Chair’s Update
Jeffrey Hord, MD, FAAP

As my four years as Chair of SOHO come to a close, I thank you for giving me this opportunity. It has been a privilege to represent you and our subspecialty within the AAP. Please welcome your new SOHO Chair, Dr. Zora Rogers, from Children’s Health in Dallas. She will begin her term on November 1.

The success of the AAP Section on Hematology/Oncology (SOHO) depends upon the volunteer efforts of its members. I want to extend my appreciation to Dr. Eric Werner for his service on the Executive Committee and Education Subcommittee and Dr. Greg Hale for his service on the Executive Committee, as they complete their terms on October 31. I want to welcome two new members to the Executive Committee, Dr. Cynthia Wetmore and Dr. Carl Allen as well as Dr. Mary Jane Hogan who will assume the Education Subcommittee Chair position as of November 1.

The 42 section members who have served on the seven Pediatric Hematology Oncology (PHO) Review Groups since they were first formed in January 2014 as a collaborative initiative with ASPHO, have played a valuable role and have reviewed nearly 60 policies, chapters, and coding documents. Their first terms will end at the end of 2016 and I extend my sincere appreciation for their efforts. Based upon the success of the original PHO Review Groups, the Executive Committee has decided to add 3 additional review groups in the areas of transfusion medicine, neuro-oncology, and oncology supportive care. Please look for a call for volunteers for all 10 review groups later this fall through both SOHO and ASPHO communications.

I also want to thank the multiple Section members currently writing and revising multiple policies including:

- AAP Clinical Practice Guideline: Strategies to Treat and Manage Infantile Hemangiomas (Dr. Francine Blei)
- Supervision of Children with Sickle Cell Disease (Drs. Zora Rogers, Banu Aygun, Rachelle Nuss)
- Policy Statement on Cord Blood Banking for Future Transplantation (Drs. Bertram Lubin and Mitch Cairo)
- Preservation of Fertility in Pediatric and Adolescent Patients with Cancer (Dr. Stephanie Savelli)
- Treatment of Iron Deficiency and Iron Deficiency Anemia (Drs. Jacquelyn Powers, George Buchanan and Matthew Heeney).

Believing in the value of collaboration, SOHO continues to work with other advocacy, professional, and accreditation organizations. Dr. Maria Velez has agreed to continue...
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Chairperson: Jeffrey Hord, MD, FAAP

Executive Committee:
Gary Crouch, MD, FAAP
Gregory Hale, MD, FAAP
James Harper, MD, FAAP
Jeffrey Lipton, MD, FAAP
Zora Rogers, MD, FAAP

Immediate Past-Chair:
Eric Werner, MD, FAAP

Liaisons:
David Dickens, MD, FAAP
Alliance for Childhood Cancer
Maria Velez, MD, FAAP
Commission on Cancer
Rebecka Meyers, MD, FAAP
AAP Section on Surgery
Gary Crouch, MD, FAAP
Council on Pediatric Subspecialties

Staff:
Suzanne Kirkwood, MS
Manager, Section on Hematology/Oncology

Journal Production Specialist
Mark A. Krajecki

Welcome to Our New Members

If you know of others who might be interested in joining the Academy and the Section please refer them to the AAP website membership page. Thank you to all who have continued to support the AAP and the Section by renewing their memberships. And welcome to new members of the Academy and the Section!

For Upcoming Newsletters . . .

We welcome your input and encourage you to submit ideas or information by email to Carl Allen, MD, FAAP at ceallen@txch.org or Suzanne Kirkwood at skirkwood@aap.org for future issues of the newsletter.

Statements and opinions expressed in this publication are those of the authors and not necessarily those of the American Academy of Pediatrics or the AAP Section on Hematology/Oncology.

to serve as the SOHO representative to the American College of Surgeons Commission on Cancer. In July, the SOHO/AAP joined the newly formed Sickle Cell Disease Coalition and in August agreed to support the American Society of Hematology’s Sickle Cell Disease Call to Action and endorsed “The State of Sickle Cell Disease: 2016 Report”. SOHO is working with the AAP Sections on Radiology and Dermatology in developing a joint program for the 2016 NCE entitled, “Vascular Anomalies: Imaging and Image Guided Treatments”.

My last plea is for each of you to please consider how you might share your expertise and talent to build a stronger SOHO. In exchange, I believe that you will greatly benefit from professional development, enhanced leadership skills, and by getting to know new colleagues from across the nation.
Telementoring: Connecting Primary and Subspecialty Care for Sickle Cell Disease

Project ECHO

Project ECHO (Extension for Community Healthcare Outcomes) is a model of health care delivery, education and care management linking expert specialist teams at academic institutions (“hub”) with primary care clinicians in local communities (“spokes”). This partnership helps patients get the right care, in the right place, at the right time, and promotes medical home goals.

Pioneered at the University of New Mexico (Project ECHO), the ECHO model breaks down the walls between specialty and primary care through the usage of videoconferencing technology to create knowledge networks and leverage scarce resources. The goal is to develop capacity to provide best practice care to treat various common and complex health conditions in medically underserved and rural locations.

The clinics are very similar to grand rounds and combine mentoring, didactics, and de-identified patient case presentations. During case-based presentations, specialists and other participants make recommendations regarding diagnosis and disease management based on the area of specialty. With continuous involvement, healthcare clinicians can increase knowledge and self-efficacy in the management of epilepsy, which in turn, enables patients to receive state-of-the-art and culturally competent care from local primary care providers, whom they know and trust in their communities. This creates a center of excellence in their community, resulting in better quality and greater access to healthcare.

AAP received funding from the Maternal Child Health Bureau to increase access to care and awareness to Children and Youth with Epilepsy (CYE). The AAP partnered with the University of New Mexico (UNM) Project ECHO in November 2013 to implement a CYE ECHO clinic, which launched on June 3rd, 2014. Due to a successful launch and the ability to leverage and enhance limited resources for specialty care required by children with complex medical conditions and the potential to replicate the ECHO model in other programs, domestically and internationally, AAP decided to expand the CYE ECHO program across five additional states (IL, CO, KS, MO, NY). Since then, AAP also received funding from Novo Nordisk to implement an ECHO clinic specifically to address endocrinology short-stature in the southeast region of Georgia.

In 2015, the AAP was designated as an ECHO Superhub. In this capacity, the AAP can conduct trainings, assist with curriculum development, provide technical assistance, and create/expand partnerships. The AAP trained a group from Cincinnati Children’s Hospital to implement an ECHO focused on Sickle Cell Disease (SCD). They launched their first session on March 17th, 2016.

Sickle Treatment Outcomes Research in the Midwest (STORM)

Sickle Treatment and Outcomes Research in the Midwest (STORM) is a regional learning network funded by the Health Resource and Services Administration (HRSA) as one of the SCD Treatment Demonstration Projects. While the Regional Coordinating Center is based at Cincinnati Children's Hospital Medical Center (CCHMC), STORM's regional spread includes pediatric and adult providers in Indiana, Illinois, Michigan, Minnesota, Ohio and Wisconsin. STORM's overall goal is to increase the number of healthcare providers who are knowledgeable about evidence-based management and treatment of SCD, specifically prescribing hydroxyurea.

Because SCD is a rare disorder with limited access to providers, especially for adults, a regional approach to Project ECHO was implemented. The STORM TeleECHO “hub” team is led by the STORM Regional Coordinating Center multidisciplinary team within the Cincinnati Comprehensive Sickle Cell Center. STORM state teams throughout the regional network are actively recruiting providers to participate in the monthly STORM TeleECHO clinics.

The didactic curriculum, presented by nationally recognized hematologists, is based on the National Heart Lung and Blood Institutes’ Evidence-Based Management of Sickle Cell Disease: Expert Panel Report, which was released in 2014.
Topics include management of complications for both pediatric and adult patients, as well as how to best coordinate care between hematologists and primary care providers.

During the first six months of STORM TeleECHO, both primary care providers and hematologists, have been enthusiastic about this telementoring opportunity to learn more about evidence-based care of SCD, but also to begin to network with other providers across the region.

The project, funded through August 2017, will continue recruiting providers to participate in this successful program. Registration information is available for providers on the STORM website at http://sicklestorm.org/echo.html.

References:
1. About ECHO. Retrieved from http://echo.unm.edu/about-echo/

The 2017 ASPHO Pediatric Hematology/Oncology Review Review Course

The 2017 ASPHO Review Course, offered every other year by the American Society of Pediatric Hematology/Oncology (ASPHO), is being held February 2-5, 2017, in Irving, Texas. The highly-regarded intensive course is a practical investment for physicians planning to take the American Board of Pediatrics (ABP) examination for initial certification in pediatric hematology/oncology as well as for those preparing for Part 3 of the ABP’s Program for Maintenance of Certification in the Pediatric Subspecialties. The course is also a beneficial tool for fellows in training and practitioners who wish to review the established standards of care.

Highlights of the course include:
• 26 lectures on specific content for the subboard exam
• Take-home study materials, including a comprehensive syllabus and a compendium of 300 multiple-choice self-assessment test questions
• Free online access to recordings of the presentations
• Up to 25 AMA PRA Category 1 Credits™

The program also offers access to expert faculty, slide presentations, a networking reception, a certificate of completion, and more. Registration opens in October. Please visit www.aspho.org/review or call 847-375-4716 for more information.

ASPHO is accredited by the AACME to provide continuing medical education for physicians.

A Commentary on the TWiTCH Study

Aniket Saha, MD, MS, BiLO
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Greenville, SC

The TWiTCH (TCD with Transfusions Changing to Hydroxyurea) study was designed to answer the question of whether hydroxyurea could be used as an alternative to the standard of care (chronic transfusions) as primary stroke prevention in patients with sickle cell disease (SCD) who had elevated transcranial doppler (TCD) velocities.

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The study was a multi-centered, randomized, phase 3, non-inferiority clinical trial. Eligible patients included those who were aged between 4-16, had elevated TCD velocities, had been chronically transfused for at least 12 months and had no history of stroke, transient ischemic attack (TIA) or vasculopathy. Following a screening protocol (which included MRI, MRA, Ferriscan, TCD, etc), patients were openly randomized to the standard of care (continued chronic transfusions) versus hydroxyurea treatment arms. On the standard treatment arm, patients were transfused at the discretion of the treating physician to keep the hemoglobin S% less than 30%. Patients on the experimental arm began treatment with hydroxyurea (20 mg/kg) and the dose was increased until they reached a MTD. The patients on the experimental arm had their transfusions weaned over several months using a standard protocol. The length of follow-up was 24 months and the primary outcome was TCD velocities at that point. Of the 159 patients who enrolled onto the study, 121 met screening criteria, 60 were randomized to the experimental arm and 61 were in the standard arm. TCD velocities were followed every 12 weeks. MRI and MRA were followed, as were liver iron stores. The first interim analysis was done after all patients had entered the study and 37% of patients exited the study. Non-inferiority between the two arms was present. This was confirmed on repeat analysis when 50% of the patients had exited the study. The study was terminated early. The primary end point was met and there were no differences in the TCD velocities at 24 months between the two arms. No strokes occurred in either group (3 TIAs were seen in each group), there were no new cerebral infarcts in either group and no major differences were noted in terms of serious adverse events amongst the two groups.

The TWiTCH study has several important implications, some of which are listed below:

1. It successfully completed a large, complex, multi-centered, randomized trial in a population in whom the enrollment and completion of trials have, unfortunately, been a challenge. It is clear that well-designed studies such as this are necessary and this will hopefully open the door for more opportunities, including funding, for similar studies to help advance and improve the clinical outcomes in patients with SCD.

2. It gives pediatric hematologists an excellent and viable option, in terms of primary stroke prevention, in a high-risk patient group. Historically, the cumulative incidence of stroke is around 10% of children with SCD and amongst those with elevated TCD velocities (>200 cm/s), the incidence is 40%. The STOP and STOP2 trials had long established, as standard of care, that chronic transfusions were the best way to prevent stroke in this population and that stopping transfusions was not a good alternative. As important as those trials were, hydroxyurea was not tested as an alternative until the current TWiTCH study. Now, based on the results of the early-terminated, highly convincing current study, following 12 months of chronic transfusions, we can now switch these high-risk patients to hydroxyurea, and keep their TCD velocities low and continue to have primary stroke prevention.

3. Finally, it also shows that iron overload in these high-risk patients who were previously chronically transfused and switched to hydroxyurea, can be successfully treated with serial phlebotomy. In fact, in the TWiTCH study, those in the experimental arm had lower serum ferritin and liver iron concentration, when compared with the standard of care group. Furthermore, the change of iron burden within the hydroxyurea group was also significant. The risk of infectious and other complications related to chronic exposure to blood products, while not reported in the study, is obvious.

It will be interesting to see if there are long-term follow-up data being collected in the study participants. One concern this hematologist has is with regards to long-term compliance with an oral daily medication. It could be argued that a patient on chronic transfusions could be expected to show-up for their transfusions and as a result, receive ‘directly observed therapy.’ That benefit is lost when patients are in charge of their own medication and compliance has the potential to become less than optimal.

Despite that concern, this study is seminal and it has given pediatric hematologists an excellent option in primary prevention of stroke in a high risk SCD patient population.
New Focus on the “Second Victims” of Medical Errors

Eric Werner, MD, MMM, FAAP
SOHO Education Subcommittee Chair
Chief Medical Quality Officer
Children's Specialty Group
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In the nearly two decades since the Institute of Medicine report “To Err Is Human” there has been increased focus on the systems-based defects that cause most medical errors and efforts to address these faults. Health care institutions have developed processes to investigate medical errors and have moved from “deny and defend” to disclosure programs that share investigation outcomes and improvement plans with injured patients and their families.

Attention is now being paid to the “second victims” of the faulty processes that contributed to the error, the involved health care workers. Providers involved in medical errors associated with an adverse outcome were instructed not to speak about the events outside of legal counsel for fear of these conversations being subject to legal discovery. Real consequences of such events for physicians included loss of privileges, position and professional respect. For most providers; however, the personal impact has been greater than the professional one. Physical symptoms such as sleeping disturbance, decreased appetite and fatigue may occur as well as psychologic effects like guilt, anxiety and loss of confidence. Examples exist of providers who have left health care entirely or even worse, committed suicide after roles in medical errors.

For many years, the culture in medicine and medical education has been punitive when adverse events occur, but this is changing. While there is little literature on the optimal approach to helping providers work through these stresses, many health care institutions are developing support programs. At St. Jude Children’s Research Hospital, Burlison et al developed and validated a Second Victim Experience and Support Tool to assess effects on health care workers and the quality of available support services. (Burlison, J.D. J Patient Saf, 2014). Risk managers and legal counsel often have experience in helping providers through this period, but they may have other obligations including managing the potential legal ramifications of the event. Training colleagues to become knowledgeable, early responding peer support has great potential. Such individuals can acknowledge the stresses the second victim is having, be a positive and active listener, share similar experiences and validate her/his worth. While there are financial, time and other barriers to professional intervention for second victims, leadership should help providers move past these obstacles when symptoms so dictate. Attending physicians need to be especially sensitive to the impact that medical errors can have on trainees, even when there is not a serious adverse outcome.

Alongside programs designed to prevent medical errors, health care institutions and their leaders need to develop support systems that address the often serious consequences that occur in health care workers including attending and trainee physicians associated with such events.

Additional Reading:
Pediatric hematology/oncology and quality/safety go hand-in-hand, because we were one of the first specialties to consistently practice evidence-base medicine, follow outcomes and improve them through constant modifications of our treatments (plan-do-study-act or versions of protocols). The American Society for Hematology has published “Choosing Wisely” guidelines for 3 years in a row, and several other societies (such as the National Heart, Lung and Blood Institute’s sickle cell guidelines, or the Chest guidelines for Antithrombotic Therapy for VTE Disease) have produced evidence-based materials to guide our treatments in hematology. On the other hand, we are also a discipline that creates one of the highest numbers of errors, mostly due to the complexity of our therapies and the constant need to adjust to the organ function of our patients.

But despite all this, there is still room for improvement. We still have too much unnecessary variability in our care processes, which not only confuses our patients, families and trainees, but also creates the potential for errors. For example, just consider the different opinions regarding a “safe” absolute neutrophil count to allow discharge, or the ongoing discussions about antibiotic prophylaxis for neutropenic patients, hospitalizations for patients with immune thrombocytopenia, and the need to pre-operatively transfuse (or not) a patient with sickle cell disease who also happens to be a Jehovah’s witness. We have variability in regards to mouth care of patients on chemotherapy, choice of central line, whether to give high-dose methotrexate as an in- or out-patient, and when to recommend the wearing of a mask.

As the above list shows, it is mainly in the areas of supportive care where our opinions differ. The reason is the fact that although we are great at writing protocols to answer a research question, we are not as facile in using quality improvement (QI) methods to start tackling some of these other issues. Fortunately, education about QI is now incorporated into student and resident curricula, and many of our fellows have at least a basic understanding of quality, safety and process improvement.

Many organizations have a strong emphasis on eliminating harm to children (for example, Solutions for Patient Safety, http://www.solutionsforpatientsafety.org/, now has over 100 children’s hospitals as members), and the goal of becoming a High Reliability Organization (HRO) has been integrated into many strategic plans (Table 1 - See Page 8). Organizations such as hospitals, nuclear power plants, and air traffic control agencies operate in fast-paced, high-risk circumstances, but seek to ensure high levels of safety for customers (patients), employees and the public. Safety is one of many organizational goals that can be pursued with high reliability. Reliability is measured as the inverse of the system’s failure rate. It is estimated that the U.S. health system has a failure rate of 1 in 10 (i.e., we do it right only about 90% of the time); we thus perform at a level of 10⁻¹. A performance level of 10⁻¹ relies on basic standardization, such as guidelines, standardized order templates, memory aids such as checklists, feedback mechanisms regarding compliance with standards and awareness-raising, as well as training of new staff. To get to the next level (10⁻², or 95% reliability) we need to implement real-time identification of failures, introduce redundancy, such as double verification of chemotherapy orders, and create an environment that makes the right way the easy way to do it. Level 3 (roughly 99% or fewer than five failures per 1,000) starts to get to the core principles of HRO, but high reliability industries such as the nuclear power industry function at or above the 10⁻⁶ level. We already know that very high performance levels are possible to achieve in medicine: the safety of blood transfusions or general anesthesia approaches a low failure rate of 10⁻⁵, similar to the airline industry.

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Table 1

High Reliability Principles

**Preoccupation with failure:** Real time awareness of failures, achieved by daily monitoring of processes, reporting of near misses, and an enhanced sensitivity to processes that could potentially fail before they actually do.

**Reluctance to simplify:** The first, obvious explanation for a failure may not be the right one, and it is rarely a single issue that leads to the error. We need to dig deeper!

**Sensitivity to operations:** Leaders and staff are constantly aware of how processes and systems affect the organization. Any process that does not work is highlighted and modified in real time. Transparency is a valuable tool to increase sensitivity to operations and to constantly improve.

**Commitment to resilience:** Failures and especially near-miss situations are considered learning opportunities. High reliability organizations are constantly learning, improving, and testing new ways of operating. This takes skilled people that have the appropriate tools, as well as adequate time to evaluate, measure and implement. A commonly used tool is the Plan-Do-Study-Act (PDSA) cycle.

**Deference to expertise:** This includes taking advantage of the different levels and areas of expertise that team members contribute, and the recognition that the most senior person is often not the most knowledgeable. Just like we use multi-disciplinary teams to discuss patient care, we may use the same approach to discuss any real or potential failures.


How does the AAP help you to become an expert in quality and safety? The Council on Quality Improvement and Patient Safety (COQIPS, [https://www.aap.org/en-us/about-the-aap/Committees-Councils-Sections/coqips/](https://www.aap.org/en-us/about-the-aap/Committees-Councils-Sections/coqips/)) is focused on innovations and activities supporting the integration of policy and practice, education, advocacy, and implementation around the topic areas of quality improvement and patient safety. We have several subcommittees, including Committees for Education, Measurement, Policy and Advocacy Committee (PAC), Guidelines, Evidence and Transparency (GETS), Patient Safety, Implementation Committee, Membership and Bylaws Committee. The council offers many opportunities to network, learn, and lead in the areas of membership, implementation, education, evidence-based guidelines, quality measurement, and patient safety. We encourage you to join us if you are interested in working to improve the quality of care for children. We would love to welcome you as a member! [Join here](https://www.aap.org/en-us/about-the-aap/Committees-Councils-Sections/coqips/)

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Hot Papers in Pediatric Hematology/Oncology

**Reviewed by:** Mary Jane Staba Hogan, MD, FAAP, Assistant Clinical Professor, Pediatric Hematology Oncology, Yale University School of Medicine, New Haven, Connecticut.


Investigators from multiple international institutions analyzed cytogenetic data from 427 children with relapsed B-cell precursor acute lymphoblastic leukemia treated on the ALLR3 trial, of which 238 with marrow relapse were screened for selected copy number alterations and mutations predictive of outcome post-relapse. This study confirmed previous

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research from different chemotherapy regimens showing a higher prevalence of \textit{TP53} and \textit{NR3C1} alterations noted at first relapse. They discovered that \textit{TP53} alterations and deletions of \textit{NR3C1} or \textit{BTG1} were associated with a higher risk of progression (hazard ratio 2.36; 95% confidence interval, 1.51–3.70, \textit{P}<.001, and 2.15; 1.32–3.48, \textit{P}=.002). In addition, NRAS mutations were associated with an increased risk of progression among standard-risk patients with high hyperdiploidy (3.17; 1.15–8.71, \textit{P}=.026). Patients classified clinically as standard and high risk had distinct genetic profiles at relapse. The outcome of clinical standard-risk patients with high-risk cytogenetics was equivalent to clinical high-risk patients. Practical implications include screening children at relapse for similar genetic abnormalities, integrating genetic with clinical risk factors to stratify relapsed patients to treatments and to discover potential, targeted, relapse therapies.


Investigators from multiple institutions conducted an international, open-labeled, randomized, phase 3 trial comparing post-operative cisplatin, doxorubicin and methotrexate (MAP) versus MAP with ifosfamide and etoposide (MAPIE) in patients with newly diagnosed, resectable, high-grade osteosarcoma who had a poor response (≥10% viable tumor) to preoperative chemotherapy. Between 2005 and 2011, 618 patients were randomly assigned; 310 to receive MAP and 308 to receive MAPIE. Median follow-up was 62.1 months (IQR 46.6–76.6). Event-free survival events, deaths and ultimately event-free survival did not differ between treatment groups (hazard ratio [HR] 0.98 [95% CI 0.78–1.23]). In addition, the most common grade 3–4 adverse events, neutropenia, thrombocytopenia and febrile neutropenia without documented infection were similar between treatment groups. However, MAPIE was associated with more frequent grade 4 non-hematological toxicity than MAP (35 [12%] of 301 in the MAP group vs 71 [24%] of 298 in the MAPIE group).

Although prior studies suggested improved outcomes by adding intensification post-operatively for poor-responders, only one of these studies was randomized comparing upfront three-drug therapy to three-drug therapy plus ifosfamide. That prior study’s post-operative treatment was non-randomized to include ifosfamide. The authors conclude that the findings of this randomized trial do not support intensification of postoperative chemotherapy in patients with poor response to pre-operative therapy and that new targeted drugs are needed to improve outcomes.


This synopsis and commentary on clinical guidelines released between July 2014 and February 2016 pertain to survivors of Hodgkin (HL) or diffuse large B-cell lymphomas (DLBCL) that have completed therapy and are without clinical or radiographic evidence of disease (in remission). Investigators from the National Comprehensive Cancer Network (NCCN) and the European Society of Medical Oncology (ESMO) developed follow-up surveillance imaging recommendations to decrease the amount of unnecessary positron emission tomography (PET) and computed tomography (CT) radiation exposure, contrast-induced nephropathy and false-positive results without disturbing outcomes. Although there are no designated pediatric oncologists on either committee, these suggestions based on retrospective, non-randomized studies and consensus opinions, may be helpful for affected adolescent and young adults. However, since patients with HL and DLBCL are heterogeneous, clinical judgment should ultimately guide decision-making. The Children’s Oncology Group currently has slightly different guidelines from the following listed in this update:

- For HL patients in remission, image as clinically indicated (for abnormal laboratory results, atypical examination findings, or abnormal symptoms). Surveillance with PET/CT scans is not recommended.
- For women with HL and prior chest/axillary radiation, image with yearly magnetic resonance imaging or mammograms starting 8 to 10 years after therapy completion or at age 40 years, whichever comes first.
- For DLBCL patients in remission, image as clinically indicated (NCCN) or at 6, 12, and 24 months only (ESMO).

The primary approach to treat symptomatic patients with Immune Thrombocytopenia (ITP) has historically been to play a good defense and suppress the action of acquired anti-platelet antibodies. The use of thrombopoietin receptor agonists (TPO-RA) has, for the first time, allowed hematologists to play offense and encourage platelet production to out produce the anti-platelet antibodies, reduce bleeding and improve health-related quality of life. The Pediatric ITP Consortium of North America (ICON) set out to study the clinical approach to treating pediatric ITP through a multi-institutional retrospective review. Of the patients enrolled from twelve participating centers, 79 reported use of a TPO-RA. Although most of these patients had chronic ITP, 18% had newly diagnosed, symptomatic ITP that was refractory to first line therapy. The results highlight the diverse off-label use of TPO-RA agents and help advocate for a prospective trial to study the efficacy of TPO-RA in the newly diagnosed and persistent clinical periods. The study demonstrates the efficacy for these agents to raise platelet counts and reduce symptomatic bleeding. This helps reinforces the results of previously published randomized clinical trials that demonstrate similar efficacy to raise platelet number in pediatric patients.


The hyperinflammatory disorder hemophagocytic lymphohistiocytosis (HLH) is clinically feared by providers for its multisystem involvement, challenging diagnosis and therapy, and often grim prognosis. The authors of this article and their colleagues who study immune regulatory disorders have sparked a new wave of understanding in the field as they are better able to define and identify a growing number of genetic defects that result in this downstream hyperinflammatory state. This recent publication by Bernice et al highlights the role Cytotoxic T Lymphocyte antigen-4 (CTLA-4) plays as a critical inhibitory checkpoint of immune activation and two novel genetic diseases that result in CTLA-4 deficiency. Understanding the pathways that control basic communication between T and B cells allows us to better classify symptomatic patients. This understanding allows us to move forward with less fear and more hope for more targeted therapies and better clinical outcomes.


The decision to carry out splenectomy in any patient with chronic hemolytic anemia is never easy. Providers and parents question the efficacy, safety and degree of resulting complications of splenectomy, most notably acquired immunodeficiency. Efforts both in Europe and in North America to study these questions have started to turn consensus opinion into evidence-based practice. In this article by Guizzetti, the current literature evaluating total versus partial splenectomy in the pediatric Hereditary Spherocytosis (HS) population is reviewed. This meta-analysis of recently published articles found that partial splenectomy (PS) significantly increased the hemoglobin concentration by an average of 2.2 g/dl and total splenectomy (TS) resulted in an average increase of 3.6 g/dl. Both procedures improved clinical outcome and fatal events were extremely rare across all reported studies. Length of hospital stay and duration of surgery were comparable between groups. The article fuels a dialog on a very common and difficult to study clinical intervention. The author advocates for new consensus guidelines for the monitoring HS patients, to increase efforts to measure splenic function, advocates for a registry to track outcomes and encourages the development of a prospective study to formally compare the clinical outcome of patients who receive PS versus TS.


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Hereditary Hemorrhagic Telangiectasia (HHT), also known as Osler-Weber-Rendu disease, is an inherited vascular condition that results in the development of arteriovenous malformations of various size throughout the body. Increased clinical recognition of pediatric HHT has resulted in the formation of a growing number of pediatric HHT “centers of excellence” nationally and internationally. However, diagnostic and treatment guidelines continue to remain primarily based on adult data. The authors of this article aimed to study the treatment of epistaxis in adult HHT patients. As epistaxis is a symptom of particular importance to the pediatric population, the study is of particular importance to ongoing efforts to improve quality of life in our pediatric HHT patients. Whitehead et al carried out a randomized clinical trial to determine whether use of topical bevacizumab, estriol, or tranexamic acid could reduce HHT-related epistaxis. Ultimately, none of the topical therapies significantly reduced epistaxis in the study population. As pediatric specific studies remain quite limited, these results will help pediatric vascular anomalists standardize the treatment of epistaxis in pediatric HHT.

Inspiring story by Dr. Howard Pearson about Paul Newman’s Hole in the Wall Gang Camp for children with life-threatening diseases

“Fulfilling Paul Newman’s Dream—‘Raising a Little Hell’ and Healing at The Hole in the Wall Gang Camp” is the amazing true story of a magical place created for children with life-threatening diseases under the leadership of actor/philanthropist Paul Newman. Paul wanted a place where children battling cancer and other diseases could just be kids and “raise a little hell.” The book was co-authored by former AAP President (1992-93) and section member Howard “Doc” Pearson, MD, and his daughter-in-law, Mary Lou Shefsky, MPH. Paul relied on Doc to make The Hole in the Wall Gang Camp in Connecticut medically safe for the children. Doc was the Camp’s Founding Medical Director and its physician in residence for 14 summers. He served on Camp’s Board of Directors until 2014. A distinguished academic pediatric hematologist/oncologist, Dr. Pearson has over 300 published medical articles and several textbooks. A graduate of Harvard Medical School, he was Chairman of the Department of Pediatrics at Yale Medical School for 12 years, and is now (at age 86) a Yale Professor Emeritus residing in Orange, CT.

In this memoir, Doc describes how The Hole in the Wall Gang Camp (“Camp”) happened through Paul Newman’s hands-on involvement and conveys its dynamics and exuberance that have helped children and their families experience healing since 1988. Included are 200 photographs that bring the program to life, as well as the stories of the nine towering totem poles that Doc carved to reflect Camp’s history, promise, hope, whimsy, and fulfillment. “Fulfilling Paul Newman’s Dream” is now available from on-line vendors (e.g., amazon.com and barnesandnoble.com) at a list price of $19.95. Any profits will be donated to the Camp, a nonprofit organization. Visit the book’s website: www.fulfillingpaulnewmansdream.org

Join the AAP Mentorship Program

Mentorship is one of the most important tools for professional development and has been linked to greater productivity, career advancement, and professional satisfaction. The AAP recognizes that mentorship is critical in helping to nurture and grow our future leaders and that a mentorship program is a key opportunity to engage new and existing members. The AAP Mentorship Program seeks to establish mentoring relationships between trainees/early career physicians and practicing AAP member physicians. Click here for more information and to join the program.

Please note: Mentors are asked to commit at least one full year. However, the program offers opportunities for short-term “flash” mentoring. Mentors/mentees will be asked to set regular phone meetings to discuss mentee goals, objectives, and progress. Mentors/mentees should also answer all communications in a timely manner.
NHLBI Announces New Strategic Vision

More than a year ago, the National Heart, Lung, and Blood Institute (NHLBI) reached out to its stakeholders from across the United States and around the globe. More than 4,000 individuals answered the call to identify the most critical research questions and challenges for the next decade. The final result, *Charting the Future Together: The NHLBI Strategic Vision*, can be found [here](#).

**What’s the Latest with the Flu: Information from the AAP**

The American Academy of Pediatrics (AAP) policy “Recommendations for Prevention and Control of Influenza in Children, 2016-2017” offers updated recommendations for routine use of seasonal influenza vaccine and antiviral medications for the prevention and treatment of influenza in children. Important details are highlighted in the AAP News articles “Intranasal Flu MISSED its Target” and “AAP Updates Recommendations for Flu Vaccine in Children”. In addition, see the Centers for Disease Control and Prevention (CDC) Morbidity and Mortality Weekly Report “Prevention and Control of Seasonal Influenza with Vaccines: Recommendations of the Advisory Committee on Immunization Practices” for more information. [Read More](#)

**Archived Webinars:**

Rational Pain Management in Children with Chronic Medical Conditions

Pain is a common feature of chronic medical conditions in children, rational management of which often requires an interdisciplinary multi-modal approach. Numerous non-pharmacologic interventions and non-opioid medications may be effective, potentially reducing requirement for opioid. Chronic opioid may be appropriate in some settings, but is associated with significant inherent risk and frequently daunting regulatory logistics. This webinar, the fourth in a series of 6 sponsored by the American Academy of Pediatrics, will review management strategies and therapeutic options for rational pain management in children with chronic medical conditions.

This and other archived Webinars related to the topic of opioids can be accessed on the [AAP Committee on Substance Use and Prevention website](#).

Helping a Family with Traumatic Stress when a Child has Cancer

Sponsored by the National Child Traumatic Stress Network, in this first webinar of the series, Helping a Family with Traumatic Stress when a Child has Cancer, psychologist Anne Kazak will be joined by oncologist Eric Sandler and parents Vicki and Peter Brown to discuss an integrated approach to recognizing and responding to child and family traumatic stress when children have cancer. They will explore the impact of the diagnoses and treatment on the child and family, discuss cultural considerations that may intersect with a family’s response, and describe their approach for assessing, managing and treating traumatic stress. View this free webinar after creating an account at: [http://learn.nctsn.org/mod/nctsnwebinar/view.php?id=11242](http://learn.nctsn.org/mod/nctsnwebinar/view.php?id=11242)

**Financial Wellness Series from the AAP**

[Buying insurance](#) isn't hard and doesn't have to be a hassle if you know what you need. Find out what is available through your association and take part in the AAP Insurance Program’s Financial Wellness Series or short, informative videos. Subscribe to our [blogs](#) each week and [view other webinars](#) on student loan refinancing, money management and other topics.