Muscular Dystrophy Association

Spotlight: An MDA Care Center's Approach to Clinical Trial Recruitment

With more than 60 clinical studies in different stages simultaneously at Stanford Medicine, John Day, MD, PhD, one of the principal investigators, doesn't miss an opportunity to talk to patients about clinical trial options.

"Clinical trials are integral to almost everything we do," says Dr. Day, Director of the Neuromuscular Division and Clinics at Stanford. "We want families to understand that research is essential to improve diagnosis and care, as well as for developing novel treatments. Our multi-institutional Northern California meetings each year — one patient-facing with patients, clinicians, and pharma, and the other for clinicians, researchers, and pharma — are both helping to build support for all regional clinical trials. By emphasizing research, we are conveying our optimism to families that we can help and will continue to help them."

Dr. Day has been diagnosing, treating, and supporting patients with neuromuscular diseases (NMD) for more than 35 years. He has extensive experience with motor neuron disorders, myotonic dystrophy (DM), Duchenne muscular dystrophy (DMD) and other muscular dystrophies, and inherited neuropathies and myopathies. He also brings perspective from decades of clinical trials.

Approaching people with different types of diseases

NMDs may be infantile, juvenile, adolescent, or adult in onset, and these and other distinctions color the way clinicians approach patients or parents about clinical trials.

"We approach an adult who participates in decision-making differently than parents of a newborn child," Dr. Day says. "Different considerations go into structuring and orienting trials for different patients, but in all situations, the trial needs to align with goals of clinical care and personal goals of the patient and family."

For example, parents of neonates with subacutely progressive disorders, like spinal muscular atrophy (SMA), are encouraged to enroll in a clinical trial quickly and to modify their schedule to accommodate the trial. Conversely, trials involving adults with slowly progressive muscular dystrophies can be started at the convenience of the subject and integrated into participants' work or school schedules. "These subjects also need to be counseled to moderate their expectations regarding effect size," Dr. Day says.

John Day, MD, PhD
Director of the Neuromuscular
Division and Clinics
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For Dr. Day and his colleagues, clinical research remains integral to comprehensive care. "Reliable and interpretable clinical research results depend on our enrolling subjects who are receiving state-of-the-art clinical care. We also believe that we're not providing neuromuscular disease patients optimal care unless we're providing them with the opportunity to participate in frontline clinical research," he says.

According to Dr. Day, many patients travel a significant distance to be seen at the MDA Care Center at Stanford because they know the staff will try to find clinical research opportunities appropriate for their condition — whether at Stanford or elsewhere.

Making patients aware of research opportunities

Successful clinical trials depend on the patient community knowing about them and understanding their importance. Yet an MDA survey found that 76% of the NMD community has never participated in a clinical trial.

Clinician attitude and communication with patients can play an essential role in clinical trial enrollment. "We want them to understand that research is our effort to link arms with patients so that by working together we can define and control their disorder — the point of research is NOT just to achieve some abstract academic goals," Dr. Day says.

“All of our clinic’s neurologists are very aware of studies either taking place or upcoming, so they keep that in mind with new or recurring patients,” says Veronica Schnyer, Clinic Research Manager, Adult Neurology.

The Stanford Neuromuscular Recruitment Database invites recruitment for studies, drug trials, and conferences. “A few thousand people have enrolled so far to consent to be contacted,” Schnyer says. “Now, we have far more patients than studies. Sometimes a study recruiting new patients pops up in the clinic, and it’s a perfect fit.”

She says the doctors will talk to patients who may have already expressed interest in participating in studies to determine a good fit, address their concerns, and assure them of ongoing care while they’re in the study.

Helping patients make informed decisions

Discussing clinical trial opportunities with patients requires a personal approach. “Every patient and family is unique,” Schnyer says. “Some are up to date on research, while others face a new diagnosis and have limited information. We tell them about the real risks — there is no such thing as ‘no risk,’ and it’s important to disclose that. We reiterate that we are committed to monitoring safety and health throughout the study, and to doing our due diligence.”

There’s a special kind of teamwork that underlies successful recruitment for a trial. “For a center to be successful, all team members must be committed to recruitment,” Dr. Day says. “If performing studies feels more like a job to team members, potential subjects will pick up on that attitude and might be less likely to participate in those trials.”

Resources

- Submit your enrolling study in MDA’s Clinical Trial Awareness Program. MDA will help get the word out through the Quest Media blog, email, social media, and MDA.org.
- View MDA’s list of trials and studies that are currently enrolling participants.
- Share these MDA Community Education printable materials with patients:
    - Clinical Trials 101
    - Clinical Trials FAQ