# Request for Information: Call for Nominations for NICHD's Best Pharmaceuticals for Children Act Priorities in Pediatric Therapeutics 2022

## Notice Number: NOT-HD-XX-XXX

## Key Dates

**Release Date:** March 1, 2022
**Response Date:** June 1, 2022

## Related Announcements

None

## Issued by

*Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD)

## Purpose

The NICHD invites all stakeholders to submit nominations for research to be prioritized in the development of the Best Pharmaceuticals for Children Act (BPCA) Program's 2022 Priority List of Needs in Pediatric Therapeutics. The NICHD is gathering nominations for drugs and therapeutic areas (e.g., pediatric condition, subpopulation, or setting of care) that require further study in children. Suggested topics not related to a specific drug, biologic, or device (e.g., training or research collaborations) will be considered by the NICHD outside of this framework.

This is a time-sensitive Request for Information (RFI). There will be two opportunities for public input in 2022:

1. **Written responses will be accepted through June 1, 2022.** All responses will be compiled and reviewed by a committee of stakeholder representatives. The review will result in a preliminary priority list.

2. There will be an annual stakeholders meeting in December 2022 to review the final priority list and provide updates on the BPCA drug development clinical program and on the BPCA Framework Initiative.

The purpose of this RFI is to obtain knowledge and gather information regarding current gaps in pediatric therapeutics. The information from this RFI will be used for prioritization planning purposes only and shall not be construed as a solicitation for applications, abstracts, or quotations, or as an obligation on the part of the NICHD to provide any funds on the basis of responses.

### Background

The NICHD, part of the National Institutes of Health (NIH), leads a pediatric drug development program to identify and prioritize needs in pediatric therapeutics under BPCA. Originally authorized in 2002, BPCA was reauthorized in 2017 as part of the Food and Drug Administration Reauthorization Act (FDARA). The legislation has two main components:

1. Encouragement of pediatric drug testing through exclusivity incentives to private companies to conduct research in drugs used in children.

2. Authorization of a research program through the Department of Health and Human Services (HHS), with responsibility for the research program implementation through the NIH, specifically the NICHD for the development of a priority list of needs in pediatric therapeutics, and the subsequent sponsoring of clinical trials of drugs that emanate from the priority list.

Nominations for the BPCA Priority List of Needs in Pediatric Therapeutics are solicited as part of fulfilling NIH's authority and responsibility to establish an off-patent pediatric drug development program for pediatric drug testing. The BPCA Priority List consists of key therapeutic needs in the medical treatment of children and adolescents; it is organized by therapeutic area, which can be a group of conditions, a subgroup of the population, or a setting of care. The NICHD works closely in consultation with the U.S. Food and Drug Administration (FDA) and experts in pediatrics, to gather knowledge gaps in pediatric therapeutics. Each calendar year, prioritized therapeutic areas and drugs are selected for further prioritization and study implementation within the Pediatric Trials Network (https://pediatrictrials.org/).

For reference, the 2020 BPCA Priority List of Pediatric Needs is located on the BPCA website: https://www.nichd.nih.gov/research/supported/bpca/prioritizing-pediatric-therapies.

In addition to the determination of a priority list of needs in pediatric therapeutics, the BPCA program sponsored a new initiative in 2021 called the BPCA Framework to Enable Pediatric Drug Development. This project is an annotated, selective, curated collection of resources to assist drug developers, researchers, and clinicians with a universal resource of relevant topics to review before conducting pediatric drug development research. To be notified when the Framework is available, contact bpca@infinityconferences.com to be added to the BPCA Stakeholders distribution list.

## Information Requested

The NICHD is interested in concrete and relevant ideas and approaches to addressing scientific and regulatory needs in therapeutic areas, drugs, biologics, and devices in an effort to best target clinical (or translational) studies sponsored through the BPCA Program. Nominations should focus on identifying the scientific opportunity and approaches, technology, and expertise useful for a project's development rather than on a funding instrument or solicitation design.

In addition to submitting therapeutic priorities for consideration, the BPCA program is also interested in gathering data on areas that may impact the future directions of clinical therapeutic research overall. The following areas are a short list of topics of interest:

- Existing or new models for trans-agency collaborative workforce development in pharmacology research;
- Existing data or other resources related to clinical and/or translational pharmacodynamic (how the body responds to medications) biomarkers that can be considered for validation in future clinical trials; and
- Existing or new models of multi-dimensional inclusion of underserved populations (including children, women, and underrepresented minorities) in clinical therapeutic research and clinical trials.
- Best practices for widespread dissemination of clinical research data to a broad audience of researchers, clinicians, and patients/parents.

The BPCA nomination form consists of 10 comment areas and was developed to solicit information pertinent to the specific evaluation criteria described below.

## Evaluation Criteria

All nominations will be reviewed and evaluated on six key criteria, as follows:

*Relevance to BPCA mission and goals
*No disqualifying ethical concerns
*Evidence: consideration of the level of evidence available and current gaps

*Impact: potential effect on children, society, and delivery of care
*Population: consideration of the different populations that may benefit from the research
*Feasibility: consideration of the resources available to conduct the study.

Nominations must meet at least five of the six criteria to be considered.

## Prioritization Process

The NICHD has established a prioritization process through which all nominations will be considered and evaluated by leading pediatric experts and stakeholder representatives.

*Gather Nominations (March-June 2022)*
The NICHD is soliciting research nominations beginning in March 2022 by interacting with key stakeholder groups and requesting submission of the nomination form from the public and other stakeholders.

*Prioritize Therapeutic Areas (July 2022)*
The NICHD will convene key stakeholders to evaluate and prioritize the drug and Therapeutic Area nominations. From this assessment, the NICHD will finalize the top Therapeutic Areas for the next Priority List of Pediatric Needs.

## Responses

Prioritization nomination submissions in response to this RFI will be considered by the NICHD, the FDA, and an ad hoc evaluation panel to be convened by the NICHD as part of this prioritization process. **Nominations will be accepted through June 1, 2022.** This nomination form is the only vehicle by which nominations for therapeutic area, drugs, biologics, and devices will be collected. The form corresponds to one nomination; a party may complete the form multiple times if interested in submitting more than one nomination.

The preferred format is online submission of the nomination using the following URL: **https://www.surveymonkey.com/r/BPCAPrioritization2022**. As an alternative, a nomination form is also available as a PDF document by request to bpca@infinityconferences.com. The completed PDF form can be sent via email or mail to the contact listed below.

Emails may be sent to taylorpe@mail.nih.gov. Please indicate “BPCA Nomination” as the subject line of your email.

Letter responses should be addressed to the attention of Dr. Taylor-Zapata at the address below.

Responses to this RFI are voluntary. Do not include any proprietary, classified, confidential, trade secret, or sensitive information in your response. The responses will be reviewed by NIH staff, and individual feedback will not be provided to any responder. The Government will use the information submitted in response to this RFI at its discretion. The Government reserves the right to use any submitted information on public NIH websites, in reports, in summaries of the state of the science, in any possible resultant solicitation(s), grant(s), or cooperative agreement(s), or in the development of future funding opportunity announcements.

This RFI is for information and planning purposes only and shall not be construed as a solicitation, grant, or cooperative agreement, or as an obligation on the part of the Federal Government, the NIH, or individual NIH Institutes and Centers to provide support for any ideas identified in response to it. The Government will not pay for the preparation of any information submitted or for the Government's use of such information. No basis for claims against the U.S. Government shall arise as a result of a response to this request for information or from the Government's use of such information.

# Inquiries

Please direct all inquiries to:

Perdita Taylor-Zapata, M.D.
*Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD)
Telephone: 301- 496-9584
Email: taylorpe@mail.nih.gov