Avidity Biosciences Reports Fourth Quarter and Year-End 2020 Financial Results and Recent Highlights

LA JOLLA, Calif., March 15, 2021 /PRNewswire/ -- Avidity Biosciences, Inc. (Nasdaq: RNA), a biopharmaceutical company pioneering a new class of oligonucleotide-based therapies called Antibody Oligonucleotide Conjugates (AOCs™), today reported financial results for the fourth quarter and year ended December 31, 2020 and highlighted recent corporate progress.

"In 2020, we made significant advances across our AOC pipeline and platform. Our work supports Avidity's evolution to a clinical-stage company as we plan to advance AOC 1001 into the clinic in the second half of this year and progress our FSHD and DMD programs," said Sarah Boyce, President and Chief Executive Officer. "Our discovery efforts continue to focus on expanding our AOC platform into additional muscle diseases and other tissues as we begin to realize our vision of profoundly improving people's lives by revolutionizing the delivery of RNA treatments."

"Last year we built a solid foundation for growth, anchored by our successful IPO and strong financial position with $328 million in cash at year-end," said Mike MacLean, Chief Financial Officer. "We are investing in our platform and have assembled an experienced team in RNA therapeutics and rare diseases to deliver on our discovery, clinical and commercial objectives."

AOC Platform and Pipeline Highlights

- **Advanced Lead Program, AOC 1001 for DM1, and Broad Pipeline for Untreated Rare Muscle Diseases.** Avidity advanced its first-in-class, lead rare disease program, AOC 1001, toward the clinic and entered into a collaboration with Myotonic Dystrophy Clinical Research Network supporting END-DM1, a natural history study to advance the understanding of disease progression in patients with myotonic dystrophy type 1 (DM1). The company plans to initiate a Phase 1/2 clinical study of AOC 1001 in adults with DM1 in the second half of 2021.

  Avidity also advanced additional programs in its muscle franchise including a program for facioscapulohumeral muscular dystrophy (FSHD) and three programs for Duchenne muscular dystrophy (DMD). The AOC FSHD program and the lead AOC DMD program targeting Exon 44 are the most advanced. In 2022, following additional preparatory preclinical studies and regulatory clearance, Avidity plans to commence clinical trials for both of these programs.

- **Demonstrated Preclinical Proof-of-Concept in Skeletal Muscle and Other Tissues; Advancing Beyond Muscle with Discovery Efforts and Partnering.** In preclinical models, Avidity's AOCs demonstrated robust mRNA reductions in skeletal muscle, cardiac muscle, activated B- and T-cells and tumor infiltrating lymphocytes, macrophages and the liver.

  Avidity is advancing beyond muscle with its own discovery efforts and through partnering, as evidenced by its strategic collaborations with Eli Lilly in immunology and MyoKardia, a wholly-owned subsidiary of Bristol Meyers Squibb, in cardiac tissue.

Organizational Highlights

- **Appointed Experienced and Diverse Team Members to Management and Board of Directors.** Avidity has assembled a full management team with deep expertise in the discovery, development and commercialization of RNA therapeutics and rare diseases. Avidity also welcomed Jean Kim and Tamar Thompson to its Board of Directors.

Fourth Quarter and Year-End 2020 Financial Results

- **Cash, Cash Equivalents and Marketable Securities:** Cash, cash equivalents and marketable securities totaled $328.1 million as of December 31, 2020, which includes net proceeds of $274.1 million from the company's IPO in June 2020, compared to $94.6 million as of December 31, 2019.

- **Collaboration Revenue:** Collaboration revenue solely related to our partnership with Lilly, including reimbursable expenses, was $2.1 million for the fourth quarter of 2020 compared with $1.4 million for the fourth quarter of 2019, and $6.8 million for the full year 2020 compared with $2.3 million for the full year 2019.

- **Research and Development (R&D) Expenses:** R&D expenses, including external and internal costs associated with research activities, primarily relate to the progression of the company's research on AOC 1001 and other muscle programs. These expenses were $13.6 million for the fourth quarter of 2020 compared with $5.6 million for the fourth quarter of 2019, and $37.6 million for the full year 2020 compared with $14.5 million for the full year 2019. The increases were primarily driven by the progression of AOC 1001 toward the clinic, as well as other programs.

- **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 $4.8 million for the fourth quarter of 2020 compared with $1.8 million for the fourth quarter of 2019, and $13.5 million for the full year 2020 compared

with $5.1 million for the full year 2019. 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, as well as higher patent filing fees.

About Avidity Biosciences

Avidity Biosciences, Inc. is driven to change lives with a new class of therapies called Antibody Oligonucleotide Conjugates (AOCs) that are designed to overcome current limitations of oligonucleotide therapies in order to treat a wide range of serious diseases. Avidity's proprietary AOC platform combines the tissue selectivity of monoclonal antibodies and the precision of oligonucleotide therapies to access previously undruggable tissue and cell types and more effectively target underlying genetic drivers of diseases. Avidity's lead product candidate, AOC 1001, is designed to treat myotonic dystrophy type 1, and its other muscle programs are focused on the treatment of Duchenne muscular dystrophy, facioscapulohumeral muscular dystrophy, Pompe disease and muscle atrophy. In addition to its muscle franchise, Avidity has research efforts focused on immune, cardiac and other cell types.

Avidity is headquartered in La Jolla, CA. For more information about Avidity's science, pipeline and people, please visit www.aviditybiosciences.com and engage with Avidity on LinkedIn.

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 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; Avidity's evolution to a clinical-stage company; Avidity's plans to initiate a Phase 1/2 clinical trial of AOC 1001 in patients with DM1 and the expected timing thereof; Avidity's plans to submit a regulatory filing and commence a clinical trial of its AOC FSHD program and the expected timing thereof; Avidity's plans to submit a regulatory filing and commence a clinical trial of its AOC DMD program and the expected timing thereof; Avidity's plans to expand its AOC platform into additional muscle diseases and other tissues; and the broad potential of AOCs to treat 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: the company is early in development efforts and all of its development programs are in the preclinical or discovery stage; the company's approach to the discovery and development of product candidates based on the 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 the company's operations from the COVID-19 pandemic; the success of its preclinical studies and clinical trials for its product candidates; the results of preclinical studies and early clinical trials are not necessarily predictive of future results; the company may not be able to show utility of AOCs in cardiac or other tissue and may not realize any benefits from its collaborations; its 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; risks related to integration of new management personnel; and other risks described in the company's prior press releases and in its 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 December 31,**		**Twelve Months Ended December 31,**	
	2020	**2019**	**2020**	**2019**
Collaboration revenue	$ 2,142	$ 1,445	$ 6,787	$ 2,319
Operating expenses:				
Research and development	13,619	5,645	37,602	14,539
General and administrative	4,816	1,847	13,462	5,112
Total operating expenses	18,435	7,492	51,064	19,651
Loss from operations	(16,293)	(6,047)	(44,277)	(17,332)
Other income (expense), net	18	(4,354)	(78)	(7,402)
Net loss	$ (16,275)	$ (10,401)	$ (44,355)	$ (24,734)
Net loss per share, basic and diluted	$ (0.43)	$ (3.75)	$ (2.05)	$ (9.12)
Weighted-average shares outstanding, basic and diluted	37,455	2,776	21,663	2,713

Balance Sheets	**December 31,**	
	2020	**2019**
Assets		
Current assets:		
Cash, cash equivalents and marketable securities	$ 328,141	$ 94,578
Prepaid and other assets	3,537	1,098
Total current assets	331,678	95,676
Property and equipment, net	1,468	631
Restricted cash	251	—
Other assets	501	600
Total assets	$ 333,898	$ 96,907
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities:		
Accounts payable and other accrued liabilities	$ 10,897	$ 3,622
Deferred revenue, current portion	3,690	3,840
Long-term debt, current portion	—	2,774
Total current liabilities	14,587	10,236
Lease liabilities, net of current portion	938	393
Deferred revenue, net of current portion	12,150	15,100
Long-term debt, net of current portion	—	1,770
Other long-term liabilities	—	45
Total liabilities	27,675	27,544
Convertible preferred stock	—	134,720
Stockholders' equity (deficit)	306,223	(65,357)
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$ 333,898	$ 96,907

SOURCE Avidity Biosciences, Inc.

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