GOLINHEALTH

THE RAISING RARE REPORT

Examining the needs of caregivers and young people living with rare diseases during the transition to independence and adulthood.

July 28, 2025

Approximately 1 in 10 Americans are living with a rare disease,[1] and their journey typically begins in childhood.[2]

Today, thanks to advances in science, patients with rare diseases are living longer, fuller lives.[3] But as younger patients grow into adulthood, they must learn to manage their own healthcare, pursue education and careers, and maintain relationships, all while navigating systems that aren't always designed with their unique challenges in mind.

"The Raising Rare Report," developed by Golin Health, presents new research that examines the needs of caregivers and young people living with rare diseases during the pivotal transition to independence and adulthood. Launched in recognition of Rare Disease Day 2025, the research draws on firsthand insights from both groups, uncovers key gaps between what each group wants and expects, how they experience this pivotal life stage, and where healthcare communications have yet to effectively address their unmet needs. While this research focuses on individuals living with a rare disease, sentiments and insights are likely applicable to broader child and parent/caregiver behaviors outside of the rare disease community.

"The Raising Rare Report" is not just a snapshot of where we are but direction for how we move forward. These findings provide crucial insights to help communicators navigate the evolving rare disease space, address education gaps, strengthen support structures, and engage with patients and their caregivers during this critical transition.

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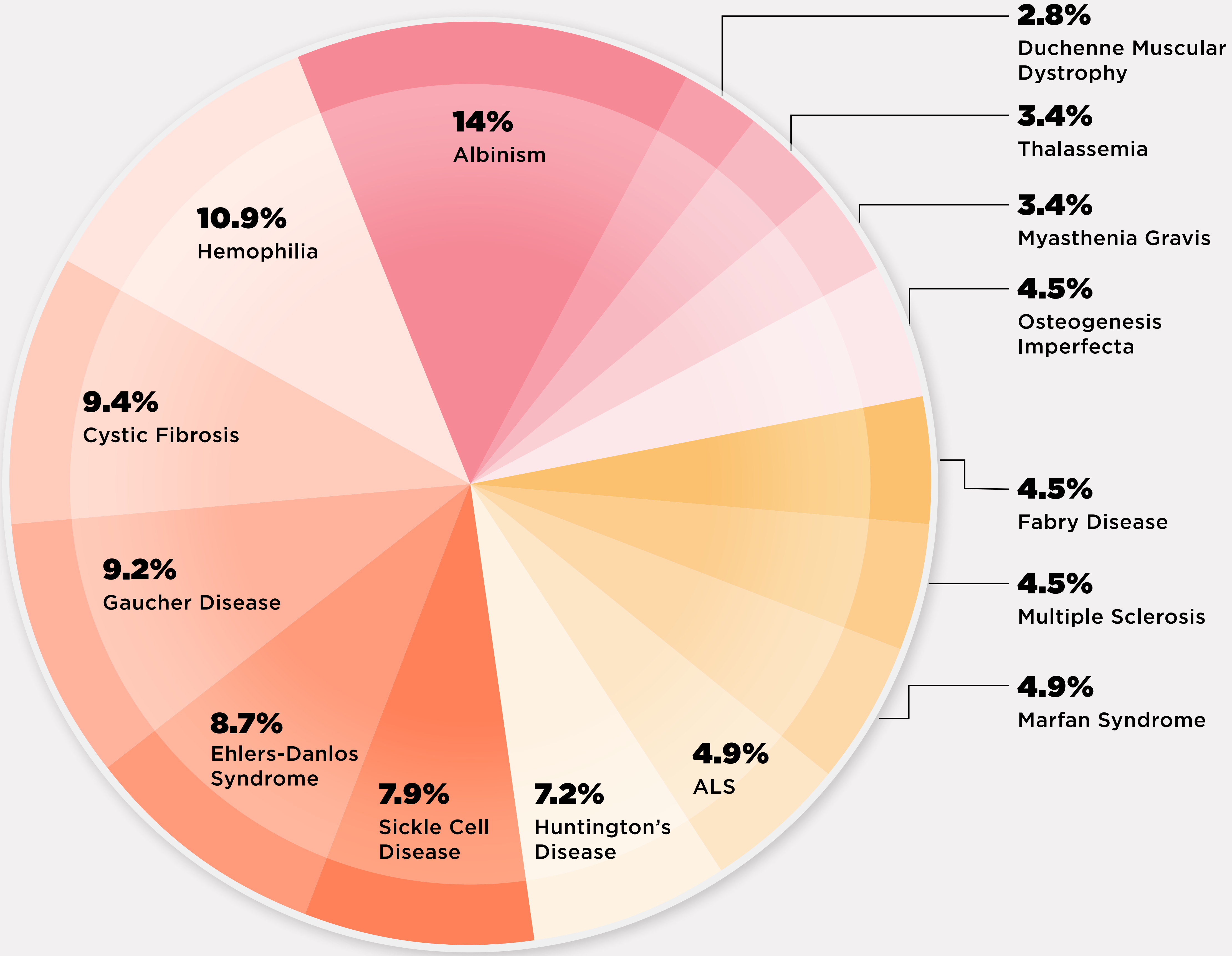
Survey Respondent Demographics: Rare Disease Patients & Caregivers

Respondents

68% rare disease patients; 16-23 years old
32% rare disease caregivers; 25-55 years old

- 37% mothers
- 25% fathers
- 25% legal guardians
- 13% grandparents

Top 15 Rare Diseases Among Respondents



Methodology

"The Raising Rare Report" was conducted as a U.S.-only survey launched on Rare Disease Day, February 28, 2025. The study focused on levels of preparedness for managing independent healthcare and the types of support needed during the transition to adulthood. A total of 261 patients aged 16 to 23 years and 120 caregivers aged 25 to 55 years participated in the study, which was fielded from April 2025 to June 2025. The research examined 175 rare diseases.

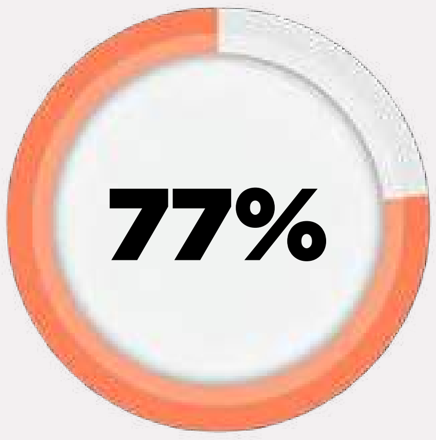
Ready or Not, Independence Is Coming

Caregivers and patients are headed in opposite directions as health ownership shifts to the patient, with patients and caregivers largely agreeing about current treatment decisions, but with patients saying they intend to change treatment plans.

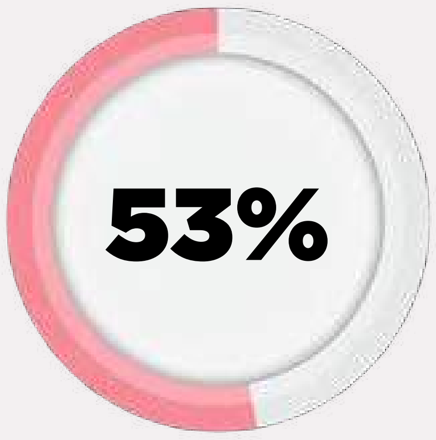
of caregivers say they agree with their loved ones on treatment decisions

of patients report the same

However,



of patients intend to change treatment plans



of caregivers expect them to remain the same

- Additionally, 67% of patients intend to adjust their daily routines and activities, and 48% plan to change their care team
- Of the patients who have already reached independence, most confirmed following through with changes to their treatment (76%), daily activities (76%), and mental health support (57%)

These data, compared with the quarter (25%) of respondents who share decision-making equally between patient and caregiver, underscore the significant lack of — or disrupted — communication between patients and caregivers.

Communication gaps widen when asked about readiness to manage care independently.

- 98% of caregivers worry their loved one's rare disease impacts their ability to plan for the future
- 79% of caregivers fear their loved one isn't equipped for health independence
- **BUT:** Among patients who achieved independence, 79% felt prepared (50% somewhat, 29% totally)

These results suggest that caregivers may be underestimating their loved one's readiness and overestimating the risks. Most adolescent patients (90%) acknowledge that their rare disease has some impact on their ability to plan for the future, but results point to deeper, potentially exacerbated, worry from caregivers. More than half of caregivers (63%) are cognizant that their loved one's rare disease significantly impacts long-term planning for milestones such as independent living, higher education, and entering the workforce.

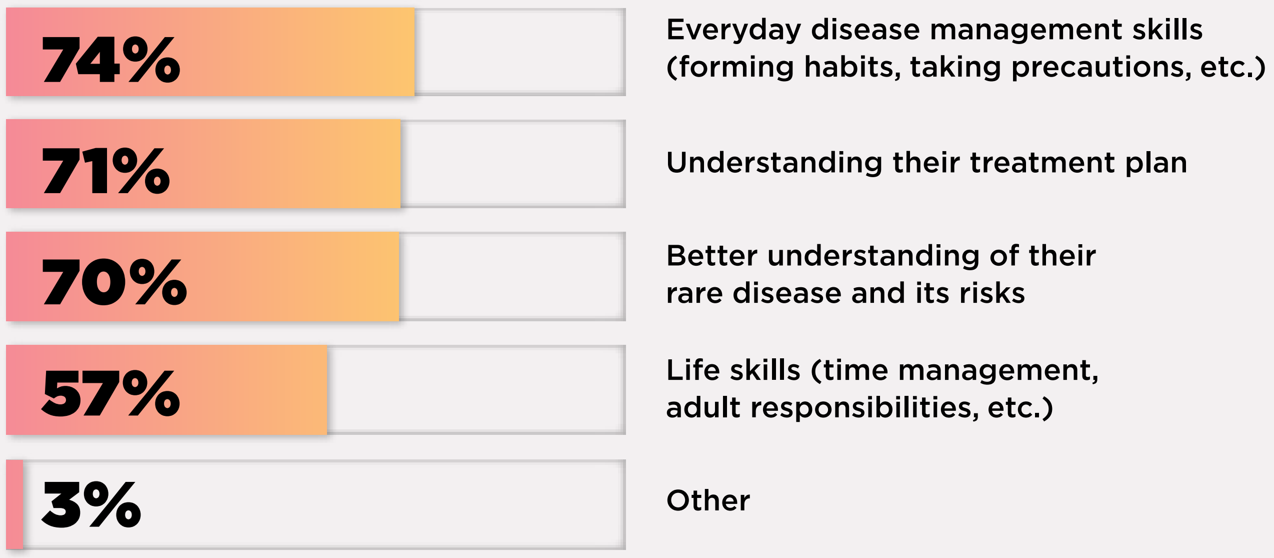
The opportunity to arm HCPs and address unmet needs

Both caregivers (70%) and patients (74%) are eager for more in-depth conversation and guidance from their care teams. Both groups (71% of caregivers; 57% of patients) are hungry to understand the long-term treatment plan and how the treatment will work (the mechanism of action described in lay terms). They're seeking help to understand how to measure treatment success and how to assess at home if the current plan is supporting their individual care priorities in a tangible way.

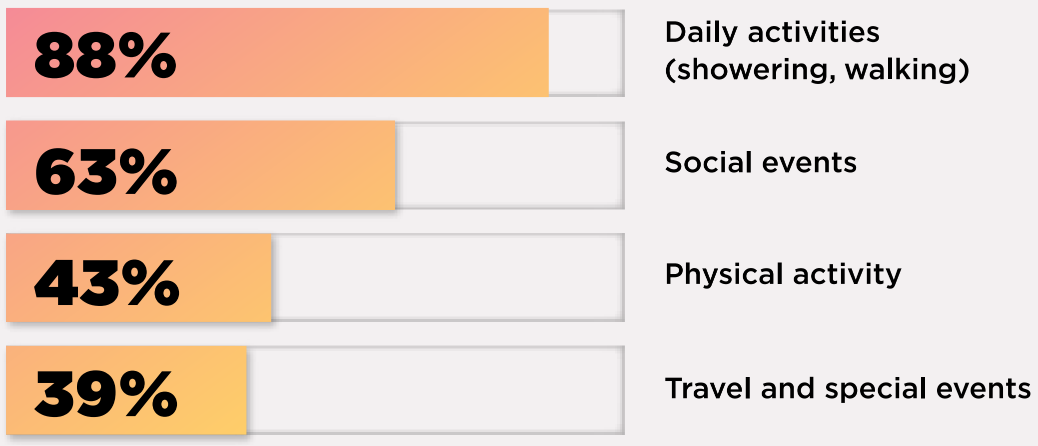
The Future Patients and Caregivers Are Preparing for (Separately)

Despite sharing a desire to complete daily activities independently, the survey exposes a critical disconnect between caregivers and patients. When caregivers think about their loved one's transition to independence, they prioritize practical preparation.

What Caregivers Feel Their Loved One Needs to Learn



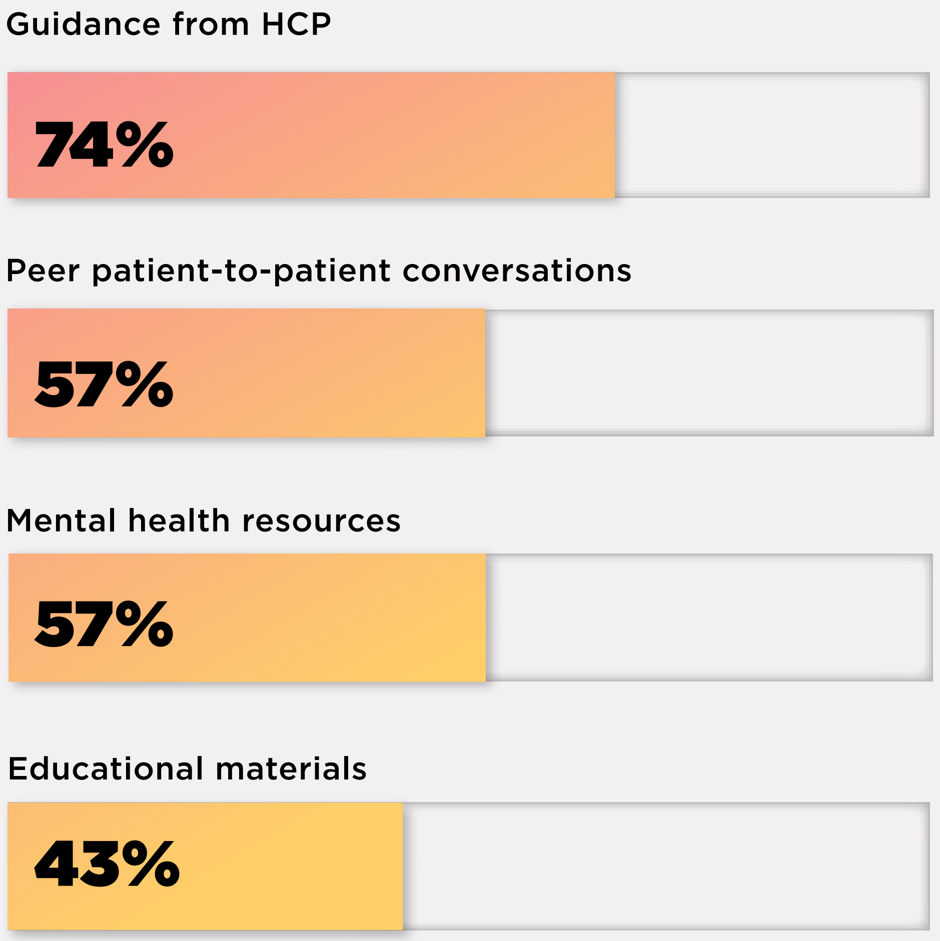
What Caregivers Prioritize When Choosing a Treatment



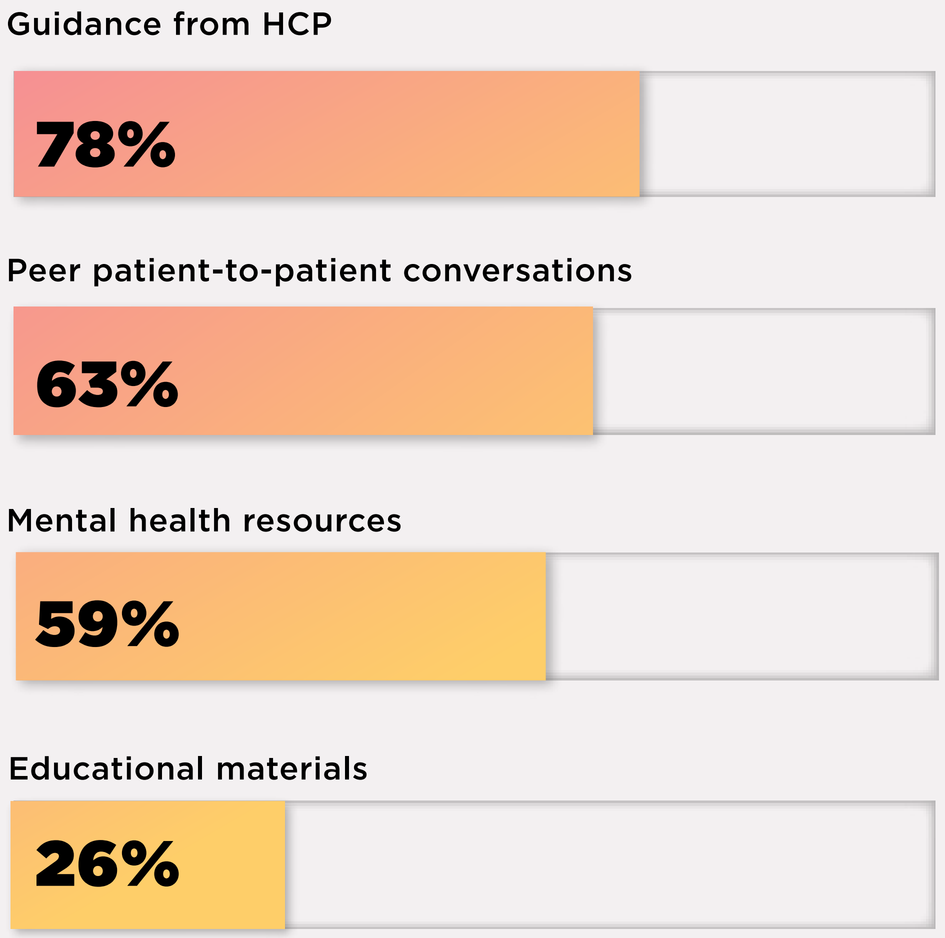
Patients are seeking connection and mental health support

Adolescents with rare diseases have a different vision for what they need to successfully manage their care.

What Patients Feel They Need for Transition Support



What Patients Prioritize When Choosing a Treatment

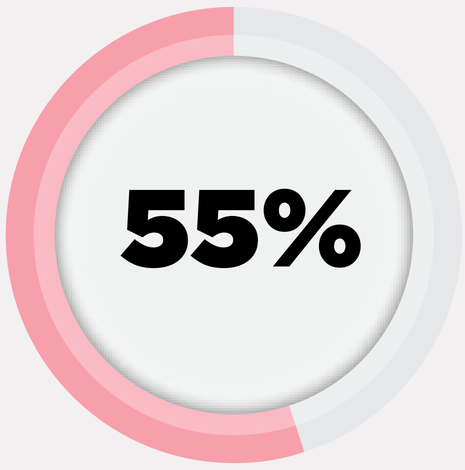


When it comes to long-term planning and key treatment priorities, patients want to improve communication with their caregivers, particularly around gaining autonomy and exploring health and human needs. Patient considerations for adulthood and preparation for it are grounded in expanding social circles and finding support to explore new interests. They seek guidance with a human touch, while caregivers tend to focus on "hard skills" that could be taught and reinforced through conversations with HCPs.

Independent patients reaffirm what tools lead to a successful transition

Data from patients who reached health independence indicate which support systems will likely prove effective. These post-transition data highlight the importance of mental health and peer-to-peer relationships, which should be considered in long-term planning before patient independence and could be integrated into HCP treatment practices.

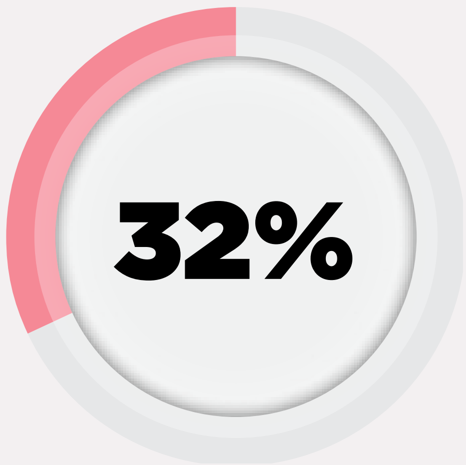
What Helped Independent Patients the Most



Mental health resources

Guidance from HCP

Peer patient-to-patient conversations

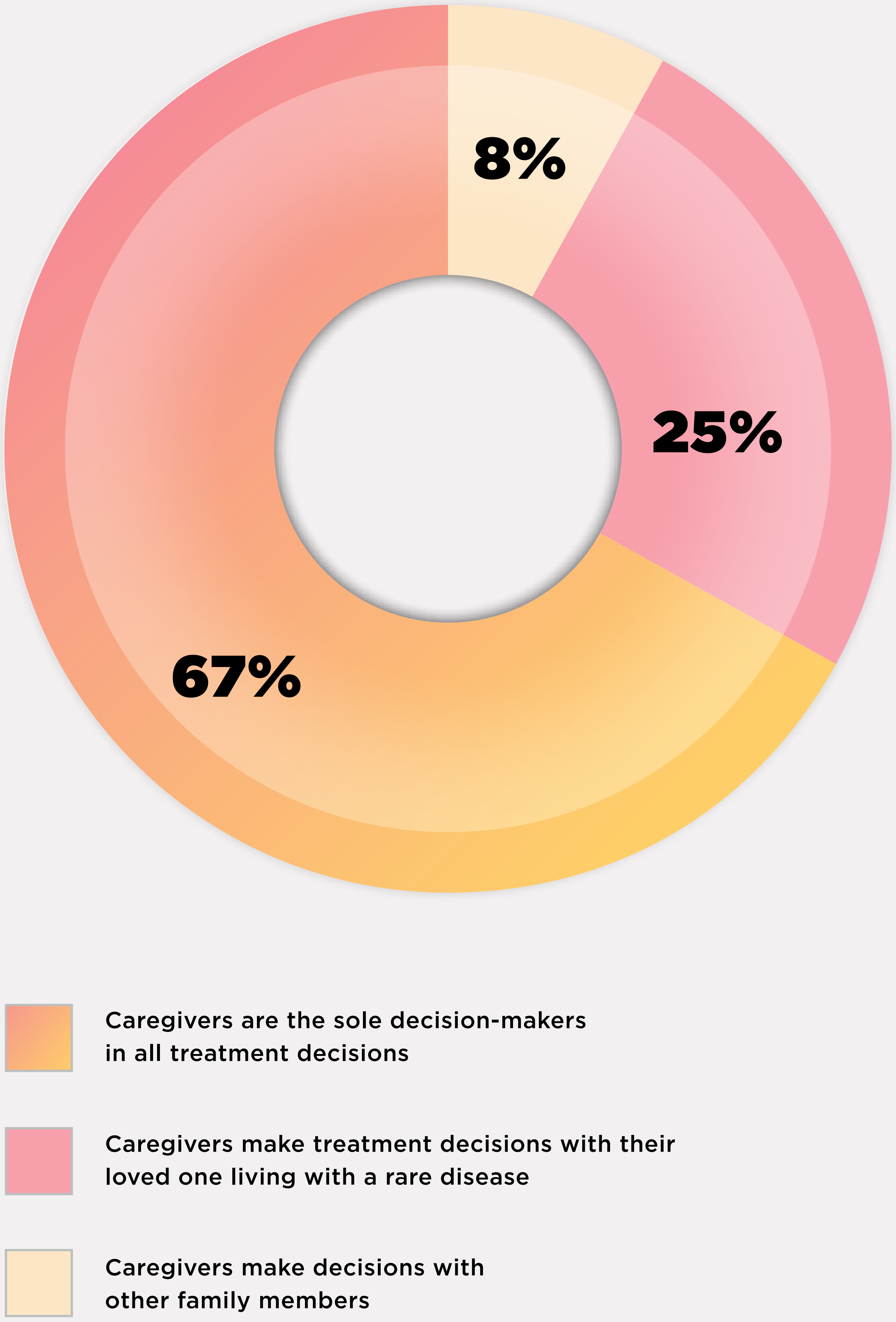


Educational materials

The Caregiver Crisis Hidden in Plain Sight

Although many patients (51%) feel their care is mutually managed, caregiver sentiments contradict this, as most caregivers see themselves as the sole decision-makers.

Caregiver Involvement in Treatment Decisions



Caregivers feel responsible for treatment decisions, and that burden is taking a toll.

73%
report an emotional impact from their role

74%
feel overwhelmed balancing everyday life with health responsibilities

53%
experience strains on their personal relationships due to caregiving

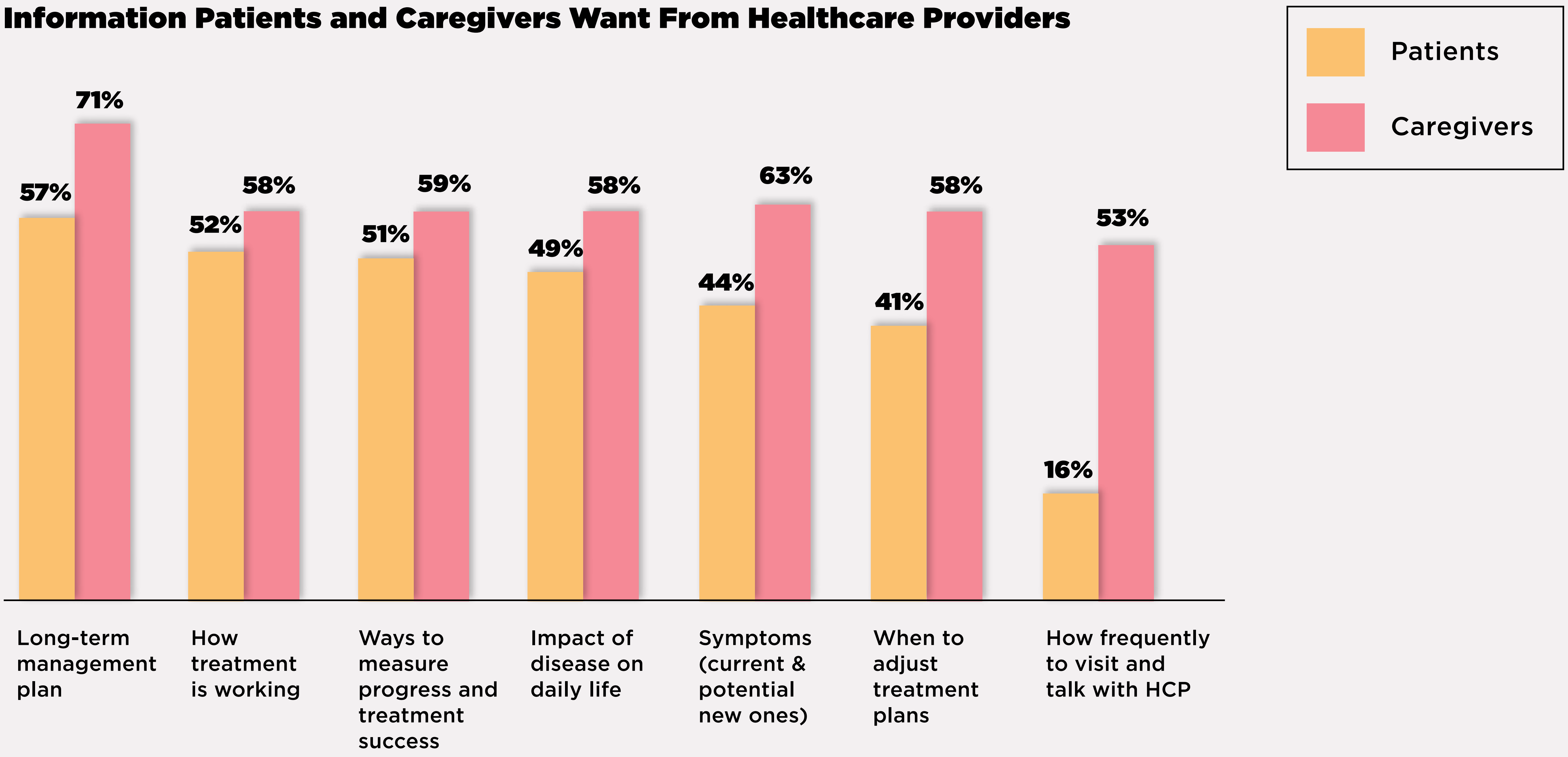
53%
report challenges focusing on daily activities

40%
experience feelings of loneliness and isolation

21%
report discomfort in social settings with others who aren't familiar with their loved one's rare disease

Caregivers face additional sources of stress. Many said that responsibilities outside of caregiving, such as work and household chores, contributed to their emotional stress. In addition, 84% of caregivers are aware of the impact the rare disease has on their loved one's well-being, signaling that they see disease management as their responsibility. However, this perception places undue strain and stress on the caregiver while delaying the adolescent patient's journey to independence.

Information Patients and Caregivers Want From Healthcare Providers



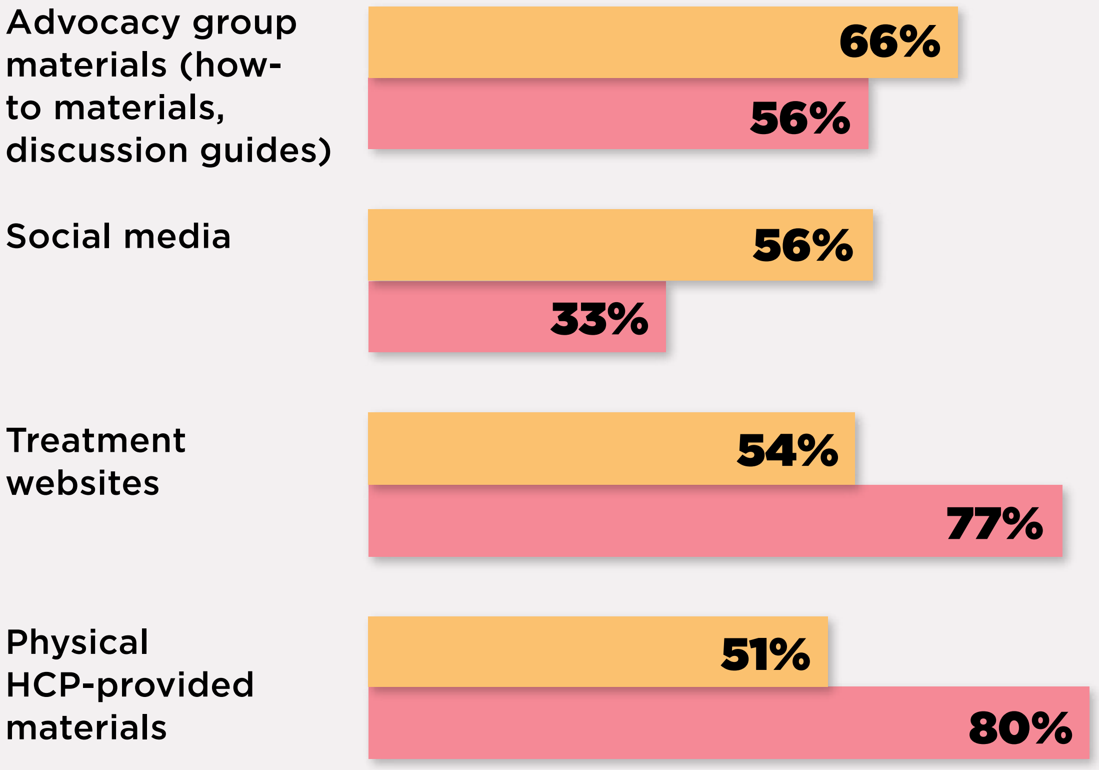
Patients and caregivers are asking for help in different places

Patients and caregivers rely on different sources, creating separate information ecosystems.

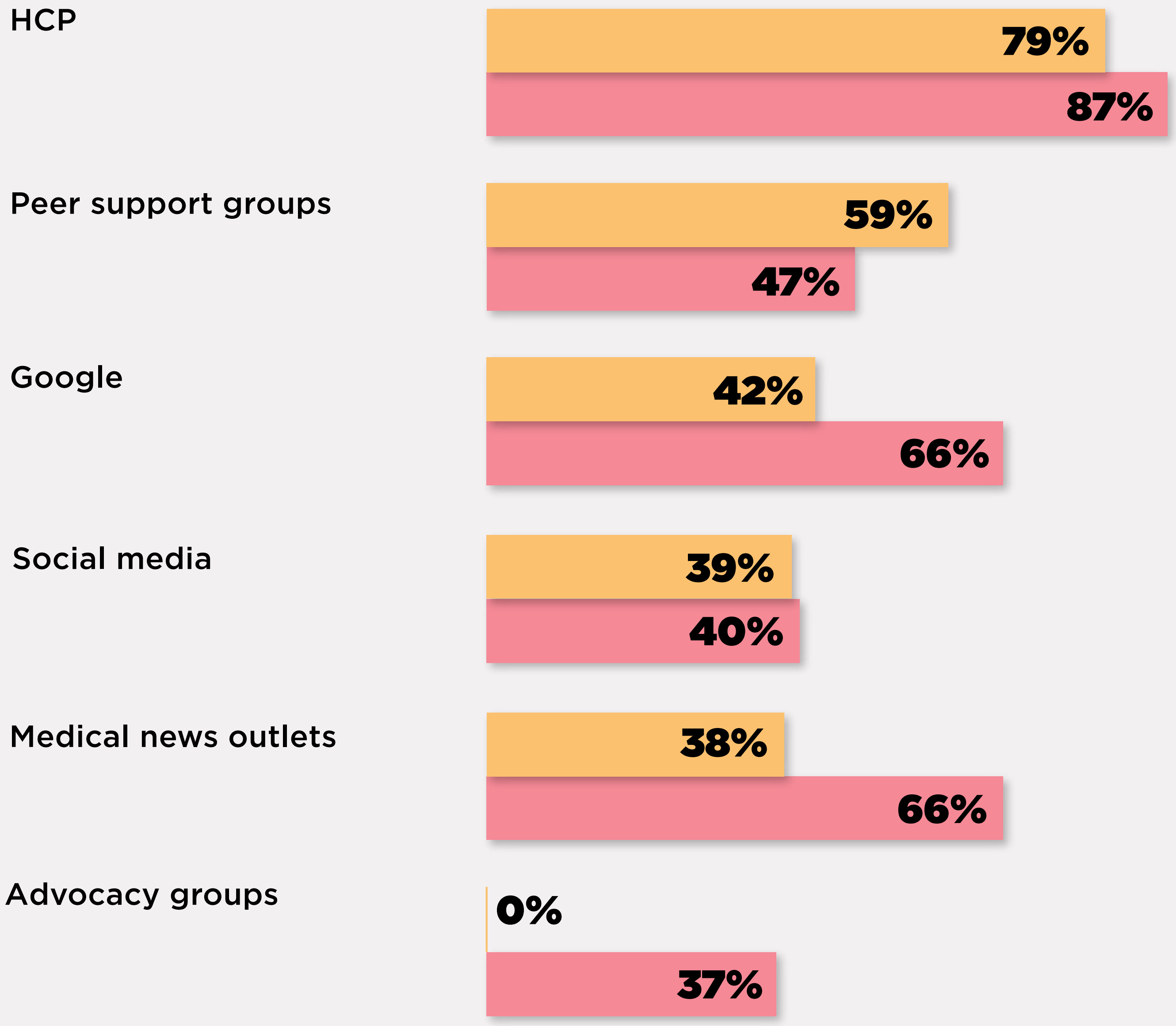
For daily management of rare diseases, patients and caregivers rely on different sources of information, with patients turning to advocacy groups and social media and caregivers consulting treatment websites and HCP-provided materials.

However, when it comes to specific information on the rare disease and treatment direction, **both groups prefer the HCP as their primary source. This finding indicates a clear opportunity for communicators to support these trusted figures with the resources and communication tools they need.**

Where Patients and Caregivers Turn for Daily Management



Preferred Sources of Information on Rare Diseases and Treatment Direction



Become a Catalyst in Rare Disease Communications

These data reveal an exciting opportunity for communications professionals to usher in a new era of rare disease engagement. We have actionable insights to reach adolescents living with rare diseases where they're at in their journey while supporting their caregivers along the way.

Real-world insights point to an opportunity — and great need — to empower rare disease doctors to engage their patients as partners and ask about their values and long-term goals so they can discover how medicine can complement their lives instead of commanding them. Learning from the successful patient-doctor dynamics emerging in oncology will help prevent disruptions in rare disease care and help maintain relationships established at diagnosis. Existing research primarily focuses on pediatric-to-adult transitions from a hospital's administrative perspective. However, our findings suggest a broader need to shift from top-down care to a collaborative model that respects patients' values and long-term goals.

In rare disease contexts, new language and approaches are needed to support shared responsibility and long-term planning between adolescents living with a rare disease, their caregivers, and medical professionals. These insights are intended to inform effective engagement strategies and tailor communications during a vulnerable transition period.

For HCP communications

There's an opportunity to arm HCPs with the information and materials that patients and their caregivers need for successful long-term planning. We can help industry professionals support HCPs with emotionally intelligent communications that close care gaps within the practice setting.

For patient, caregiver, and advocate communications

We have an opportunity to address the disruptions that prevent patients and their caregivers from having open and honest conversations that directly affect treatment choices and long-term planning. Communicators can facilitate true shared decision-making and understanding between adolescent patients and their caregivers, ensuring a balanced and united front prior to stepping into the doctor's office.

Ready to transform your rare disease communications?

The insights in this report reveal untapped opportunities to create meaningful connections between patients, caregivers, and HCPs during life's most critical transitions. As your strategic partner in rare disease communications, Golin Health can help you:

Bridge communication gaps between patients and caregivers with targeted, emotionally intelligent messaging that speaks to each group's distinct needs and priorities.

Empower healthcare providers with the resources and tools they need to have deeper, more effective conversations with their patients about long-term planning and treatment success.

Build authentic relationships within the rare disease community by demonstrating genuine understanding of the challenges young adults face and the support systems that truly matter.

Turn these insights into action. Let's discuss how the findings from "The Raising Rare Report" can inform your rare disease strategy and drive meaningful engagement with the communities you serve.

Contact Jaimee Reggio at JReggio@golin.com to explore how we can bring these insights to life for your brand.

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Bringing health within reach for more

Golin Health is a global public relations agency working to bring health within reach for more. Our science-focused, creatively driven teams craft effective solutions for healthcare brands across a range of specialties to reach key audiences and drive business impact for our clients. Centering patients and data-driven strategies in all that we do provides Golin Health with a deep understanding of the rare disease landscape and how to create change that matters through health communications.

If you are interested in working with us to bring health within reach for more, visit **golinhealth.com**

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1. National Organization for Rare Disorders. NORD 2021 annual report. Published 2022. https://rarediseases.org/wp-content/uploads/2022/11/NORD_2021_AnnualReport_WEB.pdf

2. Somanadhan S, O'Donnell R, Bracken S, et al. Children and young people's experiences of living with rare diseases: an integrative review. *J Pediatr Nurs*. 2023;68;e16-e26. doi:10.1016/j.pedn.2022.10.014

3. Chung CCY, Chu ATW, Chung BHY; Hong Kong Genome Project. Rare disease emerging as a global public health priority. *Front Public Health.* 2022;10:1028545. doi:10.3389/fpubh.2022.1028545