Avidity Biosciences Announces U.S. Managed Access Program (MAP) for Investigational Therapy del-zota in DMD44

SAN DIEGO, Nov. 19, 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 its Managed Access Program (MAP) for investigational therapy delpacibart zotadirsen (del-zota) for eligible people with Duchenne muscular dystrophy mutations amenable to exon 44 skipping (DMD44) in the United States.

"We recognize the considerable needs facing the DMD44 community given there are no approved exon skipping therapies for this disease as well as the potential of del-zota based on the unprecedented data shown in our clinical studies," said Sarah Boyce, President and Chief Executive Officer of Avidity. "We are pleased to open this MAP to enable a compliant approach to providing del-zota to eligible patients as quickly as possible."

Under an FDA-authorized treatment protocol, Avidity will provide del-zota to eligible boys and men with DMD44 through participating healthcare providers. Enrollment is anticipated to begin by year end, and participants in EXPLORE44-OLE will have the option to transition to the MAP as they complete 2 years of treatment.

Avidity aligned with FDA on a path forward for a BLA submission for del-zota following an October 2025 pre-BLA meeting, with the submission planned for 2026 for accelerated approval. Participants will transition to commercial drug supply upon future potential FDA approval and product availability.

Additional information about the MAP, including eligibility criteria will be available on www.clinicaltrials.gov.

About Duchenne muscular dystrophy (DMD)
Duchenne muscular dystrophy (DMD) causes a lack of functional dystrophin that leads to stress and tears of muscle cell membranes, resulting in muscle cell death and the progressive loss of muscle function. The dystrophin protein maintains the integrity of muscle fibers and acts as a shock absorber through its role as the foundation of a group of proteins that connects the inner and outer elements of muscle cells. People living with DMD suffer from progressive muscle weakness that typically starts at a very young age. Over time, people with Duchenne will develop problems walking and breathing, and eventually, the heart and respiratory muscles will stop working. Those living with the condition often require special aid and assistance throughout their lives and have significantly shortened life expectancy. While there are treatments approved to treat people with DMD, there remains a very high unmet need. DMD is a monogenic, X-linked, recessive disease that primarily affects males, with one in 3,500 to 5,000 boys born worldwide having Duchenne.

About del-zota
Del-zota is designed to deliver phosphorodiamidate morpholino oligomers (PMOs) to skeletal muscle and heart tissue to specifically skip exon 44 of the dystrophin gene to enable dystrophin production in people living with Duchenne muscular dystrophy with mutations amenable to exon 44 skipping (DMD44). DMD is characterized by progressive muscle degeneration and weakness due to alterations of a protein called dystrophin that protects muscle cells from injury during contraction. Del-zota consists of a proprietary monoclonal antibody that binds to the transferrin receptor 1 (TfR1) conjugated to a PMO targeting exon 44. The Phase 1/2 EXPLORE44® trial of del-zota has been completed, and the EXPLORE44 Open-Label Extension trial (EXPLORE44-OLE™) of del-zota is currently ongoing. Topline data from the completed del-zota Phase 1/2 EXPLORE44 trial demonstrated unsurpassed delivery of PMOs to skeletal muscle, robust increases in dystrophin production, significant increases in exon 44 skipping, and significant and sustained decreases of creatine kinase levels to near normal in people living with DMD44. Additionally, participants in the EXPLORE44 clinical program demonstrated reversal of disease progression across key functional endpoints including Time to Rise from Floor (TTR), 4-Stair Climb (4SC), Performance of Upper Limb (PUL) and 10-Meter Walk/Run Test (10mWRT). Safety was assessed in all participants in the EXPLORE44-OLE trial, and del-zota continued to demonstrate a favorable long-term safety and tolerability profile. Most treatment emergent adverse events (TEAEs) were mild or moderate with the most common TEAEs (occurring in greater than 3 participants) being upper respiratory tract symptoms, diarrhea, fall, backpain and headache. One participant discontinued from EXPLORE44-OLE following an event of hypersensitivity. Del-zota has received Rare Pediatric Disease, Orphan Drug, Fast Track and Breakthrough Therapy designations by the U.S. Food and Drug Administration (FDA) and Orphan designation by the European Medicines Agency (EMA).

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 the pre-BLA meeting with the FDA; the anticipated timing of a BLA submission for del-zota; the likelihood of approval of a BLA submission for del-zota; the status, progress and potential of del-zota; 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: the additional CMC data to be submitted by Avidity as requested by the FDA, among other data and information to be included in a BLA for del-zota, may not be satisfactory to the FDA; 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 data cutoff dates in the clinical trial of del-zota, and such data may not meet Avidity's or FDA's expectations; unexpected adverse side effects to, or inadequate efficacy of, del-zota that may delay or limit its development, regulatory approval and/or commercialization; later developments with the FDA that could be inconsistent with the feedback received to date regarding del-zota and which could delay its currently anticipated timelines; Avidity's approach to the discovery and development of product candidates based on its AOC™ platform is unproven; potential delays in the EXPLORE44-OLE™ study; 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; 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, 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.

Investor Contact:
Kat Lange
(619) 837-5014
investors@aviditybio.com

Media Contact:
Kristina Coppola
(619) 837-5016
media@aviditybio.com

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