June 12, 2024

We are very pleased to inform you that today we announced positive initial AOC 1020 data from the Phase 1/2 FORTITUDE™ trial in adults living with facioscapulohumeral muscular dystrophy (FSHD). AOC 1020, the first investigational therapy designed to treat the underlying cause of FSHD, demonstrated unprecedented and consistent reductions of greater than 50% in DUX4 regulated genes, trends of functional improvement, and favorable safety and tolerability in people living with FSHD.

We understand the urgency to develop a treatment for people living with FSHD who have no treatment options. With this encouraging data, we plan to accelerate initiation of registrational cohorts in the FORTITUDE trial starting in the second half of this year. The Phase 1/2 cohorts of the FORTITUDE trial are fully enrolled. Data generated from the upcoming registrational cohorts will be used as part of the data package evaluated by regulatory agencies for new drug approval. We plan to share additional information about the upcoming trial cohorts with the FSHD community as it becomes available.

We also shared today that *delpacibart braxlosiran* is the approved international nonproprietary name of AOC 1020, abbreviated as *del-brax*.

These new positive initial data are being presented this week at the 31st Annual FSHD Society International Research Congress and shared at the FSHD Connect Conference. You can view our full press release of today's FSHD news here: **Avidity Announces Unprecedented AOC 1020 Data from Phase 1/2 FORTITUDE™ Trial Demonstrating Greater Than 50 Percent Reduction in DUX4 Regulated Genes and Trends of Functional Improvement in People Living with Facioscapulohumeral Muscular Dystrophy**

In the Phase 1/2 FORTITUDE trial, *del-brax* (AOC 1020) 2 mg/kg at the four-month assessment demonstrated:

- Greater than 50% mean reductions in DUX4 regulated genes across multiple panels for DUX4 regulated gene expression in muscle
- All participants treated with *del-brax* showed reductions greater than 20% in DUX4 regulated genes
- Mean reductions of 25% or greater in novel circulating biomarker and creatine kinase
- Trends of functional improvements including increased strength in upper and lower limb muscles, and muscle function as measured by reachable workspace (RWS) compared to placebo and the ReSolve natural history study
- Trends of improvement in patient and clinician reported outcomes
- Favorable safety and tolerability with all adverse events (AEs) mild or moderate, no serious adverse events and no discontinuations

To learn more about these findings, we invite you to join us for an online webinar in partnership with the FSHD Society. The Avidity team, joined by Dr. Jeffrey Statland, University of Kansas Medical Center and FORTITUDE trial investigator, will present the FORTITUDE trial initial findings, focusing on what these results mean for the FSHD community.

Date: Thursday, June 20, 2024
Time: 10am Pacific Time / 1pm Eastern Time
How to join: You can register to join the webinar on the FSHD Society's website (**https://www.fshdsociety.org/event/fshd-university-fortitude-trial-interim-results/**). A recording of this webinar will be available on FSHD Society's FSHD University at a later date.

We look forward to advancing the FORTITUDE trial as quickly as possible as we remain steadfast in our commitment to advance meaningful therapies for the muscular dystrophy community.

We would like to thank each participant in the trial, their families, our advocacy partners as well as the investigators and their teams for their time, commitment, and continued contributions. We share the urgency for a treatment and are hopeful about the potential of *del-brax* to change the course of disease for people living with FSHD.

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

Sincerely,

The Avidity Team