Avidity Biosciences Reports First Quarter 2021 Financial Results and Recent Highlights

LA JOLLA, Calif., May 12, 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 first quarter ended March 31, 2021 and highlighted recent corporate progress.

"AOC 1001, the first program from our AOC platform, is advancing toward the clinic and is on track to begin a Phase 1/2 study in adults with DM1 in the second half of this year. Our skeletal muscle pipeline is also progressing as planned while we continue to expand our discovery efforts to other indications," said Sarah Boyce, president and chief executive officer. "We are proud to be at the forefront of RNA innovation by delivering on the promise of our AOC platform and pipeline."

Mike MacLean, chief financial officer, added, "We are off to a great start this year with a strong financial position of over $300 million at the end of the first quarter. This allows us to progress AOC 1001 while continuing to invest in our other pipeline programs and our AOC platform, which we believe is a sustainable engine with the potential to have a broad impact across both rare and other serious diseases."

AOC Platform and Pipeline Highlights

- **Advanced Lead Program, AOC 1001 for DM1, and Broad Pipeline for Untreated Rare Muscle Diseases.**

  In April 2021, Avidity presented preclinical data for AOC 1001 for myotonic dystrophy type 1 (DM1) in an oral presentation as part of the Emerging Science Session at the American Academy of Neurology (AAN) 2021 Virtual Annual Meeting. Avidity scientists have demonstrated activity and potency of siRNAs against the dystrophy protein kinase (DMPK) gene in muscle cells derived from patients with DM1. These data demonstrated activity in the nucleus and cytoplasm. The presentation also showed that single doses of AOC 1001 produced 75% reductions in DMPK mRNA expression in preclinical studies that were maintained for months post-dosing. Following regulatory clearance, Avidity remains on track with plans to begin 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. Avidity remains on track with plans to commence clinical trials for both programs in 2022, following additional preparatory preclinical studies and regulatory clearance.

Upcoming Events

- **Avidity's Virtual Investor & Analyst Event Series – Volume 1.** Recently, Avidity launched its Virtual Investor and Analyst Event Series. The first virtual event entitled "Engineering Antibody Oligonucleotide Conjugates" will be held on Wednesday, May 19th, 2021 at 8am PT/11am ET.

  The virtual event will feature a panel discussion with leading RNA therapeutics key opinion leaders Phillip D. Zamore, Ph.D., Chair, RNA Therapeutics Institute at UMASS and Steven F. Dowdy, Ph.D., Professor of Cellular and Molecular Medicine at UCSD. To register for the event or for more information, please email investors@aviditybio.com.

First Quarter 2021 Financial Results

- **Cash, Cash Equivalents and Marketable Securities:** Cash, cash equivalents and marketable securities totaled $307.9 million as of March 31, 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 $2.7 million for the first quarter of 2021 compared with $1.4 million for the first quarter of 2020.

- **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 $20.7 million for the first quarter of 2021

compared with $5.5 million for the first quarter of 2020. The increase was primarily driven by the progression of AOC 1001 toward the clinic, as well as research for 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 $5.9 million for the first quarter of 2021 compared with $2.0 million for the first quarter of 2020. The increase was 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. 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 cells, cardiac tissue 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 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 myotonic dystrophy type 1 and other planned clinical trials, and the timing thereof; 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 and all of its development programs are in the preclinical or discovery stage; 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; risks related to integration of new management personnel; 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.

Contacts:

Company:

Kathleen Gallagher
(858) 401-7900
kath.gallagher@aviditybio.com

Media and Investors:

Amy Conrad
Juniper Point
(858) 366-3243

amy@juniper-point.com

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

Statements of Operations	**Three Months Ended March 31,**	
	2021	**2020**
Collaboration revenue	$ 2,704	$ 1,358
Operating expenses:		
Research and development	20,677	5,544
General and administrative	5,884	1,964
Total operating expenses	26,561	7,508
Loss from operations	(23,857)	(6,150)
Other income (expense), net	13	65
Net loss	$ (23,844)	$ (6,085)
Net loss per share, basic and diluted	$ (0.64)	$ (2.14)
Weighted-average shares outstanding, basic and diluted	37,521	2,845

Balance Sheets	**March 31, 2021**	**December 31, 2020**
Assets		
Current assets:		
Cash, cash equivalents and marketable securities	$ 307,914	$ 328,141
Prepaid and other assets	4,036	3,537
Total current assets	311,950	331,678
Property and equipment, net	2,187	1,468
Restricted cash	251	251
Other assets	364	501
Total assets	$ 314,752	$ 333,898
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and other accrued liabilities	$ 13,157	$ 10,897
Deferred revenue, current portion	4,357	3,690
Total current liabilities	17,514	14,587
Lease liabilities, net of current portion	951	938
Deferred revenue, net of current portion	10,171	12,150
Total liabilities	28,636	27,675
Stockholders' equity	286,116	306,223
Total liabilities and stockholders' equity	$ 314,752	$ 333,898

SOURCE Avidity Biosciences, Inc.

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