



research *ready* SERIES

Research Ready: Developing a Strong PCOR Foundation in the Glut1 Deficiency Community

Background:

The Glut1 Deficiency Foundation has made significant strides in building a patient-led collaborative research network and identifying patient priorities for the most meaningful research for this rare disease; however, gaps and deficiencies remain. To take the next best steps in our efforts to continue building a strong, empowered, informed, and engaged research community, we need to better educate stakeholders on the principles of PCOR, the critical role patients play in the research process, and how they can best engage to ensure research is relevant and meaningful and more likely to improve outcomes. Patients and families clearly want and need easier, more effective treatments, so the community needs to more fully understand how best to center and focus our efforts and be able to compare these treatments against the current standard of care.

Proposed Solution:

We propose to create a model to continually assess and update priorities and needs, provide education to stakeholders on PCOR and CER principles, and enhance clinical trial readiness in hopes of accelerating progress for therapies.

Objectives:

This project aims to further refine priorities, identify needs, and educate stakeholders. The specific objectives will be to develop a model program to educate the community on the importance of PCOR to increase research literacy and clinical trial readiness; create a framework for ongoing assessment of priorities and needs to ensure research is patient-centered; and strengthen relationships and form new partnerships and collaborations across all stakeholders.

Activities/Methods:

To help meet these objectives, we will develop a PCOR educational module; host training opportunities in diverse settings; create a patient advisory board; and develop an ongoing system to capture patient needs and priorities.

Outcomes and Outputs:

Short term outcomes and outputs will include the educational program developed to train patients and families on PCOR; a patient engagement plan developed and refined to recruit participation; training sessions scheduled and held; a patient advisory board established; regular feedback analyzed and reported from the training sessions and used to update training curriculum; a project summary report created and informational resources created and disseminated in the community. Medium term outcomes will result in the most meaningful research outcomes identified based on patient priorities; increased natural history study participation; expanded biorepository samples available; and strengthened and expanded partnerships and collaborations. Long term outcomes and outputs include clinical trial and CER readiness with an educated and empowered community; clearly defined patient-centered outcomes; robust natural history data; expansive and diverse biorepository samples; continuously updated patient-led Research Compass; and a strong collaborative stakeholder network.

Patient and Stakeholder Engagement Plan:

Stakeholders include patients diagnosed with Glut1 Deficiency and their families and caregivers, clinicians who provide patient care, researchers who study to try to better understand this disease, and industry stakeholders who develop products and treatments. This project proposal was developed from engagement activities in focus groups, previous survey results, and representative stakeholder participation in the leadership team and advisory boards. Continued engagement will be achieved through the activities outlined throughout the project with frequent feedback collected and used to assess and improve participation.

Project Collaborators:

Collaborations will be created with local medical experts to participate in the regional in-person training sessions.