BPCA Funded Clinical Trials

Drug Name	NIH BPCA Priority Area	Study Description	Study Population	Study Duration	Labeling Status
Nitroprusside (SNP1)	Intensive Care	Pharmacokinetic (PK) and Pharmacodynamic (PD) of sodium nitroprusside in pediatric subjects.	<17 years	Aug 2005 - Jan 2008	Multiple labeling changes for the pediatric use of nitroprusside in December 2013. FDA Docket number: FDA-2010-N-0284
Nitroprusside (SNP2)	Intensive Care	PK, PD, safety and efficacy study of the hypotensive effect of nitroprusside in children requiring blood pressure reduction to reduce blood loss during surgery.	<17 years	Oct 2008 - Nov 2010	Multiple labeling changes for the pediatric use of nitroprusside in December 2013. FDA Docket number: FDA-2010-N-0284
Meropenem	Neonatal Infections	Safety, efficacy, PK study of meropenem in neonates with abdominal infections.	Birth - 28 days	June 2008 - Oct 2009	A label change for meropenem for intra-abdominal infections in neonates in December 2014. FDA Docket number: FDA-2011-N-0918
Lorazepam (Status 1)	Seizures	PK of Intravenous (IV) lorazepam in children treated for status epilepticus.	3 months to <18 years	Apr 2005 - Oct 2006	Label change after second study (Status 2 study).
Lorazepam (Status 2)	Seizures	Safety, efficacy, pharmacokinetics/ pharmacodynamics (PK/PD) study in children in the emergency setting (under an Exception from Informed Consent) comparing lorazepam and diazepam.	3 months to <18 years	Mar 2008 - Mar 2012	The results of these trials led to a label change for lorazepam in June 2016. FDA Docket number: FDA-2015-N-3037
Diazepam	Seizures	Analysis of PK, safety, and efficacy of diazepam using data from the Status 2 study.	3 months to <18 years	Mar 2008 - Mar 2012	Final CSR submitted to FDA in March 2017. FDA review pending for possible label change for diazepam.
Ampicillin	Neonatal Infections	PK study and retrospective safety analysis of ampicillin prescribed to infants per standard of care.	Postnatal age <30 days	Feb 2012 - Aug 2012 for PK data	Final CSR submitted to FDA in December 2014. Label change under review.

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Ampicillin	Neonatal Infections	Retrospective safety and PK study of dosing in neonates to improve label information.	Age range: <36 weeks GA	Sept 2011 - June 2013	Final CSR submitted to FDA in December 2014.
Fluconazole	Neonatal Infections	Randomized, double-blind, PK and safety prophylaxis treatment in preterm infants.	Age range: 18 - 22 months	Sept 2012 - Sept 2015	Final CSR submitted to FDA in July 2015. FDA review pending.
Clindamycin (Obesity)	MRSA Infections	Open-label, PK and safety study in obese children to determine dosing.	Age range: 2 - <18 years, obese	Sept 2012 - Sept 2016	Final CSR submitted to FDA in October 2015. Label change under review.
Acyclovir (ACY01)	Neonatal Infections	Open-label, PK study of acyclovir in preterm and term infants with suspected herpes simplex virus (HSV).	<45 days postnatal age and <34 weeks gestational age at time of initial study drug administration	Sep 2011 - June 2012	Final CSR submitted Apr 2017. Label change under review.
Acyclovir (ACY02)	Neonatal Infections	Retrospective safety and dosing analysis via chart review to evaluate the safety and efficacy of IV acyclovir in infants with herpes simplex virus (HSV) or suspected HSV.	<45 days postnatal age and <34 weeks gestational age at time of initial study drug administration	NA	Final CSR submitted Apr 2017. Label change under review.
Lithium (COLT1)	Pediatric Bipolar Disease	PK, safety, and efficacy study of lithium in children with bipolar illness.	7 years - 17 years and 11 months	Dec 2006 - Apr 2009	Final CSR submitted Nov 2015. FDA review pending.
Lithium (COLT2)	Pediatric Bipolar Disease	PK, safety, and efficacy study of lithium in children with bipolar illness.	7 years - 17 years and 11 months	June 2010 - April 2013	Final CSR submitted Dec 2015. FDA review pending.
Lisinopril	Pediatric Hypertension	Open-label, PK and safety study in children with kidney transplant.	2 - <18 years	June 2012 - Oct 2013	Final CSR submitted to FDA in December 2014. Label change based on study data in April 2016. No docket number
Mercy TAPE (device)	Pediatric Devices	Observational, 2-D and 3-D Mercy TAPE weight estimation device for children	2 months -16 years	Feb 2012 - Apr 2012	Final CSR (510k premarket notification) submitted to FDA in April 2015. Device approval obtained May 2015.
Hydroxyurea (BABY HUG)	Sickle Cell Anemia	National Heart, Lung, and Blood Institute (NHLBI) study of efficacy, safety, PK of hydroxyurea in young children with sickle cell disease.	9 months - <5 years	Oct 2003 - Sept 2009	Study unblinded January 2010 and Draft CSR submitted to FDA in May 2015. Final data submission to FDA pending. Scheduled for January 2018.
Hydroxyurea (HUB01)	Sickle Cell Anemia	Open-label, PK/bioequivalence study of liquid hydroxyurea formulation in children with sickle cell anemia.	≥2 years - ≤17 years	Jan 2012 - Nov 2013	Final CSR for PK study submitted to FDA in February 2014. Awaiting final submission of efficacy study (NHLBI Baby HUG study) for final disposition.

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Lorazepam for Sedation	Intensive Care	Safety, efficacy, pharmacokinetics/ pharmacodynamics (PK/PD) study in children on mechanical ventilation in the Intensive Care Unit (ICU) comparing lorazepam and midazolam.	<18 years	Oct 2004 - Sep 2007	CSR submitted Dec. 2011. Additional information provided March 2016. FDA review pending.
Baclofen	Cerebral Palsy	Safety, PK/PD study of oral baclofen to reduce spasticity in children with cerebral palsy	≥2 years - ≤16 years	Nov 2008 - Jan 2011	Study results submitted to the FDA in 2014. No label change anticipated at this time.
Pralidoxime	Biodefense Research	Published 2010-09-09 The U.S. Food and Drug Administration has approved the pediatric use of Protopam Chloride (pralidoxime chloride), a drug used to treat poisoning by organophosphate pesticides and chemicals (e.g., nerve agents). Protopam Chloride was approved by the FDA in 1964 to treat various types of pesticide and chemical poisoning in adults. The drug works as an antidote to pesticides and chemicals of the organophosphate call by slowing the attachment of the chemical to nerve endings.	N/A	N/A	FDA approves pediatric use of chemical poisoning treatment.
Doxycyline		PK of doxycycline administered to children per standard of care	≥1 month postnatal age - ≤17 years OR ≥5 months postnatal age if not born prematurely (i.e., ≤37 weeks gestational age); OR >17 years - <21 years if obese or receiving extracorporeal membrane oxygenation	Mar 2012 - Feb 2016	Final CSR submitted Dec 2017. Pending FDA review.

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TMP-SMX		PK of trimethoprim-sulfamethoxazole administered to children per standard of care.	<21 years of age with the exception of children <5 months postnatal age who were born prematurely (i.e., ≤37 weeks gestational age)	Nov 2011 - May 2015	Final CSR submitted Sept 2017. Pending FDA review.
Ondansetron		PK of ondansetron administered to children per standard of care.	<21 years of age	Dec 2012 - Jul 2015	Draft CSR submitted May 2017. Final CSR under development for submission to FDA.
Rifampin		PK of rifampin in infants.	infants ≤120 days gestational age	Jun 2013 - May 2015	Full CSR submitted April 2017. After FDA comments, CSR being revised. FDA submission scheduled for January 2018.
Methadone		PK, safety and genetics in children.	≥90 days to <18 years	Jan 2014 - Jan 2015	Draft CSR submitted Mar 2016. Final CSR under development to be submitted to FDA
Diuretics		Retrospective observational study analyzing PK, safety, and efficacy in extremely low birth weight infants exposed to furosemide or bumetanide.	<32 weeks gestational age	Enrollment N/A (retrospective chart review of NICU admissions from Jan 2000 to Dec 2012)	Data report created to support development of prospective furosemide study. Data report was submitted to FDA Apr 2015. Follow on study under way (SEE Furosemide).
Caffeine		Retrospective safety and dosing analysis in neonates with apnea.	<28 weeks gestational age at birth and <120 days postnatal age at earliest dose of caffeine	Enrollment N/A (retrospective chart review)	Draft CSR submitted Feb 2017. Final CSR under development and scheduled for submission to FDA February 2018.
Metronidazole		Safety and PK of multiple-dose metronidazole in premature infants.	< 32 weeks gestational age with suspected serious infection	Jan 2011 - Nov 2011	Final CSR submitted October 2012. FDA review lead to ABS01 study.
Antibiotic Safety (ABS01)		Randomized safety study evaluating ampicillin, clindamycin, metronidazole, and piperacillin-tazobactam in neonates.	Postnatal age <121 days	May 2014 - March 2017	Draft CSR for clindamycin under development.
Personal Protective Equipment (PPE)		Randomized, cross-over, simulation-based study assessing the impact of PPE on pediatric procedures.	Registered EMS providers, registered nurses, licensed physicians	Jun 2016 - Sept 2017	N/A

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Sildenafil (SIL01)		Open-label PK and safety in preterm infants with pulmonary hypertension.	≤28 weeks gestational age for cohort 1 and <32 weeks gestational age for cohort 2	Feb 2013 - Feb 2015	Study results used in protocol development for second sildenafil study (SIL02). Final Study Report drafted for trial master files, but not submitted to FDA.
Pantoprazole		Effect of obesity on the PK of pantoprazole in children and adolescents.	Age range: 6-17 years, obesity + GERD	Jul 2014 - Sept 2015	FDA determined that no label change was needed.
Current Studies					
Sildenafil (SIL02)		Randomized, placebo-controlled, sequential dose escalating, double masked safety of sildenafil in premature infants at risk of bronchopulmonary dysplasia.	<29 weeks gestational age at birth and 7-28 days postnatal age at time of randomization	Pending first enrollment	[Not applicable until after study completion.]
Furosemide		Safety of furosemide in premature infants at risk of bronchopulmonary dysplasia.	<29 weeks gestational age at birth and 7-28 days postnatal age at time of randomization	First enrollment Nov 2015	[Not applicable until after study completion.]
Antibiotic Safety 2 (ABS02)		Observational study: long-term outcomes of premature infants enrolled in the ABS01 study.	Enrolled in ABS01 study	First enrollment Nov 2017	[Not applicable until after study completion.]
Anti-epileptics (AED01)		PK of anti-epileptics in obese children.	2 years - <18 years	First enrollment Jan 2017	[Not applicable until after study completion.]
Timolol		PK, safety and efficacy in infants with infantile hemangioma.	0-84 days postnatal age	First enrollment Jul 2017	[Not applicable until after study completion.]

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Current Studies - Opportunistic Study					
BPCA Priority List of Drugs Commonly Used in Children Alfentanil Amikacin Aripiprazole Atropine IV Cefepime Ceftazidime Cidofovir ciprofloxacin clozapine Dexamethasone Diazepam etomidate Fosphenytoin haloperidol Heparin (LMW) Hydromorphone Lidocaine IV lurasidone Meropenem methylprednisolone Midazolam molindone Nafcillin (IV only) nicardipine olanzapine Oxcarbazepine pentobarbital Piperacillin-Tazobactam quetiapine Risperadone Rocuronium Timolol tobramycin Valproic Acid Vancomycin Vecuronium warfarin (oral) ziprasidone	All	Open-label, opportunistic, PK study of understudied drugs in children given as part of standard of care More than 2,650 children enrolled Approximately 40 clinical sites including U.S., Singapore, Israel, U.K., and Canada	Age range: Birth - < 21 years	Aug 2011 - ongoing	Pending submission of clinical study reports for individual drugs.