

## Avidity Biosciences Reports Fourth Quarter and Year-End 2021 Financial Results and Recent Highlights

SAN DIEGO, March 1, 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 reported financial results for the fourth quarter and year ended December 31, 2021 and highlighted recent corporate progress.

"2021 was a pivotal year for Avidity, the field of RNA therapeutics and for the DM1 community as the first patient was dosed with an AOC as part of the AOC 1001 MARINA™ trial," said Sarah Boyce, president and chief executive officer. "This milestone transitioned us into a clinical-stage biopharmaceutical company and we continue to make great strides with our advancing and expanding pipeline and AOC platform technology. By the end of 2022, we plan to have three programs in three different rare diseases in the clinic with AOC 1001, AOC 1020 and AOC 1044."

"We are in a strong financial position with \$406 million at year-end bolstered through approximately \$175 million raised in 2021, inclusive of \$155 million of net proceeds from our first follow on financing. We are well funded into 2024 which we expect will allow us to complete the MARINA™ trial, advance the AOC 1044 and AOC 1020 programs into clinical development and to continue to invest in expanding our pipeline and the AOC platform in muscle and beyond," said Mike MacLean, chief financial officer.

### 2021 Key Highlights

#### AOC 1001 Achievements

- The Company initiated the Phase 1/2 MARINA™ trial of AOC 1001 in adults with myotonic dystrophy type 1 (DM1).
- The FDA and the European Medicines Agency (EMA) granted AOC 1001 Orphan Designation and the FDA granted AOC 1001 Fast Track Designation.
- The MARINA™ trial is on track for a preliminary assessment of safety, tolerability and key biomarkers in approximately half of the trial participants in Q4 2022.

#### Pipeline Advancements

- AOC 1044 was named as the clinical development candidate for the Duchenne Muscular Dystrophy (DMD) program targeting Exon 44. AOC 1044 is in IND-enabling studies and is expected to enter the clinic by the end of 2022.
- AOC 1020 was named as the clinical development candidate for the facioscapulohumeral muscular dystrophy (FSHD) program. AOC 1020 is in IND-enabling studies and is expected to enter the clinic by the end of 2022.
- Avidity also entered a collaboration with the FSHD Clinical Trial Research Network (FSHD CTRN) to support a natural history study called the Motor Outcomes to Validate Evaluations Plus (MOVE+) Study to enhance the understanding of how to utilize whole-body MRI and other tools to identify specific biomarkers for FSHD that can potentially accelerate and support future clinical trial design.

#### Organizational Highlights

- Avidity recently appointed Steve Hughes, M.D. as chief medical officer. Dr. Hughes brings over 20 years of experience in the biotechnology industry and has extensive experience in RNA-based treatments and rare diseases.

#### Fourth Quarter and Year-End 2021 Financial Results

**Cash, Cash Equivalents and Marketable Securities:** Cash, cash equivalents and marketable securities totaled \$405.5 million as of December 31, 2021, which reflects \$174.7 million raised in 2021, inclusive of \$155.1 million of net proceeds from our first follow on financing.

- **Collaboration Revenue:** Collaboration revenue, including reimbursable expenses, primarily relates to Avidity's partnership with Eli Lilly and Company and totaled \$1.9 million for the fourth quarter of 2021 compared with \$2.1 million for the fourth quarter of 2020, and \$9.3 million for the full year 2021 compared with \$6.8 million for the full year 2020.
- **Research and Development (R&D) Expenses:** R&D expenses include external and internal costs associated with research and development activities. These expenses were \$33.0 million for the fourth quarter of 2021 compared with \$13.6 million for the fourth quarter of 2020, and \$101.2 million for the full year 2021 compared with \$37.6 million for the full year 2020. The increases were primarily driven by the advancement of AOC 1001, AOC 1020 and AOC 1044, as well as 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 \$7.4 million for the fourth

quarter of 2021 compared with \$4.8 million for the fourth quarter of 2020, and \$26.2 million for the full year 2021 compared with \$13.5 million for the full year 2020. The increases were primarily due to higher personnel costs. The full year increase was also due to higher professional fees and insurance costs.

## About Avidity Biosciences

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 has commenced clinical testing with the ongoing Phase 1/2 MARINA™ trial 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. 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: the expected timing of preliminary safety, tolerability and key biomarker data from the MARINA™ trial; the progression of clinical programs for AOC 1044 and AOC 1020 and timing of planned IND-enabling studies and clinical trials; Avidity's plans to expand its AOC platform into additional muscle diseases and other tissues; the broad potential of AOCs to treat serious 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 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 clinical trials; disruption to its operations from the COVID-19 pandemic; 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 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; 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.

## Company Contact:

Kathleen Gallagher  
(858) 401-7900  
[kath.gallagher@aviditybio.com](mailto:kath.gallagher@aviditybio.com)

## Media Contact:

Cherise Adkins  
(301) 267-4161  
[cadkins@spectrumsience.com](mailto:cadkins@spectrumsience.com)

**Avidity Biosciences, Inc.**  
**Selected Condensed Financial Information**  
**(in thousands, except per share data)**  
**(unaudited)**

Statements of Operations	Three Months Ended		Twelve Months Ended	
	December 31,		December 31,	
	2021	2020	2021	2020

Collaboration revenue	\$ 1,852	\$ 2,142	\$ 9,326	\$ 6,787
Operating expenses:				
Research and development	32,968	13,619	101,182	37,602
General and administrative	7,431	4,816	26,195	13,462
Total operating expenses	40,399	18,435	127,377	51,064
Loss from operations	(38,547)	(16,293)	(118,051)	(44,277)
Other income (expense), net	9	18	42	(78)
Net loss	\$ (38,538)	\$ (16,275)	\$ (118,009)	\$ (44,355)
Net loss per share, basic and diluted	\$ (0.82)	\$ (0.43)	\$ (2.85)	\$ (2.05)
Weighted-average shares outstanding, basic and diluted	47,215	37,455	41,428	21,663

## Balance Sheets

	December 31,	
	2021	2020
<b>Assets</b>		
Current assets:		
Cash, cash equivalents and marketable securities	\$ 405,543	\$ 328,141
Prepaid and other assets	5,598	3,537
Total current assets	411,141	331,678
Property and equipment, net	4,805	1,468
Restricted cash	251	251
Right-of-use asset	10,784	454
Other assets	599	47
Total assets	\$ 427,580	\$ 333,898
<b>Liabilities and Stockholders' Equity</b>		
Current liabilities:		
Accounts payable and other liabilities	\$ 24,794	\$ 10,897
Deferred revenue, current portion	4,864	3,690
Total current liabilities	29,658	14,587
Lease liabilities, net of current portion	9,960	938
Deferred revenue, net of current portion	6,532	12,150
Total liabilities	46,150	27,675
Stockholders' equity	381,430	306,223
Total liabilities and stockholders' equity	\$ 427,580	\$ 333,898

SOURCE Avidity Biosciences, Inc.

<https://investors.aviditybiosciences.com/2022-03-01-Avidity-Biosciences-Reports-Fourth-Quarter-and-Year-End-2021-Financial-Results-and-Recent-Highlights>