

## A Message from the Lead Principal Investigator



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PhD, MPH

Welcome to the *sixteenth* issue of the *PTN Post*, your quarterly source for information about the work of the Pediatric Trials Network (PTN).

As we near the end of the first quarter of 2016, PTN remains actively engaged with a steadfast review of ~60 medicines through an array of investigational studies. Of the many projects and initiatives, we chose a few updates to

report, including: the continuation of the Sildenafil Cohort 2 program, the new role of Child and Family Advocate, and PTN's presentations at the upcoming Pediatric Academic Society meeting in Baltimore.

With our incredible team of families, investigators, and staff, I am eager to meet the new challenges and celebrations ahead as we determine how to bring the best care to our most vulnerable population, our children. Thank you all for your continued commitment, for leading the way.

## Pharmacokinetics of Sildenafil in Premature Infants

By Maurine Morris and Tammy Day

Sildenafil is increasingly being used off-label in premature infants with little pharmacokinetic (PK) or efficacy data. The Cohort 2 portion of this Sildenafil study will examine premature infants born at <32 weeks gestational age at risk for developing bronchopulmonary dysplasia



(BPD). The first 7 participants received a single intravenous dose. Following review of the data and discussions with the Data Monitoring Committee and NICHD, a decision was made to enroll an additional 9 participants at a dose of 0.125 mg/kg.

“The results of this study,” states Co-Investigator, Matt Laughon, “will inform the dose for the next: a phase II dose-ranging study of sildenafil to prevent BPD. Currently, no drug has an FDA indication to prevent BPD, and the current trial is the first step in that direction.”

The Sildenafil Cohort 2 enrolled its first subject in September 2014—currently, 7 subjects are enrolled from four sites: Riley Hospital for Children at Indiana University: Shawn Ahlfield, MD, Greg Sokol, MD and Lucy Smiley; University of Alabama at Birmingham: Namasivayam Ambalavanan, MD, Tara McNair and Vivien Phillips; Duke University Medical Center: Michael Cotten, MD, Kimberley Fisher, PhD, Cindy Redd

and Kim Cicio; and University of Louisville School of Medicine and Kosair Children's Hospital: Dan Stewart, MD, Janice Sullivan, MD and Andrew Michael.

Coming soon – The PTN website, our hub for all things PTN is getting a well-deserved facelift. We plan to showcase our new and improved site at the upcoming Pediatric Academic Society conference in May.



We welcome your input about topics of interest for future issues. Please contact us with your suggestions via the [PTN website](#).



News Bite

Fifty-eight sites across US and Canada have participated in the [SCAMP](#) study. As of the end of February, 162 participants were enrolled. With great effort from all participating sites and study teams, we will reach our enrollment goal of 284 participants by end of 2016.

## PTN Presents at the 2016 Pediatric Academic Society (PAS) Meeting in Baltimore, April 30–May 3

### PLATFORM PRESENTATION:

**Sunday, May 1 at 4:15 PM**

*Dosing of antimicrobials in the neonatal in the NICU: Does clinical practice reflect published recommendations?* **M England**, RG Greenberg, RH Clark, M Laughon, M Cohen-Wolkowicz, DK Benjamin Jr, and PB Smith on behalf of the Administrative Core Committee of the Best Pharmaceuticals for Children Act – Pediatric Trials Network.

### POSTER PRESENTATION:

**Tuesday, May 3 at 7:30 AM**

*Using Population Pharmacokinetics and Electronic Health Records to Assess Piperacillin Safety in Infants.* **S Salerno**, C Hornik, M Cohen-Wolkowicz, PB Smith, R Clark, and D Gonzalez on behalf of the Administrative Core Committee of the Best Pharmaceuticals for Children Act – Pediatric Trials Network.

### PLATFORM PRESENTATION:

**Saturday, April 30, 2016 at 8:00 AM**

*Use of Pediatric and Adult Midazolam Population Pharmacokinetics to Assess IM Dosing and Early Drug Exposure for Status Epilepticus.* **E Capparelli**, K Chiswell, PB Smith, D Siegel, S Weinstein, S Muchohi, M Reed, J Barrett, S de Wildt, E Jaqc-Aigrain, J Ma, T Glauser for the Pediatric Trials Network.

## PTN's Child and Family Advocate

In many medical arenas and in a variety of roles, patient advocates offer a unique perspective on clinical research from the parent, the family, and even the child's perspective. Many clinical trials are starting to include a patient advocate on their trial teams to ensure trial design is feasible and acceptable. This role is particularly important in pediatric research and therefore, we added a Child and Family Advocate to the network.

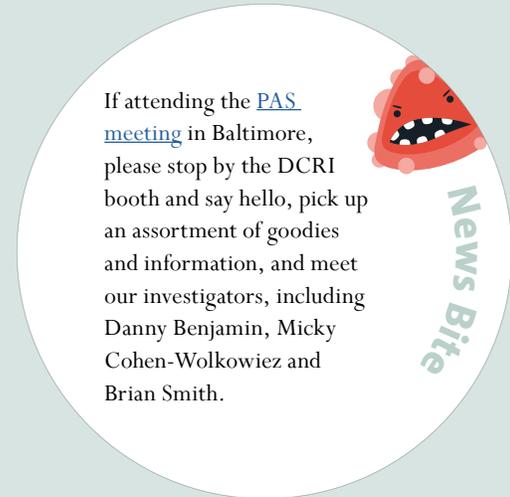
For PTN, the advocate will review trial documents, such as protocols, informed consent forms, patient recruitment material, and presentations prior to implementing clinical trials or enrolling children. We will address the advocate's concerns prior to implementing new studies.

PTN's Senior Project Leader, Katherine Berezny further explains: "Since children see adults as authoritative figures, and are sometimes unable or unwilling to voice their opinion, this position is critical. It's good for researches to have a reality check on visit schedules, procedures, and risks before approaching parents and their children."



News Bite

The [Methadone](#) trial completed enrollment, and the [Furosemide](#) trial has activated 9 sites, and enrolled the first patient on November 27, 2015. To find out more about these trials, visit the [PTN Website](#).



News Bite

If attending the [PAS meeting](#) in Baltimore, please stop by the DCRI booth and say hello, pick up an assortment of goodies and information, and meet our investigators, including Danny Benjamin, Micky Cohen-Wolkowicz and Brian Smith.

The Pediatric Trials Network (PTN) is made possible by the Best Pharmaceuticals for Children Act (BPCA). The BPCA, first enacted in 2002, provides mechanisms for studying on- and off-patent drugs in children. Visit us on the web at [www.pediatrictrials.org](http://www.pediatrictrials.org).

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