Best Pharmaceuticals for Children Act

BPCA Funded Clinical Trials - Legacy Studies

NIH *Eunice Kennedy Shriver* National Institute of Child Health and Human Development

Best Pharmaceuticals for Children Act (BPCA)

Drug Name	NIH BPCA Priority Area	Study Description	Study Population	Study Duration	Labeling Status	DASH Link	FDA Docket
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.	Dash link pending	FDA-2012-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.	https://dash.nichd.nih.gov/study/14	FDA-2012-N-0284
Meropenem	Neonatal Infections	Safety, efficacy, PK study of meropenem in neonates with intra-abdominal infections.	Birth - 28 days	June 2008 - Oct 2009	A label change for meropenem for intra-abdominal infections in neonates in December 2014.	https://dash.nichd.nih.gov/study/2091	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	The results of these trials led to a label change in May 2016.	https://dash.nichd.nih.gov/study/18571	FDA-2015-N-3037
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 May 2016.	https://dash.nichd.nih.gov/study/18573	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 January 2017. Label change November 2021.	https://dash.nichd.nih.gov/study/18570	No Docket Available
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	The results of these trials led to a label change in Oct 2018. Final CSR submitted Nov 2015.	https://dash.nichd.nih.gov/study/16018	FDA-2018-N-2986

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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	The results of these trials led to a label change in Oct 2018. Final CSR submitted Nov 2015.	https://dash.nichd.nih.gov/study/16020	FDA-2018-N-2986
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. FDA requested re-analyses of some endpoints, revised data	No DASH link anticipated	No Docket Available
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 completed. IND withdrawn Nov 2018. No label change anticipated.	No DASH link anticipated	No Docket Available
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. IND withdrawn Sep 2015. No label change anticipated at this time.	DASH link pending	No Docket Available
Baclofen	Cerebral Palsy	Retrospective safety/efficacy chart review, children with spastic cerebral palsy	≥2 years - ≤16 years	Mar 1990 - Nov 2006	Draft CSR was submitted in Dec 2013, but label change not possible.	https://dash.nichd.nih.gov/study/416073	No Docket Available
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	The results of these trials led to a label change in Sep 2010 for pediatric use of chemical poisoning treatment.	No DASH link anticipated	No Docket Available