Avidity Biosciences Enters into Collaboration to Study the Natural History of Myotonic Dystrophy Type 1

SAN DIEGO, Sept. 8, 2020 /PRNewswire/ -- Avidity Biosciences, Inc. (Nasdaq: RNA), a biopharmaceutical company pioneering a new class of oligonucleotide-based therapies called Antibody Oligonucleotide Conjugates (AOCs™), today announced it entered into a collaboration supporting END-DM1, a natural history study to advance the understanding of disease progression in patients with myotonic dystrophy type 1 (DM1). END-DM1 (Establishing Biomarkers and Clinical Endpoints in Myotonic Dystrophy Type 1) is a non-interventional study designed and run by the Myotonic Dystrophy Clinical Research Network (DMCRN), a network of medical centers that aims to support future clinical trials of potential therapies for DM1 through the generation of evidence around endpoint measures and testing methods.

"By supporting END-DM1, we strengthen our commitment to patients and physicians to advance the knowledge of the natural progression, burden and premature mortality of this severe genetic disease," said Jae Kim, M.D., Chief Medical Officer of Avidity. "The findings from END-DM1 will help inform the clinical development of our lead program, AOC 1001, and we look forward to initiating a Phase 1/2 clinical trial in 2021 in adults with myotonic dystrophy type 1."

END-DM1 is designed to evaluate the full spectrum of disease severity and serve as the foundation for therapeutic development in DM1. END-DM1 plans to enroll approximately 650 people with DM1 aged 18-70 across nine DMCRN study sites in the U.S. and five sites in Europe. In addition to Avidity, supporters of END-DM1 include the U.S. Food & Drug Administration (FDA), the Myotonic Dystrophy Foundation, the Wyck Foundation, the Muscular Dystrophy Association, and other biotechnology companies.

"We are thrilled to have robust support for END-DM1 from leading industry sponsors, such as Avidity," said Nicholas Johnson, M.D., a co-principal investigator for END-DM1 at VCU Health in Richmond, Virginia. "This partnership allows for us to share the collective learnings about the disease and level of unmet need for patients living with DM1 to optimize therapeutic strategies to further accelerate the development of promising new therapeutics, like AOC 1001."

For more information about END-DM1, including study requirements and a list of participating sites, please visit www.myotonic.org.

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. 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 (GI) complications, and cognitive and behavioral impairment. Currently, there are no disease-modifying treatments for patients living with DM1.

AOC 1001, Avidity's lead program utilizing its AOC platform, is potentially the first disease-modifying treatment for DM1. AOC 1001 is designed to address the underlying cause of DM1 by reducing levels of mutant DMPK mRNA. AOC 1001 consists of a proprietary mAb that binds to transferrin receptor 1 (TfR1) conjugated with a siRNA. 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. Avidity expects to submit an investigational new drug application for AOC 1001 in 2021 and plans to initiate a Phase 1/2 clinical trial in DM1 patients by the end of 2021.

For more information about AOC 1001, please visit www.aviditybiosciences.com/programs.

About Avidity Biosciences

Avidity Biosciences, Inc. is pioneering a new class of oligonucleotide-based therapies called AOCs designed to overcome the current limitations of oligonucleotide therapies in order to treat a wide range of serious diseases. Avidity utilizes its proprietary AOC platform to design, engineer and develop therapeutics that combine the tissue selectivity of monoclonal antibodies and the precision of oligonucleotide therapies in order to access previously undruggable tissue and cell types and more effectively target underlying genetic drivers of diseases. Avidity's lead product candidate, AOC 1001, is designed to treat myotonic dystrophy type 1, and its four other muscle programs are focused on the treatment of muscle atrophy, Duchenne muscular dystrophy, facioscapulohumeral muscular dystrophy and Pompe disease. In addition to its muscle franchise, Avidity has

research efforts focused on immune and other cell types.

About VCU and VCU Health

Virginia Commonwealth University is a major, urban public research university with national and international rankings in sponsored research. Located in downtown Richmond, VCU enrolls more than 30,000 students in 233 degree and certificate programs in the arts, sciences and humanities. Twenty-two of the programs are unique in Virginia, many of them crossing the disciplines of VCU's 11 schools and three colleges. The VCU Health brand represents the VCU health sciences academic programs, the VCU Massey Cancer Center and the VCU Health System, which comprises VCU Medical Center (the only academic medical center in the region), Community Memorial Hospital, Children's Hospital of Richmond at VCU, MCV Physicians and Virginia Premier Health Plan. For more, please visit www.vcu.edu and vcuhealth.org.

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 planned enrollment of END-DM1 and the study's ability to inform clinical development plans for AOC 1001; the potential to develop a meaningful pipeline of novel AOC therapeutics; the selection of clinical candidates; the initiation of first-in-human studies; the anticipated timing of filing an IND for the treatment of DM1; and the broad potential of AOCs to treat serious diseases. 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 all 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 management 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.

Contacts:

Company:

Mike MacLean
(858) 401-7900
mikemaclean@avidity bio.com

Media and Investors:

Amy Conrad
Juniper Point
(858) 366-3243
amy@juniper-point.com

SOURCE Avidity Biosciences, Inc.
