Patient-driven insights into the impact of COVID-19 on the rare disease community

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Xperiome™

Magnolia innovation

Introduction

The COVID-19 pandemic has had a profound impact on people around the world, but arguably none more so than the rare disease community.

Xperiome, a global healthtech company specializing in rare disease, and Magnolia Innovation, a healthcare research consultancy, partnered up to explore how COVID-19 has impacted the rare disease community, including changes in patient and caregiver behaviors and the emotional toll of the pandemic.

The survey elicited eye-opening responses that paint a picture of isolation, uncertainty, and a heightened sense of urgency for disease stability.

As we are now well into the second winter season of the global COVID-19 pandemic, this report considers how pharma can reflect on the lessons learned and play a part in equipping the rare disease community with the resources and information they need to feel empowered within the present-day uncertainties.

Survey: COVID-19 and You
141 respondents
41 rare conditions
55% US / 18% UK / 7% Canada / 20% Rest of World

Overview

Survey findings

The survey uncovered many insights into the lives of rare disease patients and their caregivers during the pandemic, but three key themes prevailed across the responses:

Rare disease patients have been thinking a lot more about their disease as a result of COVID-19

The pandemic has created a heightened sense of urgency for many individuals with rare disease — patients found themselves experiencing increased access challenges, concerns about immunosuppression, and a desire for better disease stability to reduce risk of hospital exposure.

Overburdened healthcare systems impelled many rare disease patients to become more of their own self-advocates, yet they have struggled to find reliable information

Despite an increase in questions and online searches for answers, patients felt information sources were often unreliable and many questions were left unanswered.

There is an opportunity for pharma to re-establish its commitment to rare disease patients through acknowledging their needs in the wake of the pandemic

While the rapid vaccine development was viewed as a major success for pharma, there was minimal positive movement in terms of patients' trust in and perceived support from pharma.

Key Insights

Rare disease patients have been thinking a lot more about their disease as a result of COVID-19

Despite growing self-advocacy, patients have struggled to find quality sources for information given the overburdened healthcare system and lack of trust in online channels

There is an opportunity for pharma and patient advocacy groups, working together, to grow their presence and demonstrate commitment to rare disease patients

Insight 1:

Rare disease patients have been thinking a lot more about their disease as a result of COVID-19

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Some patients felt that having a rare disease prepared them for the pandemic.

- Isolation and additional health precautions (e.g., mask wearing, social distancing) were situations many rare disease patients were already familiar with prior to the pandemic
- Some hoped it would make employers more sensitive and amenable to the needs of employees with chronic conditions; for instance, recognizing the ability for people with rare diseases or disabilities to work remotely even after the pandemic is over

However, many patients felt neglected, and uncertain about the future.

- Patients felt abandoned by a system now focused on COVID-19, and uncertain of whether things would ever get better
- Refusal by some members of the general public to wear masks and follow COVID-19 precautions also caused frustration by putting an already at-risk population at even greater risk
- Some also noted hearing dismissive comments about rare disease patients and the risks of COVID-19 (e.g., “COVID-19 was only going to kill the elderly and the sick”, “only sick people end up in the hospital”)

“This is a taste of what I live normally, isolated from many friends and activities.”

- Idiopathic Pulmonary Fibrosis Patient

“We are more at risk and COVID-19 has made us very unsocial as we can't meet up with others or go shopping, in fear.”

- Myasthenia Gravis Patient

“It has meant that I've been unable to meet with my consultant or have x-rays to monitor my condition. I feel that I've been robbed of more than a year of my life.”

- Idiopathic Pulmonary Fibrosis Patient

Rare disease patients have been thinking a lot more about their disease as a result of COVID-19

Patients have spent more time thinking about their health and actively looking into information related to their disease during the pandemic.

- Patients reported often feeling cautious, worried/anxious, depressed, and concerned having to deal with the difficulties of isolation, constant symptom monitoring (e.g., wondering if symptoms are COVID-19 vs. their condition), and accessing medical care and treatments
- This persistent concern exacerbated by the pandemic led many to spend more time seeking out information related to how COVID-19 might affect their disease

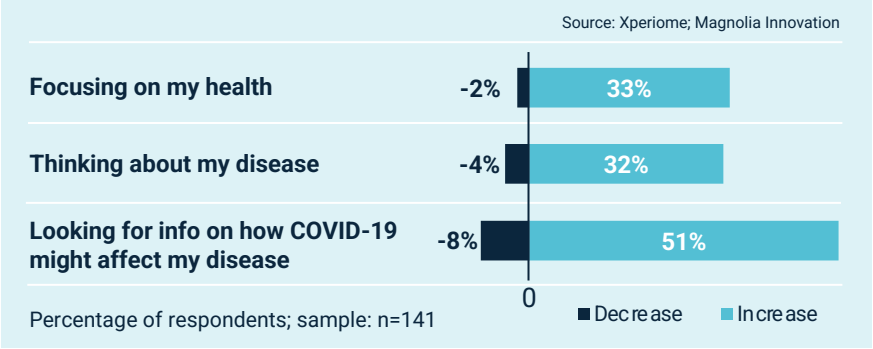

Figure 1: Change in focus on health

"It just brings out fear of how it might affect us since we're already dealing with a life-threatening illness – COVID-19 just compounds that fear."

- Sickle Cell Beta Thalassemia Patient

"I would like to not be immuno-compromised. It scares me to think I might get COVID-19 and possibly die."

- Myasthenia Gravis Patient

Rare disease patients have been thinking a lot more about their disease as a result of COVID-19

The link between rare diseases and the immune system made some feel especially vulnerable.

- For some, understanding that they were immunocompromised caused them to become hyper-focused on the potential impact of COVID-19
- A lack of reliable information led to stress and anxiety for many
  - Especially for the newly diagnosed – these individuals were likely to experience even greater anxiety due to the limited familiarity with their disease compounded by concerns around the impact of COVID-19

Qualitatively, some reported a significant impact on mental health and well-being.

- This varied by type of disease and prognosis, but newly diagnosed patients felt especially isolated due to separation from friends and family while processing their diagnosis
- Stress and anxiety of the pandemic was also reported to exacerbate symptoms for many

"Because I am VERY vulnerable to possibly catching COVID-19, it scares and worries me. And if I do catch COVID-19 am I going to beat it or is it going to beat me?"

- Sickle Cell Beta Thalassemia Patient

"I was concerned about the risks of my treatments regarding COVID-19. I took those risks for granted without much thought until COVID-19."

- Myasthenia Gravis Patient

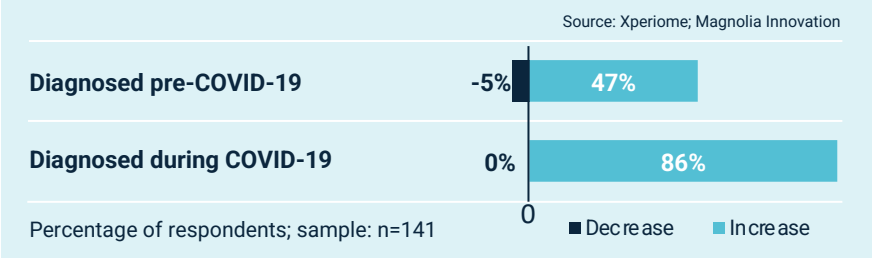

Figure 2: Change in looking for information about disease

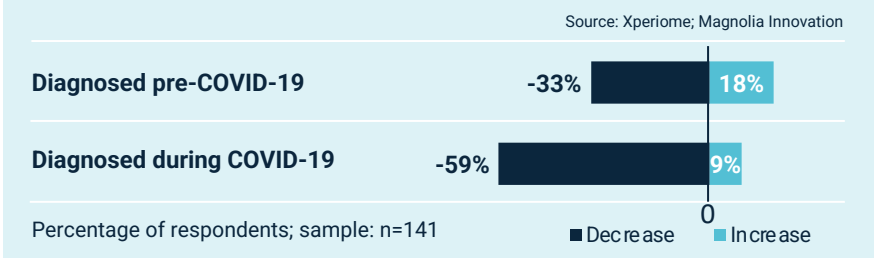

Figure 3: Change in ability to manage a rare disease

Rare disease patients have been thinking a lot more about their disease as a result of COVID-19

Increased access challenges, concerns about immunosuppression, and desire for better disease stability drove a greater urgency and interest in new treatments.

- Fears of COVID-19 exposure led many rare disease patients to place more importance on whether their treatments would reduce the risk of hospitalization
- This has caused patients to take on an “optimization mindset” as treatments they once felt satisfied and accustomed to no longer feel like enough

Respondents reported feeling the need to be more cognizant of their current treatments and learn more about other treatment options that could further optimize their outcomes and reduce COVID-19-related risks.

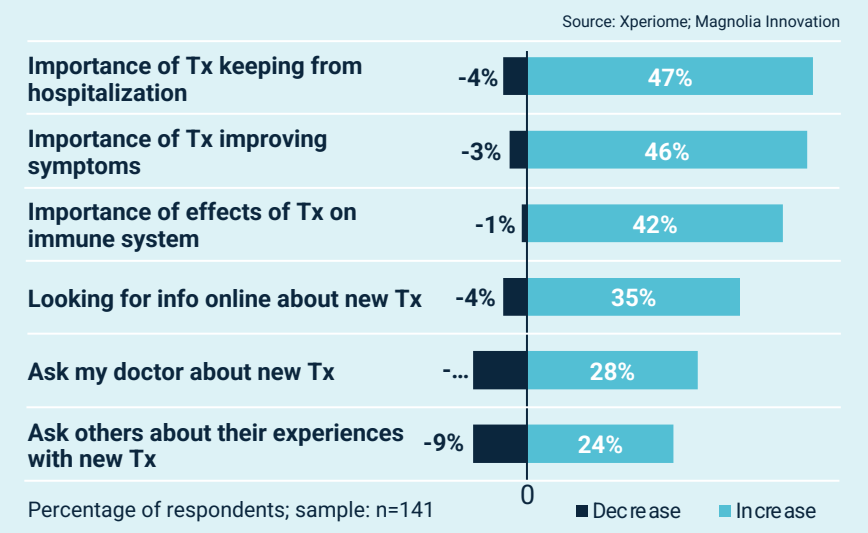

Figure 4: Change in health-related concerns and activities

“I wish I wasn't taking an immunosuppressant during a pandemic, but it has helped me with my rare disease.”

- Myasthenia Gravis Patient

I'm terrified. I don't want to go near the Doctor’s rooms or hospitals as I'm more at risk there.”

- Marburg Acute Multiple Sclerosis Patient

Insight 2:

Despite growing self-advocacy, patients have struggled to find quality sources for information given the overburdened healthcare system and lack of trust in online channels

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Increased pressure on hospitals and the care system resulted in challenges with accessing consistent and quality medical care.

- HCPs were spread thin, and many patients felt rushed during appointments or concerned they were not receiving appropriate care
- Some patients also reported shortages of specific medications - either through prioritization of COVID-19 patients, or due to supply chain issues

Limited healthcare capacity for in-person visits, combined with video consultations not always being available, inhibited essential disease monitoring and treatment management.

- In some cases, this led to an inability to refill prescriptions, leaving patients without potentially life-saving medicines or driving them to seek out new physicians or providers
- As a result the lack of in-person doctor's visits or limited access to treatment have caused some patients to experience exacerbations or worsening of their disease

The limited access to providers forced patients to become self-advocates, turning to digital resources and social media to search for answers.

"It has meant that I've been unable to meet with my consultant or have x-rays to monitor my condition. I feel that I've been robbed of more than a year of my life."

- Idiopathic Pulmonary Fibrosis Patient

"My treating neurologist refused to take video appointments, and won't refill my prescription for Mestinon, and so I've had to stop taking my treatment."

- Myasthenia Gravis Patient

"Surgeries and appointments many of us were waiting for over six months to a year had to be temporarily cancelled and put off."

- Sensorineural Hearing Loss-Early Graying-Essential Tremor Syndrome Patient

Despite growing self-advocacy, patients have struggled to find quality sources for information given the overburdened healthcare system and lack of trust in online channels

However, patients reported an absence of validated resources about COVID-19 and rare diseases.

– Patients struggled to find reliable information on effects of COVID-19, vaccines, and treatments for rare disease populations

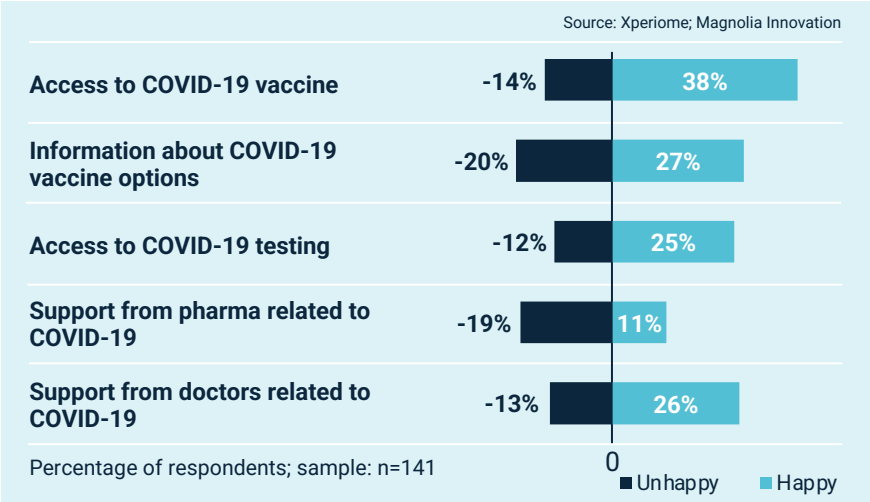

Figure 5: Satisfaction with COVID-19 support

"The fact doctors are pushing vaccines with little regard for our pre-existing conditions is upsetting. No research has been done ensuring that there will be no negative impact."

- Sickle Cell Anemia Patient

"There's lots of ways to tell if a source is trustworthy, but mostly, that it comes from a reputable source. A trusted source that I know verify some information and follows the general rule of journalistic ethics."

- Myasthenia Gravis Patient

Despite growing self-advocacy, patients have struggled to find quality sources for information given the overburdened healthcare system and lack of trust in online channels

Patients reported a greater shift to use of virtual channels due to COVID-19, including;

Increased use of online searches.

- This included looking for information on how COVID-19 may affect their disease and potential new therapies, as well as the impact therapies may have on their immune system

Increased desire to connect with others.

- More than a quarter of rare disease patients expressed an increased desire to connect with others, in person, online or both
- This shift is likely due to the increased levels of uncertainty and feelings of isolation from typical support systems (friends, family, doctors) due to COVID-19 restrictions

The desire to seek out information resulted in:

- Higher engagement with social media and other online sources likely driven by hyper focus on disease
- Continued searching for answers about their disease as COVID-19 evolves; likely to continue adding to frustration as new questions emerge while answers remain limited

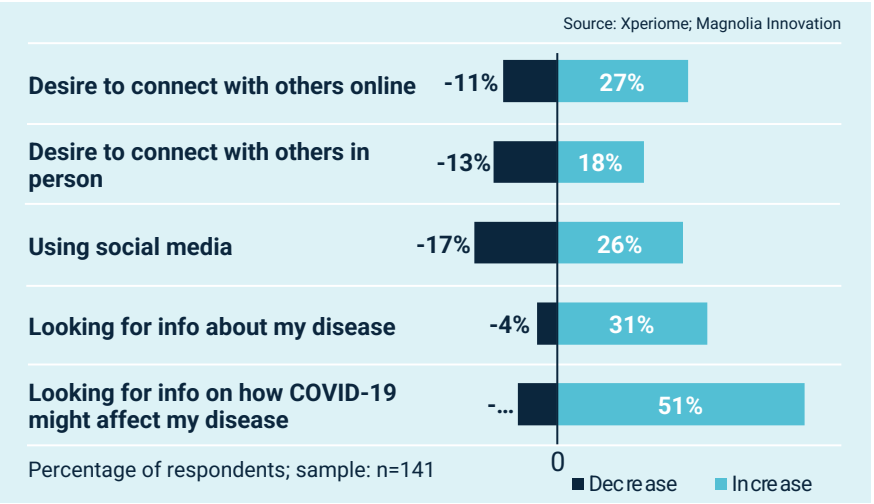

Figure 6: Change in need for social support and information

Despite growing self-advocacy, patients have struggled to find quality sources for information given the overburdened healthcare system and lack of trust in online channels

Despite increased use of virtual channels (i.e., social media, online sources) for information on their diseases, COVID-19 has led to a growing distrust in these outlets.

- A disconnect emerged between patients yearning for more information on their disease and COVID-19 but a decreased trust in these information sources

Trust in sources has significantly decreased during COVID-19, while need for information has increased, suggesting needs for information have not been met.

- Patients with rare diseases struggled to find trusted and consistent sources of information
- There has been a loss of trust in institutions like government, media, and social media due to inconsistent or questionable information
- The social isolation and frustration with a dismissive general public further exacerbated emotional burden
- Some felt that political biases may impact reputability of media sources, adding to greater general scrutiny and skepticism around COVID-19 information

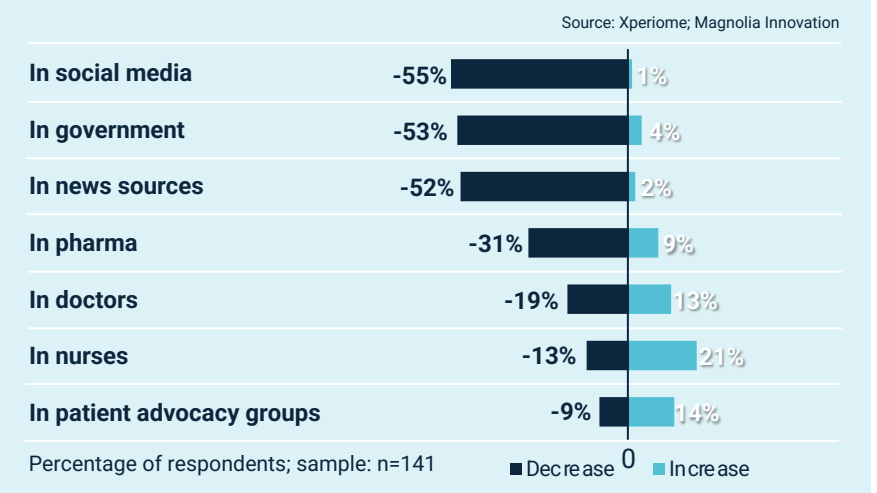

Figure 7: Change in trust

"There's so much misinformation online these days…It kind of made me pull away from social media. I just didn't want to see it anymore."

- Myasthenia Gravis Patient

"I look more to scientific sources. I don't trust news sources because the way they report is piecemeal. It causes confusion because people don't come away with the full story."

- Sickle Cell Patient

Insight 3:

There is an opportunity for pharma and patient advocacy groups, working together, to grow their presence and demonstrate commitment to rare disease patients

There is an opportunity for pharma and PAGs, working together, to grow their presence and demonstrate commitment to rare disease patients

Perceptions of pharma and patient advocacy groups (PAGs) shifted less dramatically than other informational sources.

- Despite patients' desire to access information online, the most common sources (news, social media and government) showed a significant decline in trust
- Rare disease patients were less likely to express decreased trust in pharma, with some increasing trust due to certain manufacturers' vaccine response

PAGs saw the least decline in trust, and pharma the greatest increase in trust during COVID-19.

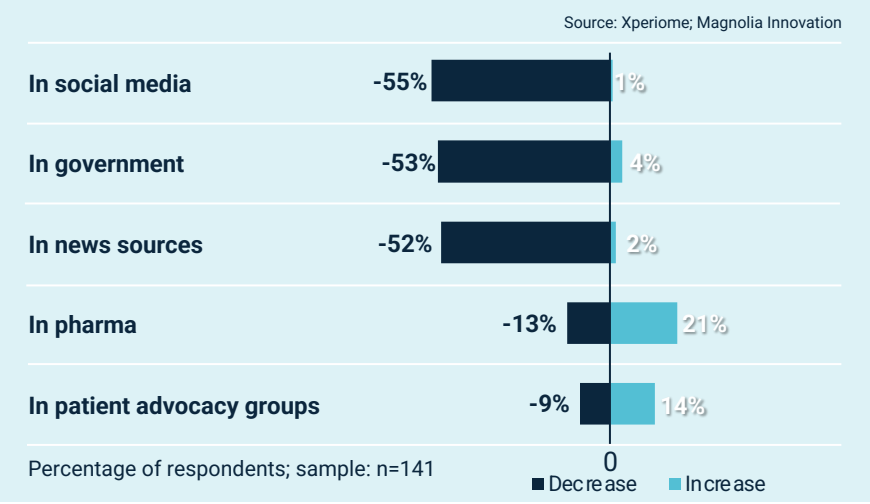

Figure 8: Change in trust (select groups)

"My trust for pharmaceutical companies remains the same and we need them more than ever before because lives are at stake worldwide, but extreme professional ethics must be revisited and evaluated 24/7 as well."

- Hemophilia Patient

"I no longer trust the CDC, WHO or news because of conflicting information. Keeping schools open is a risk to all of us, while everything else is still closed or limited in opening. They want kids in school and there really isn't any accountability for those who become sick or die. Families and people will suffer..."

- Marburg Acute Multiple Sclerosis Patient

There is an opportunity for pharma and PAGs, working together, to grow their presence and demonstrate commitment to rare disease patients

Patients reported minimal support from pharma companies during COVID-19

- Some patients reported dissatisfaction with the level of support from pharma companies, due in part to skepticism around the speed and profit motives surrounding vaccine development
- Some mentioned manufacturer supply issues due to redirected products to COVID-19 patients, leaving rare disease patients without essential medications

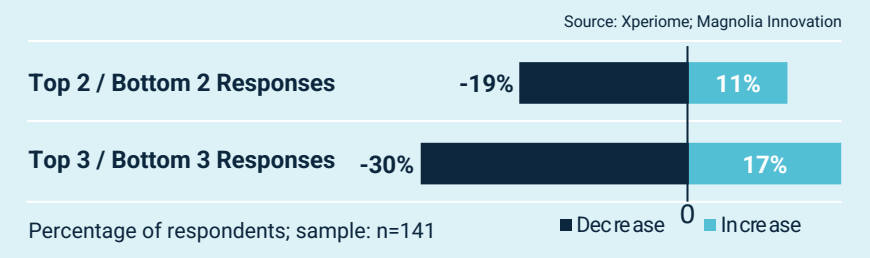

Figure 9: Satisfaction with COVID-19 support from pharma

Respondents were asked on a 7-point scale level of increased or decreased change (i.e., -3=decreased significantly, -2=decreased, -1=decreased slightly, 0=no change, 1=increased slightly, 2= increased, 3= increased significantly).
Top 2/bottom 2 box indicates % of respondents selecting 2/-2 ("increased"/"decreased") or 3/-3 ("increased significantly"/"decreased significantly").
Top 3 box indicates % of respondents indicating any level of increase or decrease.

"Many drugs are not easily available, so we have to settle for alternatives."

- Myasthenia Gravis Patient

"If they can come up with a vaccine in such a short time, why aren't there more cures for cancer and other diseases?"

- Myasthenia Gravis Patient

"[I want] more research and information about the relationship between immunosuppressants, COVID-19 vaccines and treatment options."

- Myasthenia Gravis Patient

Discussion

Opportunities for pharma and patient advocacy groups

Discussion

The COVID-19 pandemic has highlighted opportunities for pharma and patient advocacy groups (PAGs), working together, to grow their presence and demonstrate their commitment to rare disease patients.

Of all support sources, pharma and PAGs saw the least change in patients' trust. Those with better experiences and improved trust in pharma and PAGs tended to have conditions with fairly high visibility and relatively recent new drug approvals (e.g., Cystic Fibrosis, Idiopathic Pulmonary Fibrosis, Myasthenia Gravis, Thalassemia).

This indicates an opportunity to grow presence by supporting rare disease patients during the pandemic, fill the information void, and form a more positive reputation as a trusted resource, especially for less visible disease communities.

For some, the development of vaccines has created a halo effect of positive perceptions that extends beyond vaccine manufacturers, further providing an opportunity across the pharma industry to build on the changing perspective.

Opportunity

Bring together key stakeholders to extend patient support beyond pharma alone

Become a trusted source of information and support for rare disease patients

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Given increased uncertainty and decreased trust in other institutions during the pandemic, there is a clear opportunity for pharma to step in as a trusted source of information and support for rare disease patients by:

Broadening the understanding of how COVID-19 disease, treatments, and vaccines impact the rare disease population

- Invest in studies, real-world evidence, and publications focusing on rare disease patients
- Partner with patient support groups for further advocacy in the rare disease community
- Build trust through consistent messaging

Providing educational materials to support conversations around rare disease and COVID-19

- Direct patients to trusted sources or develop new materials to fill information gaps
- Provide materials to physicians to support patient conversations

Leading the conversation by reaching out to patients and physicians

- Engage with patients to understand their needs and concerns
- Design support services with rare disease patients in mind

Extending patient support beyond pharma

- Connect patients with holistic support services, such as family or mental health support

"Every company that I get medication from either sent email or sent things directly in the mail about COVID-19. I feel very supported and with sickle cell disease that has not always been the case."

- Sickle Cell Patient

Become a trusted source of information and support for rare disease patients

Partnering with other trusted organizations will enhance the value of support and potentially increase the level of trust from the rare disease community

Work with academic institutions to run and publish studies

- Enhance trust in studies by partnering with academia
- Opportunity to expand the breadth of research beyond traditional pharma-focused objectives

Partner with patient support groups for further advocacy in the rare disease community

- Opportunity for pharma/PAGs to work together and be proactive sources of information moving forward, driven by understanding the immediate concerns for patients as the healthcare landscape continues to evolve

Recognizing that many of these questions are difficult to address, a demonstrated effort and commitment to standing alongside rare disease patients in the journey to finding the answers is a first step in the right direction.

"Pharmaceutical companies have been stepping up to help fund national conventions."

- Sickle Cell Patient

"I generally expect [information] to flow through the Myasthenia Gravis Foundation and get sent out in things like their newsletters or on their website."

- Myasthenia Gravis Patient

About

About Xperiome

Xperiome is a global healthtech company that helps pharma get to know rare disease patients and their conditions faster by delivering deep insights into patient experience.

Our platform, Raremark, brings together thousands of patients and caregivers who live with any of the 7000+ rare diseases to connect with others, share experiences, and navigate every stage of their health journey.

This helps members better understand, manage, and talk about the condition that's part of their life and provides data-driven insights into the rare experience. Through these insights we are able to match research-ready members to clinical and real-world studies, faster and smarter than ever before.

Find out more at xperiome.com
contact@xperiome.com

About Magnolia Innovation

Magnolia Innovation is a healthcare research consultancy based in the United States.

Magnolia provides strategic research and analytics services to biopharmaceutical and health-tech companies with a particular focus on the rare disease space.

Our team of healthcare research analysts specialize in complex and rare diseases and aim to drive innovation in patient care through deep capabilities in product development, marketing and market research.

Find out more at magnoliainnovation.com