Duke University Department of Pediatrics Your Pediatrics Alumni Community

SPRING 2017

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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.

MESSAGE FROM THE CHAIR

Welcome to the spring 2017 issue of the Pediatrics Alumni Organization newsletter. During the period since the last newsletter, our outstanding faculty, staff, trainees and students have made extraordinary achievements in all aspects of our mission, including clinical programs, educational activities, and research and advocacy initiatives.

I would like to highlight that the Duke Department of Pediatrics has been ranked third nationally on the list of NIH funding for pediatrics clinical science departments. As you know, success in securing NIH funding is considered a strong indicator of the quality of an institution's research. The number of people contributing to our department's success in research is commendable, and we hope to continue to build on this as we mentor and support junior research faculty.

In addition, we continue to recruit bright and motivated residents and subspecialty fellows who add impressive diversity and talent to our community. This year, the department matched 15 interns into our categorical pediatrics program, six into our medicine/pediatrics program and two who will train in child neurology. Our newest trainees geographically hail from as close as our own Duke School of Medicine to as far away as the state of California. We are thrilled they have chosen Duke as the place to establish their identities as pediatricians.

I would also like to thank all who attended and participated in this year's Pediatrics Research Retreat, which was held on April 18 and featured keynote speaker Robert M. Califf, MD, Former Commissioner of the U.S. Food and Drug Administration. It was another outstanding year showcasing the science and discovery accomplished by our talented faculty and staff with a record number of abstracts submitted.

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.



More extremely preterm babies survive, live without neurological impairment

Babies born at just 22 to 24 weeks of pregnancy continue to have sobering outlooks — only about 1 in 3 survive.

But according to a new study led by Duke Health and appearing Feb. 16 in the *New England Journal of Medicine*, those rates are showing small but measurable improvement. Compared to extremely preterm babies born a decade earlier, the study found a larger percentage are developing into toddlers without signs of moderate or severe cognitive and motor delay.

Changes to prenatal care, including greater use of steroids in mothers at risk for preterm birth, could have contributed to increased survival and fewer signs of developmental delay in these infants, the authors said.



"The findings are encouraging," said lead author Noelle Younge, MD, a neonatologist and assistant professor of pediatrics at Duke. "We see evidence of improvement over time. But we do need to keep an eye on the overall numbers, as a large percentage of infants born at this stage still do not survive. Those who survive without significant impairment at about age 2 are still at

risk for numerous other challenges to their overall health."

The researchers analyzed the records of 4,274 infants born between the 22nd and 24th week of pregnancy, far earlier than the 37 to 40 weeks of a full-term pregnancy. The babies were hospitalized at 11 academic medical centers in the Neonatal Research Network, part of the Eunice Kennedy Shriver National Institute of Child Health and Human Development at the National Institutes of Health.

In addition to their work with the Neonatal Research Network studying strategies to improve outcomes for preterm babies, the Duke researchers continue to study environmental and genetic factors, as well as the babies' gut bacteria and metabolomics.

"We're always looking at how we can make further headway and continue to improve survival and reduce illness in this population," said senior author **C. Michael Cotten, MD**, a neonatologist and professor of pediatrics at Duke. "The results of this study are encouraging, but there's still a long way to go."



Welcome to the newest faculty members of the Department of Pediatrics!

Eliana M. Perrin, MD, MPH Primary Care

Charles Wood, MD, MSHP Primary Care

Charlene Wong, MD, MSHP Primary Care

For additional details about our new faculty, please visit: pediatrics.duke.edu

Duke Endowment grant will support community-based programs to reduce childhood obesity

The Duke Endowment has awarded a \$749,000 grant to Duke Health to support programs aimed at reducing obesity, improving health, and preventing chronic disease among vulnerable children in North Carolina.

The grant will fund a program that partners communities with health care providers in at least six communities across North Carolina, to increase the intensity and efficacy of pediatric obesity treatment. Low-income children and their families will receive clinical care and free access to supervised exercise classes, cooking classes, gardening classes, and parent support groups.

"The future health and well being of children is critically undermined by the epidemic of childhood obesity," says **Sarah Armstrong, MD**, associate professor of pediatrics and director of the Duke Healthy Lifestyles program. "Our aim is to disseminate the community partnership healthcare model across North Carolinia."



Gene therapy for Pompe disease effective in mice, poised for human trials

After decades investigating a rare, life-threatening condition that cripples the muscles, Duke Health researchers have developed a gene therapy they hope could enhance or even replace the only FDAapproved treatment currently available to patients.

The gene therapy, demonstrated in mice, is described in a new study published online in the journal *Molecular Therapy - Methods* & *Clinical Development*. The therapy uses a modified virus to deliver a gene to the liver where it produces GAA, an enzyme missing in people with Pompe disease.

Study authors have received approval from the FDA to launch a Phase 1 clinical trial in humans and are currently working to secure funding.

Pompe disease is an inherited condition that affects approximately 1 in 20,000 babies and can also appear in adulthood. People with the condition lack the enzyme GAA, which means their bodies can't metabolize the sugar, glycogen. As a result, glycogen builds up in the muscles. In babies, this leads to improper muscle development and, if undiagnosed and untreated, can lead to respiratory problems, heart failure and death.

"The outlook for Pompe disease is much improved since enzyme replacement has become available — it can reverse involvement of the heart and prolong survival," said senior author **Dwight Koeberl, MD**, **PhD**, professor of pediatrics and a medical genetics specialist at Duke.

"But not everyone responds to this treatment," Koeberl said.

"Many patients make some antibodies, and this can really interfere with treatment. Some infants still die from Pompe disease. Others have to add immune suppression to their treatment, which can lead to other complications. Gene therapy could help these patients."



The emerging gene therapy, which uses an inactivated form of adeno-associated virus (AAV), is

just the latest development for a team of scientists at Duke that has been working for three decades to study the causes and potential treatments for glycogen-storage diseases and specifically Pompe.

Medical students recognize Chung with Golden Apple Award

Aimee Chung, MD, assistant professor of pediatrics and medicine, received the 2017 Duke Medicine Golden Apple Award for Clinical Science Faculty.



The award, presented during the annual Student-Faculty Show, is the most prestigious teaching award presented by the Duke University School of Medicine student body to recognize physician-

teachers that the medical students feel have played an exceptionally effective and dedicated role in their education.

Awardees are selected first by open nomination, then by a vote of the student body. Three awards are given each year: one each to a member of the preclinical (basic science) faculty, clinical faculty, and clinical house staff.

This year, over 100 individuals were nominated for a Golden Apple, highlighting not only the highly selective nature of the award, but also the incredible dedication of each awardee to medical student education.

Chung completed her medical training at East Carolina University in 2005 and medicine-pediatrics residency at the Duke University School of Medicine in 2009.

Permar and Gbadegesin assume new leadership roles

Ann M. Reed, MD, chair of the Department of Pediatrics and physician-in-chief of Duke Children's, recently



announced that **Sallie Permar, MD, PhD,** has been named the new director of the Duke Pediatric Research Scholars Program for Physician-Scientist Development, and **Rasheed Gbadegesin, MBBS,** has been named the associate program director.

In their new positions, Permar and Gbadegesin will work with the vice chair of education and vice chair of research to recruit and retain top trainees, preferably with established track records of success in the clinical and basic research worlds, and support their dual interests during and after residency and fellowship training. Support will include keeping the residents' research interests viable during the challenges of clinical training, connecting with likeminded physician-scientists and mentors throughout the training processes, and streamlining the transition to fellowship training and junior faculty positions.

In addition, Permar has accepted the integral position of director of the Duke Children's Discovery Institute. In this position, she will lead the effort to discover and develop a research program specifically focused on the early influences of health and disease.

This initiative will comprise constituents throughout Duke University including the School of Medicine's Departments of Pediatrics, Medicine, Obstetrics and Gynecology, Psychiatry, Immunology,



Molecular Genetics and Microbiology, as well as the Center for the Genomics of Microbial Systems, Child Development Center, Law School, Duke Information Initiative and School of Engineering.

"This unique collaboration will ultimately inform pediatric prevention and cure strategies for childhood and chronic diseases, with the overarching goal to improve pediatric health and eliminate pediatric origins of chronic diseases," Permar said.

New Division Chiefs named for Hospital Medicine, Emergency Medicine

Beginning July 1, the Division of Hospital & Emergency Medicine will be divided into two divisions—the Division of Hospital Medicine and the Division of Emergency Medicine. Katy Bartlett, MD, has been named the new chief of the Division of Hospital Medicine, and Clay Bordley, MD, will continue as chief of the Division of Emergency Medicine.

Bartlett joined the Duke faculty in 2004 as a pediatric hospitalist and has served as an associate program director for the Duke Pediatric Residency Program since 2008. In this role, she has assisted with curriculum development, implemented milestone-based evaluations, recruited diverse classes of

residents and mentored numerous trainees. Bartlett has specific interests in teaching evidencebased medicine, quality improvement, and cultural competence.





members. He led the division's expansion of sedation services and the creation of the Children's Procedural Sedation Unit. He has also served as the medical director of the Pediatric Emergency Department and medical control for pediatrics for the Duke Transfer Center. Bordley's clinical and research interests are focused on the care of children with medical and surgical emergencies. His current work is focused on procedural sedation, bronchiolitis and the emergent care of children with fever and neutropenia.

Duke Children's establishes Jeffrey Modell Diagnostic and Research Center

The Jeffrey Modell Foundation (JMF), in partnership with CSL Behring, announced the establishment of the Jeffrey Modell Diagnostic and Research Center at a dedication ceremony at Duke Children's Hospital earlier this year. The Jeffrey Modell Foundation is a global non-profit

organization dedicated to primary immunodeficiency (PI) research, physician education, patient support, public awareness, advocacy and newborn screening. The center will serve the Triangle area and



North Carolina's Piedmont Region, offering advanced diagnostic evaluation to patients with a suspected PI, a group of disorders arising from defects in the immune system carried by the genes and characterized by infections that can be recurring, persistent, debilitating and chronic. John Sleasman, MD, chief of the Divisions of Allergy and Immunology and Pulmonary and Sleep Medicine in the Duke Department of Pediatrics, will serve as the center's inaugural director.

"While we have many goals for this new center, one of the most important goals is for the earliest and most precise diagnosis, followed by appropriate and meaningful treatments of primary immunodeficiency," said Vicki Modell.



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NOTEWORTHY

Patient Safety and Quality Conference sets record for crowd, abstracts

Enhancing workforce resiliency was front and center on March 23, as Duke Health hosted its 12th annual Patient Safety and Quality Conference. More than 600 people turned out, marking the third consecutive year of record attendance.

A record 152 abstracts were submitted this year, with more than 430 individuals contributing to the performance improvements described in the abstracts. The top 87 abstracts – also a record – were on display at the conference.

In addition to safety and quality in patient care, the conference also highlights Duke University Health System's (DUHS') devotion to its value of teamwork, with scores of individuals and groups working together to make the event happen.

Notably, members of the Department of Pediatrics team won two of the top awards at this year's conference, including the Rebecca Kirkland Award for the presentation entitled, "Achieving High Reliability Pediatric Medication Safety Through Multidisciplinary Adverse Drug Event Review" and the Learner Award for the presentation entitled, "Multimodal Intervention Improves Inpatient Discharge Process."

"These awards clearly demonstrate our overall multidisciplinary approach to patient safety and clinical quality," said Ann Reed, MD, chair of the Department of Pediatrics and physician-in-chief of Duke Children's. "Our abstract winners are highly deserving of this recognition." <image>

UPCOMING EVENTS

Duke Pediatrics Alumni Reception

Sunday, May 7, 2017 8:00 – 10:00 PM

Intercontinental San Francisco 888 Howard St., San Francisco, CA 94103

This reception is scheduled in conjunction with the Pediatric Academic Societies (PAS) annual meeting, May 6 – 9, 2017. We hope you'll join us.

RSVP to Diane Crayton at diane.crayton@duke.edu.

MAKE A GIFT

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.

Pediatrics Excellence Fund

The Pediatrics Excellence Fund provides support for the most immediate needs of the Department of Pediatrics.

You can make your gift quickly and securely online by visiting: SupportMedTraining.duke.edu.

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