Avidity Biosciences' Del-zota Demonstrated Reversal of Disease Progression Across Key Functional Endpoints in EXPLORE44® and EXPLORE44-OLE™ Phase 1/2 Trial in People Living with DMD44

-- Unprecedented improvement compared to baseline and natural history in multiple functional measures including Time to Rise from Floor (TTR), 4-Stair Climb (4SC), Performance of Upper Limb (PUL) and 10-Meter Walk/Run Test (10mWRT) at approximately one year --

-- Unprecedented rapid reduction in creatine kinase (CK) to near normal levels maintained over 16 months of follow-up and 25% increase of normal in dystrophin production, reflecting sustained muscle fiber protection --

-- Avidity remains on track to submit Biologics License Application (BLA) at year end 2025 for accelerated approval --

-- Investor and analyst webcast event today at8:00 a.m. ET --

SAN DIEGO, Sept. 10, 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 positive new data from participants treated continuously with del-zota for one year in the EXPLORE44® and EXPLORE44-OLE™ trials. These data demonstrated reversal of disease progression and unprecedented improvement compared to baseline and natural history across multiple functional measures. Additional data from the EXPLORE44 program will be presented at upcoming scientific congresses.

DMD is a rare genetic condition characterized by progressive muscle damage and weakness beginning at a very young age due to the absence of dystrophin protein from birth. Del-zota is designed to deliver phosphorodiamidate morpholino oligomers (PMOs) to skeletal muscle and heart tissue to specifically skip exon 44 of the dystrophin mRNA and enable production of functional, near-full length dystrophin. Near-full length dystrophin retains key functional domains and may offer improved muscle protection for people living with DMD44.

"For the first time, we have data showing that sustained muscle protection leads to meaningful improvements across multiple key functional measures in DMD," said Sarah Boyce, President and Chief Executive Officer of Avidity. "These unprecedented data underscore the impact of our revolutionary targeted approach to deliver RNA directly to muscle. We are acting with urgency to rapidly advance the del-zota development program and remain on track to submit a Biologics License Application (BLA) to FDA at year end 2025 for accelerated approval. We extend our deepest appreciation for the continued dedication of the investigators and their teams and, most importantly, the participants in our clinical trials and their families as we pursue a new treatment option for this relentless and devastating disease."

Data from EXPLORE44® Clinical Development Program

Trial participants treated with del-zota demonstrated statistically significant increases of approximately 25 percent of normal in dystrophin production and restored total dystrophin up to 58 percent of normal. Creatine kinase (CK) levels rapidly reduced by greater than 80 percent compared to baseline and were sustained at near normal levels throughout the duration of evaluation with participants followed for up to 16 months. Additionally, 50 percent of participants had CK levels within the normal range at one year of treatment.

A total of 17 participants (12 ambulatory and 5 non-ambulatory) who began on the del-zota treated arm of EXPLORE44® and continued into the EXPLORE44-OLE™ have been followed for approximately one year. Given the study design, some participants received 5 mg/kg once every six weeks (Q6W) and some received 10 mg/kg once every eight weeks during EXPLORE44. All participants were transitioned to the 5 mg/kg (Q6W) dosing schedule during EXPLORE44-OLE. Not all participants could complete all assessments. Functional data[i] from these pooled dosing cohorts for del-zota treated participants, compared to DMD44 natural history (PRO-DMD-01), demonstrated improvement:

- **4-Stair Climb (4SC):** Improved from baseline by 2.1 seconds. In contrast, the natural history group declined from baseline by 2.7 seconds (DMD44 Nat Hx N=22; del-zota N=10).
- **10-Meter Walk/Run Test (10mWRT):** Improved from baseline by 0.7 seconds. In contrast, the natural history group declined from baseline by 1.5 seconds (DMD44 Nat Hx N=22; del-zota N=10).
- **Time to Rise from Floor (TTR):** Improved from baseline by 3.2 seconds. In contrast, the natural history group declined from baseline by 1.6 seconds (DMD Nat Hx N=19; del-zota N=6).
- **North Star Ambulatory Assessment (NSAA):** Remained stable. In contrast, the natural history group declined from baseline by 2.4 points (DMD44 Nat Hx N=20; del-zota N=10).
- **Performance of Upper Limb (PUL2):** Improved from baseline by 1.5 points. In contrast, the natural history group declined from baseline by 0.7 points.[ii] Similar PUL improvements were seen in both ambulatory and non-ambulatory

participants (DMD44 Nat Hx N=27; del-zota N=17).

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.

Avidity remains on track to submit a BLA to the U.S. Food and Drug Administration (FDA) at year end 2025. This will be the Company's first of three planned BLA submissions over a 12-month period. Avidity continues to prepare for a confirmatory study to support full global approval.

Video Webcast Information
The Company is hosting an investor and analyst event on Wednesday, September 10, 2025, at 8:00 a.m. ET. The virtual event will be available via a live video webcast and can be accessed here or from the "Events and Presentations" page in the "Investors" section of Avidity's website. A replay of the webcast will be archived on Avidity's website following the event.

About the EXPLORE44® Phase 1/2 Trial
The EXPLORE44® trial was a randomized, placebo-controlled, double-blind, Phase 1/2 clinical trial that enrolled 26 participants with Duchenne muscular dystrophy mutations amenable to exon 44 skipping (DMD44). The study was designed to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamic effects of single and multiple ascending doses of del-zota administered intravenously in healthy volunteers and participants living with DMD44. The EXPLORE44 trial assessed exon skipping and dystrophin protein levels in participants with DMD44. Participants with DMD44 had the option to enroll into EXPLORE44-OLE™, an open-label extension study, at the end of the post-treatment period. For more information about the EXPLORE44 trial, visit the EXPLORE44 study website or visit https://www.clinicaltrials.gov and search for NCT05670730.

About the Phase 2 EXPLORE44-OLE™ Trial
EXPLORE44-OLE™ is an open-label, multi-center trial designed to evaluate the long-term safety, tolerability, pharmacokinetics, pharmacodynamic effects and efficacy of del-zota in participants with DMD44. Enrollment has been completed in the EXPLORE44-OLE study, with 23 participants who were previously enrolled in the Phase 1/2 EXPLORE44® trial and 16 participants who directly enrolled in the EXPLORE44-OLE study. The trial includes ambulatory and non-ambulatory participants. Participants in the EXPLORE44-OLE study are receiving 5 mg/kg of del-zota every six weeks. The total duration of active treatment with del-zota in the EXPLORE44-OLE study is approximately 24 months. Once participants have completed active treatment, there will be a three-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 NCT06244082.

About the PRO-DMD-01 Natural History Study
PRO-DMD-01 was an observational, prospective natural history study (N=269) intended to study the progression of subjects with Duchenne muscular dystrophy (DMD). In collaboration with the Analysis Group®, experts in the analysis of DMD progression based on natural history, a matched subset of DMD44 participants (N=22) was utilized to analyze progression on key ambulatory functional endpoints at 12 months.[iii] For more information on this study clickhere or visit http://www.clinicaltrials.gov and search NCT01753804.

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). 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 meaningfulness of the del-zota functional data; the potential for del-zota to reverse the progression of DMD44; the status of the clinical study of del-zota; Avidity's plans to submit a BLA for del-zota and the timing thereof; Avidity's plans to present additional data from the EXPLORE44® program and the timing thereof; the characterization of data associated with del-zota and the impact of such data on the advancement of del-zota; Avidity's plans for DMD candidates beyond del-zota for DMD44; the design, goals and status of the EXPLORE44 and EXPLORE44-OLE™ studies; Avidity's plans and expectations to advance its clinical programs, and the timing thereof; 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 data cutoff dates in the clinical trial of del-zota, and such data may not meet Avidity's or regulators' 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 and other global regulators 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.

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[i] 4SC, 10mWRT, TTR and NSAA only performed in ambulatory participants. PUL performed in ambulatory and non-ambulatory participants.
[ii] A different NH comparator was used for the PUL 2.0 assessment. Brogna, C., Pane, M., Coratti, G., D'Amico, A., Pegoraro, E., Bello, L., Sansone, V. A. M., Albamonte, E., Messina, S., Pini, A., D'Angelo, M. G., Bruno, C., Mongini, T., Ricci, F. S., Berardinelli, A., Battini, R., Masson, R., Bertini, E. S., Politano, L., & Mercuri, E.; Italian DMD Group. (2023). Upper limb changes in DMD patients amenable to skipping exons 44, 45, 51 and 53: A 24-month study. *Children, 10*(4), 746. https://doi.org/10.3390/children10040746
[iii] This publication is based on research using data from data contributor CureDuchenne that has been made available through Vivli, Inc. Vivli has not contributed to or approved, and is not in any way responsible for, the contents of this publication.

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https://investors.aviditybiosciences.com/2025-09-10-Avidity-Biosciences-Del-zota-Demonstrated-Reversal-of-Disease-Progression-Across-Key-Functional-Endpoints-in-EXPLORE44-R-and-EXPLORE44-OLE-TM-Phase-1-2-Trial-in-People-Living-with-DMD44