European Medicines Agency (EMA) Grants Orphan Designation for AOC 1001

LA JOLLA, Calif., Aug. 24, 2021 /PRNewswire/ -- Avidity Biosciences, Inc. (Nasdaq: RNA), a biopharmaceutical company committed to delivering a new class of RNA therapeutics called Antibody Oligonucleotide Conjugates (AOCs™), announced that the European Commission (EC) has granted orphan designation for AOC 1001. The U.S. Food and Drug administration also granted orphan drug designation to AOC 1001 earlier this summer.

The U.S. Food and Drug Administration (FDA) cleared Avidity to proceed with the Phase 1/2 MARINA™ trial of AOC 1001 in adults with myotonic dystrophy type 1 (DM1) in the United States.

"We are pleased that both the EC and the FDA have granted AOC 1001 Orphan Designation, further validating AOCs as a powerful new class of drugs that can bring benefit to people suffering from untreated and undertreated diseases," said Sarah Boyce, president and chief executive officer of Avidity. "AOC 1001 is designed to address the root cause of DM1 and has the potential to be a first-in-class treatment for people living with this debilitating rare disease."

The EC grants orphan designation to drugs and biologics intended for the treatment, diagnosis or prevention of rare, life-threatening or chronically debilitating diseases or conditions that impact fewer than 5 in 10,000 patients in the European Union. Orphan designation allows companies certain benefits, including reduced regulatory fees, clinical protocol assistance, research grants and 10 years of market exclusivity following regulatory approval.

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 and EMA have granted Orphan Designation for AOC 1001.

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 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 myotonic dystrophy type 1 and the potential for AOC 1001 to be a treatment for DM1, and whether the FDA or EMA will grant AOC 1001 years of market exclusivity if approved, or significant development incentives, including reduced regulatory fees or clinical protocol assistance, research grants. 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; 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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