# Avidity Biosciences Reports Third Quarter 2023 Financial Results and Recent Highlights

*Avidity reported new positive AOC 1001 data at World Muscle Society (WMS) Congress in October demonstrating consistent improvement in multiple additional functional endpoints and favorable long-term safety and tolerability in people living with myotonic dystrophy type 1 (DM1)*

*AOC 1044 receives Orphan Designation for the treatment of DMD in US and EU*

*Avidity remains on track to report data readouts from three clinical development programs – DM1, DMD and FSHD – over the next nine months*

SAN DIEGO, Nov. 8, 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 and recent highlights for the third quarter ended September 30, 2023. Avidity ended the third quarter of 2023 with cash, cash equivalents and marketable securities totaling $542.6 million.

"In early October we announced new positive AOC 1001 data that demonstrated improvement in multiple additional functional endpoints, as well as favorable long-term safety and tolerability, in people living with DM1," said Sarah Boyce, president and chief executive officer. "In addition, we announced the completion of the 12-participant dose escalation from 2 mg/kg to 4 mg/kg as part of the partial clinical hold easement and look forward to finalizing the Phase 3 study design and global regulatory path for AOC 1001. We continue to make significant strides across all three of our clinical development programs, remaining on track to report data this year for AOC 1044 in DMD and in the first half of 2024 for both AOC 1001 in DM1 and AOC 1020 in FSHD".

"With funding into the second half of 2025 and continued execution across our portfolio, we remain very confident in the progress we are making in advancing our clinical development programs for DM1, DMD and FSHD," said Mike MacLean, chief financial officer and chief business officer. "The recently presented positive AOC 1001 data at World Muscle Society (WMS) Congress reinforces our confidence in our AOC platform, which spans multiple therapeutic areas, and our ability to deliver on our vision to profoundly improve people's lives by revolutionizing the delivery of RNA therapeutics".

**Recent Highlights**

- In October 2023, Avidity announced new positive AOC 1001 data demonstrating improvement in multiple additional functional endpoints and favorable long-term safety and tolerability in people with DM1 at WMS. The new data demonstrated improvement in additional functional measures including hand grip, muscle strength (Manual Muscle Testing composite score and both upper and lower Quantitative Muscle Testing composite scores) and patient reported outcomes, augmenting previously reported positive data showing improvements in myotonia, muscle strength and mobility.
- Data from the dose escalation of 12 participants from 2 mg/kg to 4 mg/kg of AOC 1001 as part of the easement of the partial clinical hold showed no neurological events and no MRI changes following dosing. Avidity continues to work as quickly as possible to resolve the partial clinical hold. In parallel, the company is finalizing a Phase 3 study design and a global regulatory path for AOC 1001.
- Avidity has initiated dosing participants living with Duchenne muscular dystrophy with mutations amenable to exon 44 skipping (DMD44).
- In August and October 2023, the FDA and EMA respectively, granted Orphan Designation for AOC 1044 for DMD44.

**Upcoming Milestones**

- The company continues to advance three distinct rare disease clinical programs with AOC 1001 for DM1, AOC 1020 for the treatment of facioscapulohumeral muscular dystrophy (FSHD) and AOC 1044 for the treatment of DMD44. Upcoming milestones include:
    - Data from healthy volunteers in the EXPLORE44™ trial planned for Q4 of this year
    - A first look at efficacy data from the MARINA-OLE™ trial planned for first half of 2024
    - Data from a preliminary assessment in approximately half of participants in the FORTITUDE™ trial planned for the first half of 2024

**Third Quarter 2023 Financial Results**

**Cash, Cash Equivalents and Marketable Securities:** Cash, cash equivalents and marketable securities totaled $542.6 million as of September 30, 2023, compared to $610.7 million as of December 31, 2022.

- **Collaboration Revenue:** Collaboration revenue, including reimbursable expenses, primarily relates to Avidity's partnership with Eli Lilly and Company and totaled $2.8 million for the third quarter of 2023 compared with $2.5 million for the third quarter of 2022, and $7.4 million for the first nine months of 2023 compared with $6.5 million for the first nine months of 2022.
- **Research and Development (R&D) Expenses:** R&D expenses include external and internal costs associated with research and development activities. These expenses were $47.7 million for the third quarter of 2023 compared with $37.3 million for the third quarter of 2022, and $138.2 million for the first nine months of 2023 compared with $104.8 million for the first nine months of 2022. The increases were primarily driven by the advancement of AOC 1001, AOC 1020 and AOC 1044, as well as external and internal 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 $13.7 million for the third quarter of 2023 compared with $10.1 million for the third quarter of 2022, and $38.1 million for the first nine months of 2023 compared with $27.3 million for the first nine months of 2022. 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 is revolutionizing the field of RNA with its proprietary AOCs, which are designed to combine the specificity of monoclonal antibodies with the precision of oligonucleotide therapies to address targets and diseases previously unreachable with existing RNA therapies. Utilizing its proprietary AOC platform, Avidity demonstrated the first-ever successful targeted delivery of RNA into muscle and is leading the field with clinical development programs for three rare muscle diseases: myotonic dystrophy type 1 (DM1), Duchenne muscular dystrophy (DMD) and facioscapulohumeral muscular dystrophy (FSHD). Avidity is broadening the reach of AOCs with its advancing and expanding pipeline including programs in cardiology and immunology through internal discovery efforts and key partnerships. Avidity is headquartered in San Diego, CA. For more information about our AOC platform, clinical development pipeline and people, please visit www.aviditybiosciences.com and engage with us on LinkedIn and X.

**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: Avidity's efforts to resolve the partial clinical hold related to AOC 1001; the characterization of safety, tolerability and functional data associated with AOC 1001; expectations related to the MARINA-OLE™ study and AOC 1001; the anticipated timing of release of data from the MARINA-OLE, EXPLORE44™ and FORTITUDE™ trials; expectations regarding a Phase 3 study and global regulatory path for AOC 1001; Avidity's balance sheet and the ability of Avidity's cash balance to meet the company's operational needs; plans for the progression of clinical programs for AOC 1001, AOC 1044 and AOC 1020 and the timing thereof; the continued advancement of programs with collaboration partners, including Eli Lilly and Company; the potential of Avidity's product candidates to treat rare diseases and Avidity's efforts to bring them to people suffering from applicable diseases; and the potential of AOCs to target a range of different cells and tissues beyond the liver, and to treat cardiac and immunological 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 Avidity's business, including, without limitation: Avidity may not be able to resolve the partial clinical hold related to the serious adverse event which occurred in the Phase 1/2 MARINA® trial, which may result in delays in the clinical development of AOC 1001; additional participant data related to AOC 1001 that continues to become available may be inconsistent with the data produced as of the most recent data cutoff, and further analysis of existing data and analysis of new data may lead to conclusions different from those established as of such data cutoff; unexpected adverse side effects to, or inadequate efficacy of, Avidity's product candidates that may delay or limit their development, regulatory approval and/or commercialization, or may result in additional clinical holds which may not be timely lifted, 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, data readouts 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; Avidity may not realize the expected benefits of its collaborations; regulatory developments in the United States and foreign countries; Avidity could exhaust its available capital resources sooner than it currently expects and fail to raise additional needed funds; and other risks described in Avidity's Annual Report on Form 10-K for the fiscal year ended December 31, 2022, filed with the Securities and Exchange Commission (SEC) on February 28, 2023, and in subsequent filings with the 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.

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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,** | |
|---|---|---|---|---|
| | **2023** | **2022** | **2023** | **2022** |
| Collaboration revenue | $ 2,818 | $ 2,482 | $ 7,367 | $ 6,455 |
| Operating expenses: | | | | |
| Research and development | 47,714 | 37,317 | 138,151 | 104,794 |
| General and administrative | 13,729 | 10,094 | 38,071 | 27,349 |
| Total operating expenses | 61,443 | 47,411 | 176,222 | 132,143 |
| Loss from operations | (58,625) | (44,929) | (168,855) | (125,688) |
| Other income, net | 6,267 | 1,330 | 17,078 | 2,164 |
| Net loss | $ (52,358) | $ (43,599) | $ (151,777) | $ (123,524) |
| Net loss per share, basic and diluted | $ (0.71) | $ (0.82) | $ (2.11) | $ (2.45) |
| Weighted-average shares outstanding, basic and diluted | 74,097 | 53,069 | 71,987 | 50,432 |

| **Balance Sheets** | **September 30, 2023** | **December 31, 2022** |
|---|---|---|
| **Assets** | | |

| | | |
|---|---|---|
| Current assets: | | |
| Cash, cash equivalents and marketable securities | $ 542,583 | $ 610,727 |
| Prepaid and other assets | 12,904 | 12,215 |
| Total current assets | 555,487 | 622,942 |
| Property and equipment, net | 8,130 | 6,254 |
| Restricted cash | 295 | 251 |
| Right-of-use asset | 8,907 | 8,755 |
| Other assets | 344 | 598 |
| Total assets | $ 573,163 | $ 638,800 |
| **Liabilities and Stockholders' Equity** | | |
| Current liabilities: | | |
| Accounts payable and other liabilities | $ 46,748 | $ 46,867 |
| Deferred revenue, current portion | 1,968 | 5,041 |
| Total current liabilities | 48,716 | 51,908 |
| Lease liabilities, net of current portion | 6,979 | 7,582 |
| Deferred revenue, net of current portion | — | 1,235 |
| Total liabilities | 55,695 | 60,725 |
| Stockholders' equity | 517,468 | 578,075 |
| Total liabilities and stockholders' equity | $ 573,163 | $ 638,800 |

SOURCE Avidity Biosciences, Inc.

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https://aviditybiosciences.investorroom.com/2023-11-08-Avidity-Biosciences-Reports-Third-Quarter-2023-Financial-Results-and-Recent-Highlights