Avidity Biosciences Receives FDA Breakthrough Therapy Designation for Delpacibart Etedesiran (AOC 1001) for Treatment of Myotonic Dystrophy Type 1

Avidity initiating global Phase 3 HARBOR™ study for delpacibart etedesiran this quarter

Delpacibart etedesiran data from MARINA-OLE™ showed reversal of disease progression in multiple functional measures in DM1 compared to END-DM1 natural history data

SAN DIEGO, May 8, 2024 /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 Breakthrough Therapy designation to delpacibart etedesiran (AOC 1001), the company's lead clinical development program, for the treatment of myotonic dystrophy type 1 (DM1). Delpacibart etedesiran, abbreviated as del-desiran, is an investigational treatment designed to address the root cause of DM1, an underrecognized, progressive and often fatal neuromuscular disease with no approved therapies.

"We are pleased that the FDA has granted Breakthrough Therapy designation to del-desiran for myotonic dystrophy type 1, underscoring the potential of del-desiran to be an effective treatment and the urgency of bringing this treatment to people living with DM1," said Sarah Boyce, president and chief executive officer at Avidity. "Initiation is underway for our global Phase 3 HARBOR™ study as we focus on rapidly advancing del-desiran for people living with DM1, who currently have no treatment options to address the underlying cause of this devastating rare muscle disease."

Avidity is initiating the global pivotal HARBOR™ study of del-desiran this quarter. The primary endpoint in the Phase 3 HARBOR trial is video hand opening time (vHOT) and key secondary endpoints include muscle strength as measured by hand grip strength and quantitative muscle testing (QMT) total score, and activities of daily living as measured by DM1-Activ. Avidity recently reported positive long-term MARINA-OLE™ data demonstrating reversal of disease progression in adults living with DM1 across multiple endpoints including vHOT, muscle strength and DM1-Activ when compared to natural history data.

In addition to receiving FDA Breakthrough Therapy designation, del-desiran has previously been granted Orphan Drug and Fast Track designations by the FDA and Orphan designation by the European Medicines Agency (EMA) for the treatment of DM1.

About the Phase 2 MARINA-OLE™ Study
MARINA-OLE™ is an open-label, multi-center trial designed to evaluate the long-term safety and tolerability of del-desiran (AOC 1001) in participants with DM1 who were previously enrolled in the MARINA® Phase 1/2 trial. This trial will continue to evaluate the safety, tolerability, PK, PD, and efficacy of del-desiran in participants enrolled in the randomized, placebo-controlled, Phase 1/2 MARINA clinical trial. Participants enrolled in the MARINA-OLE study receive quarterly doses of del-desiran regardless of whether they received active treatment or placebo in the MARINA study. The total duration of active treatment with del-desiran in the MARINA-OLE study is approximately 24 months. Once patients have completed active treatment, there will be a nine-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 Del-desiran (AOC 1001)
Del-desiran (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. Del-desiran consists of a proprietary monoclonal antibody that binds to the transferrin receptor 1 (TfR1) conjugated with a siRNA targeting DMPK mRNA. In preclinical studies, del-desiran 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. Del-desiran is currently in Phase 1/2 development with the completed MARINA® trial and the ongoing MARINA-OLE™ trial in adults with DM1. The U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) have granted Orphan Designation for del-desiran and the FDA has granted del-desiran 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 approved treatments for people living with DM1.

About Avidity
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 is revolutionizing the field of RNA with its proprietary AOCs, which are

designed to combine the specificity of monoclonal antibodies with the precision of oligonucleotide therapies to address targets and diseases previously unreachable with existing RNA therapies. Utilizing its proprietary AOC platform, Avidity demonstrated the first-ever successful targeted delivery of RNA into muscle and is leading the field with clinical development programs for three rare muscle diseases: myotonic dystrophy type 1 (DM1), Duchenne muscular dystrophy (DMD) and facioscapulohumeral muscular dystrophy (FSHD). Avidity is broadening the reach of AOCs with its advancing and expanding pipeline including programs in cardiology and immunology through internal discovery efforts and key partnerships. Avidity is headquartered in San Diego, CA. For more information about our AOC platform, clinical development pipeline and people, please visit www.aviditybiosciences.com and engage with us on LinkedIn and X.

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 implications of Breakthrough Therapy designation for the advancement of del-desiran; the global pivotal Phase 3 HARBOR™ trial for del-desiran, the timing of its initiation and key endpoints to be used therein; the MARINA-OLE™ study, its design, goals, dosage amounts and timelines; the potential of Avidity's product candidates, including del-desiran, to treat rare diseases and Avidity's efforts to bring them to people suffering from applicable diseases; the potential of AOCs to target a range of different cells and tissues beyond the liver, and to treat cardiac and immunological diseases; and Avidity's plans to expand its AOC platform and to invest in its pipeline programs. This press release also contains estimates and other statistical data made by independent parties and by us. This data involves a number of assumptions and limitations, and the reader is cautioned not to give undue weight to such estimates.

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 Avidity's business, including, without limitation: Avidity may not be able to resolve the partial clinical hold related to the serious adverse event which occurred in the Phase 1/2 MARINA trial; additional participant data related to del-desiran that continues to become available may be inconsistent with the data produced as of the date hereof, and further analysis of existing data and analysis of new data may lead to conclusions different from those established as of the date hereof; unexpected adverse side effects to, or inadequate efficacy of, Avidity's product candidates that may delay or limit their development, regulatory approval and/or commercialization, or may result in additional clinical holds which may not be timely lifted, recalls or product liability claims; 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, data readouts and completion of preclinical studies or clinical trials; Avidity's dependence on third parties in connection with preclinical and clinical testing and product manufacturing; regulatory developments in the United States and foreign countries; and other risks described in Avidity's Annual Report on Form 10-K for the fiscal year ended December 31, 2023, filed with the Securities and Exchange Commission (SEC) on February 28, 2024, and in subsequent filings with the 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 arise 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 Contact:
Geoffrey Grande, CFA
(619) 837-5014
investors@aviditybio.com

Media Contact:
Navjot Rai
(619) 837-5016
media@aviditybio.com

SOURCE Avidity Biosciences, Inc.

---

https://investors.aviditybiosciences.com/2024-05-08-Avidity-Biosciences-Receives-FDA-Breakthrough-Therapy-Designation-for-Delpacibart-Etedesiran-AOC-1001-for-Treatment-of-Myotonic-Dystrophy-Type-1