

Avidity Biosciences Reports Second Quarter 2023 Financial Results and Recent Highlights

Advancing three clinical development programs – DM1, DMD, FSHD - with data anticipated from each program over the next 12 months

SAN DIEGO, Aug. 8, 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 and recent highlights for the second quarter ended June 30, 2023. Avidity ended the second quarter of 2023 with cash, cash equivalents and marketable securities totaling \$577 million.

"We are executing well across all three of our clinical development programs in three distinct rare muscle diseases – DM1, DMD and FSHD. We plan to report data from each of these clinical programs over the next 12 months," said Sarah Boyce, president and chief executive officer at Avidity. "With the positive topline data from MARINA™ and the easing of the partial clinical hold, we are working to finalize the Phase 3 study design and global regulatory path for AOC 1001 in DM1, while advancing our programs for DMD and FSHD. There are no approved treatment options for these muscle diseases. We are working to bring these much-needed therapies to people as quickly as possible."

"At the close of Q2, we maintain a strong cash balance of \$577 million. We are funded into the second half of 2025, allowing us to execute on advancing our clinical development programs in rare muscle diseases, expanding our AOC platform and broadening our pipeline in immunology, cardiology and other select indications outside of muscle," said Mike MacLean, chief financial officer and chief business officer at Avidity. "As leaders in the RNA field, we are committed to investing in our platform, pipeline and people as we revolutionize a new class of targeted RNA therapeutics for people living with rare diseases."

Recent Highlights

- The company is now enrolling participants living with Duchenne muscular dystrophy with mutations amenable to exon 44 skipping (DMD44) into the AOC 1044 EXPLORE44™ trial
- The Phase 1/2 MARINA trial concluded with 38 participants enrolled; 37 participants completed MARINA and have rolled over into the MARINA-OLE™
- In May, the U.S. Food and Drug Administration (FDA) eased the partial clinical hold on AOC 1001. Avidity is now dose escalating approximately 12 participants from 2 mg/kg to 4 mg/kg of AOC 1001 in the MARINA-OLE; in parallel, the company is finalizing a Phase 3 study design and a global regulatory path for AOC 1001
- In April, FDA granted Fast Track Designation for AOC 1044
- In April, AOC 1001 topline data demonstrating functional improvement, disease modification and favorable safety and tolerability profile in people living with myotonic dystrophy type 1 (DM1) were presented at the AAN Annual meeting. AOC 1001 provided directional improvements in multiple functional endpoint assessments including myotonia, measures of strength and mobility, as well as meaningful DMPK reduction and splicing changes.

Upcoming Milestones

- The company continues to advance three distinct rare disease clinical programs with AOC 1001 for DM1, AOC 1020 for the treatment of facioscapulohumeral muscular dystrophy (FSHD) and AOC 1044 for the treatment of DMD44. Upcoming milestones include:
 - Data from healthy volunteers in the EXPLORE44 trial planned for the fourth quarter of 2023
 - A first look at data from the MARINA-OLE trial planned for first half of 2024
 - Data from a preliminary assessment in approximately half of participants in the FORTITUDE™ trial planned for the first half of 2024

Second Quarter 2023 Financial Results

Cash, Cash Equivalents and Marketable Securities: Cash, cash equivalents and marketable securities totaled \$576.5 million as of June 30, 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 Eli Lilly and Company and totaled \$2.3 million for the second quarter of 2023 compared with \$2.2 million for the second quarter of 2022, and \$4.5 million for the first six months of 2023 compared with \$4.0 million for the first six months 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 \$42.6 million for the second quarter of 2023 compared with \$39.8 million for the second quarter of 2022, and \$90.4 million for the first six months of 2023 compared with \$67.5 million

for the first six months 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 \$12.3 million for the second quarter of 2023 compared with \$8.7 million for the second quarter of 2022, and \$24.3 million for the first six months of 2023 compared with \$17.3 million for the first six months 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 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 [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: the anticipated timing of release of data from the MARINA-OLE™, EXPLORE44™ and FORTITUDE™ trials; the design of and prospects for a Phase 3 trial and regulatory pathway for AOC 1001; plans the progression of clinical programs for AOC 1001, AOC 1044 and AOC 1020 and the timing thereof; the sufficiency of Avidity's cash balance to meet the company's operational needs and the expected cash runway; 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; the continued advancement of programs with collaboration partners, including Eli Lilly and Company; and Avidity's plans to expand its AOC platform and to invest in its pipeline programs.

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: 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 participant data related to AOC 1001 that continues to become available may be inconsistent with the data produced as of the most recent date cutoff, and further analysis of existing data and analysis of new data may lead to conclusions different from those established as of such date cutoff; 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, 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, including acceptance of INDs and similar foreign regulatory filings and the proposed design of future clinical trials; Fast Track Designation by the FDA may not lead to a faster development or regulatory review or approval process; 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 June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Collaboration revenue	\$ 2,316	\$ 2,178	\$ 4,549	\$ 3,973
Operating expenses:				
Research and development	42,672	39,789	90,437	67,477
General and administrative	12,278	8,688	24,342	17,255
Total operating expenses	54,950	48,477	114,779	84,732
Loss from operations	(52,634)	(46,299)	(110,230)	(80,759)
Other income, net	5,609	609	10,811	834
Net loss	\$ (47,025)	\$ (45,690)	\$ (99,419)	\$ (79,925)
Net loss per share, basic and diluted	\$ (0.66)	\$ (0.92)	\$ (1.40)	\$ (1.63)
Weighted-average shares outstanding, basic and diluted	71,390	49,927	70,914	49,091

Balance Sheets

Assets

Current assets:

	June 30, 2023	December 31, 2022
Cash, cash equivalents and marketable securities	\$ 576,503	\$ 610,727
Prepaid and other assets	18,031	12,215
Total current assets	594,534	622,942
Property and equipment, net	7,776	6,254
Restricted cash	295	251
Right-of-use asset	7,755	8,755
Other assets	424	598
Total assets	\$ 610,784	\$ 638,800

Liabilities and Stockholders' Equity

Current liabilities:

Accounts payable and other liabilities	\$ 41,434	\$ 46,867
Deferred revenue, current portion	3,810	5,041
Total current liabilities	45,244	51,908
Lease liabilities, net of current portion	6,362	7,582
Deferred revenue, net of current portion	—	1,235
Total liabilities	51,606	60,725
Stockholders' equity	559,178	578,075
Total liabilities and stockholders' equity	\$ 610,784	\$ 638,800

SOURCE Avidity Biosciences, Inc.

