A Message from the Lead Principal Investigator

In the last issue of the *PTN Post*, we noted that the completion of the meropenem study, and the resulting label change allowing treatment of abdominal infections in infants under 3 months of age, laid the ground work for the Pediatric Trials Network. The label change is an important milestone and underscores the continuing significance of the work we do. With that in mind, I’d like to review the progress we have made to date in other therapeutic areas.

In addition to meropenem, two other advances were made through work under the Best Pharmaceuticals for Children Act. The use of sodium nitroprusside was approved in 2014 for blood pressure management in children, with label changes for efficacy, safety, and dosing. More recently, the TAPE study resulted in FDA marketing clearance for a new device, the first of its class, to more accurately assess weight to determine pediatric dosing.

The submission of the clinical study report, sometimes referred to as CSR, is an important event for each molecule studied by the PTN. When we submit the CSR under our current relationship with NIH and FDA, it’s a signal that we believe that sufficient data have been collected to secure a labeling change. In effect, we are asking the FDA if they agree that a labeling change is warranted, and if not, what the next steps are for the PTN.

As of June 1st of this year, CSRs for BPCA legacy (prior to PTN) and PTN-led studies submitted to the FDA include the following:

- **Lorazepam** for seizure control to determine efficacy, safety, and dosing (BPCA legacy study and PTN)
- **Isotretinoin** for treatment of neuroblastoma to determine efficacy, safety, and dosing (BPCA legacy)
- **Hydroxyurea** for treatment of sickle cell disease to determine PK and bioavailability of a liquid formulation (this is a PTN partnership submission with NHLBI)
- **Ampicillin** for treatment of complicated infections in pre-term neonates to determine safety and dosing (PTN)
- **Fluconazole** for prevention of candidiasis in neonates and infants to determine efficacy, safety, and dosing, prevention of candida infections for infants and children on ECMO, and treatment of invasive candida for infants <12 months of age (PTN)
- **Lisinopril** for treatment of hypertension in pediatric renal transplant patients to determine PK and safety (PTN)
- **Clindamycin** for treatment of complicated infections to determine PK and safety for obese children (PTN)

With an eye toward the future, we anticipate submitting data for the following trials in 2016.

- **Diazepam** for seizure control to determine improved dosing (BPCA legacy and PTN)
- **Lorazepam** for sedation to determine efficacy, safety, and dosing (PTN legacy and PTN)
- **Clindamycin** for treatment of soft tissue infection to determine efficacy, safety, and dosing (PTN in partnership with NIAID)
- **Sulfamethoxazole and trimethoprim** for treatment of soft tissue infection to determine efficacy, safety, and dosing (PTN in partnership with NIAID)
- **Caffeine citrate** for treatment of apnea of prematurity to determine efficacy, safety, and dosing (PTN in partnership with NHLBI)
- **Methadone** for treatment of pain and opiate withdrawal to determine safety and dosing (PTN)
- **Rifaximin** for treatment of complicated infections in neonates to determine safety and dosing (PTN)
- **Clindamycin** for treatment of complicated infections in neonates to determine safety and dosing (PTN)

(continued on next page)
A Message from the Lead Principal Investigator (continued)

Thanks to the hard work and dedication of PTN investigators and sites, the list of accomplishments is long, but there is much more work to be done to determine the safest and most effective use of medications and devices in the pediatric population. This issue of the PTN Post celebrates the progress we’ve made and the work that lies ahead. You will also read about opportunities to learn more through upcoming meetings and contributions to the literature. As always, we welcome your input about topics of interest for future issues. Please contact us with your suggestions via the PTN website.

Since October of 2014, PTN faculty members have published ten manuscripts in professional journals. Refer to the list below for more information about these PTN-led studies.


For a full listing of PTN publications, click here.