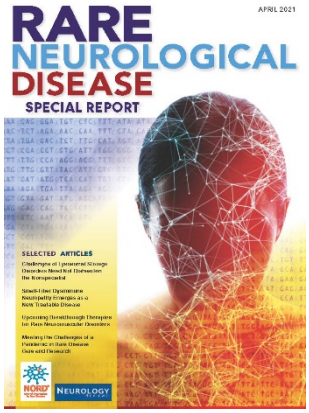
Neurology Reviews and NORD publish their annual Rare Neurological Disease Special Report

The 7th edition of the annual report

Parsippany, NJ – June 07, 2021 – Frontline Medical Communications (FMC) and the National Organization for Rare Disorders (NORD)® announce the release of the 7th annual ***Rare Neurological Disease Special Report***. Despite the difficulties brought on by the pandemic, science continued behind-the-scenes headway within rare diseases, and we are proud to bring the spotlight back to this unheralded progress. From the identification of genetic markers that improve diagnostics to potential and newly available treatments in the neurology and neuromuscular space, research and technology are able to find ways to meet, and work around, the many challenges posed by the COVID-19 outbreak.



The **NIH Rare Diseases Clinical Research Network (RDCRN**) summary shares insights gained from the shifting research environment induced by the pandemic. Some research was halted as a result of the impracticalities of in-person visits, staffing cuts, and strained fundraising efforts due to social-distancing restrictions, yet all constituent reports found a common "silver-lining" thread. Technologies stepped up to improve telemedicine and videoconferencing while in-home services helped increase sample collection efforts to spare the patient and caregiver a trip to the clinic. Many organizations used these telehealth advances to pivot toward improving patient outreach and widening much-needed diversity to bolster future study participation. As Glenn S. Williams, group editor for *Neurology Reviews* and *MDedge Neurology*, states, overcoming barriers is something "the rare disease community knows all too well."

This issue is a testament to continued evolutions in the rare disease world, with articles underscoring a new genetic treatment target in one subtype of **amyotrophic lateral sclerosis (ALS)** and additional discoveries of possible gene-based causes for other types, as well as new developments for **neuromyelitis optica spectrum disorder (NMOSD)**. An interview with the Muscular Dystrophy Association's chief research officer and executive vice president, Sharon Hesterlee, PhD, provides an in-depth discussion regarding **Duchenne muscular dystrophy (DMD)** diagnostics, prognosis, treatments in the pipeline, and the outlook for gene therapy. Attention is also drawn to new disease findings, such as **small-fiber dysimmune neuropathy** and **molybdenum cofactor deficiency**, and the value of **newborn screening (NBS)** programs. Despite the slowdown of clinical trials as sites shifted to COVID-19 patient care, one review of promising neuromuscular disease therapies notes an uptick in active trials beginning in the fall of 2020, and highlights some novel options approved by the FDA *during* the pandemic for **ALS, DMD, myasthenia gravis, and mitochondrial disease**. Additionally, reports indicate a host of recently approved treatments and trials of new therapeutic targets for **rare epilepsy syndromes**. An included supplement addresses gene therapy in monogenic neurodegenerative disease, balanced later in the issue with input by four experts on the ethics and reality of gene therapy.

NORD has not wavered in its commitment to advocacy, patient services, and education in the face of hardships brought on by the epidemic. As Peter Saltonstall, president and CEO, affirms, its website (www.rarediseases.org) continues to bolster support through provision of on-demand CME programs and the Rare Disease Video Library. The **Living Rare, Living Stronger Patient and Family Forum** is scheduled for June 2021 as a virtual event, as is this year's ***Rare Diseases and Orphan Products Breakthrough Summit*** in October. New on the docket is a newsletter geared for healthcare professionals set to launch later this year.

To learn more about the rare disease initiatives of *Neurology Reviews* and the NORD partnership, contact Elizabeth Katz at 973-224-7951 or ekatz@mdedge.com. Details and information on all FMC/MDedge digital brands, print publications, and custom programs are available at www.mdedge.com/neurology; visit weekly for the latest innovative programs and multimedia initiatives.

About *Neurology Reviews* and *MDedge Neurology*

Launched in 1993, *Neurology Reviews®* is the first and original news source in neurology. *Neurology Reviews®* has a 27-year history of providing independent, unbiased news to neurologists and clinicians interested in the neurosciences. *Neurology Reviews®* covers medical conferences and clinical research findings, as well as specialty trends, expert opinions, and the breadth of influences affecting the practice of neurology. Experienced medical journalists deliver timely, relevant, and insightful news affecting the practice of neurology and all its subspecialties. In addition to the monthly print issue reaching nearly 25,000 neurologists and clinicians interested in neuroscience, the *Neurology Reviews®* website ***https://www.mdedge.com/neurology***, part of the **MDedge™** web portal, features online ahead of print conference reporting, audio and video interviews, disease-specific microsites, self-assessment quizzes, supplements, sponsored educational programs, a calendar of relevant medical meetings, and a career center listing job openings around the country. *Neurology Reviews®* provides its content in print, through an app, on a mobile-friendly website, in digital editions, and through targeted e-blasts.

About the National Organization for Rare Disorders (NORD)®

The National Organization for Rare Disorders (NORD)® is the leading independent advocacy organization representing all patients and families affected by rare diseases. NORD is committed to the identification, treatment, and cure of the more than 7,000 rare diseases that affect 25 to 30 million Americans. NORD began as a small group of patient advocates who formed a coalition to unify and mobilize support to pass the Orphan Drug Act of 1983. For 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 represents more than 300 disease-specific member organizations and collaborates globally to raise awareness around rare disease and advance access to treatments for our community. For more information, see **https://rarediseases.org/.**

About Frontline Medical Communications

Frontline Medical Communications Inc. (FMC) is one of the healthcare industry's largest medical communications companies and a leader in digital, print, and live events. The company leads in HCP-level targeting and is ranked 1st in print reach and 2nd in combined web and print engagements. With **MDedge®**, our state-of-the-art integrated web portal and audited email database, FMC meets the marketing challenges of our clients with superior reach, optimal sponsorship opportunities, and flexible advertising programs. We reach 1.2 million+ physicians, NPs, PAs, HCPs, and key healthcare decision makers through more than 30 legacy media brands serving 20 distinct markets, who access our content through an array of digital, print, and face-to-face channels and social media platforms. FMC delivers award-winning, indexed clinical reviews; practice and policy information; and medical news daily from on-site reporting at major medical meetings, many in collaboration with notable societies, medical associations, and opinion leaders. FMC produces live events, and CME in affiliation with Global Academy for Medical Education, LLC (globalacademycme.com).

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