



This document is scheduled to be published in the Federal Register on 08/26/2015 and available online at <http://federalregister.gov/a/2015-21155>, and on [FDsys.gov](http://FDsys.gov)

[Billing Code: 4140-01-P]

DEPARTMENT OF HEALTH AND HUMAN SERVICES

National Institutes of Health

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

AGENCY: National Institutes of Health, The Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)

ACTION: Notice.

SUMMARY: The National Institutes of Health (NIH) hereby announces the Best Pharmaceuticals for Children Act (BPCA) Priority List of Needs in Pediatric Therapeutics for 2015. The Best Pharmaceuticals for Children Act (BPCA) seeks to improve the level of information on the safe and effective use of pharmaceuticals used to treat children. The BPCA requires that the NIH identify the drugs of highest priority for study in pediatric populations, and publish a list of drugs/needs in pediatric therapeutics. This notice fulfills the requirement to publish that list.

ADDRESS: All nominations of pediatric therapeutics for future consideration by NICHHD should be submitted to Dr. Perdita Taylor-Zapata at [taylorpe@mail.nih.gov](mailto:taylorpe@mail.nih.gov).

FOR FURTHER INFORMATION CONTACT: Dr. Perdita Taylor-Zapata via email at [taylorpe@mail.nih.gov](mailto:taylorpe@mail.nih.gov); by phone at 301-496-9584; or by fax at 301-480-2897.

SUPPLEMENTAL INFORMATION: The pediatric medical community, the public health community, and government agencies have long recognized multiple gaps in knowledge regarding the use of therapeutics in children including the correct dosage, appropriate indications, side effects, and safety concerns of pharmaceuticals in the short- and long term. These gaps have frequently resulted in inadequate labeling for pediatric use and in wide-spread off-label use of prescription drugs in children. Off-label use of a drug substantially limits the ability to gain clinical information of the drug product such as appropriate dosing of a drug, changes in drug metabolism and response during growth and development, and important short- and long-term effects.

The NICHHD, the Food and Drug Administration (FDA), other federal agencies, and various non-profit and commercial organizations are taking steps to fill knowledge gaps that exist in pediatric therapeutics and to promote an increase in evidence-based data about medications used in children.

#### **Update on BPCA Prioritization**

The BPCA requires that the NIH, in consultation with the Food and Drug Administration and experts in pediatric research, identify the drugs and therapeutic areas of highest priority for study in pediatric populations. The NIH's authority and

responsibility outlined in the BPCA legislation is to establish a program for pediatric drug testing and development and to publish a list of information needs regarding drugs used to treat children. The drug development program consists of a series of clinical trials in various therapeutic areas, which may be based on proposed pediatric study requests (PPSRs) submitted to FDA and/or from written requests (WR) received from the FDA. The BPCA Priority List consists of key therapeutic needs in the medical treatment of children and adolescents identified for further study; it is organized by therapeutic area, which can be a group of conditions, a subgroup of the population, or a setting of care. The first priority list of off-patent drugs needing further study under the 2002 BPCA legislation was published in January 2003 in the Federal Register (FR Vol. 68, No. 13; Tuesday, January 21, 2003: 2789-2790). The most recent priority list was published August 25, 2014; all Federal Register notices can be found on the BPCA website: <http://bpcanichd.nih.gov/prioritization/status.cfm>. The BPCA legislation requires the NIH to update the priority list every three years. This publication serves as an update to the BPCA priority list of needs in pediatric therapeutics.

The Obstetric and Pediatric Pharmacology and Therapeutics Branch of the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD), NIH, has developed a prioritization process for the determination of the needs in pediatric therapeutics. There are two main phases: Phase I of the prioritization process entails identifying therapeutic areas, which are general categories of conditions, diseases, settings of care, or populations with multiple therapeutic needs. The NICHD solicits input from experts in pediatric research, general pediatric and subspecialty care, organizations focused on specific conditions, and professional societies to determine

these therapeutic areas that need further study. Each year, the NICHD revisits the current list of needs in pediatric therapeutics, prioritizes three therapeutic areas of interests for that calendar year, and develops working groups in the prioritized areas.

Recommendations from these working groups are then presented to the NICHD and the FDA at the annual BPCA meeting. Previous meeting minutes can be found on the BPCA website: [http://bpca.nichd.nih.gov/prioritization/meeting\\_summary.cfm](http://bpca.nichd.nih.gov/prioritization/meeting_summary.cfm). Phase II of the prioritization process includes an extensive review and ranking of all stakeholder nominations received based on key criteria for prioritization, such as relevance, gaps in labeling information, affected populations and feasibility. Please visit the BPCA website for more details ([http://bpca.nichd.nih.gov/prioritization/priority\\_list.cfm](http://bpca.nichd.nih.gov/prioritization/priority_list.cfm)).

Below is an updated list of therapeutic areas and drugs that have been prioritized for study since the inception of the BPCA and a summary of the NICHD's plans and progress in all of these areas to date. The NICHD welcomes input from the pediatric medical community on additional gaps in pediatric therapeutics for future consideration. All nominations should be submitted to Dr. Perdita Taylor-Zapata at the address in the ADDRESS section of this notice.

### **Priority List of Needs in Pediatric Therapeutics 2015**

In accordance with the BPCA legislation, the list outlines priority needs in pediatric therapeutics for multiple therapeutic areas listed below. The complete list can be found on the BPCA website at the following address: <http://bpca.nichd.nih.gov>.

- [Table 1: Infectious Disease Priorities](#)
- [Table 2: Cardiovascular Disease Priorities](#)
- [Table 3: Respiratory Disease Priorities](#)

- Table 4: Intensive Care Priorities
- Table 5: Bio-defense Research Priorities
- Table 6: Pediatric Cancer Priorities
- Table 7: Psychiatric Disorder Priorities
- Table 8: Neurological Disease Priorities
- Table 9: Neonatal Research Priorities
- Table 10: Adolescent Research Priorities
- Table 11: Hematologic Disease Priorities
- Table 12: Endocrine Disease Priorities and Diseases with Limited Alternative Therapies
- Table 13: Dermatologic Disease Priorities
- Table 14: Gastrointestinal Disease Priorities
- Table 15: Renal Disease Priorities
- Table 16: Rheumatologic Disease Priorities
- Table 17: Special Considerations.

Dated: August 19, 2015.

---

Francis S. Collins, M.D., Ph.D.

Director

National Institutes of Health

[FR Doc. 2015-21155 Filed: 8/25/2015 08:45 am; Publication Date: 8/26/2015]