



## Background

Inborn errors of metabolism (IBEM) are rare conditions that interfere with the processing of food by our body, and can lead to devastating consequences such as cognitive impairment, organ damage, and even death. Many patients with IBEM are identified by Newborn Bloodspot Screening (NBS) programs which allow for early detection and treatment. As better techniques have allowed more conditions of IBEM to be detected, some conditions identified at NBS have uncertain clinical consequence. Lack of data and variability in policy and practice regarding these conditions has led to an inconsistent approach to care management.

## Purpose

The purpose of the Inborn Errors of Metabolism Collaborative (IBEMC) QUEST project is to build a network of patient partners and stakeholders (providers, public health professionals, researchers) focused on linking data available in the IBEMC to patient-reported outcomes to facilitate comparative effectiveness research. A strong emphasis will be placed on building partnerships and fostering co-learning among all members of the network.

## Participation

Members of QUEST will participate actively in monthly webinars lasting one hour each, and attend two in person meetings (1.5 days each). Webinars will be a combination of education, skill building, and facilitated discussions. Some webinars will be targeted to specific members (**Table 1**); however, all members of QUEST will be invited and encouraged to attend all webinars.

**Table 1: Webinar Topics and Expected Participants.**

Topic	# of Webinars	Required Participants
Introduction to Skype/Practice Session	2	patient partners
Intro to PCORI and QUEST	1	patient partners and stakeholders
Advocacy and Partnering with Professionals	1	patient partners and stakeholders
Effective Meetings and the 3 C's	1	patient partners and stakeholders
What is CER?	1	patient partners and stakeholders
Working with Patient Partners	1	stakeholders
Patient-Reported Outcomes	1	patient partners and stakeholders
Introduction to Research	1	patient partners and stakeholders

## Roles and Responsibilities

**Project Lead:** An individual who oversees the administration and activities of QUEST.

### Responsibilities

- Participate in all decision making for QUEST
- Assure compliance with all administrative requirements
- Assure project costs stay within budget
- Provide oversight and facilitate conflict resolution



**Lead Patient Partner:** An individual who represents the voice of patients on the leadership team.

Responsibilities

- Participate in all decision making for QUEST
- Provide mentorship and advocacy training to patient partners
- Assure that education, training, and facilitated discussions are inclusive and patient friendly
- Participate in the development of grant proposals for patient centered outcomes research

**Lead Clinician:** An individual who represents the voice of clinicians on the leadership team.

Responsibilities

- Participate in all decision making for QUEST
- Assure that education, training, and facilitated discussions are clinically relevant and accurate
- Participate in the development of grant proposals for patient centered outcomes research

**Patient Engagement Consultant:** An individual who provides expertise and leadership in overall project activities

Responsibilities

- Provide mentorship and expert advice to project staff
- Assure that education, training, and facilitated discussions are family-centered
- Assist in the development of grant proposals for patient centered outcomes research

**Project Coordinator:** An individual who provides day to day management and coordination of QUEST activities

Responsibilities

- Oversee the day to day business of QUEST
- Assure that project deliverables are met in timely fashion
- Participate in the development of grant proposals for patient centered outcomes research

**Patient Partner:** An individual with an IBEM, or an individual who cares for someone with an IBEM

Responsibilities

- Actively participates in webinars and in-person meetings
- Identify outcomes important to patients and caregivers
- Suggest and help develop ideas for patient centered outcomes research studies
- Participate in the development of grant proposals for patient centered outcomes research

**IBEM Provider Stakeholder:** An individual who provides care for persons with IBEM of uncertain clinical consequence, such as a physician or a nutritionist

Responsibilities

- Actively participate in webinars and in-person meeting
- Help identify gaps in current knowledge concerning management of IBEM
- Identify important clinical outcomes
- Participate in the development of grant proposals for patient centered outcomes research

**Public Health Stakeholder:** An individual who works in public health and has an interest in improving outcomes for IBEM of uncertain clinical consequence

Responsibilities

- Actively participate in webinars and in-person meeting
- Help identify gaps in current knowledge concerning management of IBEM
- Identify important public health outcomes
- Participate in the development of grant proposals for patient centered outcomes research

## Organizational Structure



**Organizational structure of QUEST.** Patient partners and other stakeholders reside at the center of QUEST. All work accomplished by QUEST will be guided by patient partners and other stakeholders. The leadership team provides critical direction and fosters co-learning among patient partners and other stakeholders.

### Decision Making

Decisions about administrative activities, such as scheduling webinars and meetings, will be made by project leadership with the input of patient partner and other stakeholder members. Every effort will be made to accommodate the schedules of all partners. Opportunities to listen to webinars and confer with project staff will be made available to QUEST members unable to attend learning opportunities at the scheduled times.

Decisions surrounding all other aspects of the project including recommendations of research topics, study designs, important outcomes, and dissemination plans will be made in collaboration with all partners.

### Changes in Governance

QUEST will operate as a living entity. As the group matures, it is possible that modifications to the governance document will be required. At any time during the project, any member of QUEST may point out a need or desire for a change in the governance document. At such time, the leadership team will arrange a group discussion around the proposed changes and whether or not they should be adopted.