# Study Proposal Submission to the NICHD Pediatric Trials Network (PTN)

1. The Pediatric Trials Network is charged with the mission of developing and conducting pharmacokinetic, safety, and efficacy studies of U.S. marketed drugs (primarily off-patent) in pediatric populations. Studies are conducted in accordance with the Best Pharmaceuticals for Children Act; the ultimate goal is to submit study results to FDA for review to improve drug product labels for the pediatric population. The Pediatric Trials Network welcomes study proposals from external investigators in support of this mission. Proposals that include study drugs listed on the BPCA drug priority list will be given preference. Study proposals should follow the format and include all applicable content outlines in the proposal template available at <https://pediatrictrials.org/future-research/xxx>. The following are key elements of proposals: Information about the therapeutic product of interest including routes of administration and formulations, and manufacturers.
2. The current labeling of the product of interest including adult and pediatric indications.
3. The regulatory goal for the proposed therapeutic of interest.
4. Background and significance of the public health impact of the therapeutic of interest in the condition of interest. This should include pharmacoepidemiologic data about on or off-label usage of the therapeutic of interest in adult or pediatric populations when applicable.
5. The regulatory plan and strategy. Note that the plan and strategy should clearly outline how information about the therapeutic of interest will be added to the label but does not have to be completed using a single study or project. To be comprehensive and clearly interpretable, the regulatory plan and strategy should include information about current labeling, and the information needed to support the FDA pediatric extrapolation route chosen for the therapeutic of interest, as applicable. Examples include descriptions of disease similarity between adults and children, exposure-response relationships of the therapeutic of interest in adults and children, and pharmacokinetic and safety data in adults or children. See the following links for additional information regarding pediatric extrapolation.
* <https://www.ncbi.nlm.nih.gov/pubmed/22025597>
* <https://onlinelibrary.wiley.com/doi/abs/10.1002/9781118312087.ch11>
* <https://www.ncbi.nlm.nih.gov/pubmed/28890858>
1. Proposed key protocol design features, including, as applicable: study design, number of participants, number of sites, primary objective and endpoint, secondary objective(s) and endpoint(s), exploratory objective(s) and endpoint(s), description of intervention, draft schedule of assessments table, and any other information deemed essential to the review process.

The PTN leadership will screen study proposals, primarily to assess fit with the PTN mission. Study proposals that pass the initial screening process will then be reviewed by the PTN Steering Committee on a quarterly schedule.

PTN Steering Committee reviews will be conducted similar to an NIH study section, with a primary and secondary reviewer assigned to each study proposal. Study proposals will be judged from 1-10, with 1 being the best score, based on the following criteria:

* Potential public health impact
* Study Feasibility
* Study Design
* Priority within PTN
* Total summary score

Investigators will receive written feedback from the Steering Committee review process within one month of the quarterly PTN Steering Committee meeting. Decisions on whether the PTN will convert the study proposal to a PTN protocol and the timing of protocol execution will be made based on PTN Steering Committee feedback NICHD funding availability, and NICHD priorities. Of note, decisions on study execution are typically made approximately one year in advance.

For additional information regarding the Pediatric Trials Network, please use the following websites as guidance.

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

Investigators interested in discussing project proposals prior to submission for review may contact the PTN Program Manager at PTN-Program-Manager@dm.duke.edu.