

Avidity Biosciences Reports Second Quarter 2022 Financial Results and Recent Highlights

Company remains on track to have three programs in the clinic by the end of this year

Programs are for three distinct rare diseases - myotonic dystrophy type 1 (DM1), facioscapulohumeral muscular dystrophy (FSHD) and Duchenne muscular dystrophy (DMD)

SAN DIEGO, Aug. 9, 2022 /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 financial results for the second quarter ended June 30, 2022 and highlighted recent corporate progress.

"We are very pleased with the team's execution on our ambitious goal of having three AOC programs addressing three distinct rare diseases in the clinic by the end of this year," said Sarah Boyce, president and chief executive officer. "Our AOC 1001 program for DM1 is progressing with patients from the MARINA™ trial now enrolling in the recently initiated MARINA-OLE™ trial. We remain on track to report a preliminary assessment from MARINA in the fourth quarter. We also continue to collaborate with the FSHD and DMD communities as we work to advance AOC 1020 and AOC 1044 into the clinic by the end of this year."

"Our strong cash balance of over \$400 million, inclusive of additional funds raised subsequent to June 30th, positions us to be well funded through 2024. This allows us to meaningfully advance our DM1, FSHD and DMD programs, as well as other programs in skeletal muscle and expand the platform into other tissues and cell types," said Mike MacLean, chief financial and chief business officer.

Recent Highlights

- Commenced enrolling patients from the MARINA study into a Phase 2 open-label extension study (MARINA-OLE) of AOC 1001 in adults with DM1. Click [here](#) for more information on the MARINA-OLE or visit www.clinicaltrials.gov and search for NCT05479981.
- [Presented](#) data supporting Avidity's approach of directly targeting DUX4 with AOC 1020 at the 29th Annual FSHD Society International Research Congress. Key highlight from the conference:
 - Data from a FSHD mouse model showed that a single intravenous dose with the murine version of AOC 1020 prevented development of muscle weakness demonstrated by three functional assays - treadmill running, in vivo force and compound muscle action potential.

Second Quarter 2022 Financial Results

- **Cash, Cash Equivalents and Marketable Securities:** Cash, cash equivalents and marketable securities totaled \$398.2 million as of June 30, 2022, compared to \$405.5 million as of December 31, 2021. In addition, subsequent to June 30th, we have raised \$18.7 million through our "at the market" program.
- **Collaboration Revenue:** Collaboration revenue, including reimbursable expenses, primarily relates to Avidity's partnership with Eli Lilly and Company (Lilly) and totaled \$2.2 million for the second quarter of 2022 compared with \$2.6 million for the second quarter of 2021, and \$4.0 million for the first six months of 2022 compared with \$5.3 million for the first six months of 2021. The decrease was primarily due to timing of reimbursable collaboration-related research and development expenses resulting in the recognition of lower corresponding revenue under the collaboration with Lilly.
- **Research and Development (R&D) Expenses:** R&D expenses include external and internal costs associated with research and development activities. These expenses were \$39.8 million for the second quarter of 2022 compared with \$22.7 million for the second quarter of 2021, and \$67.5 million for the first six months of 2022 compared with \$43.4 million for the first six months of 2021. The increase was 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 \$8.7 million for the second quarter of 2022 compared with \$6.3 million for the second quarter of 2021, and \$17.3 million for the first six months of 2022 compared with \$12.2 million for the first six months of 2021. The increase was primarily due to higher personnel costs and professional fees as well as facilities 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'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 is on track to have three programs in clinical development by the end of 2022. The company's lead product candidate, AOC 1001, is designed to treat patients with myotonic dystrophy type 1 (DM1). AOC 1001 is currently in Phase 1/2 development with the ongoing MARINA™ trial and MARINA-OLE™ in adults with DM1. The next programs in the company's advancing and expanding pipeline are AOC 1044, the lead of three programs for the treatment of DMD, and AOC 1020, designed to treat people living with FSHD. Avidity anticipates both programs will enter the clinic by the end of 2022. 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: the expected timing for obtaining and disclosing preliminary data from the MARINA™ trial; the progression of clinical programs for AOC 1044 and AOC 1020 and timing thereof; the broad potential of AOCs to treat serious diseases of skeletal muscle and other tissues and cell types; and the sufficiency of the company's current financial position to fund its development programs, investments in its pipeline and platform, and operations through 2024. 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 the business, including, without limitation: 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; the results of preclinical studies and early clinical trials are not necessarily predictive of future results; Avidity's dependence on third parties in connection with preclinical and clinical testing and product manufacturing; unexpected adverse side effects or inadequate efficacy of its product candidates that may limit their development, regulatory approval and/or commercialization, or may result in recalls or product liability claims; 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 use its available capital resources sooner than it currently expects; disruption to its operations from the COVID-19 pandemic or the war in Ukraine; and other risks described in prior press releases and in filings with the Securities and Exchange Commission (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 exist 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,	
	2022	2021	2022	2021
			\$	\$
Collaboration revenue	\$ 2,178	\$ 2,607	3,973	5,311
Operating expenses:				
Research and development	39,789	22,706	67,477	43,383
General and administrative	8,688	6,268	17,255	12,152
Total operating expenses	48,477	28,974	84,732	55,535
Loss from operations	(46,299)	(26,367)	(80,759)	(50,224)
Other income (expense), net	609	14	834	27
			\$	\$
Net loss	\$ (45,690)	\$ (26,353)	(79,925)	(50,197)
			\$	\$
Net loss per share, basic and diluted	\$ (0.92)	\$ (0.70)	(1.63)	(1.34)

Weighted-average shares outstanding, basic and diluted	49,927	37,583	49,091	37,552
Balance Sheets			June 30, 2022	December 31, 2021
Assets				
Current assets:				
Cash, cash equivalents and marketable securities			\$ 398,218	\$ 405,543
Prepaid and other assets			6,033	5,598
Total current assets			404,251	411,141
Property and equipment, net			6,137	4,805
Restricted cash			251	251
Right-of-use asset			9,747	10,784
Other assets			415	599
			\$ 420,801	\$ 427,580
Total assets				
Liabilities and Stockholders' Equity				
Current liabilities:				
Accounts payable and other liabilities			\$ 28,307	\$ 24,794
Deferred revenue, current portion			5,380	4,864
Total current liabilities			33,687	29,658
Lease liabilities, net of current portion			8,753	9,960
Deferred revenue, net of current portion			4,010	6,532
Total liabilities			46,450	46,150
Stockholders' equity			374,351	381,430
Total liabilities and stockholders' equity			\$ 420,801	\$ 427,580

SOURCE Avidity Biosciences, Inc.

<https://investors.aviditybiosciences.com/2022-08-09-Avidity-Biosciences-Reports-Second-Quarter-2022-Financial-Results-and-Recent-Highlights>