

Living With a Rare Disease: The Unique Considerations for Your Patient Support Program

*People living with a rare disease have specific support needs
— are you considering these for your patient support program?*

The diagnosis and treatment journey for any condition can be complex and challenging, and it's important for healthcare professionals to understand the barriers that people face during different stages of their journey in order to support them in the most effective way. People living with a rare disease and adolescents living with long-term conditions have specific support needs that are often neglected in patient support program designs. This can lead to several challenges along their care journey, with a major challenge being non-adherence to their treatment plans.

To enhance the care experience, increase adherence, and ultimately improve patient outcomes for those living with rare, chronic conditions, it is important to understand the challenges patients face on a day-to-day basis and provide the necessary support on their treatment journey. In an IQVIA webinar, IQVIA experts, Dr. Jessica Walburn, Dr. Clare Moloney, Katelyn Jones, and Evelyn Walsh, covered the key barriers that need to be addressed when designing PSPs for these niche populations.

Key barriers for people living with a rare disease

Non-adherence to treatment is a challenge that needs to be addressed in PSPs. It is estimated that 30–50% of people with long-term conditions do not take their medication as prescribed, regardless of their condition or sociodemographic characteristics.¹ And there is no evidence to say that rates of non-adherence are different for those living with rare disease — without the right support, it could even be higher. But what is driving this non-adherence? What are the key barriers that these populations are facing? Some of these are highlighted below.

- **Stigma and shame, leading to isolation and lack of social support.**
 - » People living with a rare disease can feel stigmatized or isolated, particularly if it is a condition that is poorly understood by others. Due to this, they may isolate themselves from society, further limiting their access to support from friends, family, and healthcare professionals.



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- **Poor emotional wellbeing.**
 - » Feeling poorly understood by others, especially during adolescence, could increase the chances of depression, low mood, or poor quality of life, which can affect their motivation to manage their condition, including adherence to medication.
- **Healthcare fatigue.**
 - » It can take a long period of time for people living with a rare disease to receive a diagnosis. This can impact their perception on the healthcare industry, reducing trust in the guidance given, which can drive non-adherent behaviors.

Unique support needs for adolescents living with a long-term condition

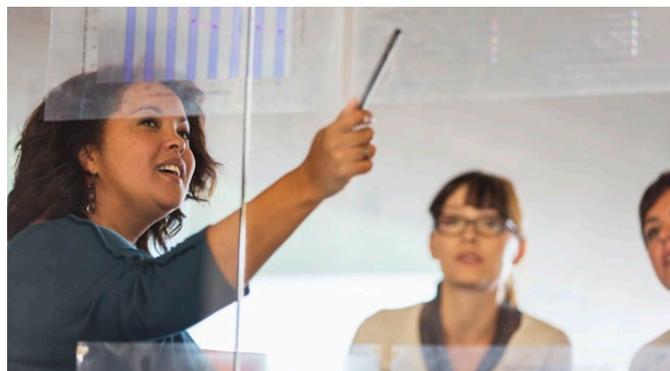
Many rare diseases begin in childhood and continue through to adolescence. And while adolescent patients share many of the same challenges as adult patients, they are also faced with challenges that are specific to their stage of life, such as physical and emotional changes which can impact their experience of disease, treatment journey and adherence.

A behavioral analysis of the literature, using the capability, opportunity and motivation model of behavior (COM-B)², identified key drivers of non-adherence in adolescent populations.

• Capability factors

- » **Physical and emotional transformations.** During the teenage years of life, adolescents are undergoing numerous physical, mental and emotional changes that impact their perspective on many aspects of their life. This can make it hard to establish a consistent response to symptoms and increase susceptibility of stress and mental health issues.
- » **Developing organization and prioritization skills.** Adolescents face the challenge of navigating the competing demands of school, extracurricular activities and other life commitments with limited time management skills, which can make it difficult to stay on top of treatment plans.
- » **Difficulty accessing high-quality information.** There is a high pressure to self-educate on their condition due to lack of HCP knowledge, but they are faced with the challenge of interpreting complex, inconsistent information online.

Adolescents can be highly conscious of the way they look, which may lead to greater concerns about a treatment if there are potential side effects, such as weight gain or acne.



• Motivation factors

- » **Developing concept of themselves.** Resistance to accept their condition as part of their identity can lead to denial and low motivation to follow treatment recommendations.
- » **Ongoing brain development.** It is likely that adolescents have underdeveloped impulse control and limited risk-assessment skills, which can either lead to intentional rebellion against medical advice or misperceptions of the disease.
- » **High concerns about treatment.** Adolescents can be highly conscious of the way they look, which may lead to greater concerns about a treatment if there are potential side effects, such as weight gain or acne.
- » **High emotional burden.** Living with uncertainty can cause frustration and an increased risk of mood states such as anxiety, which is directly correlated with non-adherence.

• Opportunity factors

- » **Social stigma.** Adolescents often have a strong desire to conform with their peers, yet living with a rare disease can make them feel different. This can lead to them feeling embarrassed to take medication in front of others.
- » **Poor delegation of responsibility.** Transitioning into adulthood, they are likely to take more ownership over their care — but if this is not handled carefully, they could feel unsupported or discouraged.
- » **Poor access to social support.** Without people around them in a similar circumstance, adolescents may fear disclosing information and ultimately feel isolated.

These insights from the literature are further supported by the experiences of our PSP nurse teams:

“In my experience to date, I have found that one of the main challenges such adolescents face is the ‘workload’ or ‘burden’ they encounter on a daily basis.” – Bernie Healy, Nurse Adviser.

“[For a person living with a rare disease] their friends may appear to have freedom and are able to change plans at the last minute, but this is very difficult if you’re dealing with a rare disease and medications that need to be timed. So, sometimes, in order to be like their peers, they’re going to be non-adherent.” – Evelyn Walsh, Nurse Adviser.

Practical solutions to improving PSP design

Understanding the challenges that people and adolescents living with a rare, chronic condition face is the first step to improving their treatment journey. The next step is using these insights to inform practical solutions and improve your PSP design. Categorization of these factors using the COM-B model allows for consideration of the different range of solutions needed. For example, addressing challenges with organization and prioritization requires a different approach to supporting people experiencing emotional burden.

Below are some of the elements to consider when designing your PSP to ensure it is addressing the key challenges and offering the necessary support.

- **Ensure the content is suitable for all ages:** Rare diseases are mostly genetic and usually begin in childhood, so it is important to deliver content that is appropriate for all ages, with consideration of family and other caregivers too.
- **Take an innovative approach:** There is a lack of existing research about the drivers of non-adherence in these populations, therefore innovative approaches to support design are often required.
- **Apply and adapt existing theories:** As there is less information and existing evidence to inform design, application of established theories of behavior change is crucial.

Improve your PSP design for better experiences, better results

Development challenges, stigma, social isolation, lack of specialist services, and complex treatment pathways are just some of the challenges that people and adolescents living with a rare, chronic condition face. By considering practical design solutions that can be tailored to specific needs, you can ensure that your PSP is better meeting the needs of these groups to improve their care journey.



- **Facilitate access to treatment:** Due to complex prescription pathways for rare disease, taking steps to facilitate access to treatment is important.
- **Ensure your enrolment pathway is smooth:** The population is small, so it is essential to enroll a high percentage. That means seamless enrollment is pivotal to achieving optimal recruitment.
- **Provide 1-1 interaction:** Filling the “support gap” with 1-1 contact can be very useful to provide immediate positive outcomes and enhance engagement.
- **Engage with stakeholders:** The rare disease community is smaller, so the potential positive impact of early engagement with stakeholders is greater.

Through working closely with patients, our IQVIA nurses and digital and behavioral science experts understand the patient experience and how to provide optimal support. Contact us today to find out how you can leverage our experience and expertise to improve your PSP design, to ultimately create better experiences and better results.

References:

1. Sabaté E. Adherence to Long-Term Therapies: Evidence for Action. World Health Organization; 2003.
2. Michie, S., van Stralen, M & West, R. The behaviour change wheel: A new method for characterising and designing behaviour change interventions. Implementation Science, 6, 42. <https://implementationscience.biomedcentral.com/articles/10.1186/1748-5908-6-42>