June 9, 2025

Dear FSHD Community,

Today, we are very pleased to share multiple milestones related to the delpacibart braxlosiran (del-brax) program in facioscapulohumeral muscular dystrophy (FSHD). We have confirmed with the FDA that the accelerated approval pathway is open for del-brax in the U.S., initiated the global, confirmatory Phase 3 FORWARD™ clinical trial, and provided positive topline data from the dose escalation cohorts of the Phase 1/2 FORTITUDE™ trial. The topline data are being presented this week at the 32nd Annual FSHD Society International Research Congress (IRC).

We would like to extend our heartfelt gratitude to all participants in the FORTITUDE clinical trial, as well as their families, and our advocacy partners and the investigators and their teams for their time and continued commitment. We know that time is muscle and continue to share the sense of urgency and the desire to bring the first approved therapy to the FSHD community as quickly as possible. For the U.S. community, the potential of an accelerated approval pathway brings us much closer to our mutual goals.

You can view our press releases of today's FSHD news here:

Avidity Biosciences Announces the Accelerated Approval Regulatory Pathway in the U.S. is Open for Del-Brax and Initiates the Global, Confirmatory Phase 3 FORWARD™ Study in FSHD

Avidity Biosciences Announces Positive Topline Phase 1/2 FORTITUDE™ Data Demonstrating Consistent Improvement Across Multiple Functional Measures Compared to Placebo in Del-Brax Treated FSHD Participants

Webinar in Partnership with The FSHD Society:

We are pleased to invite you to join us for a webinar in partnership with The FSHD Society on June 24th, 2025. The Avidity team, joined by Dr. Nick Johnson, associate professor of Neurology and Human and Molecular Genetics and vice chair of research in Neurology at Virginia Commonwealth University, will review the results from FORTITUDE. Please see information below to register:

Date: Tuesday, June 24th, 2025
Time: 10:00am Pacific Time / 1:00pm Eastern Time
Register Here:
https://us02web.zoom.us/webinar/register/WN_cf9OJF8lSDKEWmbuxA4DDw

Introducing the FORWARD™ Study: A Global Phase 3 clinical trial of del-brax in FSHD

Today we also announced the initiation of the FORWARD study to continue the advancement of del-brax. FORWARD is a global, confirmatory, Phase 3, randomized, placebo-controlled, double-blind,

18-month study involving approximately 200 people (ages 16-70) living with FSHD in North America, Europe and Japan. It will assess the effectiveness of del-brax by measuring functional mobility and muscle strength. We expect there will be great interest in enrolling in this global study. To learn more, contact your treatment physician or visit www.forwardstudy.com.

We want to thank the entire patient community for your time, commitment and continued contributions in assisting Avidity with the development of del-brax. We are so grateful to the current and future participants, their families, the investigators and their teams as we work together to advance del-brax in clinical development and toward potential accelerated approval.

Sincerely,

The Avidity Team