Transcript Details

This is a transcript of an educational program. Details about the program and additional media formats for the program are accessible by visiting: https://reachmd.com/programs/neurofrontiers/next-steps-in-gene-therapy-research-for-duchenne-muscular-dystrophy/32957/

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Next Steps in Gene Therapy Research for Duchenne Muscular Dystrophy

ReachMD Announcer:
This is *NeuroFrontiers* on ReachMD. On this episode, we'll hear from Dr. Craig McDonald, who's the Chair of the Department of Physical Medicine and Rehabilitation at UC Davis. He'll be discussing the future of gene therapy for Duchenne muscular dystrophy and the role that caregiver-reported scales play. Here's Dr. McDonald now.

Dr. McDonald:
I think, increasingly, gene therapy with treatments such as delandistrogene moxeparvovec are really becoming a standard of care in the treatment of patients with Duchenne muscular dystrophy and certainly ambulatory patients with Duchenne muscular dystrophy as well, where most of the available data has been focused on.

So in addition to other treatments such as standard of care steroid treatment, I think that most clinicians are beginning to utilize gene therapy to improve and stabilize disease progression in patients with Duchenne muscular dystrophy.

Our next steps in terms of research and clinical practice for patients with Duchenne muscular dystrophy is to continue to perform clinical trials in more severely affected Duchenne muscular dystrophy patients who are nonambulatory.

So there is an active clinical trial that is ongoing right now focusing on nonambulatory patients. We're hopeful that the gene therapy will help stabilize the loss of upper limb function and improve arm function and that it will help stabilize pulmonary disease progression as well.

And in that study, we're also utilizing the Caregiver Global Impression of Change and Global Impression of Severity as outcome measures to help us understand the meaningfulness of treatments in the nonambulatory population as well as the ambulatory population.

There are certainly next-generation gene therapies that are under development. There are other therapeutics for Duchenne muscular dystrophy and other muscular dystrophies that are under clinical development. And I think, increasingly, we're going to be seeing these caregiver global impression scales being utilized as important outcome measures in these clinical trials, both in terms of the Global Impression of Severity and the Caregiver Global Impression of Change as well.

ReachMD Announcer:
That was Dr. Craig McDonald talking about the impact of caregiver-reported scales on future research avenues for Duchenne muscular dystrophy. To access this and other episodes in our series, visit *NeuroFrontiers* on ReachMD.com, where you can Be Part of the Knowledge. Thanks for listening!