Avidity Biosciences Reports First Quarter 2024 Financial Results and Recent Highlights

Initiation of global Phase 3 HARBOR™ trial for del-desiran (AOC 1001) in DM1 on track for this quarter

Avidity to report FSHD data from FORTITUDE™ trial this quarter and DMD data from EXPLORE44™ trial in 2H24

Presented positive long-term del-desiran data from MARINA-OLE™ showing reversal of disease progression in people living with myotonic dystrophy type 1 across multiple endpoints; same key endpoints, with vHOT as primary, agreed for phase 3 HARBOR trial

Cash position of $915 million at the end of Q124 following successful private placement

SAN DIEGO, May 9, 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 first quarter ended March 31, 2024, and highlighted recent corporate progress.

"As we move forward with the planned initiation of our global Phase 3 HARBOR™ study for del-desiran this quarter, we continue to work diligently to improve people's lives by advancing our AOC platform," said Sarah Boyce, president and chief executive officer at Avidity. "In March, we announced new long-term del-desiran data from our MARINA-OLE study showing reversal of disease progression in people living with DM1 across multiple endpoints including video hand opening time (vHOT), muscle strength and activities of daily living when compared to END-DM1 natural history data. Importantly, we also secured agreement with global regulators on the inclusion of these endpoints in the HARBOR study, including vHOT as the primary endpoint. We look forward to sharing preliminary data in approximately half of the study participants in our Phase 1/2 FORTITUDE™ trial in people living with FSHD this quarter and 5 mg/kg cohort data from our Phase 1/2 EXPLORE44™ trial of people living with DMD44 in the second half of this year."

"Following an oversubscribed equity raise of $400 million, our cash position of $915 million at the end of the first quarter provides us with funding into late 2026," said Mike MacLean, chief financial officer and chief business officer at Avidity. "With our positive long-term data for del-desiran and two additional data readouts planned from our FSHD and DMD44 clinical programs this year, we continue to make significant progress in advancing our clinical programs."

Recent Highlights

- Avidity announced that it received Breakthrough Therapy designation from the FDA for del-desiran for the treatment of DM1
- The company announced it accelerated the global Phase 3 HARBOR™ study initiation to Q2 2024 following agreement with multiple regulators on study design. The primary endpoint is video hand opening time (vHOT) and key secondary endpoints include muscle strength and activities of daily living
- Avidity introduced delpacibart etedesiran as the approved international nonproprietary name of AOC 1001, abbreviated as del-desiran
- Presented positive del-desiran long-term 4 mg/kg data from MARINA-OLE™ study in March 2024. Data showed:
    - Reversal of disease progression in people living with myotonic dystrophy type 1 (DM1) across multiple endpoints including vHOT, muscle strength and activities of daily living when compared to END-DM1 natural history data
    - Consistent and durable improvements in the following:
        - Myotonia (video hand opening time, or vHOT)
        - Multiple measures of strength:
            - Hand grip
            - Quantitative Muscle Testing (QMT) total score which includes hand grip; elbow extension and elbow flexion; knee extension and knee flexion, and ankle dorsiflexion
    - DM1-Activ, a patient reported outcome (PRO) that measures activities of daily living (e.g., taking a shower, visiting family or friends, and walking up stairs)
    - With over 265 infusions totaling 61.1 patient-years of exposure, del-desiran continues to demonstrate favorable safety and tolerability
- The FDA granted AOC 1044 with Rare Pediatric Disease Designation in February 2024 for DMD44

Upcoming Milestones

- Upcoming anticipated milestones include:
    - In the second quarter of 2024, initiation of global Phase 3 HARBOR™ trial of del-desiran for adults living with DM1
    - In the second quarter of 2024, share preliminary data in approximately half of participants in the Phase 1/2 FORTITUDE™ trial of AOC 1020 in people living with FSHD
    - In the second half of 2024, share 5 mg/kg cohort data from the Phase 1/2 EXPLORE44™ trial of AOC 1044 in people living with DMD44

First Quarter 2024 Financial Results

- **Cash, Cash Equivalents and Marketable Securities:** Cash, cash equivalents and marketable securities totaled $915.9 million as of March 31, 2024, which reflects a gross $400 million raise from a private placement.
- **Collaboration Revenue:** Collaboration revenue relates to Avidity's research collaboration and license partnerships. Collaboration revenues of $3.5 million in the first quarter of 2024 related to partnerships with Bristol Myers Squibb and Eli Lilly and Company as compared to $2.2 million related to a partnership with Eli Lilly and Company in the first quarter of 2023. There was no revenue related to the partnership with Bristol Myers Squibb in 2023.
- **Research and Development (R&D) Expenses**: R&D expenses include external and internal costs associated with research and development activities. These expenses were $66.8 million for the first quarter of 2024 compared with $47.8 million for the first quarter of 2023. The increases were primarily driven by the advancement of del-desiran, 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 $13.9 million for the first quarter of 2024 compared with $12.1 million for the first quarter of 2023. The increases were primarily due to higher personnel costs 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 the global Phase 3 HARBOR™ trial of del-desiran for people living with DM1; the anticipated release of data from the FORTITUDE™ and EXPLORE44™ trials, including the timing thereof and cohort dosage data; the characterization of data associated with del-desiran from the MARINA-OLE™ study; plans for the progression of research and development initiatives, including in cardiology and immunology; Avidity's financial position, cash balance and ability to fund its operations; Avidity's growth and related needs; and the potential of AOCs to target a range of different cells and tissues beyond the liver, and to treat cardiac and immunological diseases.

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; 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; later developments with the FDA and other global regulators that could be inconsistent with the feedback received to date regarding the proposed design and protocol for the Phase 3 HARBOR trial; 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 March 31,**	
	2024	**2023**
Collaboration revenue	$ 3,543	$ 2,233
Operating expenses:		
Research and development	66,832	47,765
General and administrative	13,898	12,064
Total operating expenses	80,730	59,829
Loss from operations	(77,187)	(57,596)
Other income, net	8,332	5,202
Net loss	$ (68,855)	$ (52,394)
Net loss per share, basic and diluted	$ (0.79)	$ (0.74)
Weighted-average shares outstanding, basic and diluted	87,212	70,433

Balance Sheets	**March 31, 2024**	**December 31, 2023**
Assets		
Current assets:		
Cash, cash equivalents and marketable securities	$ 915,873	$ 595,351
Prepaid and other assets	18,601	15,956
Total current assets	934,474	611,307
Property and equipment, net	8,655	8,381
Restricted cash	295	295
Right-of-use assets	7,625	8,271
Other assets	425	301
Total assets	$ 951,474	$ 628,555
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and other liabilities	$ 48,852	$ 52,315
Deferred revenue, current portion	26,103	28,365
Total current liabilities	74,955	80,680
Lease liabilities, net of current portion	5,421	6,213
Deferred revenue, net of current portion	40,199	40,898
Total liabilities	120,575	127,791
Stockholders' equity	830,899	500,764
Total liabilities and stockholders' equity	$ 951,474	$ 628,555

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https://investors.aviditybiosciences.com/2024-05-09-Avidity-Biosciences-Reports-First-Quarter-2024-Financial-Results-and-Recent-Highlights