Avidity Biosciences Receives FDA Orphan Drug Designation for AOC 1044 for Treatment of Duchenne Muscular Dystrophy in People with Mutations Amenable to Exon 44 Skipping

SAN DIEGO, Aug. 15, 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 the U.S. Food and Drug Administration (FDA) has granted Orphan Drug designation to AOC 1044, the company's investigational therapy in development for the treatment of Duchenne muscular dystrophy (DMD) in people with mutations amenable to exon 44 skipping (DMD44). DMD is a rare genetic condition that is characterized by progressive muscle damage and weakness due to the loss of dystrophin protein that typically starts at a very young age. Currently, there are no therapies approved targeting exon 44.

AOC 1044 is being assessed in the Phase 1/2 EXPLORE44™ clinical trial for people living with DMD44 and is the first of multiple AOCs in development at Avidity for the treatment of DMD. Avidity plans to share results from the healthy volunteer portion of the EXPLORE44 trial in the fourth quarter of 2023 and is now enrolling participants living with DMD44 into the study. In April 2023, AOC 1044 received FDA Fast Track designation for the treatment of DMD44.

"We are pleased that the FDA has granted both Orphan Drug and Fast Track designation to AOC 1044, highlighting the importance of advancing new treatments for people living with DMD," said Steve Hughes, M.D., chief medical officer at Avidity. "There are currently no treatment options that target the underlying cause of DMD44. AOC 1044 is designed to specifically skip exon 44 of the dystrophin gene to enable the production of functional dystrophin protein. We look forward to advancing AOC 1044 in clinical development and bringing this very important treatment to patients as quickly and safely as possible."

Avidity's proprietary AOCs are designed to combine the specificity of monoclonal antibodies (mAbs) with the precision of oligonucleotide therapies to target the root causes of diseases previously untreatable with RNA therapeutics. In the case of DMD, the disease is caused by a genetic mutation that prevents the body from producing the dystrophin protein, which protects muscle cells from injury during contraction. The lack of functional dystrophin leads to stress and tears of muscle cell membranes, resulting in muscle cell death, inflammation, and progressive loss of muscle function. AOC 1044 is designed to deliver phosphorodiamidate morpholino oligomers (PMOs) to skeletal muscle and heart tissue. The PMOs circumvent the mutation by causing exon 44 of the dystrophin gene to be skipped, which enables production of functional dystrophin protein.

The FDA's Office of Orphan Products Development grants orphan status to support the development of medicines for rare disorders that affect fewer than 200,000 people in the U.S. Orphan Drug designation provides certain benefits, including market exclusivity upon regulatory approval, exemption of FDA application fees, and tax credits for qualified clinical trials.

The EXPLORE44™ Phase 1/2 Trial of AOC 1044
The EXPLORE44 trial is a randomized, placebo-controlled, double-blind, Phase 1/2 clinical trial to evaluate AOC 1044 in healthy volunteers and participants with DMD mutations amenable to exon 44 skipping (DMD44). EXPLORE44 will evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamic effects of single and multiple ascending doses of AOC 1044 administered intravenously. EXPLORE44 is expected to enroll approximately 40 healthy volunteers and 24 participants with DMD44, ages seven to 27 years old. The EXPLORE44 trial will assess exon skipping and dystrophin protein levels in participants with DMD44. Participants with DMD44 will have the option to enroll into an extension study.

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 AOC 1044
AOC 1044 is designed to deliver phosphorodiamidate morpholino oligomers (PMOs) to skeletal muscle and heart tissue to specifically skip exon 44 of the dystrophin gene to enable dystrophin production in people living with Duchenne muscular dystrophy with mutations amenable to exon 44 skipping (DMD44). DMD is characterized by progressive muscle degeneration and weakness due to alterations of a protein called dystrophin that protects muscle cells from injury during contraction. AOC 1044 consists of a proprietary monoclonal antibody that binds to the transferrin receptor 1 (TfR1) conjugated with a PMO targeting exon 44. In a preclinical model of DMD, a murine active AOC produced durable exon skipping and functional dystrophin protein in skeletal muscle and heart tissue following a single intravenous dose. AOC 1044 is currently in Phase 1/2 development as part of the EXPLORE44™ trial for the treatment of DMD mutations amenable to exon 44 skipping.

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 clinical programs for AOC 1001, AOC 1044, and AOC 1020 and the timing thereof; the potential of AOC 1044 to treat people with DMD44; the number and characteristics of participants enrolling in the EXPLORE44™ trial and the timing thereof; the design, goals and prospects for success of the ongoing EXPLORE44 trial; the prospect of a related extension study and the ability of EXPLORE44 participants to enroll therein; the reporting of data from healthy volunteers in the EXPLORE44 study and the timing thereof; AOC 1044's potential to address unmet needs in patients with DMD44 and to treat the underlying cause of DMD44; expectations for Avidity's interactions with the FDA; the implications of Orphan Drug designation; Avidity's development of multiple AOCs™ to treat DMD; and the potential to broaden the reach of AOCs beyond skeletal muscle tissues. 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 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; Avidity's ability to resolve the partial clinical hold related to the Phase 1/2 MARINA™ trial of AOC 1001; potential delays in the commencement, enrollment and completion of clinical trials; unexpected adverse side effects to, 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 which may not be timely lifted, 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 preclinical 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; disruption to its operations, including the ability to enroll eligible participants in its clinical trials, from the COVID-19 pandemic or the war in Ukraine; Avidity could exhaust its available capital resources sooner than it currently expects and be unable 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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