Transcript Details
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Investigating Gene Therapy for DMD: The Role of Caregiver-Reporter Scales

ReachMD Announcer:
Welcome to *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 results from the EMBARK study, which focused on the gene therapy called delandistrogene moxeparvovec for patients with Duchenne muscular dystrophy. Here's Dr. McDonald now.

Dr. McDonald:
I think those of us that are treating patients with Duchenne muscular dystrophy really believe that gene therapy such as delandistrogene moxeparvovec is a transformational treatment, and I think the FDA places a great emphasis on evaluations by caregivers. Caregivers are increasingly playing a critical role in evaluating patients with diseases such as Duchenne muscular dystrophy, and increasingly, in clinical trials, we're using caregiver-reported scales to evaluate the effective therapy.

If we have a therapy that we think produces a transformational change in a patient, we should really be able to get the impression of a caregiver or observer that the treatment is actually having an impact on the severity and producing a noticeable change that impacts physical ability, including the ability to perform daily activities, and a child or adult's overall health as well.

So in this particular study, as an extension of the traditional clinical evaluator-utilized scales such as the North Star Ambulatory Assessment or timed function test, we actually also utilized caregiver-reported scales, namely the Caregiver Global Impression of Severity as well as the Caregiver Global Impression of Change, to evaluate the effect that gene therapy had on patients with Duchenne muscular dystrophy.

With regards to the key findings from the EMBARK study, the overall patient population actually did show statistically significant improvements in timed function tests. These tests are more sensitive to evaluating a treatment effect, but a statistically significant effect on those timed function tests of several seconds in the ability to rise from the floor or walk or run 10 meters may not be quite as impactful when being evaluated by patients' caregivers as well as potential payers. And so we added these important scales of the Caregiver Global Impression of Change as well as the Caregiver Global Impression of Severity as outcome measures for this clinical trial.

I think that, importantly, all patients in the intent-to-treat population—regardless of age—who were treated with the gene therapy showed a significant increase in the odds of achieving a better rating for the gene therapy treatment. On all four scales of the Caregiver Global Impression of Change, there was a nearly four-fold increase in the likelihood that caregivers rated patients treated with gene therapy as showing significant improvements in a variety of domains, including physical ability, ability to perform activities of daily living, and overall health, as well as severity of specific Duchenne muscular dystrophy symptoms.

And the caregivers also evaluated the overall severity of disease, and there was a significant increase in the overall odds—greater than a two-fold increased likelihood—that the caregivers would evaluate the patients treated with the gene therapy as having an improvement in disease severity as opposed to those patients that were treated with a placebo.

And so I think these Caregiver Global Impression of Change instruments are really important to the regulatory authorities such as the FDA in evaluating the clinical meaningfulness of a drug treatment, and so in this case, it really did corroborate what we were seeing on the more granular clinical evaluator assessments, such as timed function testing and functional testing that was performed by the therapist.

So, this supported the overall impression of the treating physicians that this gene therapy treatment is having a really a substantial impact on overall disease severity.

ReachMD Announcer:
That was Dr. Craig McDonald sharing key findings from the EMBARK study on the gene therapy delandistrogene moxeparvovec 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!