Avidity Biosciences Announces Completion of Enrollment for HARBOR™, the First Global Phase 3 Trial of Delpacibart Etedesiran (del-desiran) for Treatment of DM1 and Provides Guidance on Regulatory Submission

-- Topline data readout from HARBOR study anticipated in Q2 2026 --
-- Marketing application submissions for del-desiran including in U.S., EU and Japan anticipated to start in H2 2026; on track to potentially be the first globally approved drug for DM1--
-- On track to share updates from ongoing MARINA-OLE™ trial of del-desiran including long-term 4 mg/kg efficacy and safety data in Q4 2025 --

SAN DIEGO, July 28, 2025 /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 the completion of enrollment in the ongoing global Phase 3 HARBOR™ clinical trial of delpacibart etedesiran (del-desiran) for people living with myotonic dystrophy type 1 (DM1). Topline data from HARBOR, the first global Phase 3 clinical trial in DM1, are anticipated in the second quarter of 2026.

Prior to initiation of the HARBOR trial, Avidity aligned with global regulators, including FDA, on the registrational path for del-desiran. The Company plans to submit marketing applications beginning in the second half of 2026 including in the United States, European Union and Japan.

"Completing enrollment in our Phase 3 HARBOR study for del-desiran marks a significant step toward bringing the first potentially approved drug for DM1 to people around the world who are living with this devastating rare neuromuscular disease," said Steve Hughes, M.D., chief medical officer at Avidity. "The growing body of data from clinical studies to date support the promise of del-desiran, including durable improvements in multiple clinical endpoints compared to natural history data and evidence that del-desiran is addressing the underlying genetic cause of disease progression. These encouraging results suggest that this potential therapy may be transformational for patients who urgently need treatment options. We extend our deepest appreciation to the DM1 community for their ongoing support of our research and development program, especially the patients, families, global advocacy groups, healthcare providers and staff who are part of the HARBOR and MARINA-OLE™ studies."

Del-desiran is an investigational treatment designed to address the underlying genetic cause of DM1, a rare, hereditary, progressive neuromuscular disease that shortens life expectancy and requires life-long care. DM1 is characterized by multisystemic manifestations including myotonia and progressive muscle weakening and may be underrecognized because it presents heterogeneously across skeletal, cardiac, and smooth muscles, leading to impairment of the cardiovascular, gastrointestinal, respiratory, ocular, and/or endocrine systems. Currently, there are no approved drugs for people living with DM1.

Del-desiran has received Breakthrough Therapy, Orphan Drug and Fast Track designations by the U.S. Food and Drug Administration (FDA) and Orphan designation by the European Medicines Agency (EMA). Del-desiran was also the first investigational treatment for DM1 to receive Orphan Drug designation in Japan.

About the Phase 3 HARBOR™ Trial
The global Phase 3 HARBOR™ trial is a randomized, placebo-controlled, double blind pivotal study designed to evaluate del-desiran in approximately 150 people (age 16 and older) living with DM1. The trial is being conducted at approximately 40 sites globally. Patients are administered either del-desiran or placebo (1:1) every eight weeks. HARBOR is designed to assess multiple key functional aspects of DM1. The primary endpoint is video hand opening time (vHOT), a measurement of myotonia, which is a hallmark symptom of DM1. 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. All study participants, regardless of whether they receive active treatment or placebo, have the option to enroll into an open-label extension trial that is expected to begin in Q3 2025. For more information about the HARBOR trial, visit the HARBOR study website or visit http://www.clinicaltrials.gov and search for NCT06411288.

About Myotonic Dystrophy Type 1
Myotonic dystrophy type 1 (DM1) is a rare, hereditary (autosomal dominant), progressive neuromuscular disease that shortens life expectancy and requires life-long care 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. DM1 is characterized by multisystemic manifestations including myotonia and progressive muscle weakening and may be underrecognized because it presents heterogeneously across skeletal, cardiac, and smooth muscles, leading to impairment of the cardiovascular, gastrointestinal, respiratory, ocular, and/or endocrine systems. Currently, there are no approved drugs for people living with DM1.

About del-desiran

Del-desiran, utilizing Avidity's AOC platform technology, is designed to address the genetic cause of DM1 by reducing levels of toxic DMPK mRNA. Del-desiran consists of a proprietary monoclonal antibody that binds to transferrin receptor 1 (TfR1) and is conjugated to a siRNA that targets DMPK mRNA. Del-desiran is currently being assessed in the global Phase 3 HARBOR™ trial and in the ongoing MARINA-OLE™ trial in people with DM1. Long-term data from the MARINA-OLE trial showed reversal of disease progression in people living with DM1 across multiple endpoints including video hand opening time (vHOT) as a measure of hand function and myotonia, muscle strength and activities of daily living when compared to END-DM1 natural history data. Del-desiran has received Breakthrough Therapy, Orphan Drug and Fast Track designations by the U.S. Food and Drug Administration (FDA) and Orphan designation by the European Medicines Agency (EMA). Del-desiran was also the first investigational treatment for DM1 to receive Orphan Drug designation in Japan.

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 also advancing two wholly-owned precision cardiology development candidates addressing rare genetic cardiomyopathies. In addition, Avidity is broadening the reach of AOCs with its advancing and expanding pipeline including programs in cardiology and immunology through 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 characterization of data associated with del-desiran, and the impact of such data on the advancement of the del-desiran; the status of the global Phase 3 HARBOR™ trial, including but not limited to enrollment, design and goals; the regulatory status of a registrational path for del-desiran; Avidity's plans to release topline data from the HARBOR trial and the timing thereof; and Avidity's plans to submit marketing applications for del-desiran and the timing thereof.
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 and beyond its control, including, without limitation: preliminary results of a clinical trial are not necessarily indicative of final results; further analysis of existing clinical data and analysis of new data may lead to conclusions different from those established as of the respective data cutoff dates in Avidity's clinical study of del-desiran, and such data may not meet Avidity's expectations; unexpected adverse side effects to, or inadequate efficacy of, del-desiran that may delay or limit its development, regulatory approval and/or commercialization; later developments with the FDA and other global regulators that could be inconsistent with the feedback received to date regarding del-desiran and could affect the timing or likelihood of its potential approval; Avidity's approach to the discovery and development of product candidates based on its AOC™ platform is unproven; potential delays in the data readouts and completion of the HARBOR trial; Avidity's dependence on third parties in connection with clinical testing and product manufacturing; legislative, judicial and 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, 2024 and 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.

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