Avidity Biosciences Reports Third Quarter 2024 Financial Results and Recent Highlights

Enrollment in global Phase 3 HARBOR™ study for del-desiran in DM1 is on track

Avidity initiated biomarker cohort for del-brax FORTITUDE™ study for FSHD; pursuing apotential accelerated approval path for del-brax

Reported positive del-zota data from Phase 1/2 EXPLORE44™ trial for DMD44

Avidity to provide a first look at precision cardiology candidates and a glimpse at next-generation technology innovations via webcast event November 12, 2024

SAN DIEGO, Nov. 7, 2024 /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 ended September 30, 2024, and highlighted recent corporate progress.

"We are executing three potentially registrational clinical trials in three rare diseases where there are limited or no therapeutic options available. We reported positive del-zota data from the 5 mg/kg cohort of our Phase 1/2 EXPLORE44™ trial for DMD44 and have initiated enrollment in the EXPLORE44-OLE™. In addition, enrollment is on track for del-desiran's global Phase 3 HARBOR™ trial in DM1 and we initiated the biomarker cohort of our FORTITUDE™ study in FSHD, marking a key step in our strategy to pursue a potential accelerated approval path for del-brax," said Sarah Boyce, president and chief executive officer at Avidity. "As we build for the future, we look forward to sharing our exciting innovations in precision cardiology and a glimpse at our next-generation technology. We continue to build our global commercial infrastructure to provide potential new therapies to people living with these serious rare diseases as quickly as possible."

"We were pleased to complete an additional upsized public offering in August following positive clinical data from our EXPLORE44 program. With a strong cash position of approximately $1.6 billion, we remain focused on advancing our programs, bringing forward additional candidates from our DMD pipeline, executing on our precision cardiology programs and building additional capabilities, including commercial functions, as well as expanding to countries outside of the US," said Mike MacLean, chief financial officer and chief business officer at Avidity.

Recent Highlights

Del-zota (AOC 1044) for DMD44

- In August, Avidity reported positive initial del-zota data from the 5 mg/kg cohort of the Phase 1/2 EXPLORE44™ trial in people living with DMD44 demonstrating unsurpassed delivery to skeletal muscle, unprecedented, unadjusted increase of 25% in near full-length dystrophin production with a profound reduction in creatine kinase levels to near normal, and robust exon 44 skipping. Del-zota demonstrated favorable safety and tolerability with most treatment emergent adverse events mild or moderate.
- The Phase 1/2 EXPLORE44 trial is fully enrolled and ongoing.
- In addition to the participants rolling over from the Phase 1/2 EXPLORE44 trial, Avidity has begun enrolling 10-15 new participants in the EXPLORE44 Open-label Extension study (OLE).

Del-brax (AOC 1020) for FSHD

- In October, Avidity announced the initiation of the biomarker cohort in the Phase 1/2 FORTITUDE™ trial of del-brax. 2 mg/kg of del-brax will be administered every six weeks, designed to ensure continuous suppression of DUX4.
- In June of this year, Avidity reported positive initial del-brax 2 mg/kg data at four months from the Phase 1/2 FORTITUDE trial demonstrating unprecedented and consistent reductions of greater than 50% in DUX4 regulated genes, mean reductions of 25% or greater in novel circulating biomarker and creatine kinase, trends of functional improvement, and favorable safety and tolerability in people living with FSHD.
- Avidity remains on track to initiate the functional cohort in the FORTITUDE study in the first half of 2025.
- In July, the first participants from FORTITUDE began to roll over to the FORTITUDE Open-Label Extension (OLE) trial. All participants that complete FORTITUDE are eligible to enroll in the FORTITUDE-OLE™ trial.

Del-desiran (AOC 1001) for DM1

- Enrollment for the global Phase 3 HARBOR™ trial is ongoing and on track.
- In October, the U.S. Food and Drug Administration (FDA) removed the partial clinical hold on del-desiran.

Pipeline Advancements and Organizational Highlights

- In November, Avidity plans to provide a first look at precision cardiology candidates. In addition, Avidity plans to share a glimpse at next-generation technology innovations.
- In August, Avidity announced it plans to advance additional candidates from its DMD franchise following robust del-zota data; Exon 45 is currently in IND-enabling studies.

- Kathleen Gallagher was promoted to chief program officer in September 2024.

Third Quarter 2024 Financial Results

- **Cash, Cash Equivalents and Marketable Securities:** Cash, cash equivalents and marketable securities totaled $1.6 billion as of September 30, 2024, which reflects a gross $345.1 million raise from a public offering.

- **Collaboration Revenue:** Collaboration revenues of $2.3 million for the third quarter of 2024 and $7.9 million for the first nine months of 2024 primarily relate to Avidity's research collaboration and license partnership with Bristol Myers Squibb. Collaboration revenues of $2.8 million for the third quarter of 2023 and $7.4 million for the first nine months of 2023 primarily related to Avidity's research collaboration and license partnership with Eli Lilly and Company.

- **Research and Development (R&D) Expenses**: R&D expenses include external and internal costs associated with research and development activities. These expenses were $77.2 million for the third quarter of 2024 compared with $47.7 million for the third quarter of 2023, and $208.0 million for the first nine months of 2024 compared with $138.2 million for the first nine months of 2023. The increases were primarily driven by the advancement of del-desiran, del-brax and del-zota, as well as internal and external 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 $23.3 million for the third quarter of 2024 compared with $13.7 million for the third quarter of 2023, and $57.9 million for the first nine months of 2024 compared with $38.1 million for the first nine months of 2023. The increases were primarily due to higher personnel costs 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: a potential accelerated pathway for registration for del-brax, and the potential for all of Avidity's current clinical trials to be registrational; Avidity's next-generational technology innovations and the timing of its announcement; the anticipated announcement of lead precision cardiology program targets, including the timing thereof; Avidity's plans to build a global commercial infrastructure and capabilities; plans for the advancement of DMD programs beyond DMD44; plans for the initiation of the functional cohort in the FORTITUDE™ trial and the timing thereof; the characterization of data associated with Avidity's product candidates in their respective clinical trials, the conclusions drawn therefrom, and the impact of such data on the advancement of the respective product candidates; enrollment statuses of Avidity's clinical programs; the status of the HARBOR™ study; Avidity's platform, planned operations and programs; and Avidity's cash position and runway.

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 and beyond its control, including, without limitation: preliminary results of a clinical trial are not necessarily indicative of final results; further analysis of existing clinical data and analysis of new data may lead to conclusions different from those established as of the respective data cutoff dates in Avidity's clinical trials, and such data may not meet Avidity's expectations; Avidity's biomarker and planned functional cohorts in the FORTITUDE study may not support the registration of del-brax; 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; later developments with the FDA and other global regulators that could be inconsistent with the feedback received to date regarding Avidity's clinical trials; Avidity's approach to the discovery and development of product candidates based on its AOC™ platform is unproven and may not produce 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; legislative, judicial and regulatory developments in the United States and foreign countries; Avidity could exhaust its available capital resources sooner than it currently expects; and other risks described in Avidity's Annual Report on Form 10-K for the fiscal year ended December 31, 2023 and 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 Consolidated Financial Information
(in thousands, except per share data)
(unaudited)

Statements of Operations	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Collaboration revenue	$ 2,336	$ 2,818	$ 7,924	$ 7,367
Operating expenses:				
Research and development	77,197	47,714	207,968	138,151
General and administrative	23,273	13,729	57,902	38,071
Total operating expenses	100,470	61,443	265,870	176,222
Loss from operations	(98,134)	(58,625)	(257,946)	(168,855)
Other income, net	17,736	6,267	37,901	17,078
Net loss	$ (80,398)	$ (52,358)	$ (220,045)	$ (151,777)
Net loss per share, basic and diluted	$ (0.65)	$ (0.71)	$ (2.08)	$ (2.11)
Weighted-average shares outstanding, basic and diluted	123,375	74,097	105,902	71,987

Balance Sheets	September 30, 2024	December 31, 2023
Assets		
Current assets:		
Cash, cash equivalents and marketable securities	$ 1,588,593	$ 595,351
Prepaid and other assets	33,273	15,956
Total current assets	1,621,866	611,307
Property and equipment, net	9,493	8,381
Restricted cash	2,795	295
Right-of-use assets	6,299	8,271
Other assets	318	301
Total assets	$ 1,640,771	$ 628,555
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and other liabilities	$ 71,673	$ 52,315
Deferred revenue, current portion	19,660	28,365
Total current liabilities	91,333	80,680
Lease liabilities, net of current portion	3,797	6,213
Deferred revenue, net of current portion	42,261	40,898
Total liabilities	137,391	127,791
Stockholders' equity	1,503,380	500,764
Total liabilities and stockholders' equity	$ 1,640,771	$ 628,555

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