

Avidity Biosciences Joins with Patient Communities to Raise Awareness During National Muscular Dystrophy Awareness Month as it Advances Three Muscular Dystrophy Clinical Programs

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 joins with MDF community leaders, patients, caregivers, and legislators in educational briefing to highlight the latest scientific advancements in myotonic dystrophy research and development

Company advancing clinical development programs for three types of muscular dystrophy – DM1, DMD and FSHD – with planned data readouts over the next 12 months

SAN DIEGO, Sept. 7, 2023 [/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 that it is joining with patient communities to raise awareness during National Muscular Dystrophy Awareness Month, an annual observance that takes place every September to support families across the U.S. who are impacted by neuromuscular diseases. Today, Avidity will be joining Myotonic Dystrophy Foundation (MDF) community leaders, patients, caregivers, and legislators to raise awareness of myotonic dystrophy and the importance of advancing research to develop new treatments for the disease as part of a special MDF Advocacy Day on Capitol Hill in Washington, D.C. In addition, Avidity is engaging with patient communities to support World Duchenne Awareness Day, International Myotonic Dystrophy Awareness Day, FSHD Society Walk & Roll to Cure FSHD, and Global Genes Week in RARE, all taking place during National Muscular Dystrophy Awareness Month.

"We are proud to be uniting with patient communities and their families this month to raise awareness of muscular dystrophy. Today, we are joining MDF as part of an educational briefing on Capitol Hill and also, recognizing World Duchenne Awareness Day. Later this month, we will be engaging with members of the FSHD community and Global Genes," said Sarah Boyce, president and chief executive officer at Avidity. "At Avidity, we are developing a new class of treatments and advancing three distinct clinical development programs for three types of muscular dystrophy – DM1, DMD and FSHD. Many people living with these devastating muscle disorders have limited or no treatment options. It is important that we work together as a community to bring promising new treatments to patients in need as quickly as possible."

Avidity is currently advancing three muscular dystrophy clinical programs: AOC 1001 for the treatment of myotonic dystrophy type 1 (DM1), AOC 1044 for the treatment of Duchenne muscular dystrophy in people with mutations amenable to exon 44 skipping (DMD44), and AOC 1020 for the treatment of facioscapulohumeral muscular dystrophy (FSHD).

"National Muscular Dystrophy Awareness Month is an opportunity for members of the myotonic dystrophy community to unite to help patients and families affected by all types of muscular dystrophy," said Tanya Stevenson, EdD, MPH, Chief Executive Officer at the Myotonic Dystrophy Foundation (MDF). "Together with Avidity, MDF is proud to have the opportunity to educate and engage with key members of Congress, and representatives from the National Institutes of Health and the Congressionally Directed Medical Research Program, to raise new levels of awareness about myotonic dystrophy, educate about the impact of the disease on the quality of life, and to provide updates on the latest scientific developments." MDF is committed to helping patients and families by supporting and connecting the myotonic dystrophy community, providing resources and advocating for care, and accelerating research toward treatments and a cure.

In support of National Muscular Dystrophy Awareness Month, Avidity will be engaging in various activities with patient and advocacy communities, including:

- Joining leaders from the advocacy community and Congress to discuss advances in myotonic dystrophy research in an educational briefing on Capitol Hill organized by the MDF to be held September 7 beginning at 12:30 p.m. ET in the 385 Senate Russell Building in Washington, D.C.
- Participating at the [2023 MDF Annual Conference](#), taking place September 7-9, 2023.
- Supporting the Jett Foundation's Stronger than Duchenne [World Duchenne Awareness Day](#) event, taking place on September 7, 2023.
- Recognizing [International Myotonic Dystrophy Awareness Day](#) on September 15 as a proud member of the Global Alliance for Myotonic Dystrophy Awareness.
- Participating at the 2023 Global Genes [Week in RARE](#) event taking place September 18-21, 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 their [2023 Walk & Roll to Cure FSHD](#) in San Diego on September 30 and additional locations throughout the country, the only international annual event focused solely on funding progress for FSHD.

Avidity's lead program, AOC 1001, is currently being studied in the MARINA open-label extension (MARINA-OLE™) trial in adults living with DM1. The company plans to share a first look at data from the MARINA-OLE study in the first half of 2024, while in parallel, planning for a Phase 3 study. Avidity is advancing AOC 1044 in the [Phase 1/2 EXPLORE44™ trial](#) in people living with DMD44 and plans to report data from healthy volunteers in the EXPLORE44 trial in the fourth quarter of 2023. The company is also advancing AOC 1020 in the [Phase 1/2 FORTITUDE™ trial](#) in people living with FSHD with data from a preliminary assessment in approximately half of the participants in the FORTITUDE trial planned for the first half of 2024.

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 [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 anticipated timing of release of data from the MARINA-OLE™, EXPLORE44™ and FORTITUDE™ trials; plans for a Phase 3 study for AOC 1001; plans for the progression of 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 to treat cardiac and immunological diseases; the continued advancement of programs with collaboration partners; and Avidity's plans to expand its AOC platform and to invest in its 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, 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 participant data related to AOC 1001 that continues to become available may be inconsistent with the data

produced as of the most recent date cutoff, and further analysis of existing data and analysis of new data may lead to conclusions different from those established as of such date 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; 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, 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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