Avidity Biosciences Reports Second Quarter 2021 Financial Results and Recent Highlights

LA JOLLA, Calif., Aug. 9, 2021 /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 second quarter and six months ended June 30, 2021 and highlighted recent corporate progress.

"The FDA clearance to proceed with our AOC 1001 Phase 1/2 MARINA™ trial in adults with myotonic dystrophy (DM1) is a huge milestone for Avidity and our AOC platform. AOC 1001 will be the first program based on our novel technology to enter clinical development. This also marks an important step forward for the DM1 community who have no approved therapies and so desperately needs therapeutic options," said Sarah Boyce, president and chief executive officer. "In addition, over the past quarter we made significant advances in our pipeline including nominating AOC 1044 as the clinical development candidate for our lead DMD program. We remain on track for both AOC 1044 and our AOC FSHD program to advance into the clinic in 2022."

"We are well funded with $280 million at the end of Q2'21, along with an additional $155 million in estimated net proceeds from our successful financing in August 2021. Our strong financial position allows us to further progress our late stage programs while continuing to invest in our skeletal muscle pipeline and our AOC platform," said Mike MacLean, chief financial officer.

AOC Platform and Pipeline Highlights

- **Received FDA clearance to proceed with clinical studies for AOC 1001 in adults with DM1.**

  Avidity recently received clearance to proceed with the Phase 1/2 MARINA trial of AOC 1001 in adults with DM1, under its Investigational New Drug application (IND). Avidity continues to be on track to initiate the Phase 1/2 MARINA clinical trial this year. In the second half of 2022, the Company plans to conduct a preliminary assessment of safety, tolerability and key biomarkers.

  Detailed information on the MARINA study was presented during Volume 2 of Avidity's virtual investor and analyst event series. Volume 2 featured presentations on AOC 1001, the MARINA study and a presentation on the clinical impact of DM1 by Nicholas E. Johnson, MD, MSCI, FAAN, an associate professor, division chief of neuromuscular, and vice chair of research in the department of neurology at Virginia Commonwealth University. The event also featured a live Q&A session with Avidity's management team and Dr. Johnson. Volume 2 in the series follows Volume 1 which was focused on the years of engineering underpinning Avidity's AOC platform and AOC 1001. Replays of Volume 1 and Volume 2 can be found on the events page in the investors section of the Avidity website.

  In July, the FDA granted Orphan Drug Designation for AOC 1001 for the treatment of DM1. The FDA grants Orphan Drug Designation to novel drugs that seek to treat a rare disease or condition and, if the drug is approved for the designated orphan indication, provides 7 years of market exclusivity, along with certain financial incentives, including tax credits, opportunities for grant funding towards clinical trial costs and FDA user-fee waivers.

  In May, Avidity reported results from its IND-enabling toxicology study of AOC 1001. Results from the study showed the highest dose tested was the maximum feasible dose and was the no-observed adverse effect level (NOAEL). The Company did not observe any treatment-related histopathologic toxicity or any changes in safety pharmacology parameters (cardiac, respiratory and neurological). All dose levels in the study produced a greater than 80% reduction in the expression of dystrophy myotonic protein kinase (DMPK) across multiple skeletal muscles, demonstrating that pharmacology was essentially saturated even at the lowest dose tested.

- **Progressed Skeletal Muscle Pipeline including nominating AOC 1044 to move into IND-enabling studies.**

  AOC 1044 was recently nominated as the clinical development candidate for the DMD program targeting Exon 44. AOC 1044 is entering into IND-enabling studies and is on track with plans to advance into the clinic in 2022.

  Data from the AOC FSHD program targeting facioscapulohumeral muscular dystrophy (FSHD) was presented at the 28th Annual FSHD Society International Research Congress (IRC) in June. The data demonstrated promising preclinical activity in both *in vitro* and *in vivo* experiments in the cells of patients with FSHD.

Second Quarter 2021 Financial Results

- **Cash, Cash Equivalents and Marketable Securities:** Cash, cash equivalents and marketable securities totaled $279.5 million as of June 30, 2021, compared to $328.1 million as of December 31, 2020.
- **Collaboration Revenue:** Collaboration revenue, including reimbursable expenses, primarily relates to Avidity's partnership with Eli Lilly and Company and totaled $2.6 million for the second quarter of 2021 compared with $1.5 million for the second quarter of 2020, and $5.3 million for the first six months of 2021 compared with $2.9 million for the first six months of 2020.
- **Research and Development (R&D) Expenses:** R&D expenses include external and internal costs associated with research and development activities. These expenses were $22.7 million for the second quarter of 2021 compared with $9.0 million for the second quarter of 2020, and $43.4 million for the first six months of 2021 compared with $14.5 million for the first six months of 2020. The increases were primarily driven by the advancement of AOC 1001, AOC 1044 and our AOC FSHD program, as well as costs related to the expansion of our 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 $6.3 million for the second quarter of

2021 compared with $2.9 million for the second quarter of 2020, and $12.2 million for the first six months of 2021 compared with $4.9 million for the first six months of 2020. The increases were primarily due to higher personnel costs (including noncash stock-based compensation), professional fees and insurance costs related to being a public company.

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's lead product candidate, AOC 1001, is designed to treat myotonic dystrophy type 1 (DM1). The FDA has cleared Avidity to proceed with the Phase 1/2 MARINA™ trial of AOC 1001 in adults with myotonic dystrophy type 1 (DM1). Its advancing and expanding pipeline also includes programs in facioscapulohumeral muscular dystrophy (FSHD), Duchenne Muscular Dystrophy (DMD), muscle atrophy and Pompe disease. The company is planning for AOC 1044, the lead of three programs for the treatment of DMD, and its AOC FSHD program to enter the clinic in 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 La Jolla, 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 potential to develop a meaningful pipeline of novel AOC therapeutics; the initiation of a clinical trial of AOC 1001 in patients with DM1, and the timing thereof; the progression of clinical programs for AOC 1044 and the company's AOC FSHD program and timing of planned clinical studies; the expected benefits associated with Orphan Drug Designation; the sufficiency of the company's current financial position to allow it to further progress its development programs and invest in its pipeline and platform; the potential to identify new targets beyond skeletal muscle that can be targeted with Avidity's AOC approach; and the broad potential of AOCs to treat rare and serious diseases. 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; risks that the benefits associated with Orphan Drug Designation may not be realized, including that Orphan Drug exclusivity may not effectively protect a product from competition and that such exclusivity may not be maintained; 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; Avidity's dependence on third parties for existing collaborations and Avidity may not realize any benefits from such 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; 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 June 30,**		**Six Months Ended June 30,**	
	2021	**2020**	**2021**	**2020**
Collaboration revenue	$ 2,607	$ 1,541	$ 5,311	$ 2,899
Operating expenses:				
Research and development	22,706	8,984	43,383	14,528
General and administrative	6,268	2,925	12,152	4,889
Total operating expenses	28,974	11,909	55,535	19,417
Loss from operations	(26,367)	(10,368)	(50,224)	(16,518)

Other income (expense), net	14	(188)	27	(123)
Net loss	$ (26,353)	$ (10,556)	$ (50,197)	$ (16,641)
Net loss per share, basic and diluted	$ (0.70)	$ (1.23)	$ (1.34)	$ (2.91)
Weighted-average shares outstanding, basic and diluted	37,583	8,586	37,552	5,715

Balance Sheets	**June 30, 2021**	**December 31, 2020**
Assets		
Current assets:		
Cash, cash equivalents and marketable securities	$ 279,458	$ 328,141
Prepaid and other assets	7,851	3,537
Total current assets	287,309	331,678
Property and equipment, net	2,556	1,468
Restricted cash	251	251
Other assets	407	501
Total assets	$ 290,523	$ 333,898
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and other accrued liabilities	$ 11,841	$ 10,897
Deferred revenue, current portion	4,497	3,690
Total current liabilities	16,338	14,587
Lease liabilities, net of current portion	914	938
Deferred revenue, net of current portion	8,791	12,150
Total liabilities	26,043	27,675
Stockholders' equity	264,480	306,223
Total liabilities and stockholders' equity	$ 290,523	$ 333,898

SOURCE Avidity Biosciences, Inc.

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https://aviditybiosciences.investorroom.com/2021-08-09-Avidity-Biosciences-Reports-Second-Quarter-2021-Financial-Results-and-Recent-Highlights