Avidity Biosciences Announces Positive Pre-BLA Meeting with U.S. FDA for del-zota in DMD44 with a Submission Planned for Q1 2026

SAN DIEGO, Oct. 13, 2025 /PRNewswire/ -- Avidity Biosciences, Inc. (Nasdaq: RNA), a biopharmaceutical company committed to delivering a new class of RNA therapeutics called Antibody Oligonucleotide Conjugates (AOCs™) to profoundly improve people's lives, today announced that the Company completed a positive pre-BLA meeting with the U.S. Food and Drug Administration (FDA) regarding its upcoming BLA submission of delpacibart zotadirsen (del-zota). Del-zota, which received Breakthrough Therapy designation, is an investigational drug being evaluated as a potential treatment for people living with Duchenne muscular dystrophy who have gene mutations amenable to exon 44 skipping (DMD44).

The timing for the BLA submission has been updated to Q1 2026 from previous guidance of year end 2025 to ensure the FDA receives additional data to support the chemistry, manufacturing, and controls (CMC) package at time of submission.

"Our recent meeting with the FDA was highly collaborative and provided a clear path forward for our BLA submission," said Sarah Boyce, President and CEO, Avidity Biosciences. "For DMD, the accelerated approval pathway in the US is the best and fastest way to bring del-zota to people who need it. We are grateful the agency gave us clear guidance on the CMC data it needs at submission, and we believe this alignment can facilitate a successful BLA process. We greatly appreciate the dedicated, diligent reviewers from multiple functions within FDA who are enabling us to move this program forward with the urgency it deserves. We know that every minute matters for boys and young men living with DMD."

Avidity remains highly confident in the potential of del-zota and looks forward to filing a BLA for del-zota in Q1 2026, which will be the Company's first of three planned BLA submissions over a 12-month period. Additionally, Avidity continues to prepare a confirmatory study to support full global approval of del-zota.

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.

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