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Posting Date: 12/7/18
Due Date: 1/7/19

Development of Guidelines to Integrate Patient Perspectives into Clinical Trial Statistical Design

Key Dates	
Request for Proposal Released	December 7, 2018
Deadline for Questions	January 2, 2019
Responses to Questions	January 4, 2019
Deadline for Proposals	January 7, 2019
Projected Notification Date	January 11, 2019
Projected Start Date	January 29, 2019

Request for Proposals (RFP)

MDIC is seeking proposals from organizations for assistance writing a report on the lessons learned from the MDIC 2016-2018 project to test a model for using patient preference information to inform the statistical design of clinical trials. With the Novel Method for Incorporation of Patient-Centered Outcomes Research in Clinical Trial Design (PCOR) project nearing completion, we would like to develop a guideline document that synthesizes the findings of this project into actionable lessons learned and incorporates expert and stakeholder review and feedback.

Eligibility

Private-sector, nonprofit, and for-profit organizations are eligible to submit proposals.

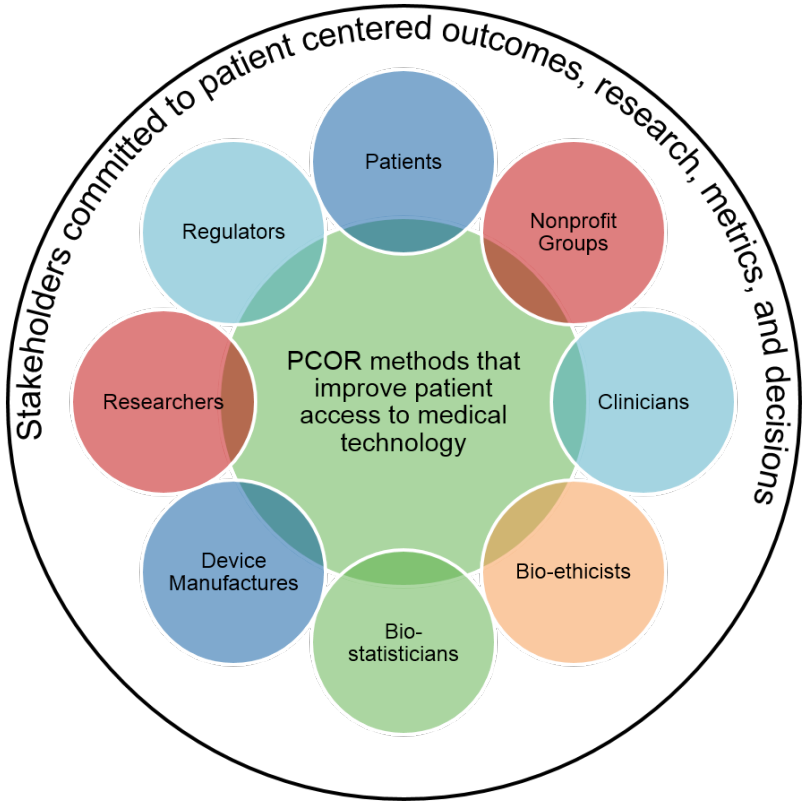
Background

For chronic debilitating and terminal illnesses with no effective treatments, the standard statistical threshold for determining therapeutic effectiveness in clinical trials may be too conservative and may not reflect patients' perspectives on the trade-off between the risk of endorsing an ineffective therapy (type I statistical errors) and the risk of rejecting an effective therapy (type II statistical errors).

Clinical trials designed and sized with consideration of patient preference information may elevate the voices of smaller patient populations and patients with rare diseases and expedite trials. By designing clinical trials that reflect patients' urgency and risk tolerance, scarce resources can be allocated more efficiently, bringing more innovative therapies to more patients, faster.

Objectives: This project developed and tested a method for incorporating patient preference information as an explicit means to set significance levels in clinical trial design. While the pilot

focuses on a specific disease state, Parkinson's disease, the method may be generalizable to other diseases and has the potential to remove barriers to therapy access by giving patients a pathway to breakthrough, lifesaving technologies based, in part, on their risk tolerance.



Specific Aims:

1. Identify the outcomes important to patients, family members, and caregivers
2. Design and conduct a patient preference assessment study
3. Design methods for clinical trials based on explicit patient input
4. Assess medical device stakeholder acceptance of clinical trial designs based on patient preference, with particular focus on regulatory and reimbursement stakeholders

Outcomes: This method is the first to incorporate patient preference information as an explicit means to set significance levels in clinical trial design. While this project focuses on a specific disease state, the method may be generalizable to other diseases. The primary outcome of this project is a method to determine an appropriate p-value threshold and trial size using patient input. This project will also lead to the development of an open-source software tool to incorporate this type of patient perspective into clinical trial design.

Impact: The direct incorporation of patient preferences into clinical trial design will address the longstanding conundrum that, in an enterprise devoted to easing the burden of disease, the

afflicted currently have little to no input into the process. This method may remove barriers to access by giving patients a pathway to breakthrough, lifesaving technologies based on their risk tolerance and the resulting potential for reduced clinical trial size.

Management of the Project

The contractor is expected to work with the MDIC Science of Patient Input Statistics and Outcomes working group. The Statistics and Outcomes working group consists of representatives from MDIC member organizations, as well as representative of the PCOR project investigator team. The working group will manage, guide, and provide feedback to the contractor.

Deliverables to be Completed within the Period of Performance

This report is part of a larger MDIC initiative to develop a Framework for Patient Input in Medical Device Clinical Trials. In order to meet the deadlines of the larger project, we would require completion of the draft report by **June 15, 2019** and completion of the final report by **August 31, 2019.** MDIC would circulate the draft report for stakeholder feedback to the broader Science of Patient Input steering committee and other relevant stakeholders. That feedback would be incorporated into the final report. We anticipate sharing the final report at MDIC's Annual Public Forum September 5, 2019.

It is expected that the contractor will work closely under the direction of the working group and will provide interim milestones, such as outlines and section drafts, so that the working group can provide regular feedback and guidance.

Submission Components

To enable MDIC to evaluate the submission, the responding proposal must include the following:

- A project proposal that may not exceed 5 pages
- A timeline for completing the deliverables within the required period of performance
- A budget for a time and materials contract that includes proposed hourly rates for all personnel who will be supporting the project, including all expected costs and expenses
- Curriculum Vitae (CVs) of key personnel with experience with projects of a similar nature (experience with medical device evidence preferred)

Period of Performance

January 29, 2019 – September 30, 2019

Review Process

MDIC staff will review received proposals. MDIC staff reserve the right to contact applicants with additional questions during the review period or conduct an interview. MDIC staff reserve

the right to consult additional external stakeholders to review applications. Any external reviews will be completed in accordance with the MDIC conflict of interest policy. Responses will be reviewed for completeness and appropriateness of the responses as they pertain to the required submission components. MDIC will consider both the programmatic aspects of the proposal, as well as the anticipated cost, with the programmatic elements of the proposal receiving greater weight. MDIC may, for example, choose a costlier proposal if its programmatic offering warrants the premium. However, as potential contractors' programmatic offerings move toward equivalency, cost will gain in importance.

MDIC's selection of a contractor will be contingent on the parties executing a mutually acceptable contract. MDIC reserves the right to terminate contract negotiations at any time and select another contractor if it determines that it is unlikely that an agreement will be executed in a timely manner.

Timeline

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Please send proposals or questions to Stephanie Christopher, schristopher@mdic.org. Deadline for proposals is January 7, 5 p.m. EDT.

About MDIC

The Medical Device Innovation Consortium (MDIC) is the first public-private partnership created with the sole objective of advancing medical device regulatory science throughout the total product life cycle. MDIC's mission is to promote public health through science and technology and to enhance trust and confidence among stakeholders. We work in the pre-competitive space to facilitate development of methods, tools, and approaches that enhance understanding and improve evaluation of product safety, quality, and effectiveness. Our initiatives improve product safety and patient access to cutting-edge medical technology while reducing cost and time to market.

For more information visit: http://www.mdic.org