Best Pharmaceuticals for Children Act (BPCA) Priority List of Needs in Pediatric Therapeutics

The National Institutes of Health (NIH) hereby announces the BPCA Priority List of Needs in Pediatric Therapeutics for 2018-2019.

Update on BPCA Prioritization

The BPCA requires that the NIH, in consultation with the Food and Drug Administration (FDA) and experts in pediatric research, develop and publish a priority list of needs in pediatric therapeutics. Part of fulfilling the NIH's authority and responsibility outlined in the BPCA legislation is to establish a program for pediatric drug development of off-patent medications and to publish a list of needs in pediatric therapeutics. The BPCA Priority List consists of key therapeutic needs in the medical treatment of children and adolescents; it is organized by therapeutic area, which can be a group of conditions, or a setting of care, or a subgroup of the population.

Below is an update of the priority list developments to date:

- Annually, the *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD) reaches out to experts in pediatrics to identify needs in pediatric therapeutics.
- All nominations received by the NICHD are reviewed, considered, and evaluated according to six key criteria:
  - Relevance to BPCA mission and goals
  - No disqualifying ethical concerns
  - Level of evidence available and current gaps
  - Potential impact on children, society, and delivery of care
  - Consideration of the different populations that may benefit from the research
  - Feasibility and availability of the resources needed to conduct the study.
- Minutes of all previous BPCA annual stakeholder and therapeutic area working group meetings, as well as other collaborations can be found on the BPCA Web site. https://bpca.nichd.nih.gov/prioritization/working_groups/Pages/annual-prioritization.aspx
- Information on the current BPCA clinical trials can be found on the PTN website. https://www.pediatrictrials.org/ptn-studies

Below is an updated list **(in bold)** of therapeutic areas and drugs that have been added to the existing BPCA list and prioritized for study for 2018-2019. The list also includes prioritized areas and drugs since the inception of the BPCA. A summary of the NICHD's plans and progress in all of areas prioritized to date is also provided.

Priority List of Needs in Pediatric Therapeutics 2018-2019

In accordance with the BPCA legislation, the following list outlines priority needs in pediatric therapeutics for the therapeutic areas listed below.

Table 1: Infectious Disease Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Skin and Soft Tissue Infections	Clindamycin	Optimal therapy and management of community-acquired skin and soft tissue infections	Pharmacokinetics (PK), safety, and efficacy clinical studies, particularly in overweight and obese children	Multiple pediatric studies by the Pediatric Trials Network (PTN) ***** Clinical study report (CSR) submitted to FDA for clindamycin use in obese patients in October 2015. FDA docket submission in November 2016. Additional data submitted November 2017. **Awaiting final disposition on potential label change**
	Trimethoprim-sulfamethoxazole	Biomarkers of disease	PK and efficacy (comparison) studies in normal weight and obese children	Pediatric studies performed by the PTN. CSR submission to the FDA June 2017. Additional data submitted December 2017. **Awaiting final disposition on potential label change**
Pediatric Infections	Doxycycline	PK, safety in children younger than 8 years	PK, safety, and efficacy clinical studies in normal weight and obese children	Pediatric opportunistic study performed by the PTN CSR submission to the FDA January 2017**. Awaiting final disposition on potential label change**
	Isavuconazole	PK, safety and efficacy data in immunocompromised children to treat serious fungal infections	PK, safety and pharmacodynamic (PD) clinical studies	Under consideration
	Daptomycin	PK and safety studies in young children for invasive gram- positive infections	Dosing and safety studies in children < 2 years	Under consideration
Tinea capitis	Griseofulvin	Safety and efficacy of higher doses in children < 20 kg with tinea capitis	PK, efficacy, and safety of higher doses in young children	Written Request (WR) received from the FDA Pediatric opportunistic study by the PTN completed. Data analysis underway

continued

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Infections in neonates	Metronidazole	PK and efficacy in neonates with abdominal infections	PK study	Pediatric PK study completed by the PTN; CSR submitted to the FDA in October 2012. Follow-up study completed. Submission to FDA anticipated for Spring 2019
	Acyclovir	Dosing, efficacy, and safety in neonates and infants with herpetic infections	PK, safety, and efficacy clinical studies in neonates and children	Pediatric PK and safety study performed by the PTN CSR submitted to the FDA for review in August 2016. Additional data submitted April 2017. **Study data submitted to NICHD Data and Specimen Hub (DASH) April 2018** **https://dash.nichd.nih.gov/Study/15961** **LABEL CHANGE in effect as of February 2019**
	Ampicillin	PK and safety in very low birth weight neonates	PK, safety clinical studies in neonates	WR received from the FDA CSR submitted to the FDA in December 2014. Addition data submitted April and October 2016**. Awaiting final disposition on potential label change**
	Fluconazole	Dosing and safety in very low birth weight neonates	PK, safety clinical studies in neonates	Pediatric PK and safety study completed by the PTN CSR submitted to the FDA in January 2015. **Awaiting final disposition on potential label change**
	Valganciclovir	PK and safety in infants exposed to CMV	Optimal PK-PD endpoints to assess efficacy and safety	Under consideration
	#Clindamycin Rifampin #Metronidazole Piperacillin-Tazobactam	PK and safety of antibiotics used in pre-term and term neonates to treat various infections		Pediatric PK and safety studies underway by PTN
Influenza	Oseltamivir	Pharmaco-epidemiology data	Impact on clinical outcomes in hospitalized children with influenza	NICHD grant funded and completed https://bpca.nichd.nih.gov/prioritization/clinicaltrials/Pages/pediatric-trials-network.aspx Pediatric opportunistic study underway by the PTN

Drug and indications **in bold** have been identified by the NICHD as a new priority and have been added to the BPCA list.
*Please refer to the PTN website for more details (www.pediatrictrials.org)

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Table 2: Cardiovascular Disease Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Pediatric Hypertension	#Hydrochlorothiazide	PK, safety, and efficacy in obese adolescents	Comparison studies, PK studies	Pediatric opportunistic study by the PTN ongoing
	Lisinopril	PK in children with kidney transplant	PK, safety, and efficacy clinical studies; formulations	Pediatric PK and safety study in renal transplant patients completed by the PTN Clinical study Report (CSR) submitted to the FDA December 2014 **LABEL CHANGE in effect as of April 2016.**
	No specific drug	Treatment options and biomarkers of end organ damage Novel devices and methods to monitor blood pressure in ambulatory setting in children	Biomarkers of disease progression and Long-term follow up studies	Co-funding with the Health Resources and Services Administration (HRSA) grant # UA6MC15585 to determine frequency of medication use via electronic health records (EHR) with the Pediatric Research in the Office Setting (PROS) Network https://www.ncbi.nlm.nih.gov/pubmed/27940711 **Workshop September 2017. Collaboration between NICHD, FDA, NHLBI. Meeting minutes on BPCA website: https://bpca.nichd.nih.gov/findingsandresources/Pages/other-collaborations.aspx**
	Sodium nitroprusside	PK, safety, and efficacy	PK, short- and long-term safety and efficacy trials for controlled hypotension	WR received from the FDA; CSR to the FDA August 2012 Redacted data submitted to the FDA docket in April and September 2012 **LABEL CHANGE in effect as of December 2013**
	Hydralazine (Intravenous)	Limited therapeutic options in ICU settings	Dosing and safety studies of intermittent use in children	Under consideration

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Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Hypotension	Dopamine	Outcome measures in neonates and children treated for hypotension	Defining outcome measures	Collaboration with existing NICHD network (Neonatal Research Network) Clinical Trial #NCT00874393 Publication: Pediatrics 2013 Jun 6;131(6):e1865-73. Epub 2013 May 6
	Epinephrine	Dosage in resuscitation in children with elevated body mass index	PK studies	Pediatric opportunistic study by the PTN ongoing
Dyslipidemia	Statins	Risk/benefit profile of long-term use in children	Novel study designs, use of surrogate markers for determining the value of long-term statin use in children	Pediatric opportunistic study by the PTN completed; Submission to FDA completed. No label change anticipated
Heart failure	Digoxin	PK and safety in infants	PK and safety in infants with single ventricle congenital heart disease	Protocol development in progress

Drug and indications in bold have been identified by the NICHD as a priority and are newly added to the BPCA list.
#Drugs listed twice for different indications or populations.

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Table 3: Respiratory Disease Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Asthma	Asthma therapeutics in young children	Objective measures of lung function and responses to therapy in children younger than 4 years	Identification of barriers to implementation of guidelines for asthma treatment Standardization of outcome measures in research **Identifying pharmacodynamics markers of treatment effects**	Trans-NIH and trans-U.S. Department of Health and Human Services (HHS) collaborations Asthma Outcome Measures meeting March 2010; published in the Supplement to *The Journal of Allergy and Clinical Immunology,* Volume 129, No. 3. March 2012 Co-funding with the HRSA grant # UA6MC15585 to determine frequency of medication use via EHR with the PROS Network **NICHD sponsoring biomarkers working group**
	Albuterol	Dose response, safety, and efficacy	Safety, efficacy, and appropriate mode of delivery in children in acute care settings	NICHD Collaborative Pediatric Critical Care Network data collection completed. https://www.ncbi.nlm.nih.gov/pubmed/22494876
	Terbutaline	Dose response, safety and efficacy	Alternative treatment options for severe asthma treatment	Concept development in progress
Pulmonary hypertension	Sildenafil	Treatment strategies and outcome measures in children with pulmonary hypertension of differing etiologies	PK and pharmacodynamics studies in neonates receiving the drug Epidemiology of differing etiologies and age appropriate outcome measures in children	Pediatric observational and PK study by the PTN ongoing Novel formulations, pre-clinical data, and clinical PK and safety protocol underway by PTN

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Table 4: Intensive Care Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Anesthesia/sedation	#Ketamine	Safety	PK, PD and safety studies of short- and long-term effects	Preclinical studies completed with the FDA/ National Center for Toxicological Research (NCTR) via Inter-Agency Agreement https://bpca.nichd.nih.gov/prioritization/clinicaltrials/Pages/pediatric-trials-network.aspx **Pediatric PK, PD and safety study underway by the PTN**
	Inhaled anesthetics	Toxicity of inhaled anesthetics in developing brains	Identification of markers of apoptosis	Preclinical studies completed with the FDA/ National Center for Toxicological Research (NCTR) via Inter-Agency Agreement https://bpca.nichd.nih.gov/prioritization/clinicaltrials/Pages/pediatric-trials-network.aspx Publication: Journal of Applied Toxicology 09/2013; 33(9). DOI:10.1002/jat.2857
	#Lorazepam	Dosing, safety	PK, safety, and efficacy trial comparing lorazepam with midazolam for sedation	WR received from the FDA Clinical trial completed; No label change anticipated.
	Dexmedetomidine	**Adjunctive use in pediatric anesthesia**	**Long-term follow up**	**Pediatric opportunistic study by the PTN ongoing**
Shock	Hydrocortisone	Dosing, duration of treatment, weaning process	PK and comparative effectiveness studies	Collaboration with the NICHD Collaborative Pediatric Critical Care Network in development
Analgesia	Hydromorphone	Pk and safety of pain medications in children	Pk, PD, and safety of analgesics in obese and non-obese children	Pediatric PK, PD and safety study underway by the PTN

Drug and indications **in bold** have been identified by the NICHD as a priority and are newly added to the BPCA list.
#Drugs listed twice for different indications or populations.

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Table 5: Biodefense Research Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Nerve agent exposure	Drug delivery systems	Need for pediatric auto-injectors	Availability and validation	Trans-HHS collaborations completed
	Midazolam	Dosing studies for treatment of seizures and in obese children	PK studies	PTN PK study completed. Manuscript in development Collaboration with NIH CounterACT Program ongoing
Cyanide toxicity	Hydroxycobalamin	Dosing and effectiveness in inhalation injuries suffered during fires	Safety and efficacy	Pediatric opportunistic study by the PTN ongoing; real-time cyanide assay developed in collaboration with NINDS https://www.ncbi.nlm.nih.gov/pubmed/23653045
Organophosphate poisoning	Pralidoxime	Dosing and safety		LABEL CHANGED as of September 2010

Drug and indications **in bold** have been identified by the NICHD as a priority and are newly added to the BPCA list.

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Table 6: Pediatric Cancer Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Neuroblastoma	13-cis-retinoic acid	New indication for neuroblastoma, pediatric formulation	PK studies, new formulation	Proposed Pediatric Study Request negotiated with the FDA; WR issued and declined by manufacturer and received from the FDA Collaboration with National Cancer Institute (NCI)/Children's Oncology Group (COG) Study complete, findings under review, and analyses ongoing. Submission to FDA pending.
Leukemias and solid tumors	#Methotrexate	Safety studies	Neurocognitive outcomes in young children with high-risk acute lymphoblastic leukemia	WR received from the FDA Collaborations with NCI/COG; data analysis ongoing
	Vincristine	PK and safety studies	PK modeling and safety studies to evaluate for neurotoxicity	WR received from the FDA Collaborations with NCI/COG; clinical trial completed; data analysis ongoing
	Daunomycin	PK studies	PK studies in children with elevated body mass index	WR received from the FDA Collaborations with NCI/COG; study completed; CSR under development
	Actinomycin-D	PK and safety studies	PK modeling and simulation, data mining for safety (hepatotoxicity)	WR received from the FDA Collaborations with NCI/COG; clinical trial completed; data analysis ongoing

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Table 7: Psychiatric Disorder Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Attention deficit and hyperactivity disorder (ADHD)	Methylphenidate	Safety and toxicity		Preclinical and clinical studies with NCTR and the National Institute of Environmental Health Sciences Publication: PLoS ONE 09/2014; 9(9):e106101. DOI:10.1371/journal.pone.0106101
Bipolar disease	Lithium	PK, safety, and efficacy	Dosing and tolerance, short- and long-term safety	WR received from the FDA PK data submitted to the FDA January 2010; safety and efficacy clinical trial completed April 2013 CSR submitted to FDA in winter 2015. Additional data submitted in 2017. **LABELCHANGED as of OCTOBER 2018**
Psychosis, aggression	Atypical antipsychotics: Risperidone Aripiprazole	Long-term safety—metabolic derangements and weight gain Pharmacoepidemiology studies	Comparative long-term safety, epidemiology research on frequency of use	Translational research; co-funding with HRSA grant # UA6MC15585 to determine frequency of use via electronic health records (EHR) with the (PROS) Network Publication in Pediatrics scheduled for 6/22/2015 PTN opportunistic study complete **PTN long-term safety study of select antipsychotics in launched in Fall 2018**

Drug and indications **in bold** have been identified by the NICHD as a priority and are newly added to the BPCA list.

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Table 8: Neurological Disease Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Cerebral palsy	Baclofen (oral)	PK, safety, and efficacy	PK and efficacy, pediatric formulation	WR received from the FDA Clinical trial completed; CSR submitted to the FDA December 2013. NO label change anticipated.
Migraines	No specific drug	Efficacy in prophylaxis	Efficacy in migraine prevention	NICHD co-fund of migraine clinical trial with NINDS grant number U01NS-076788 completed 2015
	Amitriptyline	Efficacy in prophylaxis	Efficacy in migraine prevention	Under consideration
Seizures	#Lorazepam	PK, safety, and efficacy	PK, safety, and efficacy in treating status epilepticus	WR received from the FDA PK trial data submitted to the FDA February 2009; safety and efficacy clinical trial completed CSR submitted to the FDA in October 2014 and May 2015. **LABEL CHANGED as of June 2016**
	Fosphenytoin	PK, safety	PK, safety in treating seizures in young children	Under consideration
	Diazepam	PK, safety, and efficacy	PK, safety in treating seizures in children of all ages	CSR submitted to FDA in January 2017 for potential label change. **Awaiting final disposition for potential label change.**
	Levetiracetam Valproic Acid Topiramate Oxcarbazepine	Dosing and safety studies in younger children	Establishing body weight-clearance relationship in children	Pediatric dosing studies of the steady state PK of anti-epileptics in obese children obese children are ongoing by PTN

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#Drugs listed twice for different indications or populations.

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Table 9: Neonatal Research Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Neonatal bronchopulmonary dysplasia (BPD)/ lung development	Betamethasone	Dosing, efficacy	Determination of dosing and effectiveness	Review of existing data completed. No current pathway for label change.
	Azithromycin (IV)	Dosing, efficacy	PK, efficacy in treating ureaplasma infections to prevent BPD	WR received from the FDA; NICHD grant # HD056424 funding complete; HD067126 ongoing https://bpca.nichd.nih.gov/prioritization/clinicaltrials/Pages/pediatric-trials-network.aspx
	#Hydrochlorothiazide	Dosing, safety, and efficacy	Determination of dosing and effectiveness	Pediatric opportunistic study by the PTN underway Collaboration with the NHLBI Prematurity and Respiratory Outcomes Program (PROP) network NCT01435187. Study completed. Additional data analyses underway.
	Furosemide	Dosing and safety	Determination of dosing and safety in preterm neonates	Opportunistic PTN study and retrospective analysis of diuretics in children complete **PTN PK and safety study underway**
Neonatal pain	Morphine	Dosing	Optimization of dosing and biomarkers of pain in neonates	WR received from the FDA Current NICHD grant #HD048689 funded and completed https://bpca.nichd.nih.gov/prioritization/clinicaltrials/Pages/pediatric-trials-network.aspx
	#Hydromorphone #Ketamine	Dosing	Optimization of dosing and biomarkers of pain in neonates	Pediatric opportunistic study by the PTN ongoing
Neonatal abstinence syndrome (NAS)	Methadone	PK, safety	Treatment strategies of neonatal opioid withdrawal syndrome in opioid-exposed neonates	CTSA administrative supplement completed PTN PK study completed. CSR development completed. Initial FDA submission in 2016. Revised CSR resubmitted August 2018. **FDA Meeting February 2019**
Neonatal necrotizing enterocolitis (NEC)	Meropenem	PK, safety in neonates		WR received from the FDA Clinical PK and safety trial completed; CSR to the FDA August 2011; redacted IND submission to the FDA (FDA docket number FDA-2011-N-0918). PK re-analyses completed and submitted. **LABEL CHANGED as of December 24, 2014.**

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Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Neonatal Seizures	No specific drug	Safety outcomes in medication exposure	Safety outcomes in neonates of mothers treated for seizure disorders	Co-fund with NINDS for Maternal Outcomes and Neurodevelopmental Effects of Antiepileptic drugs (MONEAD) trial 2012-2015 and 2017-2021. https://web.emmes.com/study/monead/index.htm
Apnea of Prematurity	Caffeine	Dosing and safety	Dosing and long-term safety of drug in preterm neonates	PPSR developed. NICHD co-fund of the Prematurity and Respiratory Outcomes Program (PROP). **PTN retrospective and PK CSR submitted to the FDA in April 2018. Awaiting final CSR submission scheduled for March 2019**
Exposure of medication in breastmilk	#Azithromycin #Clindamycin #Metformin Ondansetron Nifedipine Oxycodone Labetalol Tranexamic acid Escitalopram Sertraline	Dosing and safety	Opportunistic PK sampling of medications in mother-infant pairs to determine presence and relative concentrations in breast milk	PTN study developed in collaboration with FDA. 1st patient enrollment October 2018.

Drug and indications **in bold** have been identified by the NICHD as a priority and are newly added to the BPCA list.
Drugs listed twice for different indications or populations.

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Table 10: Adolescent Research Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Over-the-counter drug use	No specific drug	Health literacy		December 2007 symposium https://bpca.nichd.nih.gov/collaborativeefforts/Documents/otc_drug_use_12-06-2007.pdf
Adolescent pharmacology	No specific drug	Effects of puberty and gender on PK/ pharmacodynamics, adherence, and formulations research	Translational research, need to include adolescents in clinical trials	Pediatric Clinical Pharmacology Training grants thru NICHD and NIGMS co-funding https://bpca.nichd.nih.gov/prioritization/working_groups/Documents/adolescent_wg_07-14-2009.pdf
Obesity	Weight-based doing in pediatrics Weight loss system devices	PK, Efficacy and safety of multiple drugs	Dosing studies for multiple indication Collaborative research	PTN clinical studies underway Under consideration

Drug and indications **in bold** have been identified by the NICHD as a priority and are newly added to the BPCA list.

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Table 11: Hematologic Disease Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Sickle cell anemia	Hydroxyurea	Safety and efficacy in young children	PK, safety, and efficacy Oral formulation for children	WR received from the FDA BABY HUG trial completed in children 9–17 months of age, Draft CSR submitted in May 2013. Additional analyses underway. CSR submission pending. PK and bioavailability study conducted by PTN completed in December 2013 and submitted to FDA in February 2014 Long-term safety follow-up study under way
Thrombosis and thromboprophylaxis	Low-molecular-weight heparin	Treatment and prevention of childhood strokes and venous thrombosis	Determine validated biomarkers/ surrogate markers of anticoagulant drug including developmental hemostasis parameters and age-appropriate assays Adjunctive studies to evaluate toxicity	Pediatric opportunistic study underway by the PTN.

Drug and indications **in bold** have been identified by the NICHD as a priority and are newly added to the BPCA list.

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Table 12: Endocrine Disease Priorities and Diseases with Limited Alternative Therapies

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Fragile X	MGluR5 antagonists	Outcome measures targets for intervention	Development of MGluR5 antagonists to treat Fragile X	Development of new therapeutics co-funded with https://bpca.nichd.nih.gov/collaborativeefforts/Documents/FragileX_children_05-08-2008.pdf
Type 1 diabetes	No specific drug	Immunomodulatory therapies	Development of novel immunomodulatory therapies for children with type 1 diabetes	Collaboration with sponsored NIH networks, including TrialNET and DirectNET. Funding completed **Biomarkers of disease progress and treatment response working group in development**
Metabolic syndrome	Metformin	Dosing and toxicity	PK and toxicity data	Under consideration

Drug and indications **in bold** have been identified by the NICHD as a priority and are newly added to the BPCA list.

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Table 13: Dermatologic Diseases Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Severe inflammatory skin disease	#Methotrexate	Dosing, efficacy, and safety	Safety and efficacy in treatment of severe inflammatory disease	Co-fund of R13 workshop with NIAMS-2013 in 2014
Hemangiomas	#Timolol	PK, safety, and efficacy	PK, safety	Opportunistic study completed. **Clinical study enrolling of two doses of drug underway**

Drug and indications **in bold** have been identified by the NICHD as a priority and are newly added to the BPCA list.
#Drugs listed twice for different indications or populations.

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Table 14: Gastrointestinal Diseases Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Gastroesophageal reflux	Prokinetic drugs	Dosing, safety, and efficacy of existing drugs in neonates and infants	PK study	PTN Opportunistic study completed. Data analysis underway
	Pantoprazole	Dosing and efficacy data	Safety and effectiveness in infants	Pediatric PK/PD/Pharmacogenomics study completed. CSR submitted to FDA in Spring 2017. No label change anticipated.
Inflammatory Bowel Disease	No specific drug	Safety and efficacy of treatments in children		Participation in Gastroenterology Regulatory Endpoints and the Advancement of Therapeutics (GREAT II & III) meetings; pathways for new drug development needed.
Nausea and vomiting	Ondansetron	Dosing	PK studies in young children	Pediatric opportunistic study completed by the PTN. CSR scheduled for submission to FDA in March 2019.

Drug and indications **in bold** have been identified by the NICHD as a priority and are newly added to the BPCA list.

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Table 15: Renal Diseases Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/ or Scientific Needs	Plans and Progress
Chronic kidney disease	No specific drug	Pharmacoepidemiology data	Neurodevelopmental outcome assessments in children with CKD	Ongoing co-funding with NIDDK to evaluate outcomes of children with CKD.
Acute kidney injury	No specific drug	Drug dosing, drug interactions	Population PK studies of multiple drugs used in this patient population to prevent sub-therapeutic dosing	PTN opportunistic study ongoing
Nephrotic syndrome	No specific drug	New drug development in pediatrics	Innovative research and partnership for new drug development and repurposing	Under consideration

Drug and indications **in bold** have been identified by the NICHD as a priority and are newly added to the BPCA list.

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Table 16: Rheumatologic Disease Priorities

Current or Proposed Listed Therapeutic Area	Current or Proposed Listed Drug	Gaps in Knowledge/ Labeling	Type of BPCA Study and/or Scientific Needs	Plans and Progress
Connective tissue disorders	Hydroxychloroquine	PK and safety in children with juvenile idiopathic arthritis	PK, safety studies	Under consideration

Drug and indications **in bold** have been identified by the NICHD as a priority and are newly added to the BPCA list.

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Table 17: Special Considerations

Area of Consideration	Identified Therapeutic Area	Gaps in Knowledge/Labeling	Type of Study and/or Scientific Needs
Therapeutics in children with intellectual and developmental disabilities	No specific drug or indication	Identification of differences in drug disposition and response, including safety and efficacy outcome measures	Need for inclusion in clinical trials
Pediatric formulations	Multiple drugs and indications: Infectious diseases: HIV: antiretrovirals Tuberculosis: isoniazid Trypanosomiasis: benznidazole nifurtimox Parasitic infections: albendazole Malaria: mefloquine, sulfadoxine-pyrimethamine chlorproguanil-dapsone Hematology: hydroxyurea Oncology: 6-mercaptopurine methotrexate prednisone isotretinoin Spasticity: baclofen Hypothyroidism: l-thyroxine **Nitroglycerin Gel** **Surfactant**	Taste-masking technologies Orally dissolvable dosage forms that do not require water Heat-stable and light-stable dosage forms Safety data for excipients New technology needed to improve water solubility of intravenous formulations, reducing the need for solvents **Synthesis of existing data in use for ischemic or embolic events of peripheral vessels** **Aerosolized formulations**	Improving the technology and designs of child-friendly/easy-to-swallow dosage forms of drugs to improve adherence and effectiveness NICHD-FDA Formulations Platform https://bpca.nichd.nih.gov/prioritization/researchandcollaborations/Pages/pediatric-formulations-initiative.aspx
Pediatric devices	General Issues	Need for validation of existing devices used in children	Validation of existing methodologies
	Auto-injectors	Availability and validation of pediatric autoinjectors for biodefense countermeasures	Expansion of current methodologies, particularly in an emergency setting

continued

Area of Consideration	Identified Therapeutic Area	Gaps in Knowledge/Labeling	Type of Study and/or Scientific Needs
Opportunistic study of drugs used in children	**Multiple drugs:** **Alfentanil** **Amikacin** **Amiodarone** **Amphotericin B** **Atropine** **Bosentan** **Cefepime** **Ceftazidime** **Cidofovir** **Ciprofloxacin** **Clonidine** **Clozapine** **Dexamethasone** **Epinephrine** **Etomidate** **Fentanyl** **Fosfomycin** **Granisetron** **Haloperidol** **Labetalol** **Levofloxacin** **Lidocaine** **Lurasidone** **Methylprednisolone** **Molindone** **Nafcillin** **Nicardipine** **Olanzapine** **Oxcarbazepine** **Oxycodone** **Pentobarbital** **Piperacillin-Tazobactam** **Propofol** **Quetiapine** **Rocuronium** **Tobramycin** **Vancomycin** **Vecuronium** **Warfarin** **Ziprasidone**	**Need for doing information on drugs used in children.**	**Dosing/PK data collected from patients previously prescribed drugs listed for different indications.** **Data from this study may be used to develop full clinical studies and/or inform PK data for drug labels.** **For more information on additional drugs listed under the opportunistic clinical study, please see the PTN website.**
Electronic Health Records	**Pharmacology Research**	**Harmonization of EHRs for use in drug development research**	**Pilot studies of multicenter electronic medical records data registry under consideration**

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