A MESSAGE FROM THE LEAD PRINCIPAL INVESTIGATOR

The PTN Annual Review provides an opportunity to take a step back and appreciate the recent accomplishments of the network. Over the last year, the PTN has provided research to the U.S. Food and Drug Administration (FDA) that has resulted in label changes for lithium and acyclovir, bringing the total list of PTN-informed label changes to eleven. We have released 16 publications outlined below, met a number of key study milestones, and the work of PTN has been highlighted within the greater pediatric community.

We have made significant accomplishments this year; but our work is far from finished. We have much to do to determine the safest and most effective use of medications and devices in children. This Annual Review should serve as recognition and celebration of what we have accomplished, while providing motivation for our voyage ahead. Thank you for your continuing support and commitment to improving care for our youngest patients.

Danny Benjamin, MD, PhD, MPH

LITHIUM AND ACYCLOVIR LABEL CHANGES

Under the Best Pharmaceuticals for Children Act (BPCA), the PTN worked to provide the FDA with information to inform label changes for lithium and acyclovir over the last year. Both label changes provide doctors with the necessary information to prescribe the most appropriate doses of the medications to children.

LABEL CHANGES

As a result of research conducted through the Best Pharmaceuticals for Children Act (BPCA) program, led by the PTN, the following label changes have been made:

- **Acyclovir** for treatment of neonatal HSV infection/encephalitis (label changed for dosing and safety)
- **Ampicillin** for treatment of sepsis and/or meningitis (label changed for safety)
- **Lisinopril** for hypertension (label changed for treatment of pediatric renal transplant patients)
- **Lithium** for treatment of bipolar disorder (label changed for efficacy, safety, and dosing - BPCA legacy)
- **Lorazepam** for seizures (label changed for efficacy, safety, and dosing)
- **Mercy babyTAPE** device to assess weight without electricity in infants from birth to 90 days of age
- **2D and 3D Mercy TAPE** devices to assess weight without electricity in children 2 months through 16 years of age
- **Meropenem** for complicated abdominal infections (label changed for safety and dosing; efficacy extrapolated)
- **Propylthiouracil** for hyperthyroidism (label changed for safety and dosing)
- **Pralidoxime** (relabeled for use in organophosphate poisoning)
- **Sodium nitroprusside** for hypertension (label changed for efficacy, safety, and dosing)


PTN STUDY MILESTONES

The Long-term Antipsychotic Pediatric Safety (LAPS) Trial enrolls more than 100 patients
The enrollment of more than 100 children helps achieve nearly 30 percent of the overall enrollment goal of the LAPS Trial and is a key accomplishment for the two-year observational study. More

First participant enrolled in digoxin study
The digoxin study achieved a major study milestone by enrolling its first infant in August 2019. The study team will enroll up to 48 infants to determine the pharmacokinetics and safety of digoxin prescribed to infants with single ventricle congenital heart disease (CHD). More

Finalized first cohort of sildenafil study
The study will assess the safety of sildenafil in premature infants at risk of bronchopulmonary dysplasia (BPD) and determine preliminary effectiveness and pharmacokinetics. The study will enroll a total of up to 120 children and has rounded out the first cohort that includes 40 children. More

Database locked for SCAMP study
The study, which enrolled the first child in 2014, is designed to assess the safety, efficacy, and pharmacokinetics of three commonly prescribed antibiotic regimens for infants with intra-abdominal infections. The study database was locked in October 2018. More

PTN RESEARCH INFORMS RED BOOK UPDATE

PTN research on fluconazole dosing helped to inform a recent update to the AAP Red Book. The changes were based on a study conducted from 2008 to 2011 in collaboration with PTN that analyzed safety data on the use of fluconazole to treat infections caused by the Candida yeast. Red Book, the leading resource on pediatric infectious disease, provides the most up-to-date information on a wide variety of diseases that doctors see in children. More

THE AMERICAN ACADEMY OF PEDIATRICS (AAP) FEATURES PTN

The AAP featured the PTN’s work in an article written by Dr. Perdita Taylor-Zapata of the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) and Dr. P. Brian Smith of the PTN and the Duke Department of Pediatrics. More

DUKE STAR PROGRAM

The Summer Training in Academic Research (STAR) Program trained 25 participants during the 2019 summer academic break. The program is designed to provide a high-quality research experience for undergraduate students, high school students, and middle and high school teachers.

“The purpose of the program is to make sure we’re giving these students the opportunity to learn each day about various aspects of clinical research.”

“The purpose of the program is to make sure we’re giving these students the opportunity to learn each day about various aspects of clinical research,” said Dr. Kanecia Zimmerman, leader of the program and associate professor of pediatrics at Duke University Medical Center. “The students may have never thought seriously about a career in medical research, and they come away thinking, ‘This is actually something I can do.’”

Learn more about the 2019 STAR Program.

PTN SYMPOSIUM AT PAS MEETING

In April, PTN hosted a symposium at the annual Pediatric Academic Societies (PAS) meeting in Baltimore, Md. The symposium began with a brief overview of the PTN and the Best Pharmaceuticals for Children Act (BPCA). Dr. Matthew Laughon of the University of North Carolina at Chapel Hill and Dr. Kelly Wade of the Children’s Hospital of Philadelphia presented on real-world applications of PTN’s work and the impact of clinical research in practice, a panel of International Children’s Advisory Network (iCAN) participants discussed how researchers can better engage with participants and caregivers, and Dr. Janice Sullivan of the University of Louisville spotlighted partnering opportunities and work being done within and across research networks.