Avidity Biosciences Honors Rare Disease Day®

SAN DIEGO, Feb. 29, 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 its support for Rare Disease Day®, highlighting the importance of bringing awareness to the devastating impact rare diseases have on patients, families and caregivers worldwide.

"Today, on Rare Disease Day, we are proud to join the global community in raising awareness for people living with rare diseases," said Sarah Boyce, president and chief executive officer at Avidity. "Recently, we had the privilege of hosting individuals affected by rare muscle diseases at Avidity. Their journeys, filled with challenges, determination, and unwavering hope, exemplify the urgency of our mission to profoundly improve people's lives by revolutionizing a new class of targeted RNA therapeutics. At Avidity, we are committed to listening, learning, and partnering with the patient and advocacy community and look forward to our continued collaboration as we advance our three rare muscle disease clinical programs for DM1, DMD44 and FSHD."

Avidity has three distinct rare disease programs in clinical development: AOC 1001 for the treatment of myotonic dystrophy type 1 (DM1) currently in the MARINA open-label extension (MARINA-OLE™) trial and on-track to be studied in the global Phase 3 HARBOR™ trial planned to initiate mid-2024; AOC 1044 in the Phase 1/2 EXPLORE44™ trial for the treatment of Duchenne muscular dystrophy (DMD) mutations amenable to exon 44 skipping (DMD44), and AOC 1020 in the Phase 1/2 FORTITUDE™ trial for the treatment of facioscapulohumeral muscular dystrophy (FSHD).

"Millions of Americans live with rare diseases, yet a staggering 95% have no approved treatments," stated Annie Kennedy, Chief of Policy, Advocacy, and Patient Engagement at EveryLife Foundation for Rare Diseases. "It's essential that we highlight the importance of patient voices and advocate for policies that drive research. This can lead to breakthrough therapies that offer significant improvements for patients and their families, bringing hope to the rare disease community."

Rare Disease Day takes place on the last day of February each year with the goal to raise awareness of the impact of rare diseases worldwide. EURORDIS established Rare Disease Day in 2008 and coordinates with more than 70 national alliance patient organizations each year to honor those living with rare diseases as well as their families and caregivers.

About Myotonic Dystrophy Type 1 (DM1)
Myotonic dystrophy type 1 (DM1) is an underrecognized, progressive and often fatal disease caused by a triplet-repeat in the DMPK gene, resulting in a toxic gain of function mRNA. The disease is highly variable with respect to severity, presentation and age of onset, however all forms of DM1 are associated with high levels of disease burden and may cause premature mortality. DM1 primarily affects skeletal and cardiac muscle, however patients can suffer from a constellation of manifestations including myotonia and muscle weakness, respiratory problems, fatigue, hypersomnia, cardiac abnormalities, severe gastrointestinal complications, and cognitive and behavioral impairment. Currently, there are no approved treatments for people living with DM1.

About Duchenne Muscular Dystrophy (DMD)
Duchenne muscular dystrophy (DMD) causes a lack of functional dystrophin that leads to stress and tears of muscle cell membranes, resulting in muscle cell death and the progressive loss of muscle function. The dystrophin protein maintains the integrity of muscle fibers and acts as a shock absorber through its role as the foundation of a group of proteins that connects the inner and outer elements of muscle cells. People living with DMD suffer from progressive muscle weakness that typically starts at a very young age. Over time, people with Duchenne will develop problems walking and breathing, and eventually, the heart and respiratory muscles will stop working. Those living with the condition often require special aid and assistance throughout their lives and have significantly shortened life expectancy. While there are treatments approved to treat people with DMD, there remains a very high unmet need. DMD is a monogenic, X-linked, recessive disease that primarily affects males, with one in 3,500 to 5,000 boys born worldwide having Duchenne.

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: plans to initiate a global Phase 3 trial for people living with DM1; plans for the progression of Avidity's clinical programs for AOC 1001, AOC 1044 and AOC 1020, and the timing thereof; the potential of Avidity's product candidates to treat rare diseases and Avidity's efforts to bring them to people suffering from applicable diseases; the potential of AOCs to target a range of different cells and tissues beyond the liver; and Avidity's pipeline programs. 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 beyond its control, including, without limitation: Avidity may not be able to resolve the partial clinical hold related to the serious adverse event which occurred in the Phase 1/2 MARINA® trial, which may result in delays in the clinical development of AOC 1001; additional data related to Avidity's current clinical programs that continues to become available may be inconsistent with the data produced as of the respective data cutoff dates, further analysis of existing data and analysis of new data may lead to conclusions different from those established as of the date hereof, and such data may not meet Avidity's expectations; 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 clinical holds which may not be timely lifted (if at all), 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; the success of its preclinical studies and clinical trials for the company's product candidates; Avidity's dependence on third parties in connection with preclinical and clinical testing and product manufacturing; Avidity may not realize the expected benefits of its collaborations; regulatory developments in the United States and foreign countries; Avidity could exhaust its available capital resources sooner than it currently expects and fail to raise additional needed funds; 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. 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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