



Limb-Girdle Muscular Dystrophy Research Update

New research on [limb-girdle muscular dystrophy \(LGMD\)](#) treatment offers hope for some subtypes.

Carla D. Zingariello, DO, an assistant professor of Pediatrics at the University of Florida College of Medicine, points to several clinical trials studying treatments for subtypes of LGMD, especially LGMD2I/R9 and LGMD2E/R4.

In LGMD2I/R9, the [FORTIFY trial](#) evaluating the safety and efficacy of long-term administration of BBP-418 (Ribitol) has advanced to phase 3. This randomized, double-blind study, which includes participants ages 12 to 60, announced the dosing of the first patient in September. Preliminary data from the phase 2 study showed stability and modest improvement in motor function scales (e.g., NSAD, 10MWT, and 100MTT).

The phase 1/2 [LION-CS101 clinical trial](#) also announced the dosing of a patient with LGMD2I/R9 in August. This double-blind, randomized, placebo-controlled, dose-escalation study is evaluating the safety and tolerability of a single intravenous infusion of LION-101 in adults with LGMD2I/R9.

Of the phase 1 gene therapy studies, Dr. Zingariello considers one involving beta-sarcoglycan-related LGMD to be the most promising. It is a single-center, open-label study where two cohorts of patients with LGMD2E/R4 receive a single intravenous infusion of SRP-9003. (Find the study [here](#).)

“We have seen in other neuromuscular diseases the potential for gene therapy to stabilize muscle function or prevent loss of muscle function in pre-symptomatic individuals,” Dr. Zingariello says.

She believes all the therapies being studied have the potential to become available treatments if they go the distance. Ribitol, the farthest along, could be approved in the next couple of years. “Ribitol is the most likely to come to market, and I’m pretty excited about it,” she says. “It’s not gene replacement therapy, but it can be a difference-maker. Stability or near stability, if realized in patients treated with Ribitol (or any other therapy), would be a huge advance for patients who experience significant long-term decline and loss of activities of daily living.”

Additional LGMD Resources

Here are more ways you can stay up-to-date on LGMD:

- Review the 2022 updates to LGMD ICD-10 codes with a [webinar and flashcard](#).
- Find enrolling LGMD trials with MDA’s [Clinical Trial Finder tool](#).
- Check back for MDA’s What’s New in LGMD mini-webinar [COMING SOON](#).