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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, HHS.

ACTION: Notice.

SUMMARY: The National Institutes of Health (NIH), *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD) hereby announces the renewal of the Best Pharmaceuticals for Children Act (BPCA) Program. 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, publish a list of drugs/needs in pediatric therapeutics, and fund studies in the prioritized areas. This notice will provide a brief summary of recent changes in the legislation, a brief update on the current progress of the BPCA Program and provide the current Priority List of Needs in Pediatric Therapeutics.

ADDRESSES: The complete Priority List of Needs in Pediatric Therapeutics 2018-2019 can be found on the BPCA website at the following address:

<https://www.nichd.nih.gov/research/supported/bpca/activities>.

FOR FURTHER INFORMATION CONTACT: Dr. Perdita Taylor-Zapata via email at taylorpe@mail.nih.gov; or by phone at 301-496-9584.

SUPPLEMENTARY INFORMATION: 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 BPCA Program has been in existence since 2004 and is overseen by the Obstetric and Pediatric Pharmacology and Therapeutics Branch (OPPTB) of the NICHD. To date, the BPCA Program has prioritized over 150 drugs and therapeutic areas, funded more than 25 clinical studies, and improved the labeling to date of eight drugs and one device in the ongoing effort of advancing the knowledge of dosing, safety and effectiveness of medicines used in children. However, despite these and many other efforts, many gaps in our knowledge still remain 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 result in inadequate labeling and/or wide-spread off-label use of prescription drugs in children. Off-label use of a drug substantially limits the ability to obtain important clinical information for more generalized use of a drug product, such as characterizing changes in drug metabolism and response during growth and development, identifying precision-based responses (i.e., impact of genotype and phenotype of medication responses, the impact of obesity on dosing), and determining short- and long-term effects. The mandate of the NIH BPCA Program is to fill knowledge gaps that exist in pediatric therapeutics and to promote an increase in evidence-based data about medications used in children. Please see the BPCA website for more information:

<https://www.nichd.nih.gov/research/supported/bpca/about>

Update on the BPCA Legislation

First authorized in 2002, the Best Pharmaceuticals for Children Act (BPCA) has been reauthorized as part of larger Food and Drug Administration (FDA) user fee legislation three additional times: 2007, 2012, and now 2017. The overall mandate for the implementation of the research program at NIH has remained the same throughout, but with clarifications each time: to

prioritize testing of pediatric therapeutics that do not have labeling for pediatric use, to sponsor clinical trials and other research to provide the necessary data, and to submit those data to the FDA to begin the process of obtaining label changes and provide clinicians with the appropriate information on appropriate pediatric use and dosing. In August of 2017, the BPCA legislation was reauthorized by Congress, which renewed the NIH BPCA Program for five years (the FDA portion of the program is permanently authorized). The new legislation also permits the NIH to prioritize research on the identification of biomarkers for pediatric diseases and conditions. In addition, a new provision specifically allows the NIH to post the data from the pediatric studies it funded on its public website when it submits the report to the FDA, as required for potential label changes.

Update on BPCA Prioritization

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 has been published to the BPCA website; more information on the prioritization process, all BPCA priority lists, and all Federal Register Notices can be found on the BPCA website: <https://www.nichd.nih.gov/research/supported/bpca/prioritizing-pediatric-therapies>. The BPCA authorizing legislation requires the NIH to update the priority list every three years. This Notice serves as an update to the BPCA priority list of needs in pediatric therapeutics.

Each year, the NICHD revisits the current list of needs in pediatric therapeutics and seeks input from experts in pediatric research and medicine to determine if previous needs still exist and if new areas of needs have developed.

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. In 2017, the NIH BPCA Program focused on the following areas: Treatment options in Pediatric Hypertension, Biomarkers in Pediatric Research (various subspecialties), and Treatment strategies in several neonatal conditions (including Neonatal Opioid Withdrawal Syndrome, also known as Neonatal Abstinence Syndrome). Meeting minutes for workshops and lectures on the above topics can be found on the BPCA website

<https://www.nichd.nih.gov/research/supported/bpca/research-initiatives-collaborations>.

For 2018, the NIH BPCA Program's priorities have included: Heart failure in children, Kidney diseases, and Lactation (in particular, neonatal and infant medication exposure). The NICHD welcomes input from the pediatric medical community on additional gaps in pediatric therapeutics for future consideration. The most recent BPCA stakeholders meeting was held in Bethesda, Maryland on March 22, 2019. More information will be provided on the BPCA website as it becomes available. All inquiries should be submitted to Dr. Perdita Taylor-Zapata at the contact information above.

Priority List of Needs in Pediatric Therapeutics 2018-2019

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: <https://www.nichd.nih.gov/research/supported/bpca/activities>

- 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: April 17, 2019.

Francis S. Collins,

Director,

National Institutes of Health.

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