Overview & Background

OMB # 0925-0766

Expiration Date: 4/2023

OVERVIEW

The *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD), the National Institutes of Health (NIH), and the U.S. Department of Health and Human Services (HHS) invite all stakeholders, including physicians, clinicians, researchers, pediatric organizations and associations, patient representatives and other interested parties to submit nominations for pediatric research as it begins the Best Pharmaceuticals for Children Act (BPCA) annual prioritization process. NICHD is gathering nominations for a Therapeutic Area (e.g., pediatric condition, subpopulation or setting of care) that requires further study in children as well as the particular drug, biologic, or medical device that is being recommended for study in that area.

BACKGROUND

Nominations for the BPCA Priority List of Needs in Pediatric Therapeutics are solicited as a part of fulfilling NIH's authority and responsibility to establish a program for pediatric drug testing and development as outlined in the BPCA legislation. The 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. Each Priority List targets a few Therapeutic Areas for discussion and further prioritization.

For your reference, the 2020 BPCA Priority List of Needs in Pediatric Therapeutics is located on the BPCA website:

https://www.nichd.nih.gov/research/supported/bpca/prioritizing-pediatric-therapies. NICHD has established a prioritization process through which all nominations will be considered and evaluated by leading pediatric experts and stakeholder representatives. The final Priority List is published in the Federal Register, and sets the agenda for NICHD's focus on future BPCA-related activities.

Dates & Evaluation Criteria

DATES

There will be two opportunities for public input in 2022:

1) This nomination form 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 program.

EVALUATION CRITERIA

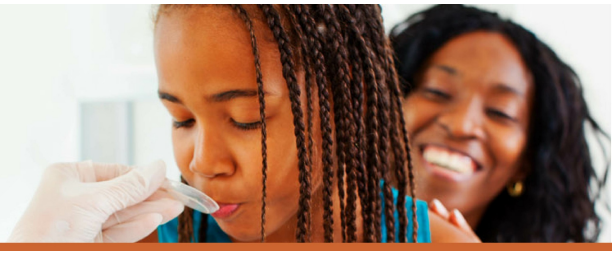
All nominations for research will be reviewed and evaluated on six key criteria, as follows:

1) Relevance to BPCA mission & goals
2) No disqualifying ethical concerns
3) Feasibility: consideration of the resources available to conduct the study
4) Impact: potential effect on children, society, and delivery of care
5) Population: consideration of the different populations that may benefit from the research
6) Evidence: consideration of the level of evidence available and current gaps

For further detail on these criteria, see the Request for Information (RFI) in the National Institutes of Health Guide at [LINK PENDING].

Best Pharmaceuticals for Children Act

NIH *Eunice Kennedy Shriver* National Institute of Child Health and Human Development



Instructions

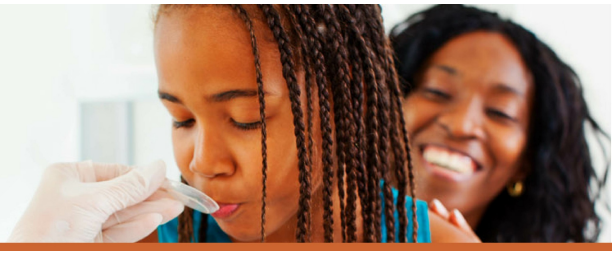
On the following pages, you have the opportunity to submit a nomination for consideration in the BPCA Priority List of Needs in Pediatric Therapeutics. This nomination form is the primary vehicle by which nominations will be collected. The form corresponds to one nomination; a party may complete the form multiple times if interested in submitting more than one nomination.

Nominations must specify the drug, biologic, or medical device to be studied and the Therapeutic Area in which it will be studied. Nominations will be categorized by Therapeutic Areas when reviewed.

Please contain all open-ended responses to 100 words or less.

Best Pharmaceuticals for Children Act

NIH *Eunice Kennedy Shriver* National Institute of Child Health and Human Development



Nomination Submission Form

*** 1. Please identify and list the proposed drug, biologic, or medical device to be investigated, along with the proposed pediatric indication of concern.**

Drug	
Biologic	
Medical Device	

2. Please list the proposed Therapeutic Area in which the proposed drug, biologic or medical device is to be investigated and the potential impact of a study in this area.

A Therapeutic Area may be a group of conditions, a subgroup of the population, or a setting of care. For example, the 2009 priority list includes the use of lorazepam for sedation in the intensive care unit. The indication is Sedation, and the Therapeutic Area would be Intensive Care.

Please consider the following in your response:

- **Prevalence of the condition/size of population subgroup/use in setting of care**
- **Morbidity of the underlying condition**
- **Severity, as indicated by mortality rate, hospital length of stay (LOS), and/or the presence of comorbidities**
- **Frequency of use of the drug, biologic, or medical device for proposed indication**
- **Availability of alternative treatments, if any**
- **Potential for research results to have multiplicative effects across Therapeutic Areas or other indications**

3. Please submit a single sentence that frames your research question about the use of the drug, biologic, or medical device. Be sure to include the specific indication and outcome measure to be investigated.

4. For the proposed nomination, what study design would be most effective in providing the needed evidence in pediatrics?

- ◯ Synthesis of existing evidence (e.g., systematic review, meta-analysis)
- ◯ Primary research using prospective data collection without randomization (e.g., observational study)
- ◯ Primary research through a prospective randomized trial
- ◯ Other (please describe)

5. Understanding that there is the potential for ethical concerns with research involving pediatric populations, please describe any potential ethical considerations relevant to the nominated research study. Please describe potential ethical considerations with respect to both the Therapeutic Area as well as the proposed drug, biologic, or medical device.

6. Please describe any existing evidence available regarding the proposed research question and the feasibility of the proposed research question, as it relates to the following considerations:

- **Existing evidence: Existence of an unmet need or gap in the available evidence; relevance to uncertainties for physicians, such as variations in clinical care or controversy over what constitutes appropriate clinical care; any economic analyses allowing calculation of health utilities; any existing efforts underway to conduct research in this area**
- **Feasibility: Resources required to conduct the study, taking into account the availability of patients, sites, and principal investigators. Please acknowledge any existing networks or resources available.**

7. Please describe any other information you would like to share that supports your nomination.

Areas for Continued Discussion/Collaboration

In addition to submitting therapeutic priorities for consideration, the BPCA program is also interested in gathering data on areas that may impact the success of clinical research. The following is a short list of areas for continued discussion and potential areas ripe for collaborative actions.

Please indicate if you have expertise or information to assist in advancing discussion and collaboration in the following areas:

8. Models for collaborative workforce development in pharmacology research

- ◯ Yes
- ◯ No

If yes, please specify.

9. Existing resources of clinical and translational biomarkers that can be considered for validation

- ◯ Yes
- ◯ No

If yes, please specify.

10. Existing models for multi-dimensional inclusion of and dissemination practices to underserved populations (including children, women, and underrepresented minorities) in clinical drug trials

- ◯ Yes
- ◯ No

If yes, please specify.

11. Best practices for widespread dissemination of clinical research data to a broad audience of researchers, clinicians, and patients/parents

- ◯ Yes
- ◯ No

If yes, please specify.

12. Please rank the following 4 areas for continued discussion in terms of priority with 1 being highest priority and 4 being lowest priority.

Models for collaborative workforce development in pharmacology research

Existing resources of clinical and translational biomarkers that can be considered for validation

Existing models for multi-dimensional inclusion of and dissemination practices to underserved populations (including children, women, and underrepresented minorities) in clinical drug trials

Best practices for widespread dissemination of clinical research data to a broad audience of researchers, clinicians, and patients/parents

13. Please indicate if each of the following areas are high priority, medium priority, or low priority:

	High Priority	Medium Priority	Low Priority
Models for collaborative workforce development in pharmacology research	○	○	○
Existing resources of clinical and translational biomarkers that can be considered for validation	○	○	○
Existing models for multi-dimensional inclusion of and dissemination practices to underserved populations (including children, women, and underrepresented minorities) in clinical drug trials	○	○	○
Best practices for widespread dissemination of clinical research data to a broad audience of researchers, clinicians, and patients/parents	○	○	○

14. Are you interested in joining a public assembly to discuss advancement in any of the areas above? If so, select the box(es) next to your preferred assemblies.

- [ ] Models for collaborative workforce development in pharmacology research
- [ ] Existing resources of clinical and translational biomarkers that can be considered for validation
- [ ] Existing models for multi-dimensional inclusion of and dissemination practices to underserved populations (including children, women, and underrepresented minorities) in clinical drug trials
- [ ] Best practices for widespread dissemination of clinical research data to a broad audience of researchers, clinicians, and patients/parents

Nominator Information

Completion of the following information is optional. If you would like to receive future BPCA communication, please provide contact information below. The answers you give will not influence the progress of your submission.

15. Please select a description that best describes your role or perspective:

- ◯ Employer
- ◯ Government - Programs (e.g., Medicare, Medicaid)
- ◯ Government - Research
- ◯ Health Care Provider
- ◯ Health Plan/Insurance Carrier
- ◯ Manufacturer (Device)
- ◯ Manufacturer (Drug or Biologic)
- ◯ Medical Administrator
- ◯ Nonprofit/Policy Institute
- ◯ Patient/Family (including family caregiver)
- ◯ Professional Association
- ◯ Public/Consumer
- ◯ Researcher

16. Who are you representing with your response?

- ◯ Self
- ◯ Organization

17. How did you hear about the opportunity to submit a nomination for the BPCA Priority List of Needs in Pediatric Therapeutics?

18. If you are not already receiving BPCA communications, would you like to be added to the BPCA Stakeholders Distribution List?

- ◯ Yes
- ◯ No

Please note that this survey is anonymous so we cannot respond to any questions/comments unless you provide your contact details. If you are interested in joining a public assembly or you would like to be added to the BPCA Stakeholders distribution list, please complete the section below. Otherwise, contact information is optional.

19. Contact Information

Name	
Organization	
Title	
Email address	
Phone number	