

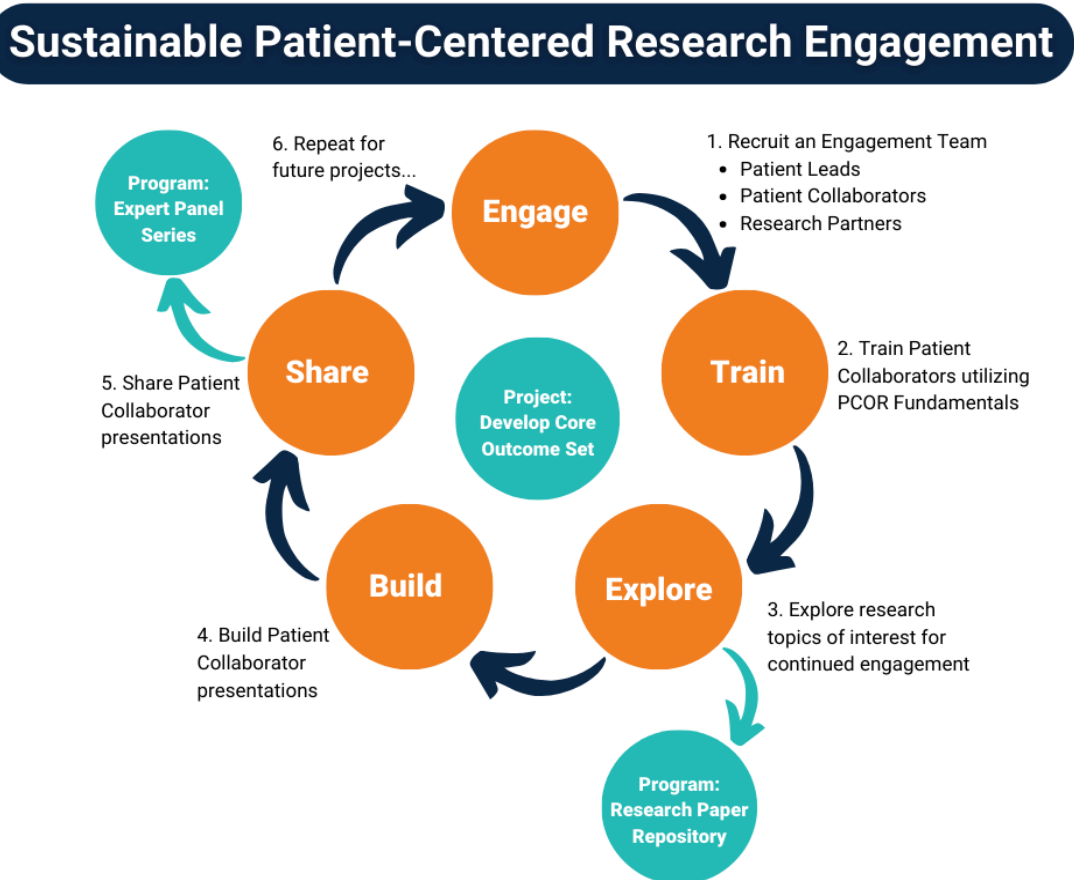
## Toolkit: Sustainable Patient-Centered Research Engagement

### Purpose

This toolkit was developed by the patient-led organization, Association for Creatine Deficiencies (ACD), and is based on learnings from the completion of a successful engagement award. It is intended to provide support to other rare disease organizations with a diverse disease community to create sustainable and effective patient engagement throughout the research process and in preparation for future research projects.

The toolkit contains an outline of six steps for continuous patient engagement as well as the projects tackled during this process as examples- both the project of intent (core outcome set development) and two projects that came about organically through patient engagement.

### Overview of the Process

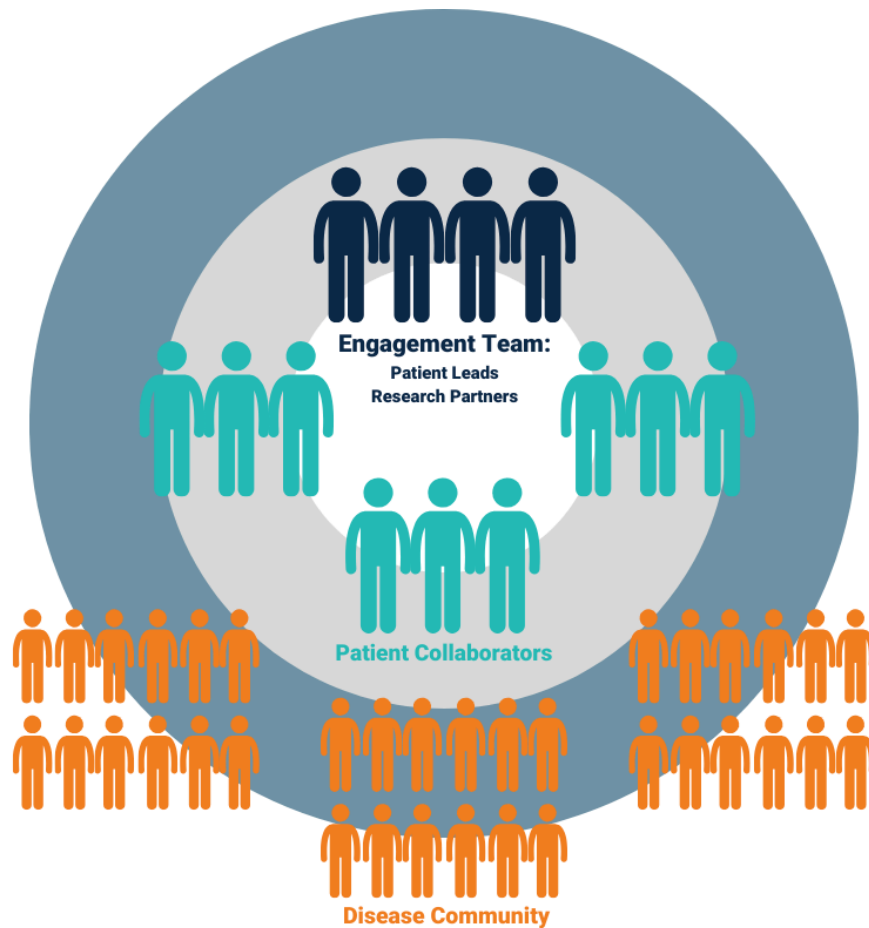


## Step 1: Engage

### *Recruit an Engagement Team*

- **Cultivate patient engagement in the Disease Community.** Positive relationships within the patient community are the cornerstone of successful recruitment for patient-centered research projects. Prior to identifying and engaging in a research project, a patient community can focus efforts toward engagement in many ways. ACD had a volunteer board member serving as the Director of Community Engagement for many years prior to receiving PCORI funding. This person reached out to each new Disease Community member as they found the organization and connected them with resources and other community members for support. As a result of these early grassroots efforts the creatine deficiency patient community is now a strong collection of caregivers. Open communications across various social media platforms, emails, virtual conferences, community events, and in-person symposiums that engage community members regularly keeps community members aware of projects and opportunities to engage. While many patient-led organizations are focused on the end goal of advancing towards research and cures, a focus on patient engagement ensures patient-centered research engagement is both possible and sustainable.
- **Identify a patient-meaningful project based on community needs.** Whether using a project template such as the examples below, developing a project from the ground up, or somewhere in-between, take care that the end result is truly meaningful to your community. In the case of ACD, a Patient Meaningful Outcomes survey was conducted and informed the initiation of the Core Outcome Set project. Another strong tool for engaging with the community to identify needs of the greatest importance is to host webinars and allow for questions and interactive discussions from participants.
- **Enlist Research Partners from the patient and scientific communities.** Identify members of both the patient and scientific communities who have related expertise, interest in advancing the project, and are willing to partner throughout- from proposal to publication. Examples may include patients, caregivers, researchers, or health care providers with backgrounds relevant to the project. These partners create a strong, vested Research Partners group that is the core of the team. Co-develop the finer details of the project together with the Research Partners to further ensure the patient-centered nature of the project.

- **Recruit Patient Collaborators from the patient community.** Share the details of the project in patient-friendly language, explaining the importance of patient collaboration throughout. Host webinars to discuss the project, allowing for questions and open discussion. Post a Patient Collaborator signup form that outlines the project, the commitment involved, and any available supports such as childcare or compensation for time to eliminate perceived and real barriers to participation. Review applications and make determinations on Patient Collaborators.
- **Enlist Patient Leads from the Patient Collaborators group to work with Research Partners in the Engagement Team.** Invite Patient Collaborators to apply for the role of Patient Lead. This leadership role is a bigger commitment and allows for more involvement in the research project. It also encourages more patient input in the development of the project. Patient Leads, together with the Research Partners, form the Engagement Team and may receive increased compensation due to increased effort.



## Step 2: Train

- **Train Patient Collaborators on PCOR and the research project in monthly workshops.** Train Patient Collaborators on PCOR fundamentals. Utilize the Research Partners to train in their areas of expertise. Schedule monthly capacity building workshops for this purpose. Allow a choice of at least two times to remove barriers such as time zone, work schedules, and patient care commitments. Schedule these workshops with as much advance notice as possible to increase attendance.

*"Having a choice of times to participate in meetings was very helpful for international participants." – ACD Patient Collaborator*

- **Gather input for continued improvement.** Creating a workshop agenda that allows for discussion and feedback is the first step to engage participants in the project. Survey Patient Collaborators and Leads to identify what is working and what is not. Continue to refine workshops to meet the needs of the participants.

## Step 3: Explore

- **Explore research topics of interest to guide your project.** Ask Patient Collaborators what areas of research they want to learn more about. This may be because a topic is interesting or because it is difficult to understand and is a barrier to the Patient Collaborator engaging with researchers. In ACD's COS project, the Patient Collaborators shared that they were intimidated and overwhelmed, especially at the time of the patient's diagnosis, when reviewing research papers. The Research Paper Repository Project was developed in response to this feedback and now hosts more than 40 family friendly summaries of the top research articles relevant to this Disease Community.

*"[Patient Collaborators] know so much! Their ability to synthesize and comment on the scientific issues was amazing." – ACD Research Partner*

## Step 4: Build Patient Collaborator Presentations

- **Prepare interview questions and presentation templates relevant to the research project.** Patient Leads and Patient Collaborators work together with the Research Team to create interview questions that will support the research project. These questions are the framework of Patient Collaborator presentations. They will vary depending on the focus of the research project and what the Research Team hopes to learn. In the case of the COS project, ACD interviews were focused on patient symptoms and outcomes important to caregivers of those with a creatine deficiency.

- **Interview Patient Collaborators.** Patient Leads meet individually with each Patient Collaborator to conduct an interview and take notes.
- **Prepare presentations.** Patient Leads create a presentation with the information from the Patient Collaborator's interview.
- **Practice sessions.** Patient Lead and Patient Collaborator go over the prepared presentation and practice it a few times. The purpose of Patient Collaborator presentations is not only to contribute to the overall research project, but also to help enable participants to engage with researchers and share their stories.

#### **Step 5: Share Family Collaborator Presentations**

- **Share presentations in monthly workshops; allow for discussion.** These presentations can often be emotional, both for the presenter and the other workshop attendees. In the COS project, workshops were 90 minutes and included three 20 minute presentations and 30 minutes for questions and discussion. Many times participants stayed in the meeting beyond the 90 minutes to continue discussions.
- **Use presentations to unite the community and elevate the patient voice.** In the COS project, Patient Collaborator presentations were de-identified and used in multi-stakeholder Expert Panel meetings where Disease Community issues were discussed. This created ongoing content for this meeting, increased awareness of symptoms often faced by patients, and fostered discussions around the specific disease and patient perspectives.

*"Hearing the case presentations was one of my favorite parts - all heartwarming, heartbreaking, sometimes funny. Always made me think." – ACD Patient Collaborator*

#### **Step 6: Repeat Future Iterations**

- **Gather feedback at the end of the project with an eye towards re-implementation.** Survey project participants to learn what worked and plan to reuse successful tools.

*"Having the caretaker perspective, and the ability to collaborate with other key perspectives allows me to gain a more all around approach to understanding the disease, the research, and the impact it all has on our entire community." – ACD Patient Collaborator*

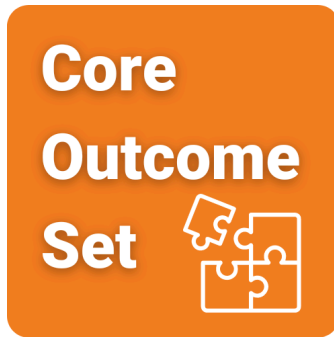


## BUILDING STRENGTH changing lives

- **Utilize an engaged and experienced community for success.** When starting a second project, engage previous Family Collaborators as mentors for their expertise. They will be strong allies to engage and support the next generation of Patient Collaborators.

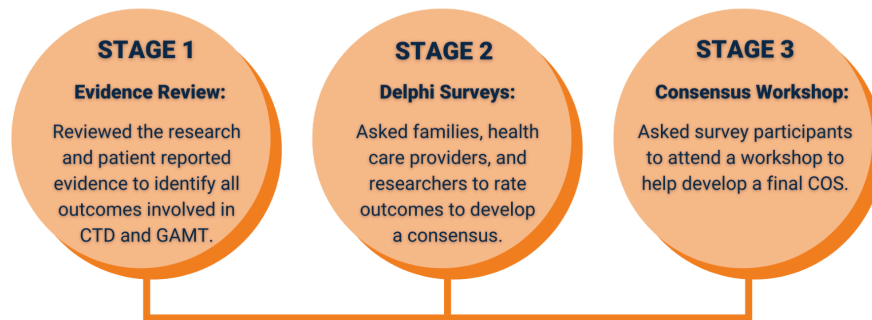
*"This project brought together families, doctors, researchers, and caregivers to work together on a task. Not only are the results of the work outstanding, it provided collaboration between the members across the globe on a regular basis." – ACD Patient Collaborator*

## ACD Patient-Centered Research Program Examples



**Developing a Core Outcome Set for CTD and GAMT Deficiency** – A patient-centered core outcome set (COS) is a critical step to ensuring clinical trials are patient focused and relevant. A COS is a small set of important outcomes recommended for measurement in all research studies for a disease. The purpose of a COS is to encourage consistency, patient relevance, and facilitate better comparison between studies. A patient-centered COS should include the outcomes that are most important to patients and caregivers. Our COS project included active partnership between patients and health professionals.

The stages of this project are detailed below.



**Stage 1 – Evidence Review:** Using our “Sustainable Patient-Centered Research Engagement” plan, Patient Collaborators were recruited and prepared to participate in this process. Parent Leads were also recruited. Potential outcomes for consideration were gathered from:

- Patient Collaborator focus groups exploring outcomes of importance
- Literature review by Research Team
- Community Meaningful Outcomes survey review

The Patient Collaborators reviewed outcome definitions to ensure family-friendly language was used making this process accessible to patients.

**Stage 2 – Delphi Surveys:** A 3-round Delphi survey was completed by multiple stakeholder groups in the disease community to narrow the list of potential outcomes. The survey and recruitment materials to participate were available in multiple languages. Patients were encouraged to invite their clinician to participate.

**Stage 3 – Consensus Workshop:** An equal number of Patient Collaborators and Research Partners met to discuss the narrowed list of potential outcomes. Patient Collaborators spent two years preparing for this meeting, were well-prepared, and consensus was easily reached on a final set of 8 core outcomes.

*"Participating in this consensus workshop allowed me to feel my opinion as a caregiver is valued [and] feel like we are working as a team with clinicians". – ACD Patient Collaborator*

*"Being able to share my thoughts regarding where goals may be measured was beneficial. Real world examples were used as part of the core outcomes and these came from myself and other parents." – ACD Patient Collaborator*



**Research Repository** – During the “Explore” stage of ACD’s project, Patient Collaborators identified access to understandable research papers as a barrier to engaging in research, expressing that this was especially overwhelming right after diagnosis when trying to “learn everything”. Discussions led to the creation of a *Research Paper Repository* with family friendly summaries of essential research papers. This resource is now shared with new community members, lessening the challenge to engaging in creatine deficiency research.



**Expert Panel Series** – The CCDS Expert Panel meeting is a platform to engage Patient Collaborators and CCDS “experts”. Discussions are centered around anonymized case narratives from Patient Collaborator interviews. Experts are asked to discuss the topics of most concern to the Patient Collaborator in an open forum with peers. This platform proved to be an excellent recruitment tool by providing a forum for fellow CCDS experts to learn from colleagues as well as a meeting for experts outside of the creatine deficiency group to engage in our community on various areas of concern raised such as- speech therapists, dieticians, gastroenterologists, child psychologists and other fields. Rich patient-focused discussions raised awareness of support needs, and created an ongoing platform to continue these discussions.