Avidity Biosciences Announces 2024 Corporate Priorities and Catalysts for Next Stage of Growth

Avidity plans to initiate global Phase 3 HARBOR™ trial of AOC 1001 for DM1 mid-2024

Company to report data from all three ongoing clinical programs in 2024
-- AOC 1001 data for DM1 in Q1, AOC 1020 data for FSHD in Q2, and AOC 1044 data for DMD44 in 2H --

Avidity to advance wholly-owned and partnered cardiology programs toward clinical development

Avidity appoints Eric B. Mosbrooker as Chief Strategy Officer

SAN DIEGO, Jan. 5, 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 its 2024 corporate priorities and catalysts for the next stage of growth. In mid-2024, Avidity plans to initiate the global Phase 3 HARBOR™ trial of AOC 1001 for adults living with myotonic dystrophy type 1 (DM1). The robust data package of AOC 1001 from the Phase 1/2 MARINA® trial and open-label extension study, MARINA-OLE™, has demonstrated consistent improvements across multiple functional endpoints including myotonia, muscle strength and mobility, and long-term favorable safety and tolerability results in people living with DM1. Avidity also plans to report data in 2024 from all three of its ongoing clinical programs targeting three distinct rare muscle diseases: DM1, Duchenne muscular dystrophy with mutations amenable to exon 44 skipping (DMD44) and facioscapulohumeral muscular dystrophy (FSHD) while advancing its cardiology programs toward clinical development. In addition, Avidity announced the appointment of Eric B. Mosbrooker as Chief Strategy Officer.

2024 Upcoming Milestones & Key Highlights

- Initiation of global Phase 3 HARBOR™ trial of AOC 1001 for DM1 in mid-2024
- Data readouts from all three clinical programs for three distinct muscle diseases
    - Q1 2024: First look at AOC 1001 efficacy data from MARINA-OLE™ trial in people living with DM1
    - Q2 2024: AOC 1020 data from a preliminary assessment in approximately half of patients in Phase 1/2 FORTITUDE™ trial in FSHD
    - 2H 2024: First look at AOC 1044 data in people living with DMD44 from Phase 1/2 EXPLORE44™ trial
- Dose-escalation of remaining study participants from 2 mg/kg to 4 mg/kg of AOC 1001 in the MARINA-OLE trial
- Advance wholly-owned and partnered cardiology programs toward clinical development following recent expansion of cardiology collaboration with Bristol Myers Squibb
- Strong cash balance with funding through 2025

"In 2024, we will continue to build on the remarkable functional data and long-term safety data from our DM1 clinical program and unprecedented data from our DMD44 clinical program while executing across multiple significant research and development initiatives. We plan to initiate the global pivotal study for people living with DM1, report data from all three of our clinical programs in rare muscle diseases, and advance our cardiology programs toward clinical development," said Sarah Boyce, president and chief executive officer. "In addition, as we evolve from a research and development company to an integrated global organization, we are excited to have Eric Mosbrooker join our leadership team. His proven track record in global commercial operations and deep understanding of our company's strategic imperatives as part of his tenure on our board will be valuable to the company in our next phase of growth."

With more than 20 years of experience in global commercial operations, Mr. Mosbrooker brings a wealth of expertise and leadership in areas including global pricing, marketing, distribution, and product launches in the life sciences industry. He has expertise in gene therapy, rare metabolic diseases, additional orphan conditions and oncology. Mr. Mosbrooker, who served two and a half years as a member of Avidity's board of directors, has stepped down from his board position to join the management team.

"I am honored to join Avidity's management team and enthusiastic about the immense potential of our AOC platform to improve the lives of people affected by serious diseases," said Mr. Mosbrooker, chief strategy officer at Avidity. "With a catalyst-rich 2024 ahead, I am excited to bring a global commercial view and strategic insights as we fulfill our mission to profoundly improve people's lives by revolutionizing a new class of targeted RNA therapeutics."

Prior to joining Avidity, Mr. Mosbrooker served as the Chief Operations Officer for Cognoa, where he spearheaded commercial initiatives, program management, product development, and business operations. He also previously held the roles of Chief Commercial Officer at Audentes Therapeutics, where he oversaw the gene therapy business unit, and Senior Vice President of the Global Orphan Business Unit at Horizon Pharmaceuticals. Mr. Mosbrooker holds a Bachelor of Science in Industrial Engineering from the University of Wisconsin – Madison.

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: Avidity's planned milestones, their prospects for success and the timing thereof; Avidity's corporate priorities; plans to initiate a global Phase 3 trial for people living with DM1; the anticipated release of data from the MARINA-OLE™, FORTITUDE™ and EXPLORE44™ trials and the timing thereof; plans for the progression of research and development initiatives, including in cardiology; Avidity's collaboration with Bristol Myers Squibb; Avidity's cash balance and ability to fund its operations; the appointment of a chief strategy officer and its importance to Avidity; Avidity's progress and evolution as a company; the potential of Avidity's product candidates to treat rare diseases and Avidity's efforts to bring them to people suffering from applicable diseases; the potential of AOCs to target a range of different cells and tissues beyond the liver, and to treat cardiac and immunological diseases; and Avidity's position in the RNA field.

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: Avidity may not be able to resolve the partial clinical hold related to the serious adverse event which occurred in the Phase 1/2 MARINA® trial, which may result in delays in the clinical development of AOC 1001; additional data related to Avidity's current clinical programs that continues to become available may be inconsistent with the data produced as of the respective data cutoff dates, further analysis of existing data and analysis of new data may lead to conclusions different from those established as of the date hereof, 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, or may result in clinical holds which may not be timely lifted (if at all), recalls or product liability claims; Avidity is early in its development efforts; Avidity's approach to the discovery and development of product candidates based on its AOC platform is unproven, and the company does not know whether it will be able to develop any products of commercial value; potential delays in the commencement, enrollment, data readouts and completion of preclinical studies or clinical trials; the success of its preclinical studies and clinical trials for the company's product candidates; Avidity's dependence on third parties in connection with preclinical and clinical testing and product manufacturing; Avidity may not realize the expected benefits of its collaborations; regulatory developments in the United States and foreign countries; Avidity could exhaust its available capital resources sooner than it currently expects and fail to raise additional needed funds; and other risks described in Avidity's Annual Report on Form 10-K for the fiscal year ended December 31, 2022, filed with the Securities and Exchange Commission (SEC) on February 28, 2023, and in 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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