Avidity Biosciences Reports Fourth Quarter and Year-End 2022 Financial Results and Recent Highlights

SAN DIEGO, Feb. 28, 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 fourth quarter and year ended December 31, 2022 and highlighted recent corporate progress.

"In 2022, we executed on our goal of advancing three rare disease programs into clinical development and demonstrated the first-ever successful targeted delivery of RNA into muscle, a revolutionary advancement for the field of RNA therapeutics," said Sarah Boyce, president and chief executive officer at Avidity. "Building on our AOC proof of platform data, 2023 will be another important year for Avidity. We look forward to Phase 1/2 MARINA™ and MARINA-OLE™ data as well as results from healthy volunteers in the Phase 1/2 EXPLORE44™ trial while continuing to expand the broad utility of our AOC platform."

"We are in a strong financial position with more than $600 million at year-end reflecting approximately $224M in net proceeds from our successful financing in December. We are well funded into mid-2025 as we advance our clinical development programs for AOC 1001, AOC 1044 and AOC 1020, as well as expand our AOC platform and pipeline programs including our recently announced internal programs in skeletal muscle and cardiology," said Mike MacLean, chief financial officer and chief business officer at Avidity.

Clinical Development Programs - Achievements & Updates

AOC 1001

- Positive interim data from the preliminary assessment of AOC 1001 in the Phase 1/2 MARINA study demonstrated first-ever successful targeted delivery of RNA to skeletal muscle, DMPK reduction, splicing improvements and early signs of clinical activity with improvement in myotonia in some participants.
- Top-line data from the MARINA trial is anticipated in 2023 and preliminary data from the MARINA-OLE trial is anticipated in late 2023.
- The company plans to give an update on the AOC 1001 partial clinical hold at the end of the first quarter in 2023.

AOC 1044

- The FDA cleared Avidity to proceed with the Phase 1/2 EXPLORE44 clinical trial of AOC 1044 for the treatment of duchenne muscular dystrophy (DMD) mutations amenable to exon 44 skipping (DMD44). AOC 1044 is the first of multiple AOCs being developed for DMD and is the first AOC PMO to advance into the clinic.
    - 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. Click here for additional details on the EXPLORE44 trial.
- Data from healthy volunteers in the EXPLORE44 trial is anticipated in the second half of 2023.

AOC 1020

- The FDA cleared Avidity to proceed with the Phase 1/2 FORTITUDE™ clinical trial of AOC 1020 in adults with facioscapulohumeral muscular dystrophy (FSHD).
    - The FORTITUDE trial is a randomized, placebo-controlled, double-blind, Phase 1/2 clinical trial designed to evaluate AOC 1020 in approximately 70 adult participants with FSHD. Click here for additional details on the FORTITUDE trial.
    - The FDA granted AOC 1020 Fast Track Designation in January 2023.
    - The FDA and the European Medicines Agency (EMA) granted AOC 1020 Orphan Designation in February 2023.
- Data from a preliminary assessment in approximately half of participants in the FORTITUDE trial is anticipated in the first half of 2024.

Pipeline Advancements

- Avidity announced that it advanced and expanded its wholly-owned early stage AOC pipeline including adding a rare cardiac program and an additional program in rare skeletal muscle disease.

Organizational Highlights

- The company announced in February that Arthur A, Levin, Ph.D., joined its board of directors and transitioned to distinguished scientist and strategic leader at Avidity.
- W. Michael Flanagan, Ph.D. was promoted in February to chief scientific and technical officer.

Fourth Quarter and Year-End 2022 Financial Results

Cash, Cash Equivalents and Marketable Securities: Cash, cash equivalents and marketable securities totaled $610.7 million as of December 31, 2022, which reflects $344.6 million raised in 2022, inclusive of $223.8 million of net proceeds from our follow-on financing. In addition, subsequent to December 31, 2022, we have raised $22.3 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 $2.7 million for the fourth quarter of 2022 compared with $1.9 million for the fourth quarter of 2021, and $9.2 million for the full year 2022 compared with $9.3 million for the full year 2021.

- **Research and Development (R&D) Expenses:** R&D expenses include external and internal costs associated with research and development activities. These expenses were $45.6 million for the fourth quarter of 2022 compared with $33.0 million for the fourth quarter of 2021, and $150.4 million for the full year 2022 compared with $101.2 million for the full year 2021. 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 $10.4 million for the fourth quarter of 2022 compared with $7.4 million for the fourth quarter of 2021, and $37.7 million for the full year 2022 compared with $26.2 million for the full year 2021. 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 resume enrollment in and complete the MARINA™ trial; the anticipated timing of results from the MARINA and MARINA-OLE™ trials; the progression of clinical programs for AOC 1001, AOC 1044 and AOC 1020 and the timing thereof; the timing of participant enrollment and data availability in the FORTITUDE™ and EXPLORE44™ trials; 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; the potential of AOCs to treat skeletal muscle, cardiac and immunological diseases; the continued advancement of collaboration programs with collaboration partners, including Eli Lilly and Company; 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 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; the Phase 1/2 MARINA trial results are based on a preliminary analysis of interim data available as of the data cutoffs, and, as with Avidity's other preclinical studies and clinical trials, the interim results may materially differ from final results of the trial; one or more of the safety or biomarker results in the Phase 1/2 MARINA trial may materially change following more comprehensive reviews of the data, as follow-up on the outcome of any particular patient continues, as and if additional patients enroll in the trial and as more patient data become available, any of which may materially alter the findings and conclusions from Avidity's preliminary analysis; unexpected adverse side effects or inadequate efficacy of Avidity's 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; 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 Avidity's operations from the COVID-19 pandemic or the war in Ukraine, which may delay or otherwise disrupt Avidity's preclinical studies, clinical trials, manufacturing and supply chain; Avidity could exhaust its available capital resources sooner than it currently expects; and other risks described 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 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.

Investor Contact:
Kathleen Gallagher
(858) 401-7900 x550
investors@aviditybio.com

Media Contact:
Navjot Rai
(858) 401-7900 x550
media@aviditybio.com

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

Statements of Operations	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2022	2021	2022	2021
Collaboration revenue	$ 2,769	$ 1,852	$ 9,224	$ 9,326
Operating expenses:				
Research and development	45,610	32,968	150,404	101,182

General and administrative	10,994	7,991	37,133	26,395
Total operating expenses	63,719	46,538	216,046	144,446
Loss from operations	(53,225)	(38,547)	(178,913)	(118,051)
Other income (expense), net	2,754	9	4,918	42
Net loss	$ (50,471)	$ (38,538)	$ (173,995)	$ (118,009)
Net loss per share, basic and diluted	$ (0.88)	$ (0.82)	$ (3.34)	$ (2.85)
Weighted-average shares outstanding, basic and diluted	57,296	47,215	52,162	41,428

Balance Sheets	**December 31, 2022**	**December 31, 2021**
Assets		
Current assets:		
Cash, cash equivalents and marketable securities	$ 610,727	$ 405,543
Prepaid and other assets	12,215	5,598
Total current assets	622,942	411,141
Property and equipment, net	6,254	4,805
Restricted cash	251	251
Right-of-use asset	8,755	10,784
Other assets	598	599
Total assets	$ 638,800	$ 427,580
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and other liabilities	$ 46,867	$ 24,794
Deferred revenue, current portion	5,041	4,864
Total current liabilities	51,908	29,658
Lease liabilities, net of current portion	7,582	9,960
Deferred revenue, net of current portion	1,235	6,532
Total liabilities	60,725	46,150
Stockholders' equity	578,075	381,430
Total liabilities and stockholders' equity	$ 638,800	$ 427,580

SOURCE Avidity Biosciences, Inc.

---

https://aviditybiosciences.investorroom.com/2023-02-28-Avidity-Biosciences-Reports-Fourth-Quarter-and-Year-End-2022-Financial-Results-and-Recent-Highlights