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DRAVET SYNDROME FOUNDATION

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Introduction

INTRODUCTION

A rare disease by definition is a disease that affects fewer than 200,000 people in the U.S. There are over 7,000 rare diseases, affecting over 30 million Americans, or 1 in 10 people. As a result, rare disease communities face unique challenges when it comes to research. Patients are difficult to locate and recruit. Clinical trials must be kept small for any hope of meeting the enrollment requirements. Funds are typically scarce for research on conditions with such a small patient population. There is a lack of understanding of patient and caregiver concerns, and existing literature on these concerns is often non-existent. Another major barrier can be the lack of investigators working on a particular rare disease.

A crucial component for rare disease populations is the establishment of strong connections between all of the stakeholders in its community - patients, caregivers, medical providers, researchers, and industry partners - particularly since rare disease research relies heavily on the patient experience and the medical professionals who care for them. By establishing an open and ongoing dialogue among community stakeholders, along with a mutual respect and understanding of what all the participants hope to achieve, optimal results can be accomplished.

One of the challenges for all rare disease patients, including those with Dravet syndrome (DS) is finding the best way to treat and manage their disease. For many of our patients, treatments are partially effective at best, and for others there are no effective treatments at all. Being a part of a connected and educated community can change that. Dravet Syndrome Foundation (DSF) established a system that allows our patient families to have greater input and an understanding of treatments, as well as the opportunity to be recognized as equal partners throughout the drug development process, and this is a key component in developing new treatments and better outcomes.

Early in the development of DSF we could see that community connections, strategic alliances, and partnerships would be critical. By growing and nurturing these relationships, we were able to not only produce meaning and value but we were able to do it in an effective and efficient manner with the limited resources we had. By offering the structural capacity needed to address specific problems and issues we are able to also ensure long-term engagement and community empowerment.

As part of a strategic work plan that was made possible through a Patient-Centered Outcomes Research Institute (PCORI) Eugene Washington PCORI Engagement Award (7881-DSF), DSF has developed this *Best Practice Paradigm for Expanding Engagement* to assist other rare diseases to help them organize and prepare their patient communities for PCOR. Through our time with PCORI, we have learned several valuable lessons including the fact that an organized, easily-reached stakeholder community is vital to any project requiring patient engagement and that stakeholders need to receive information in several different formats at more than one time to understand the process.

Key Pillars to Prepare Your Community for PCOR

KEY PILLARS TO PREPARE YOUR COMMUNITY FOR PATIENT-CENTERED OUTCOMES RESEARCH

As an advocacy organization, DSF has worked diligently to build a strong sense of community and a culture of inclusion so that our stakeholders have a feeling of belonging and are invested in our mission.

Through community feedback we were able to identify barriers and the gaps in understanding around patient-centered outcomes research (PCOR) and the importance of the patient voice. This feedback allowed us to develop tools and programs to better engage and educate our community.

This has been an evolutionary process that has required a multi-prong approach in order to reach our stakeholders in whatever way works best for them. Through our work, we identified six key pillars that we found useful in bringing the concept of PCOR to our community in a systematic and intuitive approach.





Connecting, Educating and Engaging your Rare Disease Community

CONNECTING, EDUCATING, AND ENGAGING YOUR RARE DISEASE PATIENT COMMUNITY

A movement to include the patient voice in research and decision-making has grown in the last decade, thanks in great part to the work of patients, community stakeholders, advocacy groups and nonprofit organizations such as the Patient Centered Outcomes Research Institute (PCORI). It is these community stakeholders and organizations who recognize and have shown that the patient voice is essential in all areas of research and therapy development. While there is no one-size-fits-all approach, this paradigm has been developed to help you develop, organize, and prepare your community for Patient Centered Outcomes Research (PCOR).

Your first step should be to investigate whether a patient group or network already exists in your disease state. If multiple organizations or groups exist, collaboration should be the top priority. By identifying different groups in your disease state you have the opportunity to see what each of them offer and can find the remaining areas of need and the best ways to collaborate. For instance, one group may be focused on funding research and another focused on patient advocacy. With limited time and resources, it makes sense to work together in order to avoid a duplication of efforts. If there is not an existing group for your disease state, you may want to start one. If you need to start a group, there are several approaches to take:

- **Forming an in-person support group:** This can often be accomplished under the umbrella of a broader disease group. For example, if you or your patient has a rare form of a not-as-rare disease such as epilepsy, you may want to set up a disorder-specific support group utilizing the meeting space, email list, or other resources from your local Epilepsy Foundation. Otherwise, local libraries often offer free meeting space.
- **Forming a virtual support group:** Many rare disease nonprofits have their start as a Facebook or other online platform support group, since their patient community is scattered across the world. If there is not an existing group for your disease, you may want to consider starting one. For many groups, it may be one of the strongest building blocks in your development and a primary way to interact with your patient community. Members are able to offer peer-to-peer emotional support and connect with others who understand their medical journey. It is important to assure that the group is a safe space for members where they can find support and accurate disease information and resources. You will need to establish a clear definition of this virtual community and its purpose; establish & enforce group rules; use engaged moderators; offer tools that educate members and encourage interaction; and most importantly assure that the group is a safe space that allows open and honest communication, is free of harassment, and offers emotional support and practical information. (During the COVID-19 pandemic, Zoom offers free personal accounts and waives the 40 minute time limit on free accounts to encourage virtual support.)

- **Connect with other disease groups:** There may be a larger patient group or umbrella organization that includes your rare disease and other related or similar diseases. These groups may have more resources available, such as industry, government, and research connections. Working with these larger groups when possible may allow you access to their resources, as well as their experience and expertise.
- **Forming a nonprofit organization:** Starting and sustaining a nonprofit is a significant undertaking that involves a great deal of work to establish and maintain. When you apply to the IRS for nonprofit status, you will need to have a concrete plan in place to present, including a description of your charity, the services you will provide, and who these services will assist and support. You will be required to include an estimated budget with your anticipated income and expenses. You will also need to establish a board of directors with relevant experience and skill, such as an attorney, marketing expert, or someone with deep knowledge of the community you intend to serve. Once approved, there are a number of legal filings, both on a federal and state level, that will need to be done on a regular basis to maintain your status as a nonprofit organization. There are many guidelines for establishing a nonprofit online, including <https://www.usa.gov/start-nonprofit> and <https://www.councilofnonprofits.org/tools-resources/how-start-nonprofit>.

Regardless of how you build your patient community, once it is established it is important to build a strong sense of community and a feeling of belonging, as well as educating your community on how their involvement strengthens your work. Patients want the opportunity to advocate and advance treatments and they want accurate information on promising compounds and clinical trials. Building an educated and connected community is a several-step process that requires a multi-prong approach to reach individuals in whatever way appeals to them. It is useful to offer education and resources in a variety of formats to reach patients where they are in their disease journey. The development of programs and advocacy tools are often based on the resources available at that stage of organizational growth and what are identified as the top needs of your community. Needs will vary from group to group, but some of the programs and tools that may be useful include:

- **Website:** For many groups, their first contact with a patient family is often through their website or social media page. These tools allow you the opportunity to share educational information and meaningful updates with your community members. It is important to regularly update these sites to make sure that they contain the most current and accurate information available.
- **Social media, emails & newsletters:** These offer ways to communicate with your community to offer updates on things such as regional events, fundraisers, current research, clinical trials, and other information that is important to your community. Trial and error will show you what social media channels hold the most appeal for your community. Content should be authentic and reliable. Strive for the right balance of messaging, both in terms of content and frequency.
- **Conferences or Workshops:** These in-person educational events offer the unique opportunity for community stakeholders to connect face-to-face and participate in educational sessions and interactive discussions. They are also an opportunity to directly engage and educate your community members so they better understand the drug development process, as well as the importance of patient centered research.

- **Live and on-demand educational webinars:** Available online, live educational webinars offer an opportunity for interaction and education with your patient community. It also gives participants an educational opportunity and a chance to ask questions on their top concerns, regardless of where they are physically located. By also recording and offering webinars on demand you can accommodate the busy schedules of your constituents who may be unable to join the webinar live.
- **Support groups, family networks, & parent ambassadors:** Rare disease communities, play an important role in supporting and educating patients and caregivers. These groups can help accelerate the time to diagnosis, educate on current treatments, offer practical tips on living with the disorder, and help patient families find experienced specialists. There is an opportunity to meet others with similar experiences and share openly about the disease and emotional struggles. Families often report feeling empowered and hopeful after connecting with the Dravet syndrome community, where they have the opportunity to advocate for their loved one while connecting with others who understand their medical journey

DSF facilitates these connections through the DSF Family Network <https://www.dravetfoundation.org/family-network-main-page/>. The Network offers a variety of initiatives designed to connect and strengthen families ties to the community, including online support groups, parent ambassadors, and regional in-person workshops.

- **Patient registries:** There is frequently a lack of outcome data for rare diseases. Registries allow the opportunity to document symptoms and interventions, which can lead to an improved understanding of the disease. This data collection can also lead to development of new therapies. Registries can come in a variety of forms, depending on how and why it is being used. For instance, from a patient advocacy perspective it is important to collect patient-reported outcome data on quality of life to assess and support the needs of patient families. From an academic perspective, natural history studies are essential to help understand the progression of the disease and drive future research. For industry, a registry can help gather information on secondary endpoints that are critical to the patient's quality of life and help in clinical study design.
- **Multi-stakeholder partnerships:** These shared partnerships are a vehicle through which interested community stakeholders can collaborate on specific challenges and/or explore opportunities to achieve a greater impact. Patients with rare diseases and their caregivers are often experts in their own condition. Rare disease communities have a unique connection which comes from patient support and advocacy efforts. A multi-stakeholder partnership offers the opportunity for engagement that contributes meaningfully to research and development.

Stakeholder partnerships can:

- ◆ Offer insights into disease progression and help define meaningful clinical endpoints and outcome measures.
- ◆ Offer input into clinical trial design and the informed consent process, including identification of barriers to participation.
- ◆ Convey what patients would look for in an ideal treatment based on their experiences with existing ones. For example, fewer or no side effects, less frequent administration, or different modes of administration.
- ◆ Help identify or develop tools that assess the benefit of potential therapies.
- ◆ Offer input on trial site selection.
- ◆ Assist in establishing and recruiting for patient advisory boards.
- ◆ Advocate during the regulatory review process.
- ◆ Increase awareness and educate the patient community on trial and locations.
- ◆ Play a key role in ensuring access, defining value, and informing disease management and adherence programs.

To remain successful and sustainable, partnerships should:

- ◆ Establish roles, structure, and procedures to assure an understanding of the project/commitment and assure long-term engagement.
 - ◆ Recruit individuals committed to the problem and build trust through leadership.
 - ◆ Cultivate open and frequent communication to create a positive climate of mutual respect.
 - ◆ Recognize and respect diversity among stakeholders.
 - ◆ Assure there is fiscal sponsorship/support.
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- **Web-based advocacy tools:** It is helpful to have a variety of methods to disseminate information for your disease community so that individuals can find and utilize whatever methods of delivery work best for them. Suggestions on tools that can help you unite and engage with your rare disease community include:
 - ◆ Disease brochures
 - ◆ Downloadable educational materials (infographics, checklists, etc.)
 - ◆ Resource links to programs and services
 - ◆ Patient and Caregiver surveys
 - ◆ Archived webinars / educational sessions

Identifying and Involving Other Stakeholders



IDENTIFYING AND INVOLVING OTHER KEY STAKEHOLDERS

You should establish and nurture relationships with all of the stakeholders in your community. In addition to patient families, these may include researchers, healthcare providers, industry members, governmental agencies, payers, and possibly other patient groups. Those that want to be involved will understand the importance of including the patient's voice in determining the needs of the community and the opportunity to enhance patient care. By forming these partnerships, a community is better able to address critical challenges and gaps in care.

- **Identifying researchers and clinicians:** Many rare disease groups will seek out researchers and clinicians who are focused on their disease or related diseases for guidance to form a scientific or medical advisory board to help guide their group priorities and objectives. For some groups, finding a few interested researchers and clinicians is not that difficult, but for many, especially rare disease groups, it is not only a very challenging first step, but a continuous challenge. If you are unsure where to start, some suggestions are:
 - ◆ Online: PubMed offers a searchable database of published medical articles. You can search for your disease state to find researchers working in the field who have published their research. [Clinicaltrials.gov](https://clinicaltrials.gov) will allow you to search for current clinical trials. Contact information is usually included for the Primary Investigator of the trial, which will help you identify researchers working in your disease state.
 - ◆ Personal Connections: If there are other groups or nonprofits in your disease state, speak with them about who are the experts in the field. You might also survey your patient community to learn who they see for treatment.
 - ◆ Medical centers: Search the department focused on your disease or related diseases.
 - ◆ Medical conferences: Exhibiting at medical conferences can raise awareness of your efforts among specialists and may also attract medical residents or fellows just starting out in this specialty.
 - ◆ Offer a research grant program with specific areas of focus to help you better understand your disease. Or fund young investigators to help develop researchers in your disease state.
- **Identifying other potential stakeholders:** Consider recruiting other stakeholders that can offer a different and unique perspective to community discussions. These might include:
 - ◆ Payers / insurance representatives
 - ◆ Policy makers / regulators
 - ◆ Other healthcare professionals (such as nurses, therapists, psychologists, etc.)
 - ◆ Industry members
 - ◆ Patient advocacy group leaders

- **Identifying and working with Industry:** By establishing relationships with industry, you can help drive a focus on therapy development for your disease state and associated comorbidities. When a company is evaluating whether to pursue a new treatment, it is in their best interest to collaborate with the disease patient community to assure the patient voice is at the center of the drug development process. There is a benefit to the company to involve the patient voice in all stages of development to make certain that potential therapies will address the needs of your disease community and be readily adopted. By taking a connected, data-driven, patient-focused approach from the beginning, you can alleviate some of the challenges and barriers industry faces and allow them to accelerate the development of new therapies that will offer a better quality of life for patients, based on what patients identify as their primary needs.
- **Working with governmental agencies:** Governmental agencies, such as the Food and Drug Administration (FDA), the National Institutes of Health (NIH) and the National Center for Advancing Translational Sciences (NCATS) recognize that patient perspectives are vital in the development of new treatments. Patients and caregivers can share the challenges of living with a disease that might not be easily recognized by researchers or clinicians. There are multiple opportunities to connect with the FDA in meaningful ways for your disease state. Their website offers extensive information for patients on patient engagement, treatments, drug and device approval, clinical trials, and more. <https://www.fda.gov/patients>
 - ◆ The FDA Office of Orphan Products Development (OOPD) may be available to help with the development of therapies for rare diseases. The mission of the OOPD is to advance the evaluation and development of products (drugs, biologics, devices, or medical foods) that demonstrate promise for the diagnosis and/or treatment of rare diseases or conditions. <https://www.fda.gov/about-fda/office-clinical-policy-and-programs/office-orphan-products-development>
 - ◆ The Center for Drug Evaluation and Research (CDER) Patient Focused Drug Development programs have been designed to better incorporate the patient voice in drug development, which the FDA recognizes as critical when regulatory decisions are being made for new drugs.. (<https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development>)
 - ◆ One of the programs of CDER is the Patient-Focused Drug Development (PFDD) initiative. As stated on the FDA website, this program aims to more systematically obtain the patient perspective on specific diseases and their treatments. PFDD meetings give the FDA and other key stakeholders, including medical product developers, health care providers, federal partners, an important opportunity to hear directly from patients, their families, caregivers, and patient advocates about the symptoms that matter most to them, the impact the disease has on patients' daily lives, and patients' experiences with currently available treatments. The FDA also welcomes patient organizations to apply to organize an externally-led PFDD meeting on their disease state. This offers the opportunity to stand in front of decision makers such as regulators and pharma representatives to share the burdens and challenges their disease community faces daily. (<https://www.fda.gov/industry/prescription-drug-user-fee-amendments/externally-led-patient-focused-drug-development-meetings>)

- ◆ Identify the NIH Institute that may be focused on research related to your disease. Building a relationship with that institute may allow you the opportunity to share the patient voice in any applicable grants, workshops, or other opportunities. You may also want to explore the offerings of NCATs, one of 27 Institutes and Centers at the NIH, with a focus on transforming and accelerating the translational research process. They collaborate with researchers, the public, and other stakeholder groups to design new approaches and technologies that will deliver more treatments, more quickly. (<https://ncats.nih.gov/>) They also offer a toolkit for patient-focused therapy development (<https://ncats.nih.gov/toolkit>)

Training and Engaging Your Stakeholders

TRAINING AND ENGAGING YOUR STAKEHOLDERS

Effective engagement requires a collaborative approach, open and honest communication, and proper training. A fundamental part of your strategy in training and engaging your stakeholders is building trust. Stakeholders need to know what is happening and what the impact will be for them. You need to give them space to participate and allow for shared decision-making.

Stakeholder partners will also need to understand the research process, as well as what patient centered research is, and the value of engagement. PCORI offers a variety of tools on their website to assist with this educational process.

Finally, it is important that stakeholders can see that their time and contributions are valued. Reasonable time commitment requests should be made, and partners should receive fair financial compensation for their participation.

Some guiding interactions that we have learned from our PCORI projects:

- Start at the beginning - Prioritize concerns first, and hone in on top patient concerns. Only then can you think about the end (outcome measures, design, participation).
- It's not just the patients that need training in PCOR: clinicians and researchers often need re-training to include patients in meaningful ways.
- Sometimes the education is for you - *patients have a way of making you rethink your well thought out approach.*
- Think about what is driving each stakeholder, and appeal to their needs to build engagement. (Industry = patients for meaningful trials, Patients = better treatments, Clinicians = better care, Researchers = publishable studies).

Addressing Patient Participation Barriers

ADDRESSING PATIENT PARTICIPATION BARRIERS

For your project to be successful, it is important to clearly define the goals of your engagement efforts to mobilize your community. You will need to have commitment, leadership, and vision, as well as sufficient organizational capacity.

You will also need to be aware of the various cultures of a community and other factors that may affect patient participation. Barriers to participation in engagement efforts may be related to economics, education, employment, health, culture, language, race, age, gender, ethnicity, literacy, or personal interests. Diversity must be paramount in your planning and approach to engaging your community and will require multiple engagement strategies. Some of the aspects to consider when planning to engage your stakeholders include:

- Connecting patients in lower income communities without technology access - consider in-person meetings or audio conferencing if your community is spread out geographically, rather than video conferencing.
- Language barriers - consider adding a stakeholder group member who is bilingual or bring in a translator to allow full participation.
- Resources - always offer compensation to your stakeholders for their time and participation.
- Childcare - consider offering childcare at in-person meetings or a stipend to offset childcare costs in the home so that the stakeholder can participate uninterrupted during meetings.
- Understand the possibility of frequent cancelations - building the into the event/program structure. Also consider having backup stakeholders when patients or caregivers have to step away due to life circumstances.

Identification of New Treatments

IDENTIFICATION OF NEW TREATMENTS

While you are building your community and engaging stakeholders, you can also be simultaneously on the lookout for potential opportunities for studies or the identification of new treatments. Research in rare disease etiology, mechanisms, and treatment approaches can advance progress in other rare diseases, as well as in more common diseases. While the concerns you identify will be specific to your community, some studies or treatments for another rare disease may cross over to the needs of your community as well.

- Always keep your PCOR hat on: When talking to people, think about how you could take their idea and make sure patients are included in meaningful ways. Could the project have a broader impact if it were re-designed to meet PCOR requirements?
- Focus on the new treatments that address concerns your community prioritized. Your focus and support will encourage other researchers, even those not involved in PCOR, to follow suit.
- Continually look for ways to describe, rate, and measure patient priority areas. Existing validated measurement tools may not always be relevant to your disease state.
- It benefits industry to include the patient voice throughout the drug development process, so make sure they have data that supports what you have established as the top priorities of your patient community. Examples include a caregiver survey, patient registry, or white paper.

Disseminating Information

DISSEMINATING INFORMATION

The best way to keep patients, caregivers, and other stakeholders engaged long-term is to offer frequent updates and transparency on how their participation is making an impact. In particular, these updates allow patient families to feel less isolated, better informed, and more engaged in their healthcare outcomes. In addition to in-person events, thanks to the internet and technology there are now many tools available for virtual interactions that will allow for information sharing. Some of the tools we have found to be useful to keep our community informed are:

- In-person conferences or workshops
- Video meeting platforms (such as Zoom or Facebook Live) that allow for a live virtual update and a question and answer session
- Website updates, eblasts, social media posts, and blog entries
- Stakeholder meeting transcriptions
- White papers
- Infographics
- Recorded webinars for patient and/or healthcare provider education



CONCLUSION

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While there is a great deal of work involved, the advantage to connecting and engaging your community includes the meaningful participation of patients and the interaction among diverse stakeholders who might not otherwise work together. This structured engagement can yield rich contributions with the potential to effectively generate research topics that reflect patient priorities and optimize healthcare outcomes.

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Questions on this paradigm may be directed to Mary Anne Meskis, Executive Director:

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