

ISSUES 2024THINK TANK: CREATING BETTER RARE DISEASE PATIENT SUPPORT PROGRAMS

2024 FEATURE ARTICLES JUNE PATIENT

THINK TANK: Creating Better Rare Disease Patient Support Programs

 BY
PM360 STAFF
JUNE 24, 2024

Facebook

Twitter

0
Shares



When a person is facing their own unique challenges and experiences that come with living with a rare disease, it is more important than ever that they find support from others who understand their disease and what they are going through to seek treatment. Patient support programs (PSPs) are meant to do just that. However, there is always room for improvement to empower those dealing with a rare disease to manage their own health and find the answers they need from others in their community. PM360 reached out to ten specialists who shared their own ideas on how PSPs can make difference in the lives of those who need it most.

We asked:

- What unique considerations should be taken into account when designing patient support programs (PSPs) for niche populations with rare diseases?
- How can we overcome barriers related to access, affordability, and coordination of care when it comes to rare disease populations?

- How can we enhance patient engagement in rare disease support programs? What strategies can be employed to ensure that patients actively participate, share their experiences, and contribute to program improvement?
- How can we improve access to information, educational initiatives, outreach programs, or PSPs to empower rare disease patients?

Think Tank Rare Disease Patient Support



Ed Park
Vice President of Strategy
Veeva Compass
ed.park@veeva.com

Patient support programs (PSPs) are core to improving patient outcomes, well-being, and treatment access, especially for those with rare diseases. Yet, reaching and engaging such niche patient populations and the healthcare professionals (HCPs) who service them can be a challenge. Success lies in designing PSPs centered around the patient and HCPs' treatment patterns to help reach the right patient and make a difference in their moment of need.

It all starts with deeply understanding the patient journey. How many touchpoints does the patient have with the healthcare system and which stakeholders do they interact with the most?

Anonymized patient longitudinal data is useful in mapping this, particularly in rare diseases where all aspects of the patient journey can make a difference in diagnosis and treatment, such as comorbidities, specialties, and co-therapies. With more complete data, companies can focus on HCP engagement, uncovering which HCPs meet with specific patients and in which regions, to better understand treatment patterns and tailor PSPs with the most accurate insight.

Measuring the impact of PSPs is just as important to continue delivering the best outcome for patients.

Reporting metrics based on daily patient data can help quickly measure and monitor impact to optimize future programs. For example, a biopharma may want to track the improvement in patient adherence to a therapy pre- and post-launch of a PSP across regions to gauge its impact. Ultimately, anonymized patient longitudinal data provides valuable insight into the patient's journey and pain points, helping build more effective PSPs that transform outcomes in rare diseases.



Ed Hart
Head of Design
Graphite Digital
edward.hart@graphitedigital.com

At the heart of empathetic design lies a deep understanding of the patient journey. For individuals with rare diseases, this journey is often marked by misdiagnoses, uncertainty, and a sense of isolation.

Involving patients in the design and development of support programs is crucial. People living with rare diseases face unique challenges and experiences that you can only understand by speaking to them first-hand and listening carefully to what they tell you.

Conduct diary studies or patient interviews to collect qualitative information about their experiences. Doing so before embarking on creating a product or program will allow you to highlight underserved needs.

Resist the temptation to focus solely on interactions with your treatment or organization. Explore the interactions that take place around it as well. By attempting to understand everything—their daily schedules, physical capabilities, interpersonal relationships, mental health, perceptions of HCPs, treatment pathways—you can begin to identify core opportunity areas where you can have the greatest impact. The intersection of the medical, social, and emotional journeys will highlight your focus areas.

This research can help build trust and credibility with rare disease patients, who may understandably be skeptical of healthcare systems and providers. By engaging in a transparent and collaborative research process, organizations can demonstrate their commitment to improving the lives of rare disease patients and rebuilding trust with this population, improving patient engagement, and uptake and adoption of support programs.

Involving patients in the design process ensures that their patient support programs are relevant, usable, accessible, and effective—putting patients at the center of care delivery.



Stephen Hall
Associate Director
Valid Insight, part of the Bioscript Group
stephen.hall@bioscriptgroup.com

With increasing economic pressures on healthcare systems, the capacity to afford new treatments continues to diminish. Therefore, demonstrating a product's value proposition is more important than ever. Barriers surrounding access and affordability are even greater for rare disease treatments, which tend to be higher-cost medicines backed by less data to support clinical efficacy. This is reflected in a survey by the National Organization for Rare Disorders, which showed that 61% of rare disease patients have been denied access or have faced delays in accessing treatment.¹

PSPs can help patient outcomes through better education, increased treatment adherence, and enhancements[FW1] in humanistic outcomes such as quality of life measures.^{2,3} A well-designed PSP provides data to support the improvement of patient care and demonstrates the value of a product beyond the standard efficacy and safety measures. Research demonstrates that PSPs are considered an important differentiator by payers; however, they need to be designed with genuine consideration for the patient: Developing a PSP that truly focuses on patient needs is a winwin for all involved. As rare disease patient populations are small, partnering with scientific communications and market access consultancies can help develop the specific language and communications required to engage these patients, who are often very well informed about their condition.

Better treatment adherence can reduce payer uncertainty, enhancing the value proposition for the product and providing differentiation and additional value for the pharmaceutical company. At the same time, enhanced provision of education and closer follow-up empower patients to manage their condition better and ultimately result in improved patient outcomes.



Suzy Adams
VP, Experience Strategy
Saatchi & Saatchi Wellness
suzy.adams@saatchiwellness.com

Access, affordability, and coordination of care are the most significant barriers for rare disease populations. Let's break down how to approach each challenge:

Access: We need to raise awareness of these often-invisible diseases.

Advocacy is key: By pushing for policies that improve access, we can bridge the gap between patients and the treatment they need. This requires a multi-pronged approach targeting benefits managers, insurance companies, and Pharmacy & Therapeutics (P&T) committees. Partnering with patient advocacy groups can help present real-life patient stories to highlight the human cost of delayed access, in addition to data on the economic impact of rare disease treatments (compared to untreated cases).

Affordability: The financial burden of rare diseases is immense. PSPs can play a crucial role here. Highlighting financial assistance programs, providing copay solutions, and collaborating with patient advocacy groups to explore additional resources can all make a significant difference. However, transparency is essential. Patients need clear information on what support is available and how to access it. Additionally, ensuring healthcare providers are aware of and can easily enroll patients in manufacturer cost-saving programs may be vital to patient affordability of treatment.

Coordination: Imagine a patient navigating a maze to get care. That's what it can feel like for those with rare diseases. Our programs can be the map that guides them. A system that connects patients with their entire care team—doctors, specialists, therapists—ensures everyone is on the same page. A communication platform that facilitates information exchange can streamline the entire process.

Through collaboration with patient advocacy groups, healthcare providers, and policymakers, we can dismantle these barriers and empower patients and caregivers to manage their conditions more successfully.



Shikha Singhal
Principle, Data Science
Axtria
shikha.singhal@axtria.com

We can now leverage AI and Generative AI technologies to significantly enhance patient engagement and retention in rare disease support programs. These technologies can help us create a patient centric ecosystem for holistic disease management. This ecosystem comprises multiple components:

- An empathetic disease coach that educates patients with just-in-time and on-demand information to manage their condition. The “coach” provides real-time personalized support to facilitate lifestyle changes and ensure increased adherence and persistence.
- An attractive rewards system that allocates reward points for participation and engagement. The patient could redeem these points for rewards such as free or discounted medical supplies or lower copay for a specified period.
- Most rare diseases have a substantial socio-emotional impact on the patients. Hence, designing a program that includes benefits such as therapist-on-demand, dieticianon-call, etc., is essential.
- A digital community where patients and their caregivers can share their war stories to inspire each other.

This ecosystem needs to rest on a solid data foundation for continuous measurement and enhancement, ensuring that the program stays relevant to the patients by incorporating their feedback. Programs like this involve creating a layered data architecture

consisting of a raw data layer, followed by analysis-ready data (such as patient 360 master), report-ready data for quick reports on relevant matrices, and generative AI-ready data to process unstructured datasets, like patient feedback, sentiments, conversations, etc.



Jennifer Stephenson
VP of Client Solutions
VMS BioMarketing
jennifer.stephenson@vmsbiomarketing.com

People with rare diseases are known to conduct extensive research about their symptoms, treatments, and struggles—often long before they are diagnosed or engaged in the pharma company’s patient support program. They’ve researched medical journals, subscribed to podcasts, and possibly are active in advocacy organizations. On average, they’ve visited seven physicians before getting a diagnosis.

Because the rare disease patient is typically so knowledgeable at the point they are referred to the pharma company’s PSP, it is helpful for the HCP to engage the patient in education to avoid starting the conversation with a long checklist or list of questions this patient has answered over and over. Clinical Nurse Educators or Nurse Navigators can better engage patients by starting the conversation by simply asking patients to tell their story, everything it took to get to this point, and what has been their own unique journey. Giving patients a voice to share their story in their own words helps establish a connection with the HCP to build a relationship and also uncovers the areas where the HCP must first target education and support.

Using behavioral health methodology such as motivational interviewing, the nurse educator should continue to provide education with a fundamental attitude of acceptance and empathy toward the patient in a partnership-like, unpatronizing collaboration. Many rare disease patients have done much online research. Understanding this, even in our very first conversation, we can immediately

respond by sending links to helpful articles or other resources. Utilizing technology, we can be there for the patient on a 24/7 basis.

For meaningful program development, it is crucial to continually gather insights from patients using technology such as natural language processing to help pharmaceutical companies learn more about the patients their products are supporting, understand patient perspectives, and drive evidence-based decision-making for improved research, development, and patient outcomes.



Harsha K Rajasimha, Ph.D.
Founder and CEO
Jeeva Clinical Trials
harsha@jeevatrials.com

Enhancing patient engagement in rare disease support programs requires tailored strategies that leverage innovative approaches, including AI and decentralized trials:

Personalized Communication: Utilize AI-driven communication platforms to deliver personalized messages, reminders, and educational content based on patients' preferences, needs, and disease profiles.

Virtual Support Communities Establish these communities or forums where patients can connect with peers, share experiences, and exchange information. AI-powered chatbots can facilitate discussions, answer common questions, and provide real-time support, fostering a sense of community.

Patient-Centric Design Involve patients in the co-design of PSPs to ensure that they address their specific needs, preferences, and priorities. Conduct user testing and feedback sessions to gather insights and continuously improve program offerings based on patient input.

Gamification and Incentives Challenges, quizzes, rewards, etc. make engagement more interactive and enjoyable. Offering incentives, such as gift cards or discounts on healthcare products motivate patients to participate actively and contribute valuable feedback.

Remote Monitoring and Feedback Remote monitoring devices and wearable technologies collect real-time data on patients' health status and treatment outcomes. Analyze this and provide personalized feedback and recommendations to patients and HCPs.

Empowerment through Education Provide educational resources, webinars, and workshops to empower patients with knowledge about their condition, treatment options, and self-care strategies. AI-powered virtual assistants can deliver interactive educational content tailored to each patient. learning style and preferences.



Raymond Huml
Vice President of Medical and Scientific Strategy,
Head of the Rare Disease Consortium

Syneos Health**Raymond.Huml@syneoshealth.com**

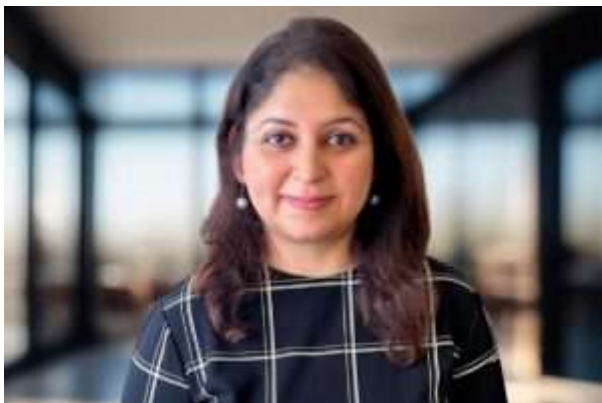
We need to seek out and listen to the voice of the patient, we need to listen to them 1:1 as well as when they utilize social media via social listening tools and include their feedback into the decision-making processes. Communications with the patient community should use clear, straightforward language.

Each community of rare disease patients faces different obstacles and at different timepoints in their patient journeys. There is also much heterogeneity within the various rare diseases. I have seen brothers with the same disease progress at different rates and a set of identical twins where one sibling advanced with the rare disease and the other was clinically unaffected. Some diseases affect one sex more than the other such as the boys in the Duchenne muscular dystrophy community.

Over half of rare diseases affect children; we not only need to listen to them but also to their caregivers. To gain input, we need to incentivize this community by providing safe spaces that remove the stigma associated with their condition. Some may wish to join a private Facebook chat room, while others may prefer to play games with financial rewards to provide disease related insights, such as patient reported outcome data in a clinical research study.

The patient support group also must consider who is taking care of patients. For children, women (typically mothers) are disproportionately caregivers, accounting for more than 80% of the total according to recent estimates.

The perspectives of the rare disease healthcare team should also be solicited. For example, the care team for patients living with muscular dystrophies may include a primary care provider, board certified neurologist, respiratory expert, orthopedist, cardiologist, physical therapist, occupational therapist, licensed clinical social worker or psychologist/psychiatrist, geneticist, and nutritionist.



Sonam Dubey
Partner
Beghou Consulting
sonam.dubey@beghouconsulting.com

The key to improving access to support programs is for pharmaceutical companies to get closer to the patient's lived experience. Mapping the patient's ecosystem and mimicking their real environment helps uncover their sources of information, the influencers who shape their perspectives, and the areas in which they gather (online hotspots where they share experiences and learn from other patients).

A key enabler of effective patient engagement and support is a customer engagement platform that leverages data science, analytics, and the power of advanced technology. These platforms can provide the data management foundation to support a company's efforts to connect with patients and improve their treatment experience. These platforms can also help companies create feedback loops that incorporate analytics-derived insights and direct patient feedback so they can continually optimize their patient-support efforts.

Additionally, companies can leverage artificial intelligence to uncover channel preferences and tailor communications in line with patient preferences and needs. They can deploy patient analytics to uncover insights that facilitate timely and personalized engagement (appointment reminders, test results, and adherence alerts).

In addition to brand-driven campaigns, companies should also engage in trust-based outreach via patient ambassadors. These patient ambassadors can share their real-life experiences with treatment and educate other patients about programs and services the company offers. These ambassadors naturally carry more credibility than branded campaigns and therefore can play an important role in educating and empowering patients.

Companies should also remember that HCPs are a crucial part of this patient-centric communication ecosystem. They should orchestrate patient and HCP focused communications to ensure they are complementary and collectively support an improved patient experience.



Maria Kirsch
President, Patient Services
EVERSANA
maria.kirsch@eversana.com

Improving access to these resources can be achieved through the development of user-friendly educational resources, including digital toolkits, webinars, and informational materials tailored to the specific needs and preferences of rare disease communities. Collaborating with patient advocacy organizations, HCPs, and industry partners to raise awareness, disseminate information, and promote PSPs can also expand reach and accessibility. Furthermore, leveraging technology, such as telemedicine and virtual support groups, can ensure equitable access to valuable resources and services for rare disease patients regardless of their location and travel capabilities.

Providing educational materials and resources in multiple languages can help ensure language barriers do not impede access to crucial information and support services for diverse patient populations.

Partnering with rare disease advocacy organizations can reach a broader audience and leverage their expertise in community engagement. Collaborative efforts can enhance the effectiveness of educational initiatives and outreach programs by tapping into existing networks and resources. Offering educational programs and training sessions for HCPs can increase awareness and understanding of rare diseases so that better-informed health providers can better educate their patients.

Organizing community outreach events like health fairs, workshops, and seminars can serve as a valuable avenue to increase awareness about rare diseases and the support services accessible to individuals affected by them. These gatherings offer a platform for patients, caregivers, healthcare providers, and industry stakeholders to come together, exchange experiences, and gain access to valuable resources.

The opinions expressed by the authors in the Think Tank section are their own and do not necessarily reflect those of their affiliated companies or organizations.