

# Navigating Complicated Ethics in Neuromuscular Disease Care

Working with patients with neuromuscular diseases (NMDs) often involves considering important ethical issues. We spoke with John Brandsema, MD, a neurologist and Neuromuscular Section Head at Children's Hospital of Philadelphia, for insight. He identified four key areas where NMD providers are commonly confronted with ethical questions.

## 1. Gene therapy

The [promise of genetically-targeted therapies](#) is a reason for optimism, but Dr. Brandsema also points out that medical professionals don't know all the short- and long-term effects of these novel therapies.

"Gene transfer isn't something we can reverse or stop like other treatments," he says. "Once the therapy is given, it's given."

Dr. Brandsema recommends having open conversations with patients and families who are interested in these therapies and sharing with them all the available information. "There can be complications, and we need to talk about possible risks and unknowns, such as durability of effect, not just possible benefits," he says.

## 2. Decision-making

When should somebody other than the patient make medical decisions? It depends on the circumstances.

"There's a spectrum across the lifespan of how people experience neuromuscular disorders," Dr. Brandsema says. Most patients don't want to lose autonomy, and it's important to respect their opinions and abilities while assessing whether aspects of their condition, such as cognitive impairment, could affect their decision-making. "Sometimes you need to involve their family and caregivers," he says. "It's important to assess the situation properly."

For children, as they age, providers should make efforts to bring them into the decision-making. "If they're old enough developmentally, they should have a voice, too," Dr. Brandsema says.

He recommends explaining treatments, clinical decisions, and clinical trials to young patients in age-appropriate ways and asking for their opinions. "The voice of the patient shouldn't only be heard if they are an adult," he says.

## 3. Clinical trial inclusiveness

One of the key ethical questions when it comes to clinical trial participation: What's being done to ensure all who qualify can participate?

Patients and their caregivers can stay up-to-date on clinical trials through online resources (e.g., [ClinicalTrials.gov](#)), and many disease advocacy groups have resources available to aid families in understanding the various research studies that are actively recruiting subjects. For example, patients can visit MDA's Clinical Trials page ([mda.org/clinical-trials](#)) to find frequently asked questions and tips on preparing for a clinical trial, as well as a clinical trial finder tool.

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Neuromuscular providers who aren't actively participating in research trials optimally are aware of the landscape so that they can counsel their patients about opportunities that may be available to them.

"We have to strive for more fairness in access to trials and we have an obligation to devote ourselves to overcoming barriers, whether it's getting to a facility, a language barrier, or other issues," Dr. Brandsema says. "The key word we should be thinking of is access. We need to address the barriers one by one to see if we can eliminate them."

## 4. End-of-life decisions

End-of-life decisions come with many ethical questions, but perhaps one of the most unique to each person living with neuromuscular disease is the optimal timing of discussing it.

"You don't want it to be a constant discussion at every visit, but having such discussions when someone is relatively well is often more successful than delaying until such decisions need to be made in emergent situations, especially if it includes a level of illness that makes the person unable to contribute to decision-making," Dr. Brandsema says. "Take stock of what collaboration is needed on an end-of-life decision: Who are the voices that need to be heard for the person living with the disease? Involve resources such as palliative care consultation when appropriate."

Keep in mind that no matter what issue you face in your practice, there's probably someone out there who has encountered it before. "With ethical questions, if you're unsure, consider talking with colleagues, including ethics teams commonly available at many Care Centers," Dr. Brandsema says. "Getting an outside perspective can help you see something more clearly, and it's good to get support."

Discussion groups, such as [MDA's Neuromuscular Disorders Discussion Group on MedShr](#), are also a good way to connect with healthcare professionals who are interested in learning from one another.