Avidity Biosciences Reports Fourth Quarter and Year-End 2023 Financial Results and Recent Highlights

Avidity is on-track to initiate global Phase 3 HARBOR™ trial of AOC 1001 for DM1 in mid-2024

Company to share first-look at long-term efficacy and safety data from MARINA-OLE™ trial in people living with DM1 at MDA Clinical & Scientific Conference and via webcast on March 4, 2024

Avidity to report data from clinical programs for people living with FSHD and DMD44 later this year

SAN DIEGO, Feb. 28, 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 reported financial results for the fourth quarter and year ended December 31, 2023, and highlighted recent corporate progress.

"With proven delivery of RNA to muscle and robust efficacy and long-term safety data from our ongoing clinical development programs, we are revolutionizing the delivery of RNA with our AOC technology," said Sarah Boyce, president and chief executive officer at Avidity. "2024 is poised to be a transformative year for Avidity as we plan to initiate the global Phase 3 HARBOR™ trial for people living with DM1 and report data from all three of our clinical development programs to treat rare muscle diseases – DM1, FSHD and DMD44. We also plan to advance our cardiology and additional skeletal muscle programs toward clinical development. We know how important these potential new treatments are for the patient community and are working diligently to bring these much-needed therapies to people living with rare diseases."

"We are pleased to be in a strong financial position, with $595 million at the close of 2023 and a cash runway through 2025," said Mike MacLean, chief financial officer and chief business officer at Avidity. "We are well positioned to rapidly advance our clinical development programs, strategically progress our pipeline and build the infrastructure needed as part of Avidity's next phase of growth."

Clinical Development Programs - Achievements & Updates

AOC 1001

- In October 2023, Avidity announced new positive AOC 1001 data demonstrating improvement in multiple additional functional endpoints and favorable long-term safety and tolerability in people living with myotonic dystrophy type 1 (DM1). These data augmented previously presented positive topline data from the Phase 1/2 MARINA® trial in April 2023 showing improvements in myotonia, muscle strength and mobility.
- Avidity will share a first-look at long-term efficacy and safety data from the MARINA-OLE™ trial of AOC 1001 in people living with DM1 via poster presentation during the 2024 MDA Clinical & Scientific Conference March 3-6 in Orlando, Florida. The company will also host a live webcast event on March 4 at 8:00 a.m. ET as part of its ongoing investor and analyst event series.
- Avidity is on-track to initiate the global Phase 3 HARBOR™ trial of AOC 1001 for adults living with DM1 in mid-2024.

AOC 1020

- In February 2023, the FDA and the European Medicines Agency (EMA) granted Orphan Designation for AOC 1020 for facioscapulohumeral muscular dystrophy (FSHD), and in January 2023, the FDA granted AOC 1020 Fast Track Designation for FSHD.
- In the second quarter of 2024, Avidity is planning to share preliminary data in approximately half of the study participants in the Phase 1/2 FORTITUDE™ trial of AOC 1020 in FSHD.

AOC 1044

- In December 2023, Avidity announced positive AOC 1044 data in healthy volunteers showing unprecedented delivery to muscle and up to 1.5% exon skipping from the Phase 1/2 EXPLORE44™ clinical trial for the treatment of Duchenne muscular dystrophy mutations amenable to exon 44 skipping (DMD44).
- In August and October 2023, the FDA and EMA, respectively, granted Orphan Designation for AOC 1044 for DMD44. The FDA granted AOC 1044 Fast Track Designation in April 2023 and Rare Pediatric Disease Designation in February 2024 for DMD44.
- In the second half of 2024, Avidity is planning to share 5 mg/kg cohort data from the Phase 1/2 EXPLORE44™ trial of people living with DMD44.

Collaboration Announcements

- In November 2023, Avidity announced the expansion of a global licensing and research collaboration with Bristol Myers Squibb. The expanded collaboration will focus on the discovery, development and commercialization of up to five cardiovascular targets with $100 million upfront and potential cumulative milestone payments to Avidity of up to $2.2 billion.

Organizational Highlights

- Avidity announced in January 2024 the appointment of Eric B. Mosbrooker as Chief Strategy Officer. Mr. Mosbrooker previously served as a member of Avidity's Board of Directors.

Fourth Quarter and Year-End 2023 Financial Results

- **Cash, Cash Equivalents and Marketable Securities:** Cash, cash equivalents and marketable securities totaled $595.4 million as of December 31, 2023, which reflects $160.5 million raised in 2023, inclusive of approximately $100 million through our collaboration with Bristol Myers Squibb and $60.5 million through our "at the market offering" program.
- **Collaboration Revenue:** Collaboration revenue, including reimbursable expenses, primarily relates to Avidity's partnership with Eli Lilly and Company and totaled $2.2 million for the fourth quarter of 2023 compared with $2.8 million for the fourth quarter of 2022, and $9.6 million for the full year 2023 compared with $9.2 million for the full year 2022.
- **Research and Development (R&D) Expenses**: R&D expenses include external and internal costs associated with research and development activities. These expenses were $52.8 million for the fourth quarter of 2023 compared with $45.6 million for the fourth quarter of 2022, and $191.0 million for the full year 2023 compared with $150.4 million for the full year 2022. The increases were primarily driven by the advancement of AOC 1001, AOC 1020 and AOC 1044, as well as internal and external costs related to the expansion of the company's overall research capabilities.
- **General and Administrative (G&A) Expenses:** G&A expenses primarily consist of employee-related expenses, professional fees, insurance costs and patent filing and maintenance fees. These expenses were $16.1 million for the fourth quarter of 2023 compared with $10.4 million for the fourth quarter of 2022, and $54.2 million for the full year 2023 compared with $37.7 million for the full year 2022. The increases were primarily due to higher personnel costs and professional fees to support the company's expanded operations.

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 focused on 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: 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; the characterization of safety, tolerability and functional data associated with Avidity's clinical development programs; plans for the progression of research and development initiatives, including in cardiology and skeletal muscle; Avidity's collaboration with Bristol Myers Squibb; Avidity's financial position, cash balance and ability to fund its operations; Avidity's growth and related needs; plans for the progression of Avidity's pipeline and clinical programs for AOC 1001, AOC 1044 and AOC 1020, and the timing thereof; 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; 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 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 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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Avidity Biosciences, Inc.
Selected Condensed Financial Information
(in thousands, except per share data)
(unaudited)

Statements of Operations	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2023	2022	2023	2022
Collaboration revenue	$ 2,193	$ 2,769	$ 9,560	$ 9,224
Operating expenses:				
Research and development	52,817	45,610	190,968	150,404
General and administrative	16,119	10,384	54,190	37,733
Total operating expenses	68,936	55,994	245,158	188,137
Loss from operations	(66,743)	(53,225)	(235,598)	(178,913)
Other income, net	6,300	2,754	23,378	4,918
Net loss	$ (60,443)	$ (50,471)	$ (212,220)	$ (173,995)
Net loss per share, basic and diluted	$ (0.79)	$ (0.88)	$ (2.91)	$ (3.34)
Weighted-average shares outstanding, basic and diluted	76,052	57,296	73,012	52,162

Balance Sheets	December 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash, cash equivalents and marketable securities	$ 595,351	$ 610,727
Prepaid and other assets	15,956	12,215
Total current assets	611,307	622,942
Property and equipment, net	8,381	6,254
Restricted cash	295	251
Right-of-use assets	8,271	8,755
Other assets	301	598
Total assets	$ 628,555	$ 638,800
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and other liabilities	$ 52,315	$ 46,867
Deferred revenue, current portion	28,365	5,041
Total current liabilities	80,680	51,908
Lease liabilities, net of current portion	6,213	7,582
Deferred revenue, net of current portion	40,898	1,235
Total liabilities	127,791	60,725
Stockholders' equity	500,764	578,075
Total liabilities and stockholders' equity	$ 628,555	$ 638,800

SOURCE Avidity Biosciences, Inc.

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