FDA Grants Fast Track Designation to AOC 1001 for the Treatment of Myotonic Dystrophy Type 1

LA JOLLA, Calif., Oct. 18, 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) has granted Fast Track Designation to its lead program, AOC 1001, for the treatment of myotonic dystrophy type 1 (DM1).

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.

"DM1 is an underrecognized, progressive and often fatal disease with no therapeutic options. Fast Track designation for AOC 1001 underscores this unmet need and allows us to expeditiously work with FDA to potentially deliver this first-in-class therapy to people living with DM1 as quickly as possible," said Sarah Boyce, president and CEO of Avidity.

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

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 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. 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 and 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 assess the activity of AOC 1001 across key biomarkers, including spliceopathy, a key 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 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 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 our current beliefs and expectations. Such forward-looking statements include, but are not limited to, statements regarding: the potential of AOC 1001 in people with myotonic dystrophy type 1 and the initiation of a clinical trial, and the timing thereof. The inclusion of forward-looking statements should not be regarded as a representation by Avidity that any of our plans will be achieved. Actual results may differ from those set forth in this press release due to the risks and uncertainties inherent in our business, including, without limitation: we are early in our development efforts and many of our development programs are in the preclinical or discovery stage; our approach to the discovery and development of product candidates based on our AOC platform is unproven, and we do not know whether we will be able to develop any products of commercial value; potential delays in the commencement, enrollment and completion of clinical trials; disruption to our operations from the COVID-19 pandemic; the success of our preclinical studies and clinical trials for our product candidates; the results of preclinical studies and early clinical trials are not necessarily predictive of future results; our dependence on third parties in connection with preclinical testing and product manufacturing; unexpected adverse side effects or inadequate efficacy of our 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 our proposed design of future clinical trials; risks related to integration of new personnel; and other risks described in our prior press releases and in our 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 we undertake 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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