

Avidity Biosciences Plans First BLA Submission and Accelerates Commercialization Preparations for Three Rare Muscle Disease Programs in 2025

Accelerated approval path confirmed for del-zota; Biologics License Application (BLA) submission planned for year end 2025

On track to complete enrollment in del-desiran HARBOR™ Phase 3 and potential registrational del-brax biomarker cohort as well as initiate global pivotal trial for del-brax by mid-2025

Eric Mosbrooker expands role to Chief Commercial Officer; Charles Calderaro III, Chief Technical Officer, and Kat Lange, Chief Business Officer, join as company prepares for global commercialization and continued pipeline expansion

Avidity to present at 43rd Annual J.P. Morgan Healthcare Conference on Tuesday, January 14

SAN DIEGO, Jan. 8, 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 progress and planning for a transformational year in 2025. These major anticipated milestones include executing on three potentially registrational programs, filing a Biologics License Application (BLA) for delpacibart zotadirsen (del-zota) for people living with Duchenne muscular dystrophy mutations amenable to exon 44 skipping (DMD44), preparing for multiple product launches in DMD44, myotonic dystrophy type 1 (DM1) and facioscapulohumeral muscular dystrophy (FSHD) and building a global, fully integrated company to support a growing AOC pipeline in neuromuscular diseases and precision cardiology.

"We look forward to an exciting year ahead as we prepare to become a commercial organization and transition to the next stage of delivering AOCs to people living with rare diseases who have no or limited treatment options. In 2025, we plan to file our first BLA, accelerate commercial preparations for three potential product launches in rapid succession and broaden our pipeline," said Sarah Boyce, President and Chief Executive Officer at Avidity. "We are very pleased that the accelerated approval path is open for del-zota in DMD44 and that global regulators are aligned on a registrational path for del-desiran in DM1. We are now setting our sights on finalizing the global registrational path for del-brax in FSHD, including a potential accelerated approval strategy in the U.S. We continue to focus on rapid execution and are on track to fully enroll del-desiran's DM1 Phase 3 HARBOR™ trial as well as the biomarker cohort of the del-brax FORTITUDE™ trial on time. As we expand the reach of the AOC platform to precision cardiology and next-generation technology innovations, we continue to make many new and revolutionary advances for patients, our company and the biotech industry."

Expanding the Leadership Team to Support Anticipated Growth

To execute on the full range of strategic initiatives and growth anticipated in 2025 and beyond, Avidity continues to enhance and deepen the company's leadership team:

- Eric B. Mosbrooker expands his role to Chief Commercial Officer (CCO) to lead Avidity's multiple global product launches;
- Charles (Chuck) Calderaro III, joins as Chief Technical Officer (CTO) to lead technical development and operations; and,
- Kat Lange joins as Senior Vice President and Chief Business Officer (CBO) to lead investor relations and business development.

"With Eric transitioning to CCO as well as Chuck and Kat joining, we are expanding our team and the organizational structure to propel Avidity forward to becoming a global, commercial company launching therapies in three separate rare diseases to profoundly improve the lives of people living with DMD44, DM1 and FSHD. As we prepare for the first of these potential launches, our early initiatives include building global medical affairs and commercial teams devoted to serving the needs of these rare disease communities that are waiting for treatments," continued Ms. Boyce.

2025 Upcoming Clinical and Regulatory Highlights

- **Delpacibart zotadirsen (del-zota) for the treatment of DMD44:**
 - Planned BLA submission year end 2025
 - The U.S. Food and Drug Administration (FDA) confirmed the accelerated approval path is available for del-zota and that the clinical data package from the EXPLORE44™ program could support a BLA filing
 - Presentation of topline data from the EXPLORE44 trial (Q1)
 - Presentation of topline data from the ongoing EXPLORE44-OLE™ trial (Q4)
- **Delpacibart etedesiran (del-desiran) for the treatment of DM1:**

- Presentation of additional data analyses from the Phase 1/2 MARINA® trial (Q1)
 - Completion of enrollment of the ongoing Phase 3 HARBOR™ trial (mid-2025)
 - Update from the ongoing MARINA-OLE™ trial including long-term 4mg/kg and safety data (Q4)
 - Planned marketing application submissions in 2026, including in the U.S. and European Union
- **Delpacibart braxlosiran (del-brax) for the treatment of FSHD:**
 - Regulatory alignment on a global Phase 3 trial design (Q2)
 - Alignment on a potential accelerated approval path for the ongoing FORTITUDE™ biomarker cohort (Q2)
 - Completion of enrollment of the FORTITUDE biomarker cohort (Q2)
 - Presentation of topline data from the FORTITUDE trial (Q2)
 - Initiation of a global, potentially registrational trial in FSHD (Q2)

J.P. Morgan Healthcare Conference – Webcast Information

Sarah Boyce, President and Chief Executive Officer, will be presenting at the **43rd Annual J.P. Morgan Healthcare Conference in San Francisco on Tuesday, January 14, 2025 at 7:30 a.m. PT / 10:30 a.m. ET.** A live webcast of the event, up-to-date event details and an archived replay will be available on the "Events and Presentations" page in the "Investors" section of Avidity's website at <https://aviditybiosciences.investorroom.com/events-and-presentations>.

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 neuromuscular 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: Avidity's plans for a BLA submission for del-zota and the timing thereof; the status of three of Avidity's programs as potentially registrational; the status of Avidity's ongoing clinical trials and cohorts therein, including but not limited to initiation, enrollment, design and goals; the ability for del-zota and del-brax to achieve accelerated approval; the presentation of additional data, analyses and other updates from Avidity's ongoing clinical programs and the timing thereof; planned marketing applications for del-desiran in the U.S. and European Union and the timing thereof; Avidity's plans to become a global commercial organization and the status of its commercialization efforts; the expansion of the AOC platform; the goals of Avidity's personnel changes and the early initiatives prior to a first potential product launch; Avidity's plans to present at the J.P. Morgan Healthcare Conference; and plans and objectives of management for future operations. 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 data and results produced in Avidity's ongoing clinical trials as of the most recent respective cutoff dates may not be indicative of final results, may not support BLA submissions or accelerated approvals, may not be satisfactory to the FDA and other regulators, and new analyses of existing data and results may produce different conclusions than established as of the date hereof; the FDA and other regulators may change their positions on the statuses of Avidity's clinical trials and product candidates; even if approved, Avidity may not be able to execute any successful product launches; Avidity's efforts to build a global commercial organization may be unsuccessful; 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; 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 Avidity's ongoing clinical trials; 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, 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.

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