours of life included respiratory distress, pain, nausea/vomiting, and anxiety; however, symptom management was very good. Utilization of services differed depending on the disease trajectory, with respite playing a critical role in the care of children with PMD. Good symptom management can be achieved.

http://www.ncbi.nlm.nih.gov/pubmed/22447847

Differences in characteristics of dying children who receive and do not receive palliative care

Keele, L., et al.

Pediatrics 2013; 132 (1): 72-78

OBJECTIVE: Comparing demographic and clinical characteristics associated with receipt of palliative care (PC) among children who died in children's hospitals to those who did not receive PC and understanding the trends in PC use. METHODS: This retrospective cohort study used the Pediatric Health Information System database. Children <18 years of age who died >/=5 days after admission to a Pediatric Health Information System hospital between January 1, 2001, and December 31, 2011 were included. Receipt of PC services was identified by the International Classification of Diseases, Ninth Revision code for PC. Diagnoses were grouped using major diagnostic codes. International Classification of Diseases codes and clinical transaction codes were used to evaluate all interventions. RESULTS: This study evaluated 24 342 children. Overall, 4% had coding for PC services. This increased from 1% to 8% over the study years. Increasing age was associated with greater receipt of PC. Children with the PC code had fewer median days in the hospital (17 vs 21), received fewer invasive interventions, and fewer died in the ICU (60% vs 80%). Receipt of PC also varied by major diagnostic codes, with the highest proportion found among children with neurologic disease. CONCLUSIONS: Most pediatric patients who died in a hospital did not have documented receipt of PC. Children receiving PC are different from those who do not in many ways, including receipt of fewer procedures. Receipt of PC has increased over time; however, it remains low, particularly among neonates and those with circulatory diseases.

http://www.ncbi.nlm.nih.gov/pubmed/23753086

Pediatric advance care planning: a systematic review

Lotz, J. D., et al.
Pediatrics 2013; 131 (3): e873-880

BACKGROUND AND OBJECTIVES: Advance care planning (ACP) is increasingly regarded as the gold standard in the care of patients with life-limiting illnesses. Research has focused on adults, but ACP is also being practiced in pediatrics. We conducted a systematic review on empirical literature on pediatric ACP (pACP) to assess current practices, effects, and perspectives of pACP. METHODS: We searched PubMed, BELIT, and PSYCinfo for empirical literature on pACP, published January 1991 through January 2012. Titles, abstracts, and full texts were screened by 3 independent reviewers for studies that met the predefined criteria. The evidence level of the studies was assessed. Relevant study outcomes were retrieved according to predefined questions. RESULTS: We included 5 qualitative and 8 quantitative studies. Only 3 pACP programs were identified, all from the United States. Two of them were informed by adult programs. Major pACP features are discussions between families and care providers, as well as advance directives. A chaplain and other providers may be involved if required. Programs vary in how well they are evaluated; only 1 was studied by using a randomized controlled trial. Preliminary data suggest that pACP can successfully be implemented and is perceived as helpful. It may be emotionally relieving and facilitate communication and decision-making. Major challenges are negative reactions from emergency services, schools, and the community. CONCLUSIONS: There are few systematic pACP programs worldwide and none in Europe. Future research should investigate the needs of all stakeholders. In particular, the perspective of professionals has so far been neglected.

http://www.ncbi.nlm.nih.gov/pubmed/23400610

The role of complementary and alternative medicine in children at the end of life [comment]

Meyer, S., et al.
J Palliat Med 2013; 16 (3): 224.

The role of complementary and alternative medicine in children at the end of life [J Palliat Med 2012]

http://www.ncbi.nlm.nih.gov/pubmed/23437831

Characteristics of family conferences at the bedside versus the conference room in pediatric critical care

October, T. W., et al.
Pediatr Crit Care Med 2013; 14 (3): e135-142

OBJECTIVE: To compare characteristics of family conferences at the bedside vs. the conference room in the PICU. DESIGN: Single-site, cohort survey study. SETTING: Thirty-three bed academic PICU in an urban setting. PARTICIPANTS: Ten PICU physicians (90.9%) providing care to 29 patients whose families participated in 58 family conferences. MEASUREMENTS AND MAIN RESULTS: Family conferences, defined as a meeting involving the parent(s) of a PICU patient and the critical care attending physician to discuss a treatment decision, redirection of care from curative to palliative, or deliver bad news, occurred most commonly among families of the sickest patients. Conferences were conducted at the bedside 20 times out of 58 (33%). Although physicians stated a general preference to discuss withdrawal or withholding care in the conference room, there was no difference in location during actual conferences. Physicians preferred the bedside when they wanted the patient to participate ($p = 0.01$) or because it was perceived to be easier ($p < 0.0005$) or faster ($p = 0.016$) to conduct, while the conference room was preferred when additional space was needed ($p < 0.0005$). Family conferences at the bedside were less likely to include a social worker ($p < 0.0005$), consultant physicians ($p = 0.043$), or father of the patient ($p = 0.006$) as compared with conferences in a conference room. Family conferences convened to discuss a treatment were followed by a decision within 24 hours (42% of the time) and a change in code status (32% of the time). In 32 of 58 family conferences (55%), the attending physician did not have a prior relationship with the family. CONCLUSION: Family conferences in the PICU are common both at the bedside and in conference rooms in a subpopulation of the most critically ill children and frequently result in a treatment decision or change in code status.

http://www.ncbi.nlm.nih.gov/pubmed/23392371

Medical complexity and pediatric emergency department and inpatient utilization

O'Mahony, L., et al.
Pediatrics 2013; 131 (2): e559-565

OBJECTIVES: To characterize the use of and disposition from a tertiary pediatric emergency department (PED) by children with chronic conditions with varying degrees of medical complexity. METHODS: We conducted a retrospective cohort study using a dataset of all registered PED patient visits at Seattle Children's Hospital from January 1, 2008, through December 31, 2009. Children's medical complexity was classified by using a validated algorithm (Clinical Risk Group software) into nonchronic and chronic conditions: episodic chronic, lifelong chronic, progressive chronic, and malignancy. Outcomes included PED length of stay (LOS) and disposition. Logistic regression generated age-adjusted odds ratios (AOR) of admission with 95% confidence intervals (CIs). RESULTS: PED visits totaled 77 748; 20% (15 433) of which were for children with chronic conditions. Compared with visits for children without chronic conditions, those for children with chronic conditions had increased PED LOS (on average, 79 minutes longer; 95% CI 77-81; $P < .0001$) and hospital (51% vs 10%) and PICU (3.2% vs 0.1%) admission rates (AOR 10.3, 95% CI 9.9-10.7 to hospital and AOR 25.0, 95% CI 17.0-36.0 to PICU). Admission rates and PED LOS increased with increasing medical complexity. CONCLUSIONS: Children with chronic conditions comprise a significant portion of annual PED visits in a tertiary pediatric center; medical complexity is associated with increased PED LOS and hospital or PICU admission. Clinical Risk Group may have utility in identifying high utilizers of PED resources and help support the development of interventions to facilitate optimal PED management, such as pre-arrival identification and individual emergency care plans.

http://www.ncbi.nlm.nih.gov/pubmed/23319525

Gaps in transitional care: what are the perceptions of adolescents, parents and providers?

Sonneveld, H. M., et al.
Child Care Health Dev 2013; 39 (1): 69-80

BACKGROUND: Several studies have investigated preferences and experiences of adolescents with different chronic conditions and their parents. Some have included the provider's perspective. Studies comparing the three perspectives on satisfaction with (transitional) care for different chronic conditions, however, are lacking. The main aim of this paper was to explore differences and similarities in perspectives between adolescents with chronic conditions, their parents and providers on transitional care. A secondary aim was to explore the extent to which such perspectives are disease-specific. METHODS: This quantitative study included 127 adolescents with juvenile rheumatoid arthritis (JRA), neuromuscular disorder with chronic ventilation (NMD), or diabetes Type I; 166 parents; and 19 care providers. To assess the experiences and perceptions of adolescents and parents on transitional care, we used the 'Mind the Gap' instrument. The survey for providers included a checklist of shortcomings in transitional care. RESULTS: Adolescents rate current care significantly worse than parents on opportunities to make their own decisions and be seen without parents present. Adolescents also rated providers' current social skills lower than parents. Adolescents are more satisfied than their parents about transitional care process aspects such as co-ordination and communication between providers, but both groups indicated that the care process offers most room for improvement. Providers reported other aspects such as adolescents' lack of responsibility with regard to self-care and parents' difficulties with ceding control to their children. When looking at the three disease groups - JRA, NMD, diabetes, we found only small differences. According to providers, shortcomings in the care process with respect to guidelines, protocols and co-ordination are most prevalent. CONCLUSION: Adolescents, parents and providers all report that there is room for improvement with regard to aspects of the care delivery process in transitional care. With respect to disease-specific issues we only found small differences.

http://www.ncbi.nlm.nih.gov/pubmed/22329453

Bereaved parents and siblings offer advice to health care providers and researchers

Steele, A. C., et al.
J Pediatr Hematol Oncol 2013; 35 (4): 253-259

OBJECTIVE: To determine how to improve care for families by obtaining their advice to health care providers and researchers after a child's death from cancer. DESIGN: Families with a surviving sibling (age, 8 to 17 y) were recruited from cancer registries at 3 hospitals in the United States and Canada 3 to 12 months (M=10.4, SD=3.5) after the child's death. SETTING: Data were collected in the home. PARTICIPANTS: Participants (N=99) included 36 mothers, 24 fathers, and 39 siblings from 40 families. OUTCOME MEASURES: Each participant completed a qualitative interview that was audio recorded, transcribed, and coded for thematic content. FINDINGS: Five major themes included the need for: (a) improved communication with the medical team, (b) more compassionate care, (c) increased access to resources, (d) ongoing research, and (e) offering praise. Interwoven within the 5 themes was a subtheme of continuity of care. CONCLUSIONS: Many participants were pleased with the care the child with cancer received, but others noted areas in need of improvement, particularly medical communication and continuity of care. Additional research is needed to inform interventions to improve services for families of children with life-limiting conditions.

http://www.ncbi.nlm.nih.gov/pubmed/23612375

Improving access to specialist multidisciplinary palliative care consultation for rural cancer patients by videoconferencing: report of a pilot project

Watanabe, S. M., et al.

Support Care Cancer 2013; 21 (4): 1201-1207

PURPOSE: Palliative care (PC) and palliative radiotherapy (RT) consultation are integral to the care of patients with advanced cancer. These services are not universally available in rural areas, and travel to urban centers to access them can be burdensome for patients and families. The objectives of our study were to assess the feasibility of using videoconferencing to provide specialist multidisciplinary PC and palliative RT consultation to cancer patients in rural areas and to explore symptom, cost, and satisfaction outcomes. METHODS: The Virtual Pain and Symptom Control and Palliative Radiotherapy Clinic was piloted from January 2008 to March 2011. Cancer patients in rural northern Alberta attended local telehealth facilities, accompanied by nurses trained in symptom assessment. The multidisciplinary team at the Cross Cancer Institute in Edmonton was linked by videoconference. Team recommendations were sent to the patients' family physicians. Data were collected on referral, clinical, and consultation characteristics and symptom, cost, and satisfaction outcomes. RESULTS: Forty-four initial consultation and 28 follow-up visits took place. Mean Edmonton Symptom Assessment Scale scores for anxiety and appetite were statistically significantly improved at the first follow-up visit ($p < 0.01$ and $p = 0.03$, respectively). Average per visit savings for patients seen by telehealth versus attending the CCI were 471.13 km, 7.96 hours, and Cdn $192.71, respectively. Patients and referring physicians indicated a high degree of satisfaction with the clinic. CONCLUSION: Delivery of specialist multidisciplinary PC consultation by videoconferencing is feasible, may improve symptoms, results in cost savings to patients and families, and is satisfactory to users.

http://www.ncbi.nlm.nih.gov/pubmed/23161339

Predicting parenting stress in caregivers of children with brain tumours

Bennett, E., et al.

Psychooncology 2013; 22 (3): 629-636

OBJECTIVE: The purpose of the study was to identify factors that contribute to parenting stress in caregivers of children diagnosed with brain tumours. METHODS: The study was cross-sectional and recruited 37 participants from a clinical database at a specialist children's hospital. Parents were sent questionnaires, which were used to measure factors related to stress in caregivers of children diagnosed with a brain tumour. Stress levels were measured using the Parenting Stress Index-Short Form (PSI/SF). Correlation analysis and multiple linear regression were used to examine the associations between parenting stress and coping styles, locus of control, parent-perceived child disability and time since diagnosis. RESULTS: Results revealed that 51% of parents were experiencing clinically significant levels of stress. The mean stress level of parents in the study was significantly higher than the PSI/SF norms ($t = 4.7$, $p < .001$). Regression analysis revealed that external locus of control and coping by accepting responsibility accounted for 67% of the variance in parenting stress. Other styles of coping, child behaviour problems and the amount of time since diagnosis were not found to be predictive of levels of parenting stress. CONCLUSIONS: There was a high prevalence of parenting stress in caregivers of children with a brain tumour. An external locus of control and coping by accepting responsibility increased the likelihood of elevated levels of stress. Results emphasised the importance of ongoing support for parents of children with brain tumours. Intervention might helpfully be centred on strategies to increase parents' internal locus of control.

http://www.ncbi.nlm.nih.gov/pubmed/22351496

Narrative medicine as a means of indirectly seeking parental opinion in children's palliative care

Campbell, L.

Int J Palliat Nurs 2013; 19 (2): 56-57.

http://www.ncbi.nlm.nih.gov/pubmed/23435533

Walking drawings and walking ability in children with cerebral palsy

Chong, J., et al.

Health Psychol 2013; 32 (6): 710-713

OBJECTIVES: To investigate whether drawings of the self walking by children with cerebral palsy (CP) were associated with walking ability and illness perceptions. METHOD: This was an exploratory study in 52 children with CP (M:F = 28:24), mean age 11.1 years (range 5-18), who were attending tertiary level outpatient clinics. Children were asked to draw a picture of themselves walking. Drawing size and content was used to investigate associations with clinical walk tests and children's own perceptions of their CP assessed using a CP version of the Brief Illness Perception Questionnaire. RESULTS: Larger drawings of the self were associated with less distance traveled, higher emotional responses to CP, and lower perceptions of pain or discomfort, independent of age. A larger self-to-overall drawing height ratio was related to walking less distance. Drawings of the self confined within buildings and the absence of other figures were also associated with reduced walking ability. CONCLUSION: Drawing size and content can reflect walking ability, as well as symptom perceptions and distress. Drawings may be useful for clinicians to use with children with cerebral palsy to aid discussion about their condition.

http://www.ncbi.nlm.nih.gov/pubmed/22369490

Socioeconomic status and in-hospital pediatric mortality

Colvin, J. D., et al.
Pediatrics 2013; 131 (1): e182-190

OBJECTIVE: Socioeconomic status (SES) is inversely related to pediatric mortality in the community. However, it is unknown if this association exists for in-hospital pediatric mortality. Our objective was to determine the association of SES with in-hospital pediatric mortality among children's hospitals and to compare observed mortality with expected mortality generated from national all-hospital inpatient data. METHODS: This is a retrospective cohort study from 2009 to 2010 of all 1,053,101 hospitalizations at 42 tertiary care, freestanding children's hospitals. The main exposure was SES, determined by the median annual household income for the patient's ZIP code. The main outcome measure was death during the admission. Primary outcomes of interest were stratified by income and diagnosis-based service lines. Observed-to-expected mortality ratios were created, and trends across quartiles of SES were examined. RESULTS: Death occurred in 8950 (0.84%) of the hospitalizations. Overall, mortality rates were associated with SES ($P < .0001$) and followed an inverse linear association ($P < .0001$). Similarly, observed-to-expected mortality was associated with SES in an inverse association ($P = .014$). However, mortality overall was less than expected for all income quartiles ($P < .05$). The association of SES and mortality varied by service line; only 3 service lines (cardiac, gastrointestinal, and neonatal) demonstrated an inverse association between SES and observed-to-expected mortality. CONCLUSIONS: Within children's hospitals, SES is inversely associated with in-hospital mortality, but is lower than expected for even the lowest SES quartile. The association between SES and mortality varies by service line. Multifaceted interventions initiated in the inpatient setting could potentially ameliorate SES disparities in in-hospital pediatric mortality.

http://www.ncbi.nlm.nih.gov/pubmed/23248226

Making meaning after the death of a child: bereaved parents share their experiences

Dokken, D.
Pediatr Nurs 2013; 39 (3): 147-150

Some 50,000 families experience the death of a child each year in the United States (Field & Behrman, 2003). Over time, some bereaved families find or develop approaches for "making meaning" from the experience in ways that allow them to maintain a sense of connection to their child, keep the child's memory alive, and help other families who may face similar circumstances. Interviews of three families demonstrate that the process of "making meaning" is individual and often builds on past strengths and interests. The stories shared by these three families demonstrate their individual approaches as well as common themes.

http://www.ncbi.nlm.nih.gov/pubmed/23926755

Psychological health in siblings who lost a brother or sister to cancer 2 to 9 years earlier

Eilegard, A., et al.

Psychooncology 2013; 22 (3): 683-691

BACKGROUND: The objective of this study was to assess long-term psychological distress in siblings who lost a brother or sister to cancer 2 to 9 years earlier, as compared with a control group of non-bereaved siblings from the general population. METHODS: During 2009, we conducted a nationwide follow-up study in Sweden by using an anonymous study-specific questionnaire. Siblings who had lost a brother or sister to cancer between the years 2000 and 2007 and also a control group of non-bereaved siblings from the general population were invited to participate. The Hospital Anxiety and Depression Scale (HADS) was used to measure psychological distress, and to test for differences in the ordinal outcome responses between the groups, we used Wilcoxon-Mann-Whitney rank-sum test. RESULTS: Among the bereaved siblings, 174/240 (73%) participated and 219/293 (75%) among the non-bereaved. Self-assessed low self-esteem ($p = 0.002$), difficulties falling asleep ($p = 0.005$), and low level of personal maturity ($p = 0.007$) at follow-up were more prevalent among bereaved siblings. However, anxiety ($p = 0.298$) and depression ($p = 0.946$), according to HADS, were similar. CONCLUSION: Bereaved siblings are at increased risk of low self-esteem, low level of personal maturity and difficulties falling asleep as compared with non-bereaved peers. Yet, the bereaved were not more likely to report anxiety or depression.

http://www.ncbi.nlm.nih.gov/pubmed/22351568

Bereaved siblings' perception of participating in research--a nationwide study

Eilegard, A., et al.

Psychooncology 2013; 22 (2): 411-416

OBJECTIVE: The objective of the present study is to examine bereaved siblings' perception of research participation. METHODS: A Swedish nationwide study on avoidable and modifiable health care-related factors in paediatric oncology among bereaved siblings who lost a brother or sister to cancer between the years 2000 and 2007 was conducted. Data are presented as proportions, and the differences between groups were statistically tested at the 5% significant level using Fisher's exact test. RESULTS: Out of 240 eligible siblings, 174 responded (73 %). None of the siblings (0/168) thought their participation would affect them negatively in the long term. However, 13% (21/168) stated it was a negative experience to fill out the questionnaire, whereas 84% (142/169) found it to be a positive experience. Women were more likely to report their participation as positive in a long-term perspective compared with men ($p = 0.018$). CONCLUSIONS: None of the bereaved siblings in this Swedish nationwide study anticipated any long-term negative effect from their research participation. A majority reported it as positive to revisit their needs and experiences throughout their brother or sister's illness and death 2-9 years following the loss. We believe that the stepwise approach used in this study contributed to the high acceptance.

http://www.ncbi.nlm.nih.gov/pubmed/22170857

A sibling death in the family: common and consequential

Fletcher, J., et al.

Demography 2013; 50 (3): 803-826

Although a large literature analyzes the determinants of child mortality and suggests policy and medical interventions aimed at its reduction, there is little existing analysis illuminating the consequences of child mortality for other family members. In particular, there is little evidence exploring the consequences of experiencing the death of a sibling on one's own development and transition to adulthood. This article examines the prevalence and consequences of experiencing a sibling death during one's childhood using two U.S. data sets. We show that even in a rich developed country, these experiences are quite common, affecting between 5 % and 8 % of the children with one or more siblings in our two data sets. We then show that these experiences are associated with important reductions in years of schooling as well as a broad range of adult socioeconomic outcomes. Our findings also suggest that sisters are far more affected than brothers and that the cause of death is an important factor in sibling effects. Overall, our findings point to important previously unexamined consequences of child mortality, adding to the societal costs associated with childhood mortality as well as suggesting additional benefits from policy and medical innovations aimed at curbing both such deaths and subsequent effects on family members.

http://www.ncbi.nlm.nih.gov/pubmed/23073753

Functional priorities in daily life for children and young people with dystonic movement disorders and their families

Gimeno, H., et al.

Eur J Paediatr Neurol 2013; 17 (2): 161-168

PURPOSE: This study aims to describe the most prevalent functional concerns of a group of young people with dystonia and their primary carers, and to explore the relationship between concerns, aetiology, severity of motor disability and manual ability. METHOD: The Canadian Occupational Performance Measure (COPM) was completed with 57 children with dystonic movement disorders (65% males/35% females, mean 11.2 years (3.5-18.1)): 25% had primary dystonia, 75% secondary dystonia. Gross motor and manual function were classified using the Gross Motor Function Classification System (GMFCS) and the Manual Ability Classification System (MACS). COPM concerns were analysed with respect to aetiology and severity of motor disability. RESULTS: Almost three quarters of the respondents were GMFCS/MACS IV-V. All respondents had at least one concern around self-care. Other concerns included access to assistive technology, pain, dressing activities, use of tools and social participation. The nature and presence of concerns did not statistically differ according to the severity of gross motor or manual function impairment, though qualitative differences were noted. No statistical difference was found in relation to aetiology. INTERPRETATION: Children and young people with dystonia have common functional concerns and priorities independent of the cause of dystonia, gross motor severity or manual function ability.

http://www.ncbi.nlm.nih.gov/pubmed/22889754

Parents' views of their child's end-of-life care: subanalysis of primary care involvement

Goldstein, R. and K. P. Rimer

J Palliat Med 2013; 16 (2): 198-202

BACKGROUND: The medical literature encourages primary care pediatricians (PCPs) to play a role in the care of patients who are dying. Actual involvement has not been investigated. OBJECTIVE: Our objective was to explore current involvement of PCPs when their patients face the end of life and bereaved parents' attitudes toward it. DESIGN: Individual, in-depth, semi-structured interviews were conducted using a focused ethnographic technique. Qualitative analysis was performed on the interviews. SETTING/SUBJECTS: Most (14/16) interviews were conducted in the family's home, involving parents of Massachusetts children who died aged 1 month to 11 years during 2005. MEASUREMENTS: Themes identified through thematic analysis of interview transcripts were utilized. RESULTS: Interviews were thematically analyzed, revealing four categories of themes: 1) the role of individual PCP in decision making and care at end of life; 2) general attitudes about the care provided by the PCP; 3) the impact of practice infrastructure on the PCP's care; and 4) bereavement involvement. From the interviews we hypothesize that PCPs play a supportive and appreciated role while having limited involvement in decision making and care; an involved PCP acting with knowledge of a family may in some cases provide profound guidance, but that kind of involvement is not typical; and although there is an articulated role for the PCP with the parents' and siblings' bereavement, it is not routinely exercised. CONCLUSIONS: The interviews suggest limited involvement by PCPs in care at the end of life and subsequent bereavement. Parents overall seem to accept this role. Further research is needed to examine these observations from the perspective of PCPs.

http://www.ncbi.nlm.nih.gov/pubmed/23098631

Becoming a parent to a child with birth asphyxia-From a traumatic delivery to living with the experience at home

Heringhaus, A., et al.

Int J Qual Stud Health Well-being 2013; 8: 1-13

The aim of this study is to describe the experiences of becoming a parent to a child with birth asphyxia treated with hypothermia in the neonatal intensive care unit (NICU). In line with the medical advances, the survival of critically ill infants with increased risk of morbidity is increasing. Children who survive birth asphyxia are at a higher risk of functional impairments, cerebral palsy (CP), or impaired vision and hearing. Since 2006, hypothermia treatment following birth asphyxia is used in many of the Swedish neonatal units to reduce the risk of brain injury. To date, research on the experience of parenthood of the child with birth asphyxia is sparse. To improve today's neonatal care delivery, health-care providers need to better understand the experiences of becoming a parent to a child with birth asphyxia. A total of 26 parents of 16 children with birth asphyxia treated with hypothermia in a Swedish NICU were interviewed. The transcribed interview texts were analysed according to a qualitative latent content analysis. We found that the experience of becoming a parent to a child with birth asphyxia treated with hypothermia at the NICU was a strenuous journey of overriding an emotional rollercoaster, that is, from being thrown into a chaotic situation which started with a traumatic delivery to later processing the difficult situation of believing the child might not survive or was to be seriously affected by the asphyxia. The prolonged parent-infant separation due to the hypothermia treatment and parents' fear of touching the infant because of the high-tech equipment seemed to hamper the parent-infant bonding. The adaption of the everyday life at home seemed to be facilitated by the follow-up information of the doctor after discharge. The results of this study underline the importance of family-centered support during and also after the NICU discharge.

http://www.ncbi.nlm.nih.gov/pubmed/23639330

I wish I could tell you but I can't: adolescents with perinatally acquired HIV and their dilemmas around self-disclosure

Hogwood, J., et al.

Clin Child Psychol Psychiatry 2013; 18 (1): 44-60

Many young people growing up with HIV are choosing not to disclose their status to others, yet are likely to face difficult decisions and conversations such as explaining school absence, taking medication, coping with physical changes and for many, parental bereavement. This study aims to describe and explore the attitudes and opinions of adolescents with perinatally acquired HIV towards disclosure. Semi-structured interviews were conducted with nine young people aged 13-19 and analysed using Interpretative Phenomenological Analysis. Four themes emerged to illuminate the young people's attitudes towards disclosure. These were 1) myths and assumptions, 2) the disclosure dilemma, 3) fear and 4) keeping HIV in its place. This study confirms that many young people with HIV are choosing not to disclose. However, it appears that it is a complex decision-making process that changes over time and is influenced by developmental factors and societal attitudes towards HIV. Recommendations are suggested for services to better support adolescents growing up with HIV.

http://www.ncbi.nlm.nih.gov/pubmed/22287554

Are the psychological needs of adolescent survivors of pediatric cancer adequately identified and treated?

Kahalley, L. S., et al.

Psychooncology 2013; 22 (2): 447-458

OBJECTIVES: To describe the psychological needs of adolescent survivors of acute lymphoblastic leukemia (ALL) or brain tumor (BT), we examined the following: (i) the occurrence of cognitive, behavioral, and emotional concerns identified during a comprehensive psychological evaluation and (ii) the frequency of referrals for psychological follow-up services to address identified concerns. METHODS: Psychological concerns were identified on measures according to predetermined criteria for 100 adolescent survivors. Referrals for psychological follow-up services were made for concerns previously unidentified in formal assessment or not adequately addressed by current services. RESULTS: Most survivors (82%) exhibited at least one concern across domains: behavioral (76%), cognitive (47%), and emotional (19%). Behavioral concerns emerged most often on scales associated with executive dysfunction, inattention, learning, and peer difficulties. Cranial radiation therapy was associated with cognitive concerns, chi(2) (1, N = 100) = 5.63, $p < 0.05$. Lower income was associated with more cognitive concerns for ALL survivors, t(47) = 3.28, $p < 0.01$, and more behavioral concerns for BT survivors, t(48) = 2.93, $p < 0.01$. Of the survivors with concerns, 38% were referred for psychological follow-up services. Lower-income ALL survivors received more referrals for follow-up, chi(2) (1, N = 41) = 8.05, $p < 0.01$. Referred survivors had more concerns across domains than non-referred survivors, ALL: t(39) = 2.96, $p < 0.01$; BT: t(39) = 3.52, $p < 0.01$. Trends suggest ALL survivors may be at risk for experiencing unaddressed cognitive needs. CONCLUSIONS: Many adolescent survivors of cancer experience psychological difficulties that are not adequately managed by current services, underscoring the need for long-term surveillance. In addition to prescribing regular psychological evaluations, clinicians should closely monitor whether current support services appropriately meet survivors' needs, particularly for lower-income survivors and those treated with cranial radiation therapy.

http://www.ncbi.nlm.nih.gov/pubmed/22278930

Understanding the concept of a "good death" among bereaved family caregivers of cancer patients in Singapore

Lee, G. L., et al.

Palliat Support Care 2013; 11 (1): 37-46

OBJECTIVE: The aim of this study was to examine the concept of a good death from the perspectives of both the dying person and the family caregiver, as perceived by bereaved family caregivers of advanced cancer patients. METHOD: The data were gathered from five focus group discussions and one face-to-face qualitative interview conducted over 8 months among 18 bereaved family caregivers recruited from a local hospice. The transcripts of the focus groups and the interview were entered into NVivo Version 8 and were analyzed using the thematic approach. RESULTS: A good death may be understood as having the biopsychosocial and spiritual aspects of life handled well at the end of life. Five major themes were identified. These were preparation for death, family and social relationships, moments at or near death, comfort and physical care, and spiritual well-being. Differences were also noted in what is important at the end of life between the patients and caregivers. Having a quick death with little suffering was perceived to be good by the patient, but the family caregiver wanted to be able to say a final goodbye to the patient. Patients tend to prefer not to die in their children's presence but the children wished to be present for the final moment. In addition, family caregivers reported it was important for them to be able to give the patients permission to die, to feel recognized for the efforts made, and to have had a fulfilling caregiving experience. SIGNIFICANCE OF RESULTS: Whereas there are global attributes of a good death, our findings suggest that patients and family caregivers may define a good death differently. Therefore, there is a need to respect, address, and reconcile the differences, so that all parties may have a good experience at the end of a person's life.

http://www.ncbi.nlm.nih.gov/pubmed/22377014

[Being a mother: encounters between mothers of children with Duchenne muscular dystrophy and nurses in Taiwan]

Lee, S. L., et al.

Hu Li Za Zhi 2013; 60 (3): 94-97

The role of "mother" is understood and represented differently by people from different cultures. In traditional Taiwanese society, mothers demonstrate their existence value by giving birth to and raising sons able to continue her husband's familial line. Sons bear the patriarchal name and care for their parents in old age. However, a son stricken, paralyzed and eventually killed by Duchenne muscular dystrophy (DMD) can destroy a mother's perceived value in this traditional social context. Mothers are thus soundless sufferers. Nurses have a critical role to play in giving encouragement and hope to mothers of children with DMD. Through their own difficult situation, these mothers can also highlight the value and importance of Taiwan's nurses, who work in conditions marked by overloading, high stress, and under-appreciation. Caring for women in critical need of empathy and support help nurses realize their own positive capacity to empower sufferers.

http://www.ncbi.nlm.nih.gov/pubmed/23729346

The effectiveness of early intervention on paternal stress for fathers of premature infants admitted to a neonatal intensive care unit

Lee, T. Y., et al.

J Adv Nurs 2013; 69 (5): 1085-1095

AIM: This article is a report of a study to evaluate the effectiveness of an intervention on fathering ability, perceived nurse's support and paternal stress after a preterm infant's admission to a neonatal intensive care unit.

http://www.ncbi.nlm.nih.gov/pubmed/22813358

The impact of cancer on the physical, psychological and social well-being of childhood cancer survivors

Li, H. C., et al.

Eur J Oncol Nurs 2013; 17 (2): 214-219

PURPOSE: Notwithstanding the advances in medical treatment, childhood cancer survivors are at risk of adverse physical, psychological and social effects of the cancer treatment. The purpose of this study was to examine the impact of cancer and its treatments on the physical, psychological and social well-being of Hong Kong Chinese childhood cancer survivors. METHOD: A total of 137 childhood cancer survivors (aged 9-16 years), who had their medical follow-up in an oncology out-patient clinic were invited to participate in the study. Participants were asked to respond to the standardized measures of depressive symptoms and self-esteem. Additionally, 15 participants from the group were selected for a semi-structured interview. RESULTS: The results revealed that more than half of the participants presented depressive symptoms. Results also found that the mean depressive symptom scores for childhood cancer survivors were statistically significant higher than those of school children without cancer ($p = 0.01$), while the mean self-esteem scores for the survivors were statistically significant lower ($p < 0.01$). Additionally, qualitative interviews indicated that cancer and its treatments have great impact on the daily life of childhood cancer survivors. CONCLUSION: The study reveals that cancer and its treatments have a great impact on the physical, psychological and social well-being of survivors. It is essential for healthcare professionals to develop appropriate interventions with the aim of promoting physical, psychological and social well-being for these children. Most importantly, it is crucial to help them develop a positive view of the impact that the cancer experience has upon their lives.

http://www.ncbi.nlm.nih.gov/pubmed/22898653

Cumulative family risk predicts sibling adjustment to childhood cancer

Long, K. A., et al.

Cancer 2013; 119 (13): 2503-2510

BACKGROUND: Prolonged, intensive treatment regimens often disrupt families of children with cancer. Siblings are at increased risk for distress, but factors underlying this risk have received limited empirical attention. In this study, the authors examined associations between the family context and sibling distress. METHODS: Siblings of children with cancer (ages 8-18 years; N = 209) and parents (186 mothers and 70 fathers) completed measures of sibling distress, family functioning, parenting, and parent post-traumatic stress. Associations between sibling distress and each family risk factor were evaluated. Then, family risks were considered simultaneously by calculating cumulative family risk index scores. RESULTS: After controlling for sociodemographic covariates, greater sibling distress was associated with more sibling-reported problems with family functioning and parental psychological control, lower sibling-reported maternal acceptance, and lower paternal self-reported acceptance. When risk factors were considered together, the results supported a quadratic model in which associations between family risk and sibling distress were stronger at higher levels of risk. CONCLUSIONS: The current findings support a contextual model of sibling adjustment to childhood cancer in which elevated distress is predicted by family risk factors, both alone and in combination.

http://www.ncbi.nlm.nih.gov/pubmed/23576115

Adolescent perspectives on phase I cancer research

Miller, V. A., et al.

Pediatr Blood Cancer 2013; 60 (5): 873-878

BACKGROUND: The aim of this study was to examine adolescent patients' perspectives on their understanding and decision making about a pediatric phase I cancer study. PROCEDURE: Participants included adolescents ages 14-21 years with cancer (N = 20), all of whom attended a phase I study consent conference. Participants responded to closed- and open-ended questions on a verbally administered structured interview, which assessed aspects of understanding and decision making about the phase I study. RESULTS: All participants decided to enroll in the phase I study. The majority of participants understood that participation was voluntary, entailed risks, and that they could withdraw. Most also believed that participation in the phase I study would increase the length of their lives. The most frequent reasons for enrolling were positive clinical benefit, needing an option, impact on quality of life, and few side effects or fewer than those of current or past treatments. Eighty-five percent of participants reported that they themselves made the final decision about enrollment in the phase I study. CONCLUSIONS: Most participants hoped or expected that the phase I study would provide a direct benefit (increased survival time or cure) and reported that they themselves were the final decision-maker about enrollment. Clinicians may underestimate the role of adolescents, especially if they believe that parents typically make such decisions. Future research should assess the actual participation of children and adolescents during the informed consent process and explore the role of hope in their decision making about phase I studies.

http://www.ncbi.nlm.nih.gov/pubmed/23034985

Mothers and fathers of children with cancer: loss of control during treatment and posttraumatic stress at later follow-up

Norberg, A. L. and K. K. Boman

Psychooncology 2013; 22 (2): 324-329

BACKGROUND: A child's cancer can lead to changes in parental role functioning, including loss of control. We studied the extent to which parental perceived loss of control during a child's cancer treatment predicted posttraumatic stress symptoms (PTSS) after completion of treatment. METHOD AND PARTICIPANTS: The sample of this longitudinal study included 62 parents (36 mothers and 26 fathers) of children currently in treatment for malignant disease (T1) and after completion of treatment (T2). Loss of control was assessed at T1 using a self-report measure, that is the loss of control module of the Parental Psychosocial Distress-Cancer questionnaire. PTSS were assessed at T2 using the Impact of Event Scale-Revised. Main analyses were carried out for mothers and fathers separately. RESULTS: The majority of the parents, 55% (n = 34), reported loss of control on more than half of the assessed domains. Only 5% (n = 3) reported no loss of control whatsoever. At T2, some degree of PTSS was reported by 89% (n = 55). These outcomes were similar for mothers and fathers. Loss of control at T1 predicted stronger PTSS at T2 primarily among mothers. CONCLUSION: The experience of loss of control during cancer treatment is a salient risk factor for later PTSS in mothers. The situational threat to the regular parental role is discussed as an explanation to this observation. Interventions should address informational needs, parent participation in care, and professional support to maintain a sense of control and functioning in their parental role.

http://www.ncbi.nlm.nih.gov/pubmed/22021113

Self-esteem of children and adolescents with chronic illness: a meta-analysis

Pinquart, M.

Child Care Health Dev 2013; 39 (2): 153-161

Chronic illness may be a risk factor for low self-esteem; however, previous meta-analyses are inconclusive whether children with a chronic illness have lower self-esteem than their healthy peers. The goal of the present study was to summarize available research in order to compare the self-esteem of children and adolescents with a chronic illness with that of healthy children. Random-effects meta-analysis was used to integrate the results of 621 empirical studies that compare levels of self-esteem of children with a chronic physical illness with healthy peers or general test norms. Studies were identified via the electronic databases Adolesc, Embase, Google Scholar, MEDLINE, PSNYDEX, PSYCINFO, and cross-referencing. Children with chronic illnesses have lower self-esteem than healthy peers or test norms (g = -0.18 standard deviation units). The lowest levels of self-esteem were observed in children with chronic fatigue syndrome and chronic headaches. Lower levels of self-esteem in children with a chronic illness were found in girls than in boys, in adolescents than in children, in children from developing or threshold countries, when results were collected from observer ratings rather than child reports, in studies published in the 1990s, and when children with chronic illnesses were directly compared with healthy children instead of test norms. Paediatricians, parents, and teachers should promote experiences of success and positive peer-relations, which are important sources of self-esteem. In addition, psychosocial interventions for children with chronic illnesses should be offered for children with reduced self-esteem.

http://www.ncbi.nlm.nih.gov/pubmed/22712715

Nausea still the poor relation in antiemetic therapy? The impact on cancer patients' quality of life and psychological adjustment of nausea, vomiting and appetite loss, individually and concurrently as part of a symptom cluster

Pirri, C., et al.

Support Care Cancer 2013; 21 (3): 735-748

PURPOSE: Despite significant antiemetic advances, almost 50% of treated cancer patients still experience nausea and vomiting (N&V). The goal of antiemetic therapy--complete prevention of treatment-induced nausea and/or vomiting (TIN+/-V)--remains elusive for several reasons. Potentially, N&V may be part of a symptom cluster where co-occurring symptoms negatively affect antiemetic management. Consequently, we examined TIN+/-V incidence and the impact of nausea, vomiting and symptom cluster(s) containing them, respectively, on patients' quality of life (QoL) and psychological adjustment across treatment. METHODS: A longitudinal secondary analysis was performed on data from a prospective, observational QoL study involving 200 newly diagnosed cancer patients who underwent combined modality treatment. QoL, psychological adjustment and patient/clinical characteristics were examined at pretreatment, on-treatment (8 weeks) and post-treatment. RESULTS: Overall, 62% of patients experienced TIN+/-V, with TIN (60%) doubling TIV incidence (27 %). Exploratory factor analyses of QoL scores at each treatment time point identified a recurrent gastrointestinal symptom cluster comprising nausea, vomiting and appetite loss. Approximately two thirds of patients reported co-occurrence of all three symptoms, which exerted synergistic effects of multiplicative proportions on overall QoL. Patients who reported co-occurrence of these symptoms during treatment experienced significantly greater QoL impairment (physical, role and social functioning, fatigue, N&V, appetite loss, overall physical health, overall QOL) and psychological distress (cancer distress, premorbid neuroticism) than those unaffected (0.001 > p </= 0.05). Moreover, nausea was more pervasive than vomiting or appetite loss across treatment and had a greater impact on overall QoL. While antiemetic therapy was effective for vomiting and helped prevent/relieve associated appetite loss, the benefits for appetite loss were seemingly constrained by its failure to exert adequate control over nausea in many patients. CONCLUSIONS: TIN+/-V still represents a very major concern for patients. Uncontrolled TIN+/-V often results in significant appetite and weight loss, leading to increased risk for malnutrition. Malnutrition and weight loss, in turn, are associated with poorer prognosis, treatment tolerance and response, performance status, QoL and survival. Consequently, a multiple symptom intervention approach focusing on N&V as core symptoms is recommended. Clinicians should genuinely consider combining essential antiemetic therapies with other evidence-based pharmacological (e.g. nausea: psychotropics, such as olanzapine) and non-pharmacological approaches (e.g. N&V: relaxation) in attempts to not only improve prevention and control of N&V for their patients, but also reduce the synergistic impact of cluster symptoms (e.g. N&V, appetite loss) as a whole and resultant QoL impairment likewise. Where associated symptoms are not adequately controlled by these antiemetic-based interventions, targeted evidence-based strategies should be supplemented.

http://www.ncbi.nlm.nih.gov/pubmed/22976921

A mixed methods analysis of songs written by bereaved preadolescents in individual music therapy

Roberts, M. and K. McFerran

J Music Ther 2013; 50 (1): 25-52

BACKGROUND: Providing opportunities for children to process loss and express grief in response to the loss of a loved one has been shown to assist with successful coping (Worden, 1996). Songwriting may be a relevant method that fosters the expression of thoughts and feelings related to the loss. OBJECTIVE: The purpose of this study was to analyse lyrics written by bereaved children during individual music therapy and to determine if they did use the opportunity to address grief through songwriting. METHODS: Participants were 14 bereaved children (13 girls, 1 boy) aged between 7 and 12 years. Children were recruited and participated in individual music therapy in their homes with a credentialed music therapist. Participants wrote a total of 49 songs and their lyrics were analysed using a mixed methods content analysis. An inductive analysis identified categories existing within the lyrics and each lyric was then deductively attributed to one of the categories. RESULTS: Results revealed that the children wrote songs about themselves, their experiences, and their relationships, including, but not limited to the topic of loss. CONCLUSIONS: It became apparent that through songwriting these children expressed their experiences of the world based on their developmental capacities and limitations.

http://www.ncbi.nlm.nih.gov/pubmed/23847863

Psychological distress in parents of children with advanced cancer

Rosenberg, A. R., et al.

JAMA Pediatr 2013; 167 (6): 537-543

IMPORTANCE: Parent psychological distress can impact the well-being of childhood cancer patients and other children in the home. Recognizing and alleviating factors of parent distress may improve overall family survivorship experiences following childhood cancer. OBJECTIVES: To describe the prevalence and factors of psychological distress (PD) among parents of children with advanced cancer. DESIGN: Cohort study embedded within a randomized clinical trial (Pediatric Quality of Life and Evaluation of Symptoms Technology [PediQUEST] study). SETTING: Multicenter study conducted at 3 children's hospitals (Boston Children's Hospital, Children's Hospital of Philadelphia, and Seattle Children's Hospital). PARTICIPANTS: Parents of children with advanced (progressive, recurrent, or refractory) cancer. MAIN OUTCOME MEASURE: Parental PD, as measured by the Kessler-6 Psychological Distress Scale. RESULTS: Eighty-six of 104 parents completed the Survey About Caring for Children With Cancer (83% participation); 81 parents had complete Kessler-6 Psychological Distress Scale data. More than 50% of parents reported high PD and 16% met criteria for serious PD (compared with US prevalence of 2%-3%). Parent perceptions of prognosis, goals of therapy, child symptoms/suffering, and financial hardship were associated with PD. In multivariate analyses, average parent Kessler-6 Psychological Distress Scale scores were higher among parents who believed their child was suffering highly and who reported great economic hardship. Conversely, PD was significantly lower among parents whose prognostic understanding was aligned with concrete goals of care. CONCLUSIONS AND RELEVANCE: Parenting a child with advanced cancer is strongly associated with high to severe levels of PD. Interventions aimed at aligning prognostic understanding with concrete care goals and easing child suffering and financial hardship may mitigate parental PD.

http://www.ncbi.nlm.nih.gov/pubmed/23545569

Parental emotional functioning declines with occurrence of clinical complications in pediatric hematopoietic stem cell transplant

Terrin, N., et al.

Support Care Cancer 2013; 21 (3): 687-695

PURPOSE: Parents' stress levels are high prior to their child's hematopoietic stem cell transplant (HSCT) and during transplant hospitalization, usually abating after discharge. Nevertheless, a subgroup of parents continues to experience frequent anxiety and mood disruption, the causes of which are not well understood. The purpose of this study was to assess whether clinical complications of HSCT could explain variation in parents' recovery of emotional functioning. METHODS: Pediatric HSCT recipients (n = 165) aged 5-18 and their parents were followed over the first year post-transplant. Health-related quality of life assessments and medical chart reviews were performed at each time period (baseline, 45 days, 3, 6, and 12 months). We tested the association between clinical complications [acute and chronic graft versus host disease (aGVHD and cGVHD), organ toxicity, and infection] and longitudinally measured parental emotional functioning, as assessed by the Child Health-Ratings Inventories. The models used maximum likelihood estimation with repeated measures. RESULTS: In adjusted analyses covering the early time period (45 days and 3 months), aGVHD grade >/=2, intermediate or poor organ toxicity, and systemic infection were associated with decreases in mean parental emotional functioning of 5.2 ($p = 0.086$), 5.8 ($p = 0.052$), and 5.1 ($p = 0.023$) points, respectively. In the later time period (6 and 12 months), systemic infection was associated with a decrease of 20 points ($p < 0.0001$). cGVHD was not significantly associated. CONCLUSIONS: When children experience clinical complications after HSCT, parental emotional functioning can be impacted. Intervening at critical junctures could mitigate potential negative consequences for parents and their children.

http://www.ncbi.nlm.nih.gov/pubmed/22936494

Correlates of social support in young adults with advanced cancer

Trevino, K. M., et al.

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OBJECTIVE: This study examined the relationship between perceived social support, quality of life (QoL), and grief in young adults with advanced cancer. METHODS: Seventy-one young adults (20-40 years) with advanced cancer were administered measures of social support, QoL, and grief. Regression analyses examined the relationship between social support and QoL and grief. RESULTS: Higher levels of total social support were associated with better psychological and existential QoL and less severe grief. Availability of someone to talk to about problems was also associated with better psychological and existential QoL and less severe grief. Tangible support was associated with better psychological and existential QoL. Availability of someone to engage in activities with was only associated with better existential QoL. CONCLUSIONS: These results suggest that enhancing social support may improve psychological well-being in this population. In addition, specific types of social support may be particularly relevant to the psychological well-being of young adults with advanced cancer.

http://www.ncbi.nlm.nih.gov/pubmed/22790223

"Not all my friends need to know": a qualitative study of teenage patients, privacy, and social media

van der Velden, M. and K. El Emam

J Am Med Inform Assoc 2013; 20 (1): 16-24

BACKGROUND: The literature describes teenagers as active users of social media, who seem to care about privacy, but who also reveal a considerable amount of personal information. There have been no studies of how they manage personal health information on social media. OBJECTIVE: To understand how chronically ill teenage patients manage their privacy on social media sites. DESIGN: A qualitative study based on a content analysis of semistructured interviews with 20 hospital patients (12-18 years). RESULTS: Most teenage patients do not disclose their personal health information on social media, even though the study found a pervasive use of Facebook. Facebook is a place to be a "regular", rather than a sick teenager. It is a place where teenage patients stay up-to-date about their social life-it is not seen as a place to discuss their diagnosis and treatment. The majority of teenage patients don't use social media to come into contact with others with similar conditions and they don't use the internet to find health information about their diagnosis. CONCLUSIONS: Social media play an important role in the social life of teenage patients. They enable young patients to be "regular" teenagers. Teenage patients' online privacy behavior is an expression of their need for self-definition and self-protection.

http://www.ncbi.nlm.nih.gov/pubmed/22771531

Psychosocial service use and unmet need among recently diagnosed adolescent and young adult cancer patients

Zebrack, B. J., et al.

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BACKGROUND: Adolescents and young adults (AYAs) with cancer demonstrate biomedical risks and psychosocial issues distinct from those of children or older adults. In this study, the authors examined and compared the extent to which AYAs treated in pediatric or adult oncology settings reported use of, and unmet need for, psychosocial support services. METHODS: Within 4 months of initial cancer diagnosis, 215 AYAs ages 14 to 39 years (99 from pediatric care settings and 116 from adult care settings; 75% response rate) were assessed for reporting use of information resources, emotional support services, and practical support services. Statistical analyses derived odds ratios and 95% confidence intervals for service use and unmet needs after controlling for race, employment/school status, sex, relationship status, severity of cancer, treatment, and treatment-related side effects. RESULTS: AYAs ages 20 to 29 years were significantly less likely than teens and older patients ages 30 to 39 years to report using professional mental health services and were significantly more likely to report an unmet need with regard to cancer information, infertility information, and diet/nutrition information. Compared with teens who were treated in pediatric facilities, AYAs who were treated in adult facilities were more likely to report an unmet need for age-appropriate Internet sites, professional mental health services, camp/retreats programs, transportation assistance, and complementary and alternative health services. CONCLUSIONS: Substantial proportions of AYAs are not getting their psychosocial care needs met. Bolstering psychosocial support staff and patient referral to community-based social service agencies and reputable Internet resources may enhance care and improve quality of life for AYAs.

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