October 30, 2024

We are very pleased to share with you that we have initiated a biomarker cohort in the Phase 1/2 FORTITUDE™ clinical trial, which is assessing the safety and efficacy of our investigational therapy delpacibart braxlosiran (formerly AOC 1020, abbreviated as del-brax) in people living with facioscapulohumeral muscular dystrophy (FSHD).

The biomarker cohort in the FORTITUDE trial will assess del-brax (2 mg/kg) administered every six weeks in people living with FSHD, ages 16-70 and will be measuring changes in DUX4 regulated biomarkers. To learn more about the study, please visit the FORTITUDE study website or go to the following link: clinicaltrials.gov.

You can view our full press release of today's FSHD news here:
Avidity Biosciences Pursues Potential Accelerated Approval Path with Initiation of Biomarker Cohort in FORTITUDE™ Trial for Delpacibart Braxlosiran (del-brax/AOC 1020) in People Living with Facioscapulohumeral Muscular Dystrophy click here to learn more.

Screening is now underway for the biomarker cohort (Cohort C) and we expect enrollment to be completed in the first half of 2025. The biomarker cohort will be conducted in the US, Canada, and the UK (same as Cohorts A and B).

At this point, we have received tremendous interest from participants to be screened for the FORTITUDE trial. Our team is moving rapidly to complete this study as we know we have more patients waiting than we can accommodate in this trial.

We anticipate the initiation of a larger functional cohort of the FORTITUDE trial to begin in the first half of 2025. Patients who have participated in a trial not conducted by Avidity may be eligible for the larger functional cohort. Due to inclusion and exclusion criteria, you would still need to undergo screening assessments to determine if you are eligible.

We are advancing our clinical studies for del-brax as quickly as possible as we understand the urgency to bring a potential new treatment to people living with FSHD.

We want to thank the entire patient community for your time, commitment and continued contributions to 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.

We encourage you to contact your doctor if you have any questions about del-brax or the FORTITUDE trial.

Sincerely,

The Avidity Team