Avidity Biosciences Reports First Quarter 2023 Financial Results and Recent Highlights

Positive topline data from AOC 1001 Phase 1/2 MARINA™ trial demonstrated functional improvement, disease modification and favorable safety and tolerability profile in people living with myotonic dystrophy type 1

Advancing three programs in clinical trials with data expected later this year - first look at data from open-label extension study, MARINA-OLE™; healthy volunteer data from EXPLORE44™

Expands research and development pipeline with rare skeletal muscle and first cardiology programs

SAN DIEGO, May 9, 2023 /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, 2023 and highlighted recent corporate progress. Avidity ended the first quarter of 2023 with cash, cash equivalents and marketable securities totaling $586.3 million.

"We are pleased with the positive AOC 1001 topline data reported at AAN demonstrating directional functional improvements across multiple functional measurements. We are focused on discussions with the FDA to define a path forward for AOC 1001 as soon as possible," said Sarah Boyce, president and chief executive officer at Avidity. "We have a number of important milestones from our pipeline expected this year including a first look at data from the MARINA-OLE™ trial and data from healthy volunteers in the Phase 1/2 EXPLORE44™ study. We are executing on our clinical and pipeline programs to develop much needed therapies for people living with devastating rare diseases."

"We ended Q1 with $586 million which puts us in a strong financial position with a runway to mid-2025. We continue to invest in our advancing and expanding pipeline in skeletal muscle and cardiology rare disease programs while also expanding the broad utility of our AOC platform through our internal discovery efforts and collaborations," said Mike MacLean, chief financial officer and chief business officer at Avidity.

Recent Highlights

- Presented topline data for MARINA™ at American Academy of Neurology (AAN) 2023 Annual Meeting in April 2023:
    - The MARINA trial concluded with the 38 participants with myotonic dystrophy type 1 (DM1). The company will continue to dose the participants at both 2 mg/kg and 4 mg/kg of AOC 1001 in the MARINA open-label extension study (MARINA-OLE™). AOC 1001 MARINA topline data demonstrated:
        - Directional improvement in multiple functional assessments including measures of myotonia, strength and mobility
        - Meaningful DMPK reduction and splicing changes in participants treated with AOC 1001 followed by directional improvements in functional measures at 2 mg/kg and 4mg/kg doses of AOC 1001
        - AOC 1001 demonstrated broad splicing improvements in more than a thousand genes impacted by DM1, confirming activity in the nucleus
        - Favorable safety and tolerability profile of AOC 1001 with most adverse events mild or moderate
- The company provided a regulatory update on the AOC 1001 partial clinical hold on new participant enrollment and provided more information on the rare serious adverse event in a single participant that led to the partial clinical hold. Avidity continues to work diligently with the FDA and remains very confident in the benefit/risk profile of AOC 1001.
- Avidity announced that it advanced and expanded its wholly-owned early stage AOC pipeline including adding a rare cardiology program and an additional program in rare skeletal muscle disease. Ongoing collaborations with Eli Lilly and Company (Lilly) and Bristol Myers Squibb (BMS) continue to advance beyond skeletal muscle in immunology and cardiology, respectively.
- The FDA granted AOC 1020 Fast Track Designation in January 2023 and in February 2023, the FDA and the European Medicines Agency (EMA) granted AOC 1020 Orphan Designation.
- The FDA also recently granted AOC 1044 Fast Track Designation in April 2023.

Upcoming Milestones

- The company continues to advance three distinct rare disease clinical programs: AOC 1001 for DM1, AOC 1020 for the treatment of facioscapulohumeral muscular dystrophy (FSHD) and AOC 1044 for the

treatment of Duchenne muscular dystrophy (DMD) mutations amenable to exon 44 skipping (DMD44). Upcoming milestones include:

- A first look at data from the MARINA-OLE trial is anticipated in late 2023.
- Data from healthy volunteers in the EXPLORE44 trial is anticipated in the second half of 2023.
- Data from a preliminary assessment in approximately half of participants in the FORTITUDE trial is anticipated in the first half of 2024.

First Quarter 2023 Financial Results

Cash, Cash Equivalents and Marketable Securities: Cash, cash equivalents and marketable securities totaled $586.3 million as of March 31, 2023, compared to $610.7 million as of December 31, 2022.

- **Collaboration Revenue:** Collaboration revenue, including reimbursable expenses, primarily relates to Avidity's partnership with Lilly and totaled $2.2 million for the first quarter of 2023 compared with $1.8 million for the first quarter of 2022.

- **Research and Development (R&D) Expenses:** R&D expenses include external and internal costs associated with research and development activities. These expenses were $47.8 million for the first quarter of 2023 compared with $27.7 million for the first quarter of 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 $12.1 million for the first quarter of 2023 compared with $8.6 million for the first quarter of 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's proprietary AOCs are designed to combine the specificity of monoclonal antibodies with the precision of oligonucleotide therapies to target the root cause of diseases previously untreatable with RNA therapeutics. Avidity's advancing and expanding pipeline has three programs in clinical development. AOC 1001 is designed to treat people with myotonic dystrophy type 1 (DM1) and is currently in Phase 1/2 development with the ongoing MARINA™ and MARINA-OLE™ trials. AOC 1020 is designed to treat people living with facioscapulohumeral muscular dystrophy (FSHD) and is currently in Phase 1/2 development with the FORTITUDE™ trial. AOC 1044 is designed for people with Duchenne muscular dystrophy (DMD) mutations amenable to exon 44 skipping and is currently in Phase 1/2 development with the EXPLORE44™ trial. AOC 1044 is the first of multiple AOCs the company is developing for DMD. Avidity is also broadening the reach of AOCs beyond muscle tissues through both internal discovery efforts and key partnerships as the company continues to deliver on the RNA revolution. Avidity is headquartered in San Diego, CA. For more information about our science, pipeline and people, please visit www.aviditybiosciences.com and engage with us on LinkedIn and Twitter.

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: expectations related to Avidity's ability to resolve the partial clinical hold and define a path forward for AOC 1001, and the timing thereof; the characterization and implications of the topline data from the Phase 1/2 MARINA™ trial; the safety and tolerability, and the benefit/risk profile of AOC 1001; the anticipated timing of data from the MARINA-OLE™, EXPLORE44™ and FORTITUDE™ trials; the progression of 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; the potential of AOCs to target a range of different cells and tissues beyond the liver, and to treat skeletal muscle, cardiac and immunological diseases; the continued advancement of collaboration programs with collaboration partners, including Eli Lilly and Company and Bristol Myers Squibb; Avidity's plans to expand its AOC platform and to invest in its pipeline programs; and the sufficiency of the company's current financial position to fund its development programs, investments in its pipeline and platform, and operations into mid-2025.

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, including, without limitation: the FDA may not remove the partial clinical hold related to the MARINA study and the analysis related to the underlying cause of the related

serious adverse event may result in delays in the MARINA study or an inability to complete the study; additional participant data related to AOC 1001 that continues to become available may be inconsistent with the data produced as of the date hereof, and further analysis of existing data and analysis of new data may lead to conclusions different from those established as of the date hereof; 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 additional clinical holds which may not be timely lifted, 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 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, including acceptance of INDs and similar foreign regulatory filings and the proposed design of future clinical trials; 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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Avidity Biosciences, Inc.
Selected Condensed Financial Information
(in thousands, except per share data)
(unaudited)

Statements of Operations	**Three Months Ended March 31,**	
	2023	**2022**
Collaboration revenue	$ 2,233	$ 1,795
Operating expenses:		
Research and development	47,765	27,688
General and administrative	12,064	8,567
Total operating expenses	59,829	36,255
Loss from operations	(57,596)	(34,460)
Other income, net	5,202	225
Net loss	$ (52,394)	$ (34,235)
Net loss per share, basic and diluted	$ (0.74)	$ (0.71)
Weighted-average shares outstanding, basic and diluted	70,433	48,246

Balance Sheets	**March 31, 2023**	**December 31, 2022**
Assets		
Current assets:		
Cash, cash equivalents and marketable securities	$ 586,300	$ 610,727
Prepaid and other assets	10,847	12,215
Total current assets	597,147	622,942
Property and equipment, net	7,845	6,254
Restricted cash	251	251
Right-of-use asset	8,259	8,755

Other assets	474	598
Total assets	$ 613,976	$ 638,800
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and other liabilities	$ 43,028	$ 46,867
Deferred revenue, current portion	5,057	5,858
Total current liabilities	48,085	52,725
Lease liabilities, net of current portion	6,976	7,582
Deferred revenue, net of current portion	—	1,439
Total liabilities	55,061	61,746
Stockholders' equity	558,915	577,054
Total liabilities and stockholders' equity	$ 613,976	$ 638,800

SOURCE Avidity Biosciences, Inc.

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https://aviditybiosciences.investorroom.com/2023-05-09-Avidity-Biosciences-Reports-First-Quarter-2023-Financial-Results-and-Recent-Highlights