

Avidity Biosciences Joins Patient and Advocacy Communities in Raising Awareness During National Muscular Dystrophy Awareness Month

Avidity supports World Duchenne Awareness Day, International Myotonic Dystrophy Awareness Day, FSHD Society Walk & Roll to Cure FSHD and Global Genes Week in RARE

Avidity reported groundbreaking data in all three clinical programs addressing rare muscular dystrophies: myotonic dystrophy type 1 (DM1), facioscapulohumeral muscular dystrophy (FSHD) and Duchenne muscular dystrophy amenable to exon 44 skipping (DMD44)

SAN DIEGO, Sept. 5, 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 joining with patient and advocacy communities to raise awareness during National Muscular Dystrophy Awareness Month, an annual observance that takes place every September to support families who are impacted by neuromuscular diseases. During the month, Avidity is engaging with patient communities to support muscular dystrophy initiatives, including World Duchenne Awareness Day, International Myotonic Dystrophy Awareness Day, FSHD Society Walk & Roll to Cure FSHD, and Global Genes Week in RARE to build broader awareness of muscular dystrophies.

"We are proud to stand alongside patient communities and their families this Muscular Dystrophy Awareness Month, raising awareness for those impacted by debilitating muscle disorders, who are facing limited or no treatment options," said Sarah Boyce, president and chief executive officer at Avidity. "This year, we reported unprecedented data across our muscular dystrophy programs - DM1, FSHD and DMD44 - and advanced our DM1 program into a global Phase 3 study. We are deeply grateful to the muscular dystrophy community for their ongoing support as we work together to advance these programs as expeditiously as possible. At Avidity, we are committed to profoundly improving people's lives by revolutionizing a new class of targeted RNA therapeutics."

Avidity has demonstrated groundbreaking data across all three rare neuromuscular disease clinical development programs: del-desiran™ for myotonic dystrophy type 1 (DM1), del-brax™ for facioscapulohumeral muscular dystrophy (FSHD) and del-zota™ for Duchenne muscular dystrophy amenable to exon 44 skipping (DMD44). The company also initiated the global Phase 3 HARBOR™ study for DM1 and is advancing additional candidates from their DMD franchise following their positive del-zota™ data. Avidity is working to accelerate the development of these important potential therapies to address the unmet need of people living with these serious rare neuromuscular diseases.

In support of National Muscular Dystrophy Awareness Month, Avidity is engaging with several leading advocacy groups and patient communities in a range of activities, including:

- Supporting the [2024 MDF Gala](#), taking place Saturday, September 14 in Los Angeles;
- Participating in the Jett Foundation's Stronger than Duchenne [World Duchenne Awareness Day](#) celebration, including virtual and in-person events in the Boston area on September 7;
- Recognizing [International Myotonic Dystrophy Awareness Day](#) on September 15 as a proud member of the Global Alliance for Myotonic Dystrophy Awareness;
- Supporting the 2024 Global Genes [Week in RARE](#) event taking place September 25-28 in Kansas City, MO, including the RARE Health Equity Forum and RARE Advocacy Summit, one of the world's largest gatherings of rare disease patients, caregivers, advocates and healthcare professionals;
- Partnering with the FSHD Society for its [2024 Walk & Roll to Cure FSHD](#) in San Diego on September 21 and additional locations across the U.S., the only international annual event focused solely on funding research progress for FSHD; and
- Presenting at the Defeat Duchenne Canada [2024 Family Forum](#) taking place September 21 in Ontario, an annual event where families and caregivers gather to learn more about the latest advancements in research, clinical trials, and advocacy initiatives.

About Myotonic Dystrophy Type 1

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: the characterization of data associated with del-desiran™, del-brax™ and del-zota™ within their respective clinical studies, and the impact of such data on the advancement of the respective product candidates; Avidity's plans and expectations to advance its clinical programs, and the timing thereof; and Avidity's platform, planned operations and 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: preliminary results of a clinical trial are not necessarily indicative of final results; further analysis of existing clinical data and analysis of new data may lead to conclusions different from those established as of the respective data cutoff dates in Avidity's clinical trials, 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; Avidity may not be able to resolve the partial clinical hold related to del-desiran™; later developments with the FDA and other global regulators that could be inconsistent with the feedback received to date regarding Avidity's clinical trials; Avidity's approach to the discovery and development of product candidates based on its AOC™ platform is unproven; 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; legislative, judicial and regulatory developments in the United States and foreign countries; Avidity could exhaust its available capital resources sooner than it currently expects; and other risks described in Avidity's Annual Report on Form 10-K for the fiscal year ended December 31, 2023 and 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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