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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 2014. The BPCA, reauthorized in 2012 as part of the Food and Drug Safety and Innovation Act, 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.

SUPPLEMENTARY INFORMATION: The pediatric medical community, the public health community, and government agencies have recognized multiple gaps in knowledge regarding the use of therapeutics in children, including the correct dose, 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 NICHD is working with the Food and Drug Administration (FDA), other federal agencies, and various nonprofit and commercial organizations to address the 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 FDA and experts in pediatric research, identify the drugs and therapeutic areas of highest priority for study in pediatric populations.

The BPCA authorizes the NIH to establish a program for pediatric drug testing and development and to publish a list of drugs/needs in pediatric therapeutics. The BPCA program consists of a series of clinical trials in various therapeutic areas, which may be based on proposed pediatric study requests (PPSRs) submitted to the FDA and/or on written requests (WR) received from the FDA. The BPCA Priority List consists of key therapeutic needs in the medical treatment of children and adolescents that need further study. It is organized by therapeutic areas, which are general categories of conditions, diseases, settings of care, or populations with multiple therapeutic needs. 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 October 24, 2012; all Federal Register Notices can be found on the BPCA website:

<http://bpcanichd.nih.gov/prioritization/status/Pages/status.aspx>. The NIH is required by the BPCA 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 NICHD developed a prioritization process for the determination of the needs in pediatric therapeutics. There are two main phases in this process. 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 the pediatric research, general pediatric and subspecialty care, and patient advocacy groups to determine these therapeutic areas that need further study. At the beginning of every year, the NICHD revisits the current list of needs in pediatric therapeutics, prioritizes three therapeutic areas of interest for that calendar year, and develops working groups in the prioritized areas. Recommendations from the therapeutic area working groups are then presented to the NICHD and the FDA at the end of the calendar year at the Institute's annual BPCA meeting. Previous meeting minutes can be found on the BPCA website:

<http://bpcanichd.nih.gov/prioritization/meetings/Pages/index.aspx>.

Phase II of the prioritization process entails an extensive review and ranking of all nominations received based on key criteria for prioritization, such as relevance, label gaps, affected

population, and feasibility. Please visit the BPCA website for more details:

([http://bpca.nichd.nih.gov/prioritization/priority\\_list/Pages/priority\\_list.aspx](http://bpca.nichd.nih.gov/prioritization/priority_list/Pages/priority_list.aspx)).

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 (contact information below).

### **Priority List of Needs in Pediatric Therapeutics 2014**

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

We would like for you to save the date of **December 10, 2014**, for our annual BPCA stakeholders meeting. The meeting will be held at the Natcher Auditorium on the NIH campus in Bethesda, Maryland, and will be streamed live via videocast for all non-local attendees.

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

Dated: August 18, 2014

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Francis S. Collins,

Director, National Institutes of Health

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