

Avidity Biosciences Granted FDA Fast Track Designation for AOC 1044 for Treatment of Duchenne Muscular Dystrophy Mutations Amenable to Exon 44 Skipping

SAN DIEGO, April 24, 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 Fast Track designation to AOC 1044 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 in males at a very young age. 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 the company is developing for DMD. Avidity plans to share results from the healthy volunteer portion of the EXPLORE44 trial in the second half of 2023.

Fast Track designation enables more frequent interactions with the FDA to expedite the development and review process for drugs intended to treat serious or life-threatening conditions and that demonstrate the potential to address unmet medical needs. Currently, there are no therapies approved targeting exon 44.

"It is very encouraging to receive FDA Fast Track designation as it further validates the potential of AOC 1044 to target the underlying cause of DMD44 and the importance of bringing people living with this devastating disease an effective treatment option. This recognition also means that now all three of our clinical-stage programs have Fast Track status, further reinforcing our efforts to make a profound difference in people's lives," said Steve Hughes, M.D., chief medical officer at Avidity. "We will continue to work closely with the FDA as we advance AOC 1044 and look forward to the anticipated data readout from the healthy volunteer portion of our Phase 1/2 EXPLORE44 clinical trial later this year."

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 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 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 1 in 3500 to 5000 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's proprietary AOCs are designed to combine the specificity of monoclonal antibodies with the precision of oligonucleotide therapies to target the root cause of diseases previously untreatable with RNA therapeutics. Avidity's advancing and expanding pipeline has three programs in clinical development. AOC 1001 is designed to treat people with myotonic dystrophy type 1 (DM1) and is currently in Phase 1/2 development with the ongoing MARINA™ and MARINA-OLE™ trials. AOC 1020 is designed to treat people living with facioscapulohumeral muscular dystrophy (FSHD) and is currently in Phase 1/2 development with the FORTITUDE™ trial. AOC 1044 is designed for people with Duchenne muscular dystrophy (DMD) mutations amenable to exon 44 skipping and is currently in Phase 1/2 development with the EXPLORE44™ trial. AOC 1044 is the first of multiple AOCs the company is developing for DMD. Avidity is also broadening the reach of AOCs beyond muscle tissues through both internal discovery efforts and key partnerships as the company continues to deliver on the RNA revolution. Avidity is headquartered in San Diego, CA. For more information about our science, 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 1020, and AOC 1044 and the timing thereof; the potential of AOC 1044 to treat people with DMD44, the enrollment of participants in the EXPLORE44™ trial, the success of the ongoing EXPLORE44 trial and 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 Fast Track 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; 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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