Avidity Biosciences Partners with the FSHD Society to Raise Awareness of Facioscapulohumeral Muscular Dystrophy on World FSHD Day

FSHD Society to host Avidity webinar on positive initial data from the Phase 1/2 FORTITUDE™ trial for people living with FSHD today at 1:00 p.m. ET

SAN DIEGO, June 20, 2024 /PRNewswire/ -- Avidity Biosciences, Inc. (Nasdaq: RNA), a biopharmaceutical company committed to delivering a new class of RNA therapeutics called Antibody Oligonucleotide Conjugates (AOCs™), today announced it is partnering with the FSHD Society and joining the global community of patients, caregivers, and healthcare providers to raise awareness of facioscapulohumeral muscular dystrophy in support of World FSHD Day. The FSHD Society is hosting a webinar with Avidity today at 1:00 p.m. ET to share recent unprecedented *del-brax* data from its Phase 1/2 FORTITUDE™ trial in people living with FSHD, a rare, hereditary disorder marked by life-long, relentless loss of muscle function, significant pain, fatigue, and progressive disability.

"This World FSHD Day, we are excited to be joining the FSHD Society to share recent unprecedented data from our FORTITUDE study with the patient and caregiver community," said Sarah Boyce, president and chief executive officer at Avidity. "We know the FSHD community is eagerly waiting for an approved therapy and we are committed to bringing forward a treatment as quickly as possible to the many families worldwide who are affected by FSHD. We are extremely thankful for the important contributions of patients, caregivers and families that continue to better inform our ongoing research and development as we advance *del-brax* for people living with FSHD."

"As the world's largest research-focused patient organization, we are dedicated to speeding the delivery of treatments and a cure for FSHD. With the remarkable data from Avidity's FORTITUDE study presented last week, we feel invigorated that there may be a potential treatment to change the course of this relentlessly progressive disease for people and families affected by FSHD," said Mark Stone, president and chief executive officer of FSHD Society. "We remain deeply committed to our collaborations with industry leaders such as Avidity who are accelerating innovative research. World FSHD Day is an important opportunity to recognize the partnership, commitment and work that is necessary to bring more support and more hope to families impacted by FSHD every day."

In the Phase 1/2 study, *del-brax*, 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. Currently, there are no approved therapies for the treatment of FSHD.

In an online webinar today hosted by 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:** 10:00 a.m. Pacific Time / 1:00 p.m. 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.

Every June 20th, people around the world join in activities to raise awareness of FSHD through World FSHD Day and to recognize patients and families worldwide who are affected by FSHD. This year, Avidity participated in the 31st Annual FSHD Society International Research Congress (FSHD IRC), where Jeffrey M. Statland, M.D., Professor of Neurology, University of Kansas Medical Center, and FORTITUDE trial investigator, presented the new initial data from the FORTITUDE study. Avidity also participated in the FSHD Connect Conference, an educational conference specifically for people living with FSHD and their families. Both conferences are organized by the FSHD Society, the world's largest research-focused patient organization for FSHD.

About the Phase 1/2 FORTITUDE™ trial
The FORTITUDE™ trial is a randomized, placebo-controlled, double-blind, Phase 1/2 clinical trial designed to evaluate single and multiple doses of *delpacibart braxlosiran* or *del-brax* (AOC 1020) in approximately 39 adult participants with facioscapulohumeral muscular dystrophy (FSHD). FORTITUDE will evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of *del-brax* administered intravenously, with the primary objective being the safety and tolerability of *del-brax* in FSHD patients. Activity of *del-brax* will be assessed using key biomarkers, including magnetic resonance imaging (MRI) measures of muscle volume and composition. Though the Phase 1/2 trial is not statistically powered to assess functional benefit, it will explore the clinical activity of *del-brax* including measures of mobility and muscle strength as well as patient reported outcomes and quality of life measures. Participants will have the option to enroll in an open-label extension study at the end of the treatment period in the FORTITUDE study. For more information about the FORTITUDE trial, visit

the FORTITUDE study website or visit http://www.clinicaltrials.gov and search for NCT05747924.

About *Del-brax* (AOC 1020)
Del-brax (AOC 1020) is designed to treat the underlying cause of FSHD, which is caused by the abnormal expression of a gene called double homeobox 4 or DUX4. The abnormal expression of DUX4 protein leads to changes in gene expression in muscle cells that are associated with the life-long, progressive loss of muscle function in patients with FSHD. *Del-brax* aims to reduce the expression of DUX4 mRNA and DUX4 protein in muscles in people with FSHD. *Del-brax* consists of a proprietary monoclonal antibody that binds to the transferrin receptor 1 (TfR1) conjugated with a siRNA targeting DUX4 mRNA. In preclinical studies, a single intravenous dose with the murine version of *del-brax* prevented development of muscle weakness demonstrated by three functional assays - treadmill running, in vivo force and compound muscle action potential. *Del-brax* is currently in Phase 1/2 development as part of the FORTITUDE™ trial in adults with FSHD. The U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) have granted Orphan designation for *del-brax* and the FDA has granted *del-brax* Fast Track designation.

About Facioscapulohumeral Muscular Dystrophy (FSHD)
Facioscapulohumeral muscular dystrophy (FSHD) is a rare, progressive, and variable hereditary muscle-weakening condition marked by significant pain, fatigue, and disability. It is characterized by progressive and often asymmetric skeletal muscle loss that initially causes weakness in muscles in the face, shoulders, arms and trunk and progresses to weakness in muscles in the lower body. FSHD is an autosomal dominant disease caused by the aberrant expression of the DUX4 (double homeobox 4) gene in the skeletal muscle, which activates genes that are toxic to muscle cells and leads to a series of downstream events that result in skeletal muscle wasting and compromised muscle function. Skeletal muscle weakness results in physical limitations throughout the whole body, including an inability to lift arms for more than a few seconds, loss of ability to show facial expressions and serious speech impediments. These symptoms cause many people affected by FSHD to become dependent on the use of a wheelchair for mobility. Currently, there are no approved treatments for people living with FSHD.

About Avidity
Avidity Biosciences, Inc.'s mission is to profoundly improve people's lives by delivering a new class of RNA therapeutics - Antibody Oligonucleotide Conjugates (AOCs™). Avidity is revolutionizing the field of RNA with its proprietary AOCs, which are designed to combine the specificity of monoclonal antibodies with the precision of oligonucleotide therapies to address targets and diseases previously unreachable with existing RNA therapies. Utilizing its proprietary AOC platform, Avidity demonstrated the first-ever successful targeted delivery of RNA into muscle and is leading the field with clinical development programs for three rare muscle diseases: myotonic dystrophy type 1 (DM1), Duchenne muscular dystrophy (DMD) and facioscapulohumeral muscular dystrophy (FSHD). Avidity is broadening the reach of AOCs with its advancing and expanding pipeline including programs in cardiology and immunology through internal discovery efforts and key partnerships. Avidity is headquartered in San Diego, CA. For more information about our AOC platform, clinical development pipeline and people, please visit www.aviditybiosciences.com and engage with us on LinkedIn and X.

Forward-Looking Statements
Avidity cautions readers that statements contained in this press release regarding matters that are not historical facts are forward-looking statements. These statements are based on the company's current beliefs and expectations. Such forward-looking statements include, but are not limited to, statements regarding: the characterization of safety, tolerability and functional data associated with *del-brax* from the Phase 1/2 FORTITUDE™ trial; the impact of such data on the advancement of *del-brax*; timeline for advancing *del-brax*; the status and potential of *del-brax* as the first investigational therapy designed to treat the underlying cause of FSHD; the potential of Avidity's product candidates to treat rare diseases and Avidity's efforts to bring them to people suffering from applicable diseases; and the potential of AOCs™ to target a range of different cells and tissues beyond the liver, and to treat cardiac and immunological diseases. This press release also contains estimates and other statistical data made by independent parties and by us. This data involves a number of assumptions and limitations, and the reader is cautioned not to give undue weight to such estimates.

The inclusion of forward-looking statements should not be regarded as a representation by Avidity that any of these plans will be achieved. Actual results may differ from those set forth in this press release due to the risks and uncertainties inherent in Avidity's business and those beyond its control, including, without limitation: preliminary results of a clinical trial are not necessarily indicative of final results and additional participant data related to *del-brax* that continues to become available may be inconsistent with the data produced as of the date hereof, and further analysis of existing data and analysis of new data may lead to conclusions different from those established as of the data cutoff; unexpected adverse side effects to, or inadequate efficacy of, Avidity's product candidates that may delay or limit their development, regulatory approval and/or commercialization, or may result in additional clinical holds which may not be timely lifted, recalls or product liability claims; Avidity is early in its development efforts; Avidity's approach to the discovery and development of product candidates based on its AOC platform is unproven, and the company does not know whether it will be able to develop any products of commercial value; potential delays in the commencement, enrollment, data readouts and completion of preclinical studies or clinical trials; Avidity's dependence on third parties in connection with preclinical and clinical testing and product manufacturing; regulatory developments in the United States and foreign countries; and other risks described in Avidity's Annual Report on Form 10-K for the fiscal year ended December 31, 2023, filed with the Securities and Exchange Commission (SEC) on February 28, 2024, and in subsequent filings with the SEC. Avidity cautions readers not to place undue reliance on these forward-looking statements, which speak only as of

the date hereof, and the company undertakes no obligation to update such statements to reflect events that occur or circumstances that arise after the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement, which is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995.

Investor Contact:
Geoffrey Grande, CFA
(619) 837-5014
investors@avidity bio.com

Media Contact:
Navjot Rai
(619) 837-5016
media@avidity bio.com

SOURCE Avidity Biosciences, Inc.
