Avidity Biosciences Announces FDA Removed Partial Clinical Hold on Delpacibart Etedesiran (del-desiran/AOC 1001)

SAN DIEGO, Oct. 3, 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 removed the partial clinical hold on delpacibart etedesiran (del-desiran/AOC 1001), an investigational treatment designed to address the root cause of myotonic dystrophy type 1 (DM1).

Del-desiran is being evaluated in the ongoingPhase 3 HARBOR™ trial in patients with DM1, an underrecognized, progressive and often fatal neuromuscular disease with no approved therapies.

Del-desiran has received Breakthrough Therapy, Orphan Drug and Fast Track designations by the FDA and Orphan designation by the European Medicines Agency (EMA).

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. 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).

About Myotonic Dystrophy Type 1
Myotonic dystrophy type 1 (DM1) is an underrecognized, autosomal dominantly inherited, 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 advancement of del-desiran™ and the timing thereof; the characterization of data associated with del-desiran™, and the impact of such data on its advancement; the design, goals, timelines and expectations related to the global Phase 3 HARBOR™ trial of del-desiran; and Avidity's platform, planned operations and programs.

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 trials, and such data may not meet Avidity's expectations; 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; the FDA lifting the partial clinical hold related to del-desiran does not foreclose the possibility of additional issues of safety related to del-desiran; later developments with the FDA and other

global regulators that could be inconsistent with the feedback received to date regarding Avidity's clinical trials; Avidity's approach to the discovery and development of product candidates based on its AOC™ platform is unproven; 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; legislative, judicial and regulatory developments in the United States and foreign countries; Avidity could exhaust its available capital resources sooner than it currently expects; and other risks described in Avidity's Annual Report on Form 10-K for the fiscal year ended December 31, 2023 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.

Investor Contact:
Mike MacLean
(619) 837-5014
investors@aviditybio.com

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

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