



COVID-19 COMMUNITY FOLLOW-UP SURVEY REPORT

92% of Rare Disease Patients Still Affected

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rarediseases.org

The National Organization for Rare Disorders (NORD®) is the leading independent advocacy organization representing over 25 million Americans affected by a rare disease. NORD is committed to the identification, treatment and cure of the more than 7,000 rare diseases, of which approximately 90% are still without an FDA-approved treatment or therapy.

NORD began as a small group of patient advocates that formed a coalition to unify and mobilize support to pass the Orphan Drug Act of 1983. For more than 37 years, NORD has led the way in voicing the needs of the rare disease community, driving supportive policies and education, advancing medical research and providing patient and family services for those who need them most. NORD is also home to over 300 disease-specific member organizations and their communities and collaborates with many other organizations on specific causes of importance to the rare disease community.

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INTRODUCTION

The COVID-19 pandemic has created tremendous hardship for the millions of Americans living with rare diseases. Six months into the public health crisis, 92% of people with rare diseases remain affected and 94% are worried.

These unprecedented times have upset the balance of a health care system that already did not work in favor of most people with rare diseases. Patients and families typically face an uphill battle trying to find a diagnosis; often encounter a lack of treatment options; experience the hope and precariousness of participating in research or clinical trials; and travel extensively to be seen by disease-specific experts—all in the hope of gaining some relief or chance at improved well-being.

On May 5, [NORD published findings](#) demonstrating that early in the pandemic people with rare diseases and their families were seeing tremendous disruptions to their health care and welfare. In this new report, responses were analyzed from a second NORD survey that was conducted the week of June 17 and some findings were compared across the two surveys. The results show how medical care for people with rare diseases continues to change with the pandemic.

Knowing the impact that COVID-19 is having on people with rare diseases helps NORD advocate for patients and answer ongoing questions, such as:

- Are people with rare disease still able to get the care they need?
- Are people with rare diseases disproportionately affected by the pandemic?
- Are new policies or programs needed to support patients, families and caregivers?

SNAPSHOT OF SURVEY AND RESPONDENTS

NORD's latest poll was conducted the week of June 17 at a time when many states were beginning to reopen and ease social distancing measures. A total of 833 individuals responded (71% self-reporting as patients; 26% family members of someone with a rare disease; 3% patient advocates/representatives), representing 48 states (Delaware and Hawaii were not represented). Approximately 80% of the patients represented in the survey responses were adult patients (Figure 1.1). For reference, the earlier survey was conducted the week of April 1 with 772 respondents (72% self-reporting as patients, the majority of whom were also adults). Thirty-six percent of respondents also participated in the first survey.

SUMMARY OF FINDINGS

COVID-19 is causing widespread effects for people with rare diseases, including:

- 79% have experienced canceled medical appointments
- 32% have had challenges accessing medical care and treatment
- 14% have had difficulties accessing medical supplies and devices, 68% of whom have had challenges accessing PPE
- 14% have experienced issues accessing medication for their rare disease, 12% of whom can no longer afford it
- 37% of households have been impacted by a loss of income
- 27% of households have experienced job loss
- 62% are concerned with medication supply shortages
- 9% of people who have lost jobs have also lost health insurance
- 6% are in households with someone who has been diagnosed with or has been suspected to have COVID-19

79% Have experienced **CANCELED** medical appointments

OBSTACLES TO MEDICAL CARE

Rare diseases do not stop in a public health crisis, yet patients have faced challenges accessing routine and specialized care. Overall, 79% of respondents have experienced canceled medical appointments, compared to 74% in April (Figure 1.2). Of those, 51% have had a medical appointment canceled by their provider.

A sampling of survey responses demonstrates the different reasons why appointments and procedures have been put on hold:

- "All therapies have been canceled."
- "[The] hospital canceled all visits to provide PPE to priority COVID hospitals."
- "I was afraid to go to the infusion center. I also could not have an Esophageal Dilation because it was considered a nonemergency surgery."

As the virus has continued to upend daily life in America, lack of reliable transportation¹ has emerged as another barrier to care, especially problematic for individuals with mobility challenges:

- "Heart ECHO postponed multiple times (no transport)."
- "Didn't seek treatment due to COVID-19 risk – no aide to assist (bedridden) and no transport (care manager paperwork wasn't filed)."
- "Ability to schedule and or drive to appointments impaired by having to accommodate household member's wildly fluctuating COVID-disrupted work schedules."

32% Have had challenges accessing medical care and treatment

Compounding these obstacles, anxiety over contracting COVID-19 is significant and 28% of respondents have canceled their own medical appointments.

Many of these respondents shared their fears about contracting the virus in a health care setting:

- “Hard to feel safe going in for lab work.”
- “Afraid to go to clinic for non-routine/non-emergency blood test.”
- “I have a number of health issues that need to be addressed but have put everything on hold because [I] don’t want to be in the hospital or have surgery (if they can do it) due to community acquired illness in hospital.”
- “Avoided treatment until couldn’t anymore.”

Some were so worried that they did not go to the emergency room when necessary. Among the 15% of respondents who needed to seek emergency room care related to their rare disease, nearly one-third (or 4% of all survey respondents) did not do so out of fear of contracting the virus. According to the data, 41% of these individuals have been extremely impacted by COVID-19. In one heart-breaking response, a person shared the loss of a spouse for this very reason: “My husband died, too scared to go to the hospital with his low immune system.”

The nature of certain rare diseases, as well as hospital visitor restrictions, have prevented patients from receiving the best care possible during the pandemic.

Survey participants told **NORD**:

- “COVID-19 means [I] cannot have an advocate with me at a hospital, making my situation even more dangerous.”
- “Patients with family members who lived in [the] same household should have had visitation rights to help with care.”
- “Knowing we are high risk for complications, not being able to be with family member in hospital, is an extra stress for deciding what appointments and treatments are worth the stress.”
- “Many people with an immune deficiency do not run fevers, including me. We do not mount enough of an immune response to cause fever... Please let people know not to make FEVER a primary criterion for COVID-19 testing.”

TELEMEDICINE

Amidst the rampant disruptions to traditional modes of care, telemedicine has emerged as a bright spot for many people with rare diseases as a way to safely and confidently access medical care without risking exposure to COVID-19.

Since the start of the outbreak, access to telemedicine has increased (Figure 1.3), in part due to advocacy efforts²:

- 83% of respondents have been offered telemedicine visits, compared to 59% in April
- 85% of individuals who experienced a canceled medical appointment were offered a telemedicine alternative, compared to 65% in April

To help understand the impact of telemedicine for the rare disease community, **NORD** asked participants about their experience and learned the following:

- 88% of those offered a telemedicine appointment accepted it, 92% of whom said it was a positive experience
- 70% of respondents would like the option of telemedicine for every appointment in the future
- 61% said that the primary reason that they would opt out of a telemedicine visit is that they prefer face-to-face interaction

Responses indicate that telehealth offers improved access to medical care and can enhance patients’ experiences, even after the COVID-19 crisis has ended:

- “Typically we have to travel over five hours (round trip) to see specialty doctors. Having a virtual appointment literally saved an entire day of travel with a two-year-old.”
- “If you could push for permanent telehealth options for Medicare patients, that would protect us now and in the future.”
- “I felt safe since I didn’t have to go into an office with sick patients.”
- “It was timely, allowing me to get back to work taking minimal time off.”
- “My daughter’s appointments at Boston Children’s were all canceled. Telehealth was very helpful as it allowed us to move forward with a trial drug therapy that would have been delayed another year despite her progressive decline in health.”

Despite potential benefits and the fact that nearly 90% of respondents accepted telemedicine when offered, the use of telemedicine nationwide has declined since

its peak in mid-April³. **NORD** has and will continue to advocate for people with rare diseases to have the best possible options and access to medical care⁴.

PPE AS A HOT BUTTON ISSUE

With anxiety raging over a second wave of COVID-19, diminished access to personal protective equipment has remained a concern for people with rare diseases. Nearly half (46%) of respondents needed PPE prior to COVID to help manage infection risks related to their rare disease, and almost 1 in 5 (18%) required PPE constantly.

Among the 14% of respondents who have had trouble accessing medical supplies and devices, PPE was by far their biggest challenge (Figure 1.4). Issues obtaining medical supplies and devices were reported widely, in 44 of the 48 states represented in the survey. Additionally, an association was found between challenges accessing PPE and greater levels of worry and impact (Figures 1.5a, 1.5b).

When asked to share other concerns or comments related to COVID-19, many respondents raised the topic of PPE and expressed fear over others’ lack of mask-wearing and how this could affect people with rare diseases:

- “I’m just generally concerned with the public’s apparent lack of consideration of at-risk populations when choosing not to follow public health precautions to limit the spread of SARS-CoV-2. I wish more people were aware of the risk they transfer to the rare-disease population.”
- “I have concerns about the spread of COVID-19 because many people do not wear masks and follow social distancing guidelines. This puts my life and lives of others at risk.”

¹ 4% of survey respondents reported that they rely on public transportation as their primary mode of transportation.

² “Telemedicine Letters to Governors.” Rare Action Network, July 21, 2020. <https://rareaction.org/policy/telemedicine-letters-to-governors>.

³ “This is supposed to be telemedicine’s time to shine. Why are doctors abandoning it?” STAT, June 25, 2020. <https://www.statnews.com/2020/06/25/telemedicine-time-to-shine-doctors-abandoning-it/>.

⁴ Readers’ advocacy can help with this issue. Those who are interested in sharing their telehealth experience may visit <https://rarediseases.org/covid-19/>.

- “Most people in my area refuse to wear masks. I wish they would so that I would feel more comfortable in venturing out.”
- “[I am concerned about] people that refuse to wear masks [and] social distance. We don’t want COVID. They don’t know if you are at a higher risk for getting it if you have a rare condition, etc. I’m a retired nurse and it’s very selfish.”
- “My husband is an essential worker so that increases my chances as well as his of gettin sick... It’s been very stressful for him and myself and we worry it’s going to get so much worse as most people in our area are not social distancing nor wearing masks or anything. Scary times.”

CHALLENGES ACCESSING MEDICATION

For the 14% who have experienced challenges accessing medication, the primary reason was due to shortages (Figure 1.6), with many people attributing this to their medicine/supplement having some potential applications for COVID:

- “It’s an OTC medicine that was mentioned as a potential COVID-19 medicine. The price has increased tenfold and it’s not readily available now.”
- “Once hydroxychloroquine [was called] a ‘miracle’ drug the pharmacy started running short on supply. I depend on that medicine to live. It took many phone calls both to pharmacies and to our insurance to get that squared away...Not only that, the price went up on the medicine. It was finally remedied, but it took hours and hours of phone calls and more than seven days to figure out.”
- “I take a mito cocktail and many of the vitamins are sold out and are no longer obtainable online.”

Further, a new trend emerged in the open-ended responses revealing that mail delays are an increasingly common problem for obtaining medication:

- “Mail order medication taking longer to receive.”
- “Delays with supply/shipment.”

- “Delivery has been very delayed from out of state.”
- “Supplies from online sources [and] shipping [is] very slow.”
- “Pharmacy work around is FedEx with [a] week delay generally.”
- “Pharmacy order VERY delayed due to COVID.”
- “Ships from overseas [with] delays and price increases.”
- “Difficulty with transportation of medication and supplies due to virus.”

MENTAL HEALTH AND ISOLATION

Months of social distancing and lockdown measures have compounded the feelings of isolation experienced by many in the rare disease community—which stem from the nature of having a medical condition held by few, and where patients and caregivers frequently encounter knowledge gaps about their disease and its progression.

Participants shared:

- “I have not left my house since February. It is extremely lonely! My family is afraid to visit because of my health and no one wants to take the chance of bringing COVID into my home. One daughter does come once a week. The first wave of this is not over and they talk about the second wave! I have difficulty walking and spend my days in bed as it is. Very lonely.”
- “I’m terrified at the blasé attitude of most people under 65 concerning wearing masks and social distancing. I am at severe risk of hospitalization, intubation and death if I contract COVID-19. I have been in home isolation since the beginning. I haven’t seen any friends and my son at a distance twice, briefly. This is killing me emotionally.”
- “Being quarantined at home has been very hard on the individual with the rare disease due to depression and no motivation to exercise at home.”
- “Family and friends do not understand why I have to cancel plans due to COVID.”

- “I worry about the mental health of my five-person family due to ‘safer at home’ recommendations that we have been closely following.”
- “The level of isolation required to stay safe or feel like you can be safe takes a HUGE toll on the patient and their families, physically, emotionally and financially.”

ECONOMIC INSTABILITY

Adding to the emotional stress is financial concern. Many said that they will need to make a choice between personal health and economic stability. Among households represented in the survey, 37% had been impacted by a loss of income and 27% had experienced job loss already.

Respondents shared:

- “I am a nurse and have been told I am too high risk to return to work until mass vaccinations happen. My unemployment will run out in the fall. Not sure what I will do at that point. May need to leave the profession.”
- “I am worried about [being] forced to make a choice in September whether to go back to work and risk my health, or lose my job. I work in special education where close contact is necessary.”
- “Pending request to continue to work from home. If denied, will go out on leave and lose income.”
- “And while I’m fortunate to be able to work from home, many high-risk people are being advised to stay home at the same time their employers are requiring them to return to work. I suppose I’m preaching to the choir here, but I hope NORD can share the message that chronically ill and high-risk people are concerned about reopening when there remains no vaccine and no treatment for COVID-19.”

ANSWERING THE UNKNOWN

Patients and caregivers told NORD that they need more information about how COVID-19 affects people with rare diseases before they can feel safe and secure about the immediate future. While this information is starting to emerge for cancer⁵ (many forms of which are rare), more data is needed for all rare diseases.

Community members said:

- “COVID info is so general, our kids are complex. Access to info for kids with multiple conditions across multiple specialties is difficult. How do we synthesize the data??”
- “There is almost no information as to what effect COVID-19 has on patients with rare medical conditions. Publishing some statistics on this would be extremely helpful so we can determine how concerned we need to be.”
- “How much does having a rare disease increase risk for COVID-19?”
- “Now that the U.S. is reopening, it’s harder to find guidance for high risk individuals. Should high risk individuals continue to stay at home if infection rates are fairly low? Can they begin to resume some normal activity with others outside their home with certain safety measures? Can high risk children safely return to school?”
- “It had been difficult to know what information about COVID is accurate. Also, as the world begins to reopen, I am not sure what to do because I am in the “high risk” category...very unsure how to proceed now. It was actually easier when we all had to follow the same restrictions.”

⁵ “For those at risk for cancer, weighing the risk of covid-19 with delays in screening and treatment.” Washington Post, July 19, 2020. https://www.washingtonpost.com/health/for-those-at-risk-for-cancer-weighing-the-risk-of-covid-19-with-delays-in-screening-and-treatment/2020/07/17/0707ea72-c13c-11ea-b4f6-cb39cd8940fb_story.html.

Participants also reported that they have encountered conflicting information about the virus itself that has not been helpful in trying to manage their health outcomes:

- “There is often conflicting information from resources (even state resources) about actual COVID numbers, cases, facts and necessary precautions.”
- “[I am concerned about] the constant change in the dos and don'ts of everything.”
- “My biggest concern is finding unbiased information regarding staying healthy when communicable diseases, such as COVID arise.”
- “Media coverage was very political and slanted, this is upsetting and needlessly upsetting to people already living stressful lives.”

- “Although I realize that this is a serious and very real condition, I sometimes wonder about the role that politics has played and has continued to play in the reporting and restrictions. The 'experts' contradict themselves frequently and reporters rarely report all of the numbers. Rarely do you hear of how many have recovered.”

With so many unknowns, how do we ensure that people with rare diseases – who make up nearly 10% of the U.S. population – continue to get proper care, even during a public health crisis? NORD's team is committed to helping those in the rare community as the COVID-19 crisis unfolds.



FIGURES

Figure 1.1
Most of the rare disease patients represented in the survey were adults.

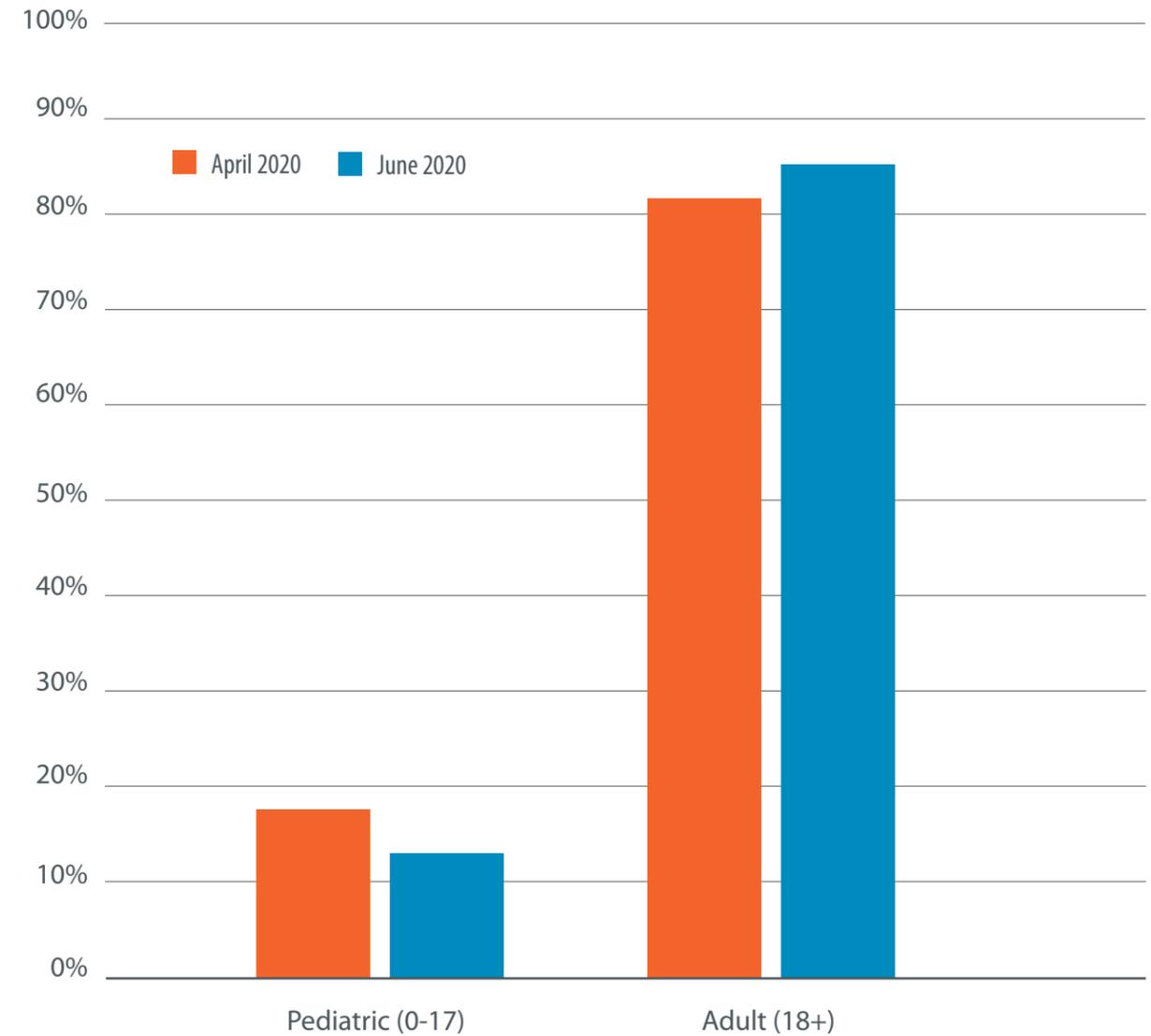


Figure 1.2
In the first six months of the COVID-19 pandemic, 79% of people with rare diseases have had a medical appointment canceled.

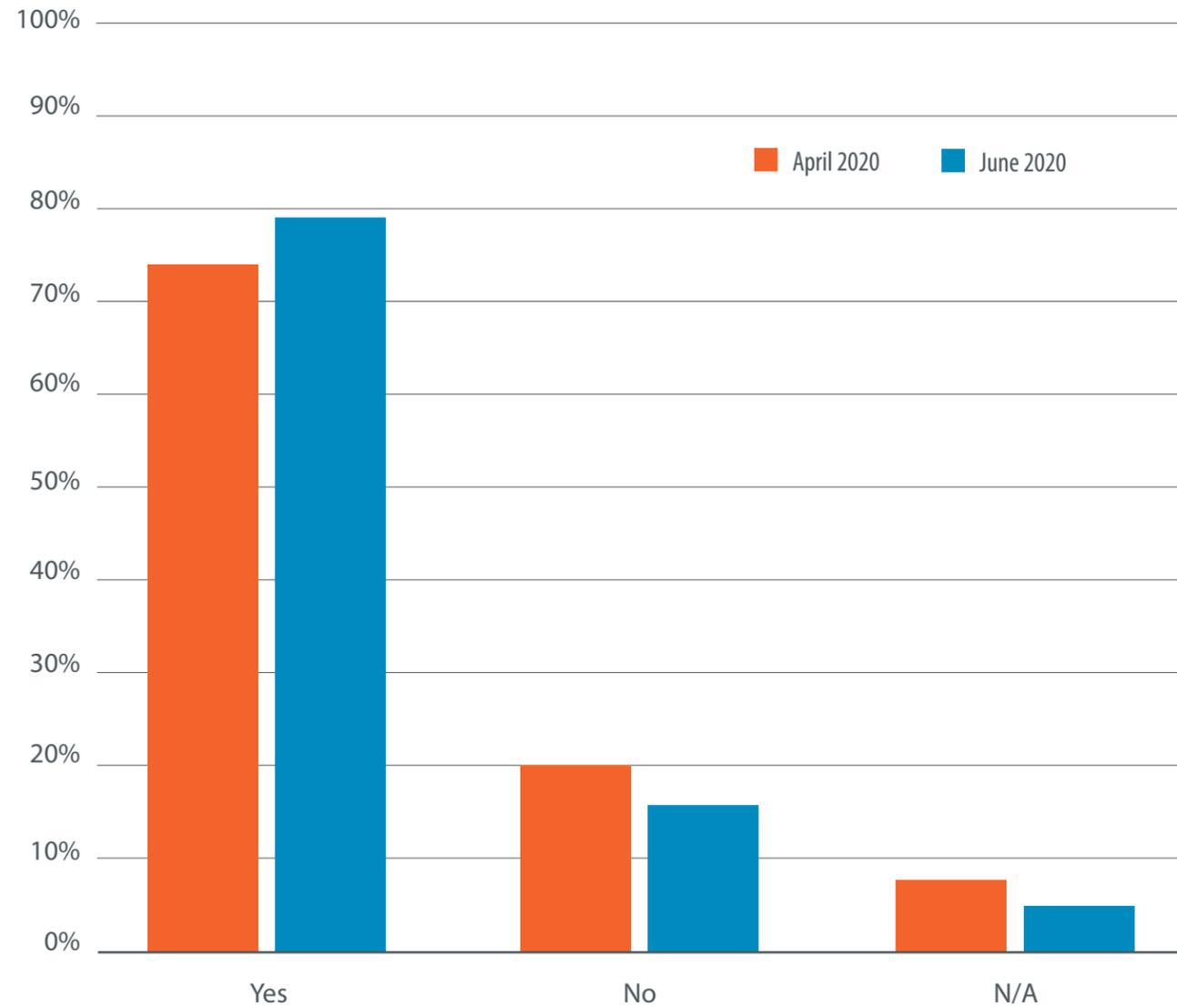


Figure 1.3
For people with rare diseases, access to telemedicine has increased since the start of the COVID-19 pandemic.

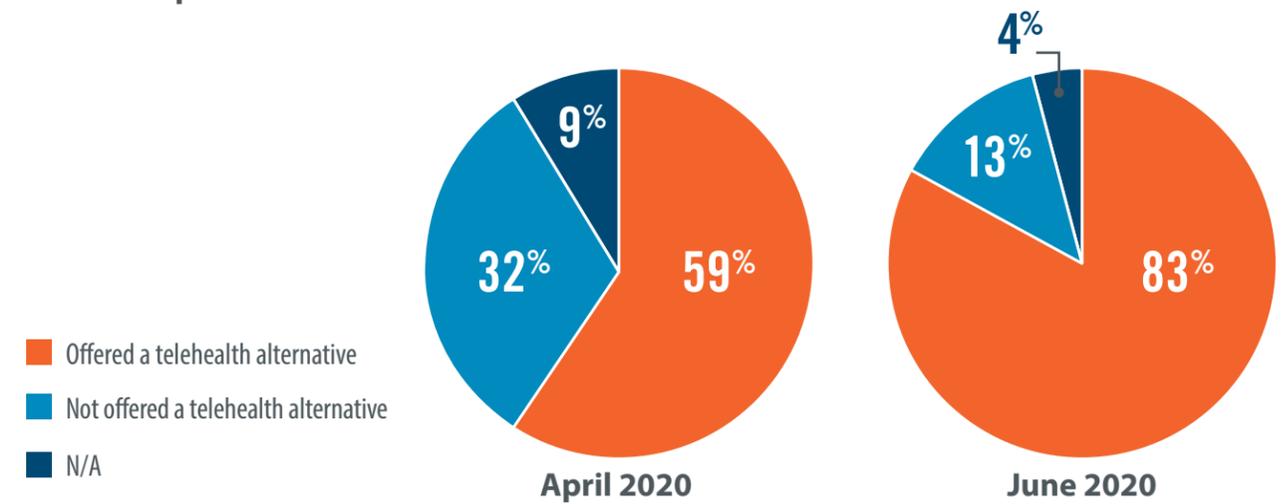


Figure 1.4
During COVID-19, 14% of respondents have had challenges with accessing medical supplies or devices for a rare disease. The challenges have remained relatively consistent since the start of the pandemic.

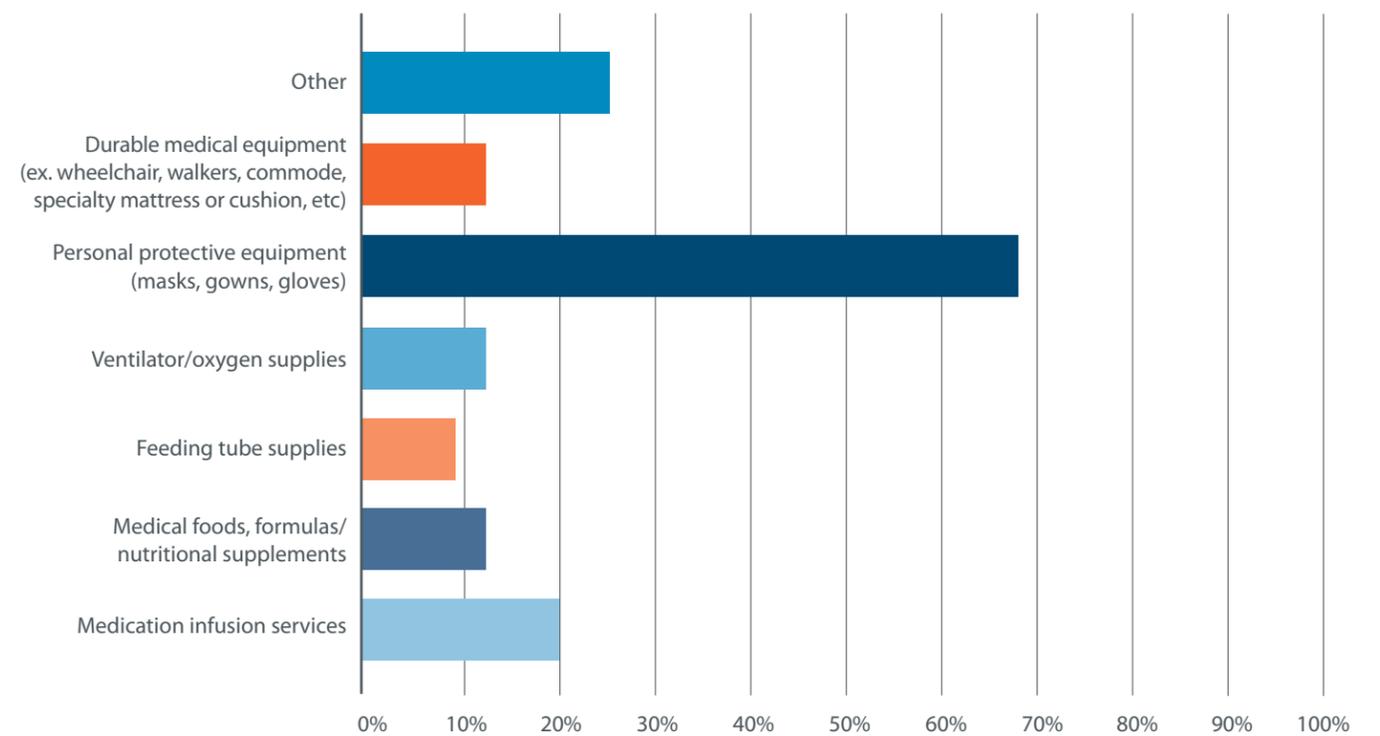


Figure 1.5a
 Prior to COVID-19, 46% of participants required PPE to help manage infection risks related to their rare disease. An association was found between being worried about COVID-19 and this reliance on PPE.

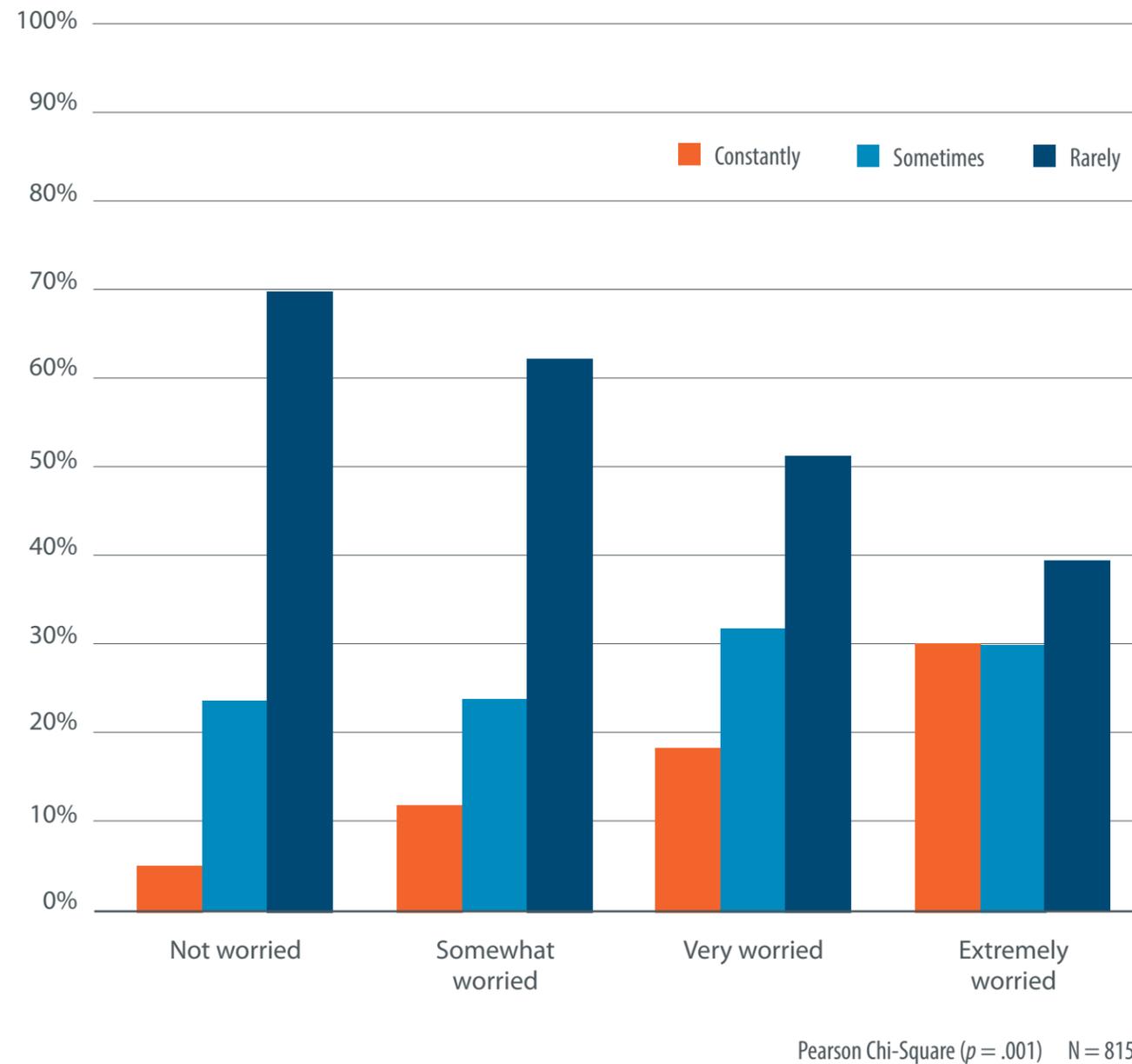


Figure 1.5b
 As association was also found between being impacted by COVID-19 and a pre-pandemic reliance on PPE.

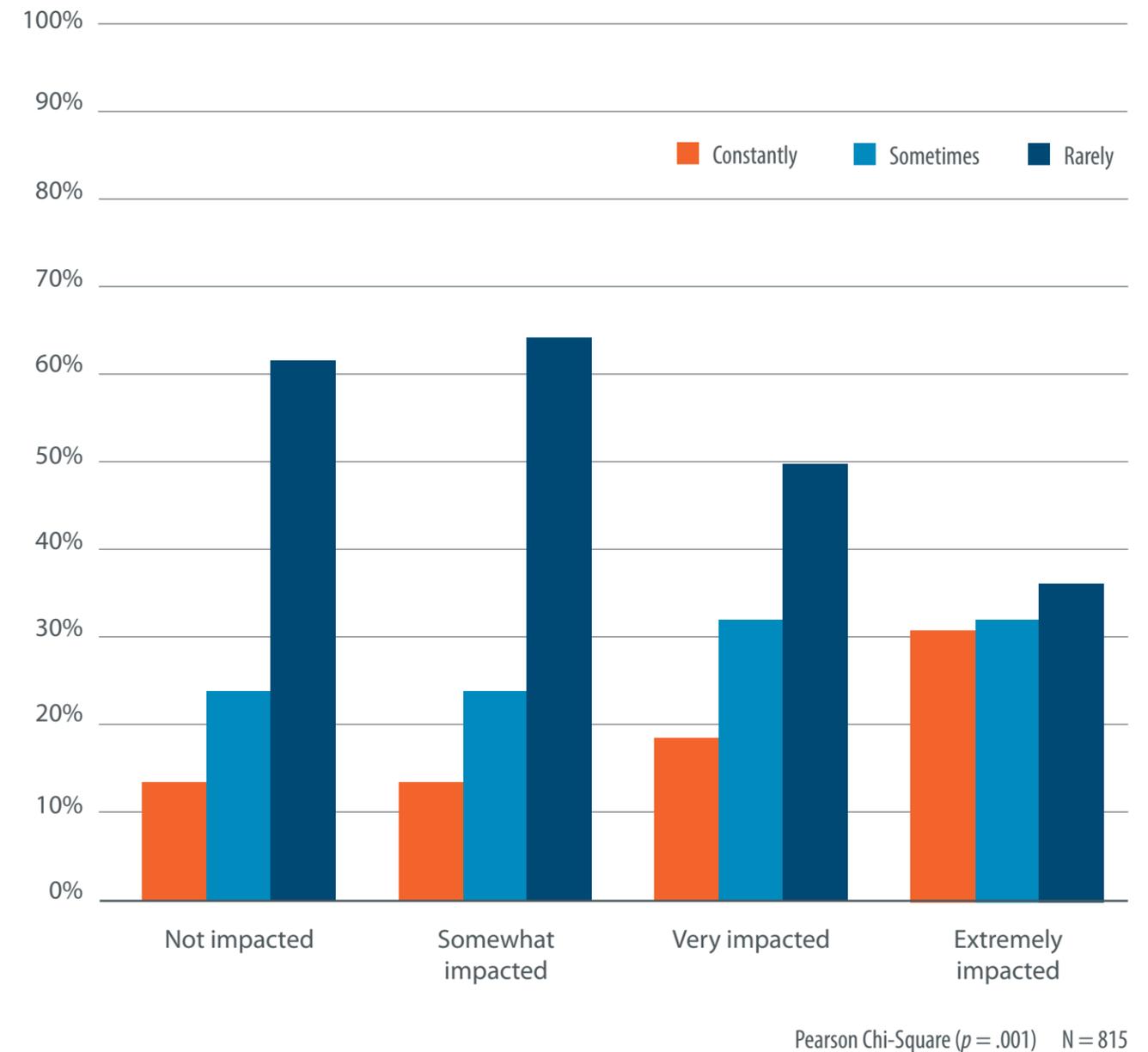
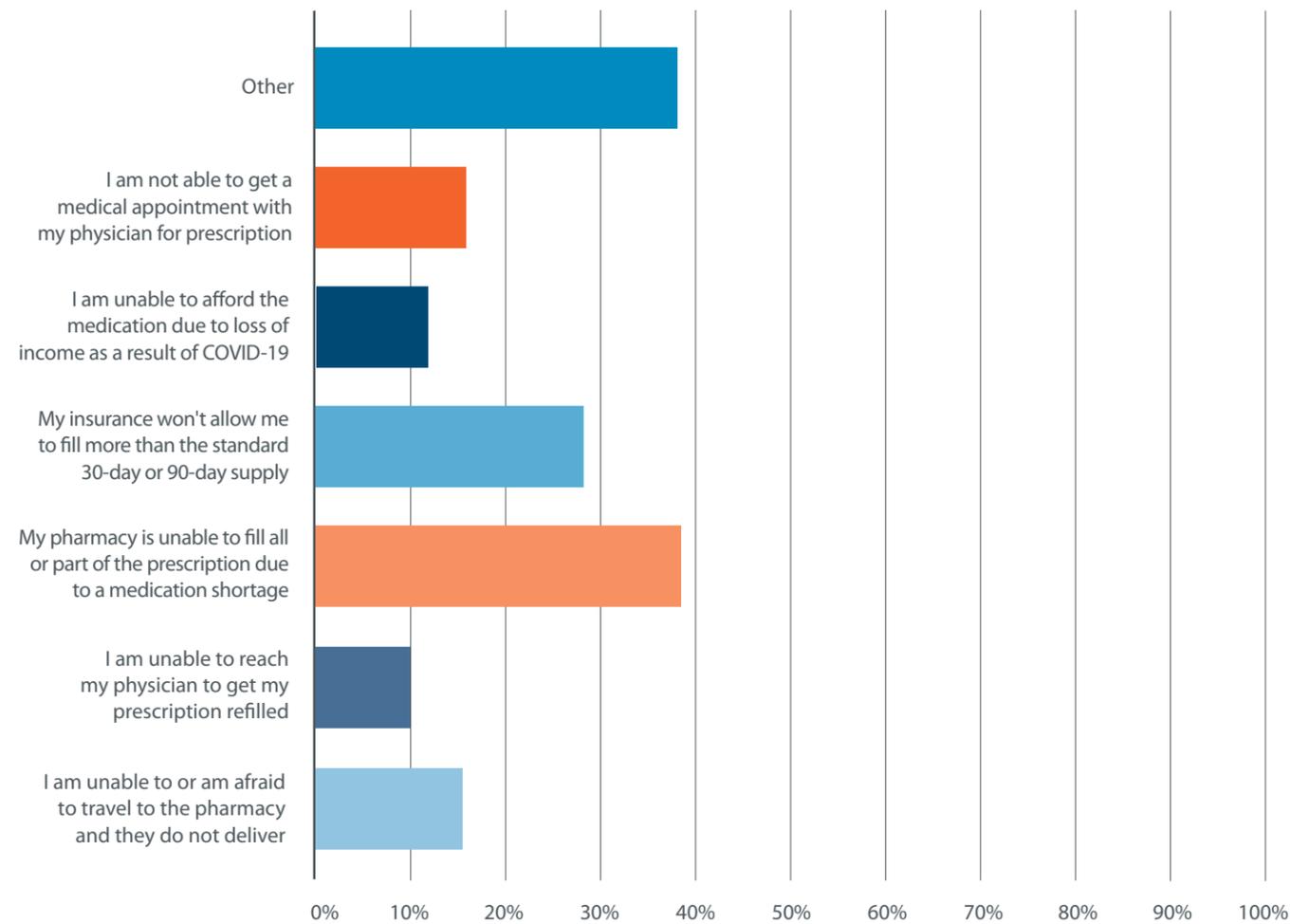


Figure 1.6
During COVID-19, 14% of participants reported challenges with getting medication for their rare disease.



RESOURCES

As part of responding to the unprecedented and evolving COVID-19 situation, NORD has developed the NORD COVID-19 Resource Center (rarediseases.org/covid-19/), offering resources to help the rare disease community, to address concerns raised by survey respondents and to share real-time updates on actions NORD has taken in response to the pandemic. Our team is working to address as many concerns as possible while also continuing NORD's other vital programs and services.

Readers of this report are encouraged to take action by joining NORD in advocacy efforts to call on lawmakers to prioritize public health in COVID-19 response legislation, to share any other concerns with respect to the COVID-19 pandemic via this [form](#), and to visit the NORD COVID-19 Resource Center for future updates.

NORD's lines are always open (rarediseases.org; orphan@rarediseases.org; 203-744-0100).

ACKNOWLEDGEMENTS

Thank you to the patients, families, caregivers and patient advocates for participating in this survey.

ABOUT RAREINSIGHTS

RareInsights™ is a NORD initiative to expand public knowledge of rare diseases and translate that knowledge into real-world solutions for patients and families.

Through this initiative, NORD is commissioning and undertaking a broad range of projects to collect and analyze empirical data for next-generation advocacy that is patient-centered and data-driven. Information is shared with the community in a variety of accessible formats, including reports, white papers, infographics, fact sheets, and more.