Avidity Biosciences Receives IND Clearance from FDA to Proceed with the Phase 1/2 MARINA™ Trial of AOC 1001 in Adults with Myotonic Dystrophy (DM1)

-First antibody oligonucleotide conjugate (AOC™) to enter the clinic-
-Volume 2 of virtual investor and analyst series today at 8:30 am ET featuring Dr. Nicholas E. Johnson, an expert and leading physician treating DM1 -

LA JOLLA, Calif., Aug. 2, 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 U.S. Food and Drug Administration (FDA) cleared the company to proceed with the Phase 1/2 MARINA™ clinical trial of AOC 1001 in adults with myotonic dystrophy type 1 (DM1).

AOC 1001, Avidity's lead program utilizing its AOC platform, is designed to address the root cause of DM1 by reducing levels of DMPK, the disease-related mRNA. AOC 1001 consists of a proprietary monoclonal antibody that binds to the transferrin receptor 1 (TfR1) conjugated with a small interfering RNA (siRNA) that targets DMPK mRNA.

"The FDA clearance to proceed with our Phase 1/2 clinical trial for AOC 1001 is a significant milestone for Avidity as we move into the clinic with our first AOC engineered to deliver to skeletal muscle," said Sarah Boyce, president and chief executive officer. "We are grateful to Dr. Johnson for joining us on today's call to discuss the current treatment of people living with DM1 and the important research that he and the Myotonic Dystrophy Clinical Research Network are conducting to further understand this progressive disease. We are continuing to engage with the DM1 patient, advocate and physician community as we actively work to get the trial up and running."

The MARINA™ Phase 1/2 Trial of AOC 1001 in Adults with DM1 and Recent Highlights

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 assess the activity of AOC 1001 across key biomarkers, including spliceopathy, a key biomarker for DM1, and knockdown of DMPK mRNA, the disease-related mRNA responsible for DM1. 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.

Recently, the FDA also granted Orphan Drug Designation to AOC 1001 for the treatment of DM1. The FDA grants Orphan Drug Designation to novel drugs that seek to treat a rare disease or condition and, if the drug is approved for the designated orphan indication, provides 7 years of market exclusivity, along with certain financial incentives, including tax credits, opportunities for grant funding towards clinical trial costs and FDA user-fee waivers.

Today's Video Webcast Information

The company is hosting Volume 2 of their virtual investor and analyst series today August 2, 2021 beginning at 8:30 am ET to further discuss the AOC 1001 program. The event is a live video webcast and can be accessed here or from the "Events and Presentations" page in the "Investors" section of Avidity's website. A replay of the webcast will be archived on Avidity's website following the event.

The management team will be joined by Dr. Nicholas E. Johnson, MD, MSCI, FAAN. Dr. Johnson is one of the principal investigators in END-DM1, an ongoing natural history study being run by the Myotonic Dystrophy Clinical Research Network (DMCRN) and will be the lead investigator in the Phase 1/2 trial for AOC 1001. Dr. Johnson is an associate professor, division chief of neuromuscular, and vice chair of research in the department of neurology at Virginia Commonwealth University.

About Myotonic Dystrophy Type 1 and AOC 1001

Myotonic dystrophy type 1 (DM1) is an underrecognized, progressive and often fatal disease caused by a triplet-repeat on 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.

AOC 1001, Avidity's lead program utilizing its AOC platform, is designed to address the root cause of DM1 by reducing levels of a disease-related mRNA. 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 a durable, dose-dependent reductions of DMPK RNA across a broad range of muscles including skeletal, cardiac, and smooth muscles. In preclinical studies, AOC 1001 had a favorable safety profile that supports advancement into the clinic. The FDA has cleared Avidity to proceed with the Phase 1/2 MARINA study of AOC 1001 in adults with DM1. FDA has granted Orphan Drug Designation for AOC 1001 for the treatment of 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 myotonic dystrophy type 1 (DM1). The FDA has cleared Avidity to proceed with the Phase 1/2 MARINA™ trial of AOC 1001 in adults with myotonic dystrophy type 1 (DM1). Its 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 initiation of a clinical trial of AOC 1001 in patients with DM1; the potential benefits associated with Orphan Drug Designation for approved drugs; plans for its other programs to enter the clinic and the timing thereof; 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 the business, including, without limitation: Avidity is early in its development efforts and all of its development programs are in the preclinical or discovery stage; 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; risks that the benefits associated with Orphan Drug Designation may not be realized, including that Orphan Drug exclusivity may not effectively protect a product from competition and that such exclusivity may not be maintained; 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; 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; risks related to integration of new management personnel; 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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