

Avidity Announces First Person Dosed with an Antibody Oligonucleotide Conjugate (AOC™)

Participant dosed with AOC 1001 in the Phase 1/2 MARINA™ trial in adults with myotonic dystrophy type 1 (DM1)

LA JOLLA, Calif., Nov. 4, 2021 /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 first participants in the Phase 1/2 MARINA trial have been dosed with Avidity's lead AOC product candidate, AOC 1001, marking the first time a person has been dosed with an AOC.

"This is a first for this new class of drugs and it is a significant milestone for the DM1 community, the Avidity team and the RNA field," said Sarah Boyce, president and CEO of Avidity. "AOCs have the potential to expand the possibilities of how we can treat diseases and our goal is to deliver meaningful drugs to patients as quickly as possible. AOC 1001's MARINA trial will offer a first glimpse of proof-of-concept data for the AOC platform to better inform the development path for DM1 as well as future treatments for other diseases with limited therapeutic options."

AOCs are designed to combine the proven technology of monoclonal antibodies with the precision and potency of oligonucleotide therapies to access previously untreatable tissue and cell types. AOC 1001 consists of a proprietary monoclonal antibody that binds to the transferrin receptor 1 (TfR1) conjugated with a small interfering RNA (siRNA). It is designed to address the root cause of DM1 by targeting DMPK, the disease-related mRNA.

Tanya Stevenson, EdD, MPH, CEO of the Myotonic Dystrophy Foundation in the U.S. stated, "This milestone represents years of research to advance treatment for DM1. Opportunities for the myotonic dystrophy community to participate in research, like the MARINA trial, are critical because the knowledge gained may help lead to earlier diagnosis, improved quality of life and, ultimately, a treatment. The MARINA trial offers much needed advancement in the treatment of DM1 and may also advance therapies for other repeat expansion diseases. We are more hopeful, encouraged and excited than ever before."

"The AOC platform was developed entirely in-house at Avidity. Our team has created this new technology through years of engineering and following the data to optimize each component of our AOCs," said Art Levin, Ph.D., Avidity's CSO. "This is a turning point for Avidity and the RNA field as AOCs target other cells and tissues that were previously unreachable. Avidity's AOCs are designed to expand the reach of oligonucleotide therapeutics to treat a broader range of diseases."

The AOC 1001 Phase 1/2 MARINA trial is enrolling adults with DM1. The first doses in the MARINA trial were administered to patients at Virginia Commonwealth University and University of Rochester Medical Center (NY). For more information on the MARINA trial, including a full list of participating sites, visit www.clinicaltrials.gov.

About AOC 1001 and the Phase 1/2 MARINA™ Trial

AOC 1001, Avidity's lead product candidate utilizing its AOC platform, is designed to address the root cause of DM1 by reducing levels of a disease-related mRNA called DMPK. AOC 1001 consists of a proprietary monoclonal antibody that binds to the transferrin receptor 1 (TfR1) conjugated with a siRNA targeting DMPK mRNA. In preclinical studies, AOC 1001 successfully delivered siRNAs to muscle cells, resulting in durable, dose-dependent reductions of DMPK RNA across a broad range of muscles including skeletal, cardiac, and smooth muscles. AOC 1001 has commenced clinical testing with the ongoing Phase 1/2 MARINA™ trial in adults with DM1. The U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) have granted Orphan Designation for AOC 1001 and the FDA has granted AOC 1001 Fast Track Designation.

The MARINA trial is a randomized, double-blind, placebo-controlled, Phase 1/2 clinical trial expected to enroll approximately 44 adults with DM1. The primary objective of this study is to evaluate the safety and tolerability of single and multiple ascending doses of AOC 1001 administered intravenously. The MARINA trial will begin to assess the activity of AOC 1001 across key biomarkers, including spliceopathy, an important biomarker for DM1, and knockdown of DMPK mRNA. Though the Phase 1/2 trial is not powered to assess functional benefit, it will explore the clinical activity of AOC 1001 including measures of mobility and muscle strength as well as patient reported outcomes and quality of life measures. Patients will have the option to enroll in an open label extension study at the end of the post-treatment period. In the second half of 2022, Avidity plans to conduct a preliminary assessment of safety, tolerability and key biomarkers in approximately half of the study participants. For more information on this study click [here](#) or visit <http://www.clinicaltrials.gov> and search for NCT05027269.

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 treatments for patients living with DM1.

About Avidity Biosciences

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 lead product candidate, AOC 1001, is designed to treat patients with myotonic dystrophy type 1 (DM1). AOC 1001 has commenced clinical testing with the ongoing Phase 1/2 MARINA™ trial in adults with DM1. It's advancing and expanding pipeline also includes programs in facioscapulohumeral muscular dystrophy (FSHD), Duchenne Muscular Dystrophy (DMD), muscle atrophy and Pompe disease. The company is planning for AOC 1044, the lead of three programs for the treatment of DMD, and its AOC FSHD program to enter the clinic in 2022. 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 La Jolla, 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 timing of commencing clinical trials and generating clinical trial data; the potential for the MARINA™ study to inform the development path for DM1 as well as future treatments for other diseases; the potential of AOC 1001 to treat patients with DM1; and the broad potential of AOCs to treat rare and serious diseases. 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 the 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; disruption to its operations from the COVID-19 pandemic; 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; unexpected adverse side effects or inadequate efficacy of its product candidates that may limit their development, regulatory approval and/or commercialization, or may result in recalls or product liability claims; and other risks described in prior press releases and in filings with the Securities and Exchange Commission (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 exist 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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