Avidity Biosciences Joins with Patients, Caregivers and Healthcare Providers Globally to Support World Facioscapulohumeral Muscular Dystrophy (FSHD) Day

Avidity is currently enrolling participants in the Phase 1/2 FORTITUDE™ study of AOC 1020 for the treatment of FSHD

Company anticipates data from a preliminary assessment in half of the participants in the FORTITUDE study in 1H 2024

SAN DIEGO, June 20, 2023 /PRNewswire/ -- Avidity Biosciences, Inc. (Nasdaq: RNA), a biopharmaceutical company committed to delivering a new class of RNA therapeutics called Antibody Oligonucleotide Conjugates (AOCs™), joins with the global community of patients, caregivers, and healthcare providers in support of World Facioscapulohumeral Muscular Dystrophy (FSHD) Day. FSHD is a serious, rare, hereditary muscle-weakening condition marked by life-long, progressive loss of muscle function that causes significant pain, fatigue, and disability. Currently, there are no approved therapies for the treatment of FSHD.

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. FSHD affects both sexes equally, with onset typically in teenage and young adult years.

"We are grateful for our partnership with the global FSHD community as we collectively work together to advance AOC 1020 for the treatment of FSHD," said Sarah Boyce, president and chief executive officer at Avidity Biosciences. "By directly targeting DUX4, AOC 1020 is designed to reduce the devastating effects of skeletal muscle wasting and progressive loss of muscle function in people living with FSHD. We are currently enrolling adults with FSHD into the AOC 1020 Phase 1/2 FORTITUDE™ study. We plan to share a preliminary assessment from the FORTITUDE study in the first half of next year."

Avidity is currently enrolling participants in FORTITUDE, an ongoing randomized, placebo-controlled, double-blind, Phase 1/2 clinical trial to evaluate the investigational therapy AOC 1020 in the treatment of FSHD. For more information about the FORTITUDE trial, visit the FORTITUDE study website or visit http://www.clinicaltrials.gov and search for NCT05747924.

"As the world's largest research-focused patient organization for FSHD, our mission is to find treatments and a cure for FSHD while empowering our families affected with FSHD, one of the most prevalent forms of muscular dystrophy," said Mark Stone, chief executive officer of FSHD Society. "We have set an extraordinary goal to ensure that the first-ever FSHD therapy would be approved by 2025. This can only be accomplished by the FSHD Community – patients & families, industry, and the medical community – resourcing and working together towards this goal. We are proud to partner with companies like Avidity who are developing much needed treatments with the aim to provide hope to families affected by FSHD."

Every June 20th, people around the world join in activities to raise awareness for FSHD through World FSHD Day and to recognize patients and families around the world who are affected by FSHD. At the 30th Annual FSHD Society International Research Congress (FSHD IRC) held in Milan, Italy, Avidity presented oral and poster presentations on AOC 1020 preclinical data, FORTITUDE study design and Health Economics and Economics Outreach (HEOR) data demonstrating disease burden among people living with FSHD. The meeting is organized by the FSHD Society, the world's largest research-focused patient organization for FSHD. In addition, Avidity participated in a panel discussion at the 2023 World FSHD Alliance Leadership Summit. The World FSHD Alliance is a network of patient advocacy groups from more than 25 countries around the world. Avidity also serves on the Global Task Force of Project Mercury, an open collaboration spearheaded by the FSHD Society to bring stakeholders from across the globe together to overcome the challenges that could slow or prevent effective therapies from getting to patients everywhere.

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 AOC 1020 in approximately 70 adult participants with facioscapulohumeral muscular dystrophy (FSHD). FORTITUDE will evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of AOC 1020 administered intravenously, with the primary objective being the safety and tolerability of AOC 1020 in FSHD patients. Activity of AOC 1020 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

AOC 1020 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 AOC 1020
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. AOC 1020 aims to reduce the expression of DUX4 mRNA and DUX4 protein in muscles in people with FSHD. AOC 1020 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 AOC 1020 prevented development of muscle weakness demonstrated by three functional assays - treadmill running, in vivo force and compound muscle action potential. AOC 1020 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 AOC 1020 and the FDA has granted AOC 1020 Fast Track designation.

About Facioscapulohumeral Muscular Dystrophy (FSHD)
Facioscapulohumeral muscular dystrophy (FSHD) 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 Twitter.

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 progression of the FORTITUDE™ trial and the timing thereof; the potential of AOC 1020 to treat people with FSHD; the enrollment of participants in the FORTITUDE trial, the success of the FORTITUDE trial and the reporting of data from the preliminary assessment of the FORTITUDE trial and the timing thereof; goals and expectations related to the FORTITUDE trial; biomarker utilization and other methodologies related to the FORTITUDE trial; the design and expected impact of AOC 1020; and AOC 1020's potential to address unmet needs in patients with FSHD. Actual results may differ from those set forth in this press release due to the risks and uncertainties inherent in the business, including, without limitation: 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 and completion of clinical trials; unexpected adverse side effects or inadequate efficacy of its product candidates that may delay or limit their development, regulatory approval and/or commercialization, or may result in clinical holds, recalls or product liability claims; the success of its preclinical studies and clinical trials for the company's product candidates; the results of preclinical studies and early clinical trials are not necessarily predictive of future results; Avidity's dependence on third parties in connection with clinical testing and product manufacturing; regulatory developments in the United States and foreign countries, including acceptance of INDs and similar foreign regulatory filings and the proposed design of future clinical trials; Avidity could exhaust its available capital resources sooner than it currently expects and fail to raise additional needed capital; and other risks described in our Annual Report on Form 10-K for the year ended December 31, 2022, filed with the Securities and Exchange Commission (SEC) on February 28, 2023, 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.

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