Avidity Biosciences Reports Third Quarter 2021 Financial Results and Recent Highlights

Initiated the Phase 1/2 MARINA™ trial of AOC 1001, marking the first AOC in clinical development
On track to have three clinical programs by the end of 2022
Volume 3, Virtual Investor and Analyst Series, on December 9 at 11am ET featuring Jeffrey Statland, M.D., an expert and leading physician treating FSHD

LA JOLLA, Calif., Nov. 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 third quarter and nine months ended September 30, 2021 and highlighted recent corporate progress.

"This past quarter we initiated our Phase 1/2 MARINA™ trial for AOC 1001, which transitions us into a clinical stage company. This trial is important for both AOC 1001 and our platform as it is expected to generate the first proof of concept data on the AOC platform's potential to deliver RNA therapy outside of the liver – a first for the field of RNA conjugates and for Avidity," said Sarah Boyce, president and chief executive officer. "This is also an important milestone for the myotonic dystrophy community who currently have no therapeutic options. We look forward to sharing preliminary data on AOC 1001 in the second half of next year as we work to achieve our ambitious agenda of having AOCs for DM1, DMD and FSHD in the clinic by the end of 2022."

"Following completion of our successful first follow-on financing in August, we are well funded into 2024 with a cash balance of $413 million at quarter end. Given these significant financial resources, we are well positioned to complete the ongoing MARINA trial for AOC 1001, as well as initiate proof of concept clinical trials in 2022 for AOC 1044, the first of our DMD programs, and for our AOC FSHD program while we continue our investment in expanding our AOC platform," said Mike MacLean, chief financial officer.

Recent Highlights:

- Initiated first clinical trial for an AOC – transitioning Avidity into a clinical stage company
    - Initiated the Phase 1/2 MARINA trial of AOC 1001 in adults with myotonic dystrophy type 1 (DM1)
        - Received clearance from the U.S. Food and Drug Administration (FDA) to proceed with the Phase 1/2 MARINA trial under Avidity's initial new drug application (IND) in July 2021
        - Enrolled the first patient in the MARINA trial in October 2021, marking the first time a person has been dosed with an AOC
    - AOC 1001 was granted Orphan Designation by the FDA and the European Medicines Agency (EMA)
    - AOC 1001 was granted Fast Track Designation by the FDA in October 2021
- Entered a collaboration with a key physician network for a natural history study in FSHD
    - Avidity is supporting the natural history study called the Motor Outcomes to Validate Evaluations Plus (MOVE+) Study run by the facioscapulohumeral muscular dystrophy (FSHD) Clinical Trial Research Network (FSHD CTRN) to enhance the understanding of how to utilize whole-body MRI and other tools to identify specific biomarkers for FSHD that can accelerate and support future clinical trial design.
- Completed a follow-on financing, resulting in net proceeds of $155.1 million

Upcoming Events

Volume 3 of Avidity's Virtual Investor and Analyst Event Series will be focused on FSHD and will be held on Thursday, December 9, 2021 at 8am PT/11am ET. To register for the live video webcast, please visit the "Events and Presentations" page in the "Investors" section of Avidity's website. A replay of the webcast will be archived on Avidity's website following the event.

Art Levin, Ph.D., Avidity's chief scientific officer, and W. Michael Flanagan, Ph.D., Avidity's chief technical officer, will discuss the scientific rationale and potential benefits of leveraging AOCs for FSHD.

The virtual event will also feature Jeffrey Statland, M.D., professor, Department of Neurology, University of Kansas Medical Center, to discuss the clinical impact of FSHD and the ongoing natural history studies. Dr. Statland is one of the principal investigators in the ongoing MOVE and MOVE+ natural history studies for people living with FSHD.

Third Quarter 2021 Financial Results

- **Cash, Cash Equivalents and Marketable Securities:** Cash, cash equivalents and marketable securities totaled $413.0 million as of September 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 $2.2 million for the third quarter of 2021 compared with $1.7 million for the third quarter of 2020, and $7.5 million for the first nine months of 2021 compared with $4.6 million for the first nine 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 $24.8 million for the third quarter of 2021 compared with $9.5 million for the third quarter of 2020, and $68.2 million for the first nine months of 2021 compared with $24.0 million for the first nine months of 2020. The increases were primarily driven by the advancement of AOC 1001, AOC 1044 and the AOC FSHD program, 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 $6.6 million for the third quarter of 2021 compared with $3.8 million for the third quarter of 2020, and $18.8 million for the first nine months of 2021 compared with $8.6 million for the first nine months of 2020. The increases were primarily due to higher personnel costs. The year-to-date 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'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. 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 potential of AOC 1001 in patients with DM1 and the expected timing of preliminary data from the MARINA trial; the progression of clinical programs for AOC 1044 and the company's AOC FSHD program and timing of planned clinical trials; the expected benefits associated with Orphan Designation and Fast Track Designation; 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 potential to apply Avidity's AOC approach to new targets beyond muscle tissues; 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 Designation may not be realized, including that Orphan Designation 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 September 30,**		**Nine Months Ended September 30,**	
	2021	**2020**	**2021**	**2020**
Collaboration revenue	$ 2,163	$ 1,746	$ 7,474	$ 4,645
Operating expenses:				
Research and development	24,831	9,455	68,214	23,983
General and administrative	6,612	3,757	18,764	8,646
Total operating expenses	31,443	13,212	86,978	32,629
Loss from operations	(29,280)	(11,466)	(79,504)	(27,984)
Other income (expense), net	6	27	33	(96)
Net loss	$ (29,274)	$ (11,439)	$ (79,471)	$ (28,080)
Net loss per share, basic and diluted	$ (0.68)	$ (0.31)	$ (2.01)	$ (1.72)
Weighted-average shares outstanding, basic and diluted	43,265	37,420	39,477	16,361

Balance Sheets	**September 30, 2021**	**December 31, 2020**

Assets		
Current assets:		
Cash, cash equivalents and marketable securities	$ 413,029	$ 328,141
Prepaid and other assets	8,929	3,537
Total current assets	421,958	331,678
Property and equipment, net	3,220	1,468
Restricted cash	251	251
Other assets	488	501
Total assets	$ 425,917	$ 333,898
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and other accrued liabilities	$ 18,157	$ 10,897
Deferred revenue, current portion	4,261	3,690
Total current liabilities	22,418	14,587
Lease liabilities, net of current portion	877	938
Deferred revenue, net of current portion	7,745	12,150
Total liabilities	31,040	27,675
Stockholders' equity	394,877	306,223
Total liabilities and stockholders' equity	$ 425,917	$ 333,898

SOURCE Avidity Biosciences, Inc.

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https://aviditybiosciences.investorroom.com/2021-11-09-Avidity-Biosciences-Reports-Third-Quarter-2021-Financial-Results-and-Recent-Highlights