



### MESSAGE FROM THE CHAIR

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Welcome to the fall 2012 issue of the Duke University Department of Pediatrics Alumni Organization newsletter. Autumn is always a dynamic time for the Department of Pediatrics—new students, trainees, and faculty members are settling in, and new programs and initiatives reflecting all levels of our mission, namely patient care, medical education, research, and advocacy, are beginning to materialize.

This summer we welcomed 12 new faculty members spanning ten of our divisions to the Department of Pediatrics. In addition, 16 new interns in Pediatrics, six new interns in Medicine-Pediatrics, and 21 new subspecialty fellows joined us from top institutions across the country, all with superb records of accomplishment. This year's Pediatric Education Day was held on October 2 and featured guest speakers Carol Carraccio, MD, MA and Robert Englander, MD, MPH, who have been instrumental in creating the national Milestones Project and in defining Entrustable Professional Activities (EPAs), both of which are key components of the ACGME's Next Accreditation System (NAS), going into effect for Pediatrics in July 2013.

As you will see inside, our faculty continue to have award-winning impact on science and medicine, with broad visibility that heightens national recognition of our department. A number of our faculty have also received new independent investigator awards from the National Institutes of Health and other federal agencies, an especially impressive accomplishment given the challenging funding environment.

It was rewarding to see friends at the Duke Pediatrics Alumni Reception last spring, and we look forward to more chances to reconnect at events this fall, including the Duke Medical Alumni Weekend that is scheduled for October 18-21. This weekend will include a breakfast for Duke Pediatrics Alumni and friends on Saturday, October 20 at 8:00a.m. in the Lobby of the McGovern-Davison Children's Health Center, an event I hope you can attend.

I look forward to the coming year with a great sense of enthusiasm about our future. I thank all of you for the continued support, and I look forward to sharing more news as the year continues to evolve.

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We would like to hear from you! If you have news about yourself or if you would like to send us comments about this newsletter, please write Duke Pediatrics Alumni News, Box 3352, Durham, NC 27710, or e-mail us at [rebecca.casey@duke.edu](mailto:rebecca.casey@duke.edu).

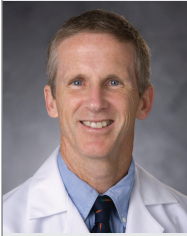


Joseph W. St. Geme III, MD  
Chair, Department of Pediatrics  
Duke Medicine



**Bordley Receives Faculty Mentor Award**

The Duke Department of Pediatrics has honored **Clay Bordley, MD, MPH**, Associate Professor of Pediatrics, Associate



Professor in Surgery, Chief of the Pediatric Division of Hospital and Emergency Medicine, and Medical Director of the Pediatric Emergency Department, with the Faculty Mentoring Award.

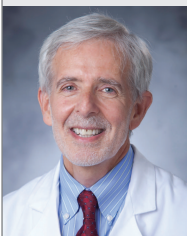
This award recognizes mentoring excellence by a department faculty member who exemplifies a deep commitment to fostering the professional and personal development of trainees and junior faculty in any or all aspects of faculty or trainee responsibilities, including patient care, education, research, and advocacy.

Bordley is nationally recognized as an innovative educator and an outstanding investigator and clinician in pediatric emergency medicine and general pediatrics. Bordley's reputation as an accessible mentor who fosters a positive environment of openness and creativity was evident in the letters of recommendation supporting his nomination.

Bordley was recognized and presented with a plaque during the final Grand Rounds of the 2011-12 academic year.

**Freemark Awarded Michael M. Frank Research Prize**

**Michael Freemark, MD, PhD**, Chief of the Pediatric Division of Endocrinology and Robert C. Atkins, MD and Veronica Atkins Professor of Pediatrics, has received the 2012 Michael M. Frank Research Prize. This

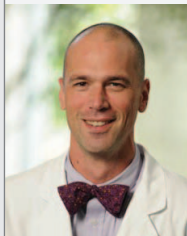


award was established in 2004 with gifts from the Duke Children's National Board of Advisors to honor Dr. Michael Frank for his service as Chairman of the Department of Pediatrics from 1990-2004 and to

recognize a member of the Duke University community who has made outstanding contributions to the health care of children. Freemark has conducted groundbreaking laboratory and clinical research in the fields of metabolism and growth over the course of his illustrious career.

**Benjamin Receives the Ruth and A. Morris Williams, Jr. Faculty Research Prize**

**Danny Benjamin, MD, PhD**, Professor of Pediatrics, Faculty Associate Director of the Duke Clinical Research Institute, and Chair of the Pediatric Trials Network, has received the 2012 Ruth and A. Morris Williams, Jr. Faculty Research Prize. This award is presented annually to a junior faculty member at the Duke University School of Medicine who demonstrates the intellectual vigor, dedication, and scientific ingenuity needed to make a



critical impact on the future of medical research.

In his role as Chair and Principal Investigator of the Pediatric Trials Network (a \$95,000,000 initiative funded by NICHD to study off-patent therapeutics in

children), Benjamin is responsible for the largest pediatric trials program in the world. His team initiated seven clinical trials and three pharmaco-epidemiology projects in 2011 and will initiate 10 clinical trials in 2012. Data from each of these trials will be submitted to the FDA for pediatric labeling.

In addition, Benjamin has made major contributions to our understanding of the diagnosis and treatment of neonatal infections, with particular emphasis on neonatal candidiasis. He has also played a leadership role on the drug development collaboration involving the FDA, the NICHD, and the European Medicines Agency.

**Smith Distinguished for Outstanding Mentorship**

**Brian Smith, MD, MPH, MHS**, Associate Professor of Pediatrics and Chief of the Division of Quantitative Sciences, has



received the 2012 Robert M. Califf Award for Outstanding Mentorship. This award was established to recognize a Duke Clinical Research Institute (DCRI) faculty member who has demonstrated dedicated

excellence in mentorship activities for DCRI research fellows, based on the example set by Robert Califf, MD, Vice Chancellor of Clinical Research and Director of the Duke Translational Medicine Institute.

Announcing the award at the annual DCRI reception, **Eric D. Peterson, MD, MPH**, Director of the Duke Clinical Research Institute, said, "Dr. Smith has a unique combination of clinical and quantitative/statistical skills that uniquely qualify him to be a successful clinical researcher and make him an excellent role model for young aspiring investigators. He possesses unflagging energy, a strong work ethic, generosity of spirit, and most importantly to the DCRI fellows, the commitment to teach."

**New Faculty in the Department of Pediatrics since July 2012**

**Jillian Crouchley, MD**  
Neonatology

**Shaequa Dasnadi, MD**  
Neonatology

**John Galiote, MD**  
Neonatology

**Heather Henderson, MD**  
Cardiology

**Sujay Kansagra, MD**  
Neurology

**Daniel Landi, MD**  
Blood and Marrow Transplantation

**Lindsay Lefler, MD**  
Primary Care

**Loren Pena, MD**  
Medical Genetics

**Jack Sharp, MD**  
Pulmonary and Sleep Medicine

**Amy Stallings, MD**  
Allergy and Immunology

**Narayanan Venkatasubramani, MD**  
Gastroenterology

**Kevin Watt, MD**  
Critical Care Medicine

**Rachel Greenberg, MD**  
Co-Chief Resident

**Charles Wood, MD**  
Co-Chief Resident

## Duke Researchers Find Gene Mutation for Childhood Disorder

Alternating hemiplegia of childhood (AHC) is a rare disorder that usually begins in infancy, with intermittent episodes of paralysis and stiffness, first affecting one side of the body, then the other. Symptoms mysteriously appear and disappear, and affected children often experience dozens of episodes per week. As they get older, children fall progressively behind their peers in both intellectual abilities and motor skills, and more than half develop epilepsy. Unfortunately, medications that work for epilepsy have been unsuccessful in controlling the recurrent attacks of paralysis, leaving parents and physicians with few options and significantly disabling those affected.

In a study that was published in *Nature Genetics* in September (2012;44:1030-1034), **Dr. Mohamad Mikati**, Chief of the Duke Pediatric Division of Neurology, and researchers at the University of Utah discovered that mutations in the ATP1A3 gene cause the disease in the majority of patients with a diagnosis of AHC.

The ATP1A3 gene encodes one piece of a key transporter molecule that normally would move sodium and potassium ions across a channel between neurons to regulate brain activity. Mutations in this gene are already known to cause another rare movement disorder called rapid onset dystonia parkinsonism, and clinical testing for mutations in this gene is readily available through a blood test.

“Out of about 100 patients that were tested, three-quarters had the mutation,” said Mikati. “Before there was just a clinical diagnosis—now, there is going to be a test that will help confirm the diagnosis. This advance means children can be diagnosed earlier and their symptoms treated more effectively.”

## Newly Discovered Breast Milk Antibodies Help Neutralize HIV

Antibodies that help stop the HIV virus have been found in breast milk. **Sallie Permar, MD, PhD**, Assistant Professor of Pediatrics in the Division of Infectious Diseases, isolated the antibodies from the B lymphocytes in the breast milk of infected mothers in Malawi and showed that the B cells in breast milk can generate neutralizing antibodies that may inhibit the virus that causes AIDS.

HIV-1 can be transmitted from mother to child via breastfeeding, posing a challenge for safe infant feeding practices in areas of high HIV-1 prevalence.

However, only one in 10 HIV-infected nursing mothers is known to pass the virus to their infants. “This low transmission rate is remarkable, because nursing children are exposed multiple times each day during their first year of life,” said Permar. “We are asking if there is an immune response that protects 90 percent of infants, and could we harness that response to develop immune system prophylaxis during breastfeeding for mothers infected with HIV-1.”

“Our work helped establish that these B cells in breast milk can produce HIV-neutralizing antibodies, so enhancing the response or getting more mucosal B-cells to produce those helpful antibodies would be useful, and this is a possible route to explore for HIV-1 vaccine development,” Permar added.

The study was published on May 18 in *PLoS One*, an open-access journal published by the Public Library of Science.

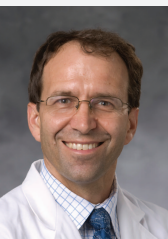


## Targeted Gene Therapy Enhances Treatment for Pompe Disease

Gene therapy to replace the protein missing in Pompe disease can be effective if the patient’s immune system does not react against the therapy. Targeted delivery of the gene to the liver, instead of throughout the body, suppresses the immune response, improving the therapeutic effect, according to a study published in the June issue of *Human Gene Therapy*.

“The current unmet medical need in Pompe disease is for prevention of immune responses against standard-of-care enzyme replacement

therapy,” says co-author **Dwight Koeberl, MD, PhD**, Associate Professor of Pediatrics in the Division of Medical Genetics. “However, we foresee a future application of the dual vector strategy described in this paper, including a liver-expressing vector along with a ubiquitously expressing vector, which might achieve much higher efficacy than either vector alone.”



In this study, **Ping Zhang, PhD**, and colleagues in Dr. Koeberl’s laboratory, targeted a gene delivery vector carrying the therapeutic gene to the livers of mice with Pompe disease. Not only did the liver-specific expression of the protein induce immune tolerance, but when combined with non-targeted delivery of the therapeutic gene, it also boosted the overall effectiveness of the treatment.

## NEW INITIATIVES

### New Federal Awards

The following new major federal awards were recently received by faculty in the Department of Pediatrics:

**Coleen Cunningham, MD**, Principal Investigator. HRSA. Title: “Ryan White Title IV Women, Infants, Children, Youth, and Affected Family Members AIDS Healthcare.” Project period: 08/01/2012 – 07/31/2015.

**Sallie Permar, MD**, Principal Investigator. NIH. Title: “Antibody Protection Against SIV Transmission.” Project period: 03/01/2012 – 02/28/2014.

**Sallie Permar, MD**, Principal Investigator. NIH. “Vaccine-Induced HIV Antibody Responses in Infants.” Project period: 07/01/2012 – 06/30/2014.

**William Steinbach, MD**, Principal Investigator. NIH. “Identification of Calcineurin-Binding Proteins in *Aspergillus fumigatus* Septum Formation.” Project period: 07/01/2012 – 06/30/2014.

**William Steinbach, MD**, Principal Investigator. NIH. “Novel Biomarkers for Early Detection of Aspergillosis.” Project period: 06/01/2012 – 05/31/2013.

**Yong-Hui Jiang, MD**, Principal Investigator. NIH. “Analysis of Shank3 Complete and Temporal and Spatial Specific Knockout Mice.” Project period: 06/01/2012 – 04/30/2017.

**Xiaoping Zhong, MD**, Principal Investigator. NIH. “TSC1-m TOR Signaling and T Cell Tolerance.” Project period: 05/01/2012 – 04/30/2017.

**Sallie Permar, MD**, Principal Investigator. NIH. “Maternal Immune Protection Against Congenital CMV Infection.” Project period: 09/30/2012 - 07/31/2017.

If you would like to receive this newsletter via e-mail,  
please send your e-mail address to [diane.crayton@duke.edu](mailto:diane.crayton@duke.edu).

## NOTEWORTHY

**Duke Children's Recognized for Top Pediatric Care**

Duke Children's Hospital & Health Center is proud to be recognized by *U.S. News & World Report* in its 2012-13 Best Children's Hospitals edition. Duke was ranked among the top 50 nationally in all 10 areas of pediatric specialty care (cancer, cardiology and heart surgery, diabetes and endocrinology, gastroenterology, neonatology, nephrology, neurology and neurosurgery, orthopedics, pulmonology, and urology), a unique distinction among pediatric centers in North Carolina.

According to *U.S. News & World Report*, these rankings "help guide patients who need high-stakes care because of the complexity or difficulty of their condition or procedure."

The results are based on an extensive analysis using a methodology that combines data from a survey of physicians, a direct survey of children's hospitals and other resources.

The data reflect hospital quality in three key aspects of hospital care: clinical outcomes such as cancer survival; the process of care, which included a hospital's national reputation among pediatric specialists and its compliance with "best practices" and steps to control infection; and adequacy of care-related indicators of quality such as patient volume, degree of nurse staffing, and availability of specialized programs. Ranking was based on a hospital's combined score in the three elements.

"We are proud and honored to be ranked by *U.S. News and World Report* in all 10 programmatic areas," says **Joseph St. Geme, MD**, Chair of the Duke Department of Pediatrics and CEO of Duke Children's Hospital & Health Center. "I firmly believe that this distinction is reflective of the breadth and depth of talent and expertise among our faculty and clinical staff."

## EDUCATION FUND

**Deborah Kredich Pediatric Education Fund**

The Deborah Kredich Pediatric Education Fund provides support for a variety of educational experiences for residents and subspecialty fellows at Duke, including participation in research projects, attendance at national meetings, involvement in community outreach programs, and hosting of a special Grand Rounds.

**Please send your donation to the Deborah Kredich Pediatric Education fund to:**

Deborah Kredich  
Pediatric Education Fund  
DUMC 2975  
Durham, NC 27710

## UPCOMING EVENTS



## The School of Medicine Medical Alumni Weekend 2012

October 18-21, 2012

Medical Alumni Weekend is scheduled for October 18 - 21 and will include breakfast for Duke Pediatrics alumni on Saturday, October 20 at 8:00am in the McGovern-Davison Children's Health Center.

We hope you'll join us. Please RSVP to Diane Crayton at:  
919-681-3260 or [diane.crayton@duke.edu](mailto:diane.crayton@duke.edu).

**Pediatric Alumni Grand Rounds 2012**

The Department of Pediatrics will host the Pediatric Alumni Grand Rounds on Tuesday, October 16, 2012, immediately preceding the annual School of Medicine Medical Alumni Weekend. This year's speaker is Leslie E. Lehmann, MD, Assistant Professor, Department of Pediatrics, Harvard Medical School and Medical Director, Pediatric Stem Cell Transplant Unit, Children's Hospital Boston.