Avidity Biosciences Enrolls Patients in the MARINA™ Open-Label Extension Study

Adults with myotonic dystrophy type 1 (DM1) in the AOC 1001 Phase 1/2 MARINA™ clinical trial can receive continuity of care as part of MARINA-OLE™

Preliminary assessment of safety, tolerability and key biomarkers from Phase 1/2 MARINA trial on track for Q4 2022

SAN DIEGO, Aug. 2, 2022 /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 it has commenced enrolling patients from the Phase 1/2 MARINA™ study into a Phase 2 open-label extension study (MARINA-OLE™) of AOC 1001 in adults with myotonic dystrophy type 1 (DM1). All patients enrolled in the randomized, placebo-controlled MARINA™ clinical trial with AOC 1001 are eligible to enroll in MARINA-OLE.

DM1 is an underrecognized, progressive and often fatal neuromuscular disease. It primarily affects skeletal and cardiac muscle with multiple organ involvement and can be highly variable with respect to severity, presentation, and age of onset. There are currently no disease-modifying treatments for people living with DM1.

"The initiation of the MARINA-OLE study marks our continued progress with AOC 1001, the first AOC in clinical development. We are pleased to provide patients in the MARINA trial with the opportunity to receive AOC 1001 on an ongoing basis," said Steve Hughes, M.D., chief medical officer. "We look forward to the preliminary assessment from the Phase 1/2 MARINA trial as well as the long-term data from the MARINA-OLE study to provide further insight into the treatment of DM1 as well as future AOC treatments for rare diseases."

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). 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.

About the Phase 2 MARINA-OLE™ Study
MARINA-OLE™ is an open-label, multi-center study designed to evaluate the long-term safety and tolerability of AOC 1001 in DM1 patients who were previously enrolled in the MARINA Phase 1/2 study. This study will continue to evaluate the safety, tolerability, PK, PD, and efficacy of AOC 1001 in patients that enrolled in the randomized, placebo-controlled, Phase 1/2 MARINA clinical study. Patients who enroll in the MARINA-OLE study will receive quarterly doses of AOC 1001 regardless of whether they received active treatment or placebo in the MARINA study. The total duration of active treatment with AOC 1001 in the MARINA-OLE is approximately 24 months. Once patients have completed active treatment, there will be a 9-month safety follow-up period. Avidity may extend active treatment beyond 24 months at a future timepoint. For more information on this study click here or visit http://www.clinicaltrials.gov and search for NCT05479981.

About the Phase 1/2 MARINA™ Trial
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 have the option to enroll in MARINA-OLE, an open label extension study, at the end of the post-treatment period. In the fourth quarter of 2022, Avidity plans to conduct a preliminary assessment of safety, tolerability and key biomarkers in approximately half of the study participants in the MARINA study. For more information on this study click here or visit http://www.clinicaltrials.gov and search for NCT05027269.

About AOC 1001
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 is currently in Phase 1/2 development with the ongoing MARINA™ trial in adults with DM1. Patients in the MARINA study are eligible to enroll in the MARINA-OLE™ study. 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.

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 is on track to have three programs in clinical development by the end of 2022. The company's lead product candidate, AOC 1001, is designed to treat patients with myotonic dystrophy type 1 (DM1). AOC 1001 is currently in Phase 1/2 development with the ongoing MARINA™ trial and MARINA-OLE™ in adults with DM1. The next programs in the company's advancing and expanding pipeline are AOC 1044, the lead of three programs for the treatment of DMD, and AOC 1020, designed to treat people living with FSHD. Avidity anticipates both programs will enter the clinic by the end of 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 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 timing of enrolling patients or commencing MARINA-OLE™; generating and assessing MARINA™ trial data; generating key biomarker data from the MARINA trial; the potential for the MARINA study and the MARINA-OLE to inform the development path for DM1 as well as future treatments for other diseases using AOCs; the potential of AOC 1001 to safely and effectively treat patients with DM1; the tolerability of AOC 1001 in patients; 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.

Investor and Media Contact:
Kath Gallagher
kath.gallagher@aviditybio.com
(858) 401-7900

SOURCE Avidity Biosciences, Inc.

TM-Open-Label-Extension-Study