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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 updates to the Best Pharmaceuticals for Children Act (BPCA) Program priorities for 2020-2021. 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 update on the current progress of the BPCA Program and provide the current Priority List of Needs in Pediatric Therapeutics.

**DATES:** The next BPCA stakeholders meeting will take place on December 14 – 15, 2020.

More information will be provided on the BPCA website as it becomes available.

**ADDRESSES:** The complete Priority List of Needs in Pediatric Therapeutics 2020-2021 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](mailto: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 one-hundred and fifty (150) drugs and therapeutic areas, funded more than forty (40) clinical studies, and improved the labeling to date of eleven (11) drugs and two (2) devices 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 knowledge still remain regarding the use of therapeutics in children including the correct dosage, appropriate indications, side effects, and safety concerns 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 metabolic pathways and response during growth and development, identifying precision-based treatments (i.e., impact of genotype and phenotype of medication responses), and determining short- and long-term effects of medications. The mandate of the NIH BPCA Program is to identify 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 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 Pub. L. 107-109, 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 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 BPCA authorizing legislation requires the NIH to hold meetings with stakeholders for input into the prioritization process and to update the priority list every three years.

The most recent BPCA stakeholders meeting was held in Bethesda, Maryland on March 22, 2019. Please save the date for **December 14-15, 2020** for the next stakeholders meeting. 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. This Notice serves as an update to the BPCA priority list of needs in pediatric therapeutics.

### **Priority List of Needs in Pediatric Therapeutics 2020-2021**

In accordance with the BPCA legislation, the list outlines priority needs in pediatric therapeutics for multiple therapeutic areas listed below. The complete 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, 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 22, 2020.

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