ENSURING ACCESS TO TELEHEALTH FOR RARE DISEASES

NORD® RARE INSIGHTS®

For more information, please contact: orphan@rarediseases.org
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.

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.

Our Mission 2
About RareInsights® 2
Telehealth Can Help Transform the Rare Disease Patient Experience 3
Policy Developments 5
What Can You Do to Help? 6

For the more than 25 million Americans with rare and undiagnosed diseases, having consistent access to health care providers is critical to their immediate and long-term health. Throughout the coronavirus pandemic, nearly 8 in 10 rare disease patients experienced canceled medical appointments.[1] Fortunately, during this time period, telehealth emerged as an increasingly common way for people to safely access medical care without risking exposure to the virus. Going forward, the majority of rare disease patients[2] say that they want the option of telehealth for their medical appointments.

For many rare diseases, there are only a handful of specialists nationwide, or even worldwide, who have expertise in that condition. As a result, patients often are forced to travel long distances to access their treating providers. Such travel involves significant expenses, time off from work and logistical challenges. For rare disease patients who are immunocompromised, it may not even be safe for them to travel these distances.

Recent surveys conducted by the National Organization for Rare Disorders (NORD) found that out of over 800 rare disease patients and caregivers, more than 83% had been offered a telehealth visit during the COVID-19 pandemic by their health provider and, of those who were offered a visit, 88% accepted.[3] Out of all patients who reported having had a telehealth visit, 92% described it as a positive experience, and 70% of respondents would like the option of telehealth for future medical appointments.[4] Sixty-one percent said that the primary reason that they would opt out of a telemedicine visit is that they prefer face-to-face interaction.[5]

88% offered a telehealth appointment during COVID-19 accepted it, 92% of whom said it was positive experience[3]

70% would like the option of telehealth for medical appointments in the future[4]

NORD has been hard at work finding ways to ensure that people with rare diseases have continued access to telehealth throughout the pandemic and beyond. Further, as part of our efforts to advance understanding of the rare disease patient experience through data under our RareInsights® initiative, we analyzed findings from NORD's recent research, including our *Barriers and Facilitators to Rare Disease Diagnosis, Care and Treatment: 30-year Follow-up* survey and two *COVID-19 Community* surveys, in conjunction with publicly-available data, to better understand and quantify the opportunities of telehealth.

TELEHEALTH CAN HELP TRANSFORM THE RARE DISEASE PATIENT EXPERIENCE

Why is access to telehealth so critical for people with rare diseases? First, patient safety is an acute concern. Many individuals living with rare diseases are immunocompromised, putting them at greater risk for complications from COVID-19 and other illnesses.

[1] "NORD COVID-19 Community Follow-Up Survey Report." National Organization for Rare Disorders, August 4, 2020. https://rarediseases.org/wp-content/uploads/2020/09/NRD-2061-RareInsights-CV19-Report-2_FNL-1.pdf

[2] *Ibid.* [3] *Ibid.* [4] *Ibid.* [5] *Ibid.*

Second, in the case of many rare diseases, there few providers who are experts in the disease and there can be a significant distance between where a patient lives and where their health care provider practices, leaving patients and often their family caregivers no choice but to take time off work to travel to see that provider. NORD's recent *Barriers and Facilitators to Rare Disease Diagnosis, Care and Treatment: 30-year Follow-up* survey found that 62% of patients have been prevented from attending work and 26% of children have missed school as a result of their rare disease.[6] Thirty-nine percent of patients travel at least 60 miles to receive medical care.[7] The burden of travel is so great that 17% have moved (or are considering relocation) to be closer to treatment to manage their rare diseases over the long-term.[8]

62% of patients have been prevented from attending work due to their rare disease.[6]

26% of children have missed school as a result of their rare disease.[6]

These concerns and challenges have been highlighted by the hundreds of rare disease patients and caregivers who have told NORD about the positive impact that telemedicine has had on their lives during the pandemic, sharing feedback such as:

- "It was timely, allowing me to get back to work taking minimal time off."

39% of patients travel at least 60 miles to receive medical care.[7]

- "We didn't have to drive 6.5 hours in one day and were able to do it in our living room. It was AWESOME! We have been able to get more doctors' appointments in because they were doing telehealth. We LOVE IT!"
- "It was great seeing my doctors without needing to go in. I have had more doctors' appointments since COVID due to the accessibility of telemedicine."
- "I did not have to drive to my doctor's office and leave my chronically ill husband for several hours."
- "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."
- "I felt safe since I didn't have to go into an office with sick patients."
- "If you could push for permanent telehealth options for Medicare patients, that would protect us now and in the future."
- "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."

Opportunities likewise exist for participation in clinical trials. For many rare disease patients, it can be a struggle to participate in clinical trials that are distant from their homes and this can have negative consequences on clinical trial adherence. At the same time, patients' interest in clinical trials is at an all-time high. Nearly nine out of 10 (88%) rare disease patients would consider using an investigational treatment, compared to 62% of patients 30 years ago.[9] Clinical trials are necessary and important, as approximately 90% of the 7,000 known rare diseases do not yet have an FDA-approved treatment.

88% of rare disease patients would consider using an investigational treatment.[9]

POLICY DEVELOPMENTS

In response to the clear call from the rare disease community reflected in our surveys, NORD's policy team went right to work advocating for immediate access to telehealth in the context of the COVID-19 public health emergency.

While the concept of telehealth is not a new development, telehealth visits accounted for less than 0.01% of total health care visits across the US before the COVID-19 public health emergency declaration.[10] By mid-April, that number had increased to 69%.[11] According to data from the Centers for Medicare & Medicaid Services (CMS), prior to the pandemic, 14,000 beneficiaries received a Medicare telehealth service each week, yet more than 10.1 million beneficiaries utilized telehealth between mid-March and early-July.[12]

In response to the pandemic, starting in early March, the federal government took important steps to temporarily expand coverage for telehealth services. This included removing barriers that had previously prohibited Medicare patients from utilizing telehealth services from the safety of their homes and allowing telehealth services only for those patients in designated rural areas. Additionally, a blanket waiver was issued permitting all physicians participating in federal health care programs (Medicare, Medicaid, TRICARE and Veterans Affairs programs) to receive payment for telemedicine services in states where they did not hold a license. Two weeks later, the US Department of Health and Human Services (HHS) Secretary Alex Azar reached out to all Governors recommending that they allow health professionals licensed or certified elsewhere in the US to practice in their states. In response to these actions by the federal government, combined with advocacy from patient groups including NORD, most states[13] modified their state licensure requirements to allow out-of-state health care providers in good standing to practice medicine, including telehealth, across state lines. Many private insurers have also temporarily expanded access to telehealth services and often reduced patient cost-sharing associated with these services to help keep patients healthy and safe at home.

As a result, people with rare diseases have had much better access to critical telemedicine services during this extraordinary time.

6 "Barriers and Facilitators to Rare Disease Diagnosis, Care and Treatment: 30-year Follow-up." National Organization for Rare Disorders, 2020.
7 *Ibid.*
8 *Ibid.*
9 *Ibid.*
10 "Telehealth: Fad or the Future." Epic Health Research Network, August 18, 2020. https://www.ehrn.org/telehealth-fad-or-the-future/.
11 *Ibid.*
12 "Trump Administration Proposes to Expand Telehealth Benefits Permanently for Medicare Beneficiaries Beyond the COVID-19 Public Health Emergency and Advances Access to Care in Rural Areas." U.S. Centers for Medicare & Medicaid, August 3, 2020. https://www.cms.gov/newsroom/press-releases/trump-administration-proposes-expand-telehealth-benefits-permanently-medicare-beneficiaries-beyond.
13 "Telemedicine Letters to Governors." Rare Action Network, September 29, 2020. https://rareaction.org/policy/telemedicine-letters-to-governors.

Currently, the Trump Administration, Congress and state governments are trying to decide which changes to telehealth should be made permanent beyond the COVID-19 public health emergency. Taking our cue from rare disease patients and providers, NORD drafted telehealth principles to shape our support or opposition to the numerous permanent changes being considered. We also joined forces with 34 other patient organizations to release coalition telehealth principles to guide policymakers seeking to ensure millions of patients with pre-existing and chronic conditions can continue to safely access appropriate telehealth services during and after the COVID-19 public health emergency. NORD has endorsed certain federal legislation[14,15,16] and has been tracking and engaging on more than 80 bills around the country related to COVID-19.

Read NORD's policy statements related to COVID-19.

WHAT CAN YOU DO TO HELP?

Many telehealth improvements are time-limited to the COVID-19 public health emergency declaration, however, our continued advocacy can help on this issue so that the positive changes that we have seen during the pandemic will endure after it ends. **Share your COVID-19 telehealth experience with NORD** and/or **send a message to your Senators and Representative asking them to protect rare disease patients in the next coronavirus relief package.** Together, we can fight to ensure that telehealth opportunities are equally and appropriately available to the benefit and not detriment of rare disease patients.

[14] "NORD's Letter of Endorsement for S. 4421, the Temporary Reciprocity to Ensure Access to Treatment (TREAT) Act." National Organization for Rare Disorders, August 12, 2020. https://rarediseases.org/wp-content/uploads/2020/08/NORD-2020-Letter-of-Support-for-S.-4421-the-TREAT-Act.pdf August 12.

[15] "Coalition Letter to Congress in Support of the Protecting Access to Post-COVID-19 Telehealth Act of 2020 (H.R. 7663)." National Organization for Rare Disorders, August 3, 2020. https://rarediseases.org/wp-content/uploads/2020/08/NORD-2020-HR-7663-Endorsement.pdf.

[16] "NORD's Letter of Endorsement for H.R. 7078, the Evaluating Disparities and Outcomes of Telehealth During the COVID-19 Emergency Act." National Organization for Rare Disorders, August 12, 2020. https://rarediseases.org/wp-content/uploads/2020/08/NORD-2020-Letter-of-Support-for-H.R.-7078.pdf.

©2020 NORD. All rights reserved. NORD, its icon and Rare Insights are registered trademarks of The National Organization for Rare Disorders. Image by Free Pik. NORD is a registered 501(c)(3) organization. NRD-2098

NORD® National Organization for Rare Disorders