

## Avidity Biosciences Reports Third Quarter 2022 Financial Results and Recent Highlights

*Company has three distinct rare disease programs in clinical development - myotonic dystrophy type 1 (DM1), facioscapulohumeral muscular dystrophy (FSHD), and Duchenne muscular dystrophy (DMD)*

*On track for preliminary assessment of AOC 1001 MARINA™ trial in fourth quarter*

SAN DIEGO, Nov. 8, 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 third quarter ended September 30, 2022 and highlighted recent corporate progress.

"Consistent with our guidance for 2022, we have progressed three programs addressing three distinct rare diseases into clinical development, and we remain on track for our preliminary assessment of the MARINA™ trial this quarter," said Sarah Boyce, president and chief executive officer. "Our team continues to execute on our plan as we advance AOC 1020 for FSHD in the FORTITUDE™ trial and AOC 1044 for DMD in the EXPLORE44™ trial. With close to 40 participants enrolled in the MARINA trial, we continue to gather data on AOC 1001 as we work to resolve the recent partial clinical hold on new participant enrollment as swiftly as possible."

"With approximately \$425 million, inclusive of additional funds raised subsequent to September 30<sup>th</sup>, we have cash runway through 2024 allowing us to invest in the expansion of our AOC platform and our three clinical stage programs addressing rare skeletal muscle diseases," said Mike MacLean, chief financial and chief business officer.

### Recent Highlights

- U.S. Food and Drug Administration (FDA) placed a partial clinical hold on new participant enrollment in the Phase 1/2 MARINA clinical trial of AOC 1001 in adults with DM1. The partial hold is in response to a serious adverse event reported in a single participant in the 4mg/kg cohort of the MARINA study. All current participants, whether they are on AOC 1001 or placebo, may continue in their current dosing cohort and roll over into the MARINA open label extension (MARINA-OLE™) where they will receive AOC 1001.
- The Company remains on track to report a preliminary assessment from the MARINA trial in the fourth quarter of 2022.
- The FDA cleared Avidity to proceed with Phase 1/2 trials for AOC 1020 for adults with FSHD and AOC 1044 for participants with DMD mutations amenable to exon 44 skipping (DMD44).
  - The FORTITUDE trial is a randomized, placebo-controlled, double-blind, Phase 1/2 clinical trial designed to evaluate AOC 1020 in 68 adult participants with FSHD. Click [here](#) for additional details on the FORTITUDE trial.
  - The EXPLORE44 trial is a randomized, placebo-controlled, double-blind, Phase 1/2 clinical trial to evaluate AOC 1044 in approximately 40 healthy volunteers and 24 participants with DMD44, ages seven to 27 years old. AOC 1044 is the first AOC PMO to enter the clinic and the first of multiple DMD programs in development at Avidity. Click [here](#) for additional details on the EXPLORE44 trial.
- Avidity also announced that it presented five poster presentations at the 27<sup>th</sup> International Hybrid Annual Congress of the World Muscle Society (WMS) in Halifax, Nova Scotia, Canada. To view those presentations, please visit the [publications page](#) on the Avidity website.

### Third Quarter 2022 Financial Results

- **Cash, Cash Equivalents and Marketable Securities:** Cash, cash equivalents and marketable securities remained constant at \$405.5 million as of September 30, 2022, and December 31, 2021, respectively. In addition, subsequent to September 30<sup>th</sup>, we have raised \$19.4 million through our "at the market" program.
- **Collaboration Revenue:** Collaboration revenue, including reimbursable expenses, which primarily relates to Avidity's partnership with Eli Lilly and Company (Lilly), increased to \$2.5 million for the third quarter of 2022 from \$2.2 million for the third quarter of 2021 primarily due to an increase of reimbursable internal costs driven by the increase in labor rates.

Collaboration revenue was \$6.5 million for the first nine months of 2022 compared with \$7.5 million for the first nine 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 \$37.3 million for the third quarter of 2022 compared with \$24.8 million for the third quarter of 2021, and \$104.8 million for the first nine months of 2022 compared with \$68.2 million for the first nine 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 \$10.1 million for the third quarter of 2022 compared with \$6.6 million for the third quarter of 2021, and \$27.3 million for the first nine months of 2022 compared with \$18.8 million for the first nine months of 2021. The increase was 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](http://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 resume enrollment in and complete the MARINA™ study, the continuation of participants in the MARINA trial and enrollment of participants into the MARINA-OLE™, and the reporting of data from the preliminary assessment of the MARINA study and the timing thereof; the progression of clinical programs for AOC 1001, AOC 1044 and AOC 1020 and the timing thereof; the initiation of the FORTITUDE™ and EXPLORE44™ trials; the potential of Avidity's product candidates to treat rare diseases; Avidity's plans to expand its AOC platform into additional muscle diseases; and the sufficiency of the company's current financial position to fund its development programs, investments in its pipeline and platform, and operations into 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 may not be able to resolve the partial clinical hold and the analysis related to the underlying cause of the serious adverse event may result in delays in the MARINA study or an inability to compete the study; unexpected adverse side effects or inadequate efficacy of its product candidates that may delay or limit their development, regulatory approval and/or commercialization, or may result in clinical holds, 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; 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; 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; disruption to its operations from the COVID-19 pandemic or the war in Ukraine; Avidity could use its available capital resources sooner than it currently expects; 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 September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Collaboration revenue	\$ 2,482	\$ 2,163	\$ 6,455	\$ 7,474
Operating expenses:				
Research and development	37,317	24,831	104,794	68,214
General and administrative	10,094	6,612	27,349	18,764
Total operating expenses	47,411	31,443	132,143	86,978
Loss from operations	(44,929)	(29,280)	(125,688)	(79,504)
Other income (expense), net	1,330	6	2,164	33
Net loss	\$ (43,599)	\$ (29,274)	\$ (123,524)	\$ (79,471)
Net loss per share, basic and diluted	\$ (0.82)	\$ (0.68)	\$ (2.45)	\$ (2.01)
Weighted-average shares outstanding, basic and diluted	53,069	43,265	50,432	39,477
<b>Balance Sheets</b>			<b>September 30,</b>	<b>December 31,</b>
<b>Assets</b>			<b>2022</b>	<b>2021</b>

Current assets:		
Cash, cash equivalents and marketable securities	\$ 405,548	\$ 405,543
Prepaid and other assets	8,916	5,598
Total current assets	<u>414,464</u>	<u>411,141</u>
Property and equipment, net	6,037	4,805
Restricted cash	251	251
Right-of-use asset	9,264	10,784
Other assets	523	599
Total assets	<u>\$ 430,539</u>	<u>\$ 427,580</u>
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable and other liabilities	\$ 37,212	\$ 24,794
Deferred revenue, current portion	5,454	4,864
Total current liabilities	<u>42,666</u>	<u>29,658</u>
Lease liabilities, net of current portion	8,185	9,960
Deferred revenue, net of current portion	2,706	6,532
Total liabilities	<u>53,557</u>	<u>46,150</u>
Stockholders' equity	376,982	381,430
Total liabilities and stockholders' equity	<u>\$ 430,539</u>	<u>\$ 427,580</u>

SOURCE Avidity Biosciences, Inc.

<https://investors.aviditybiosciences.com/2022-11-08-Avidity-Biosciences-Reports-Third-Quarter-2022-Financial-Results-and-Recent-Highlights>