Avidity Biosciences Reports Fourth Quarter 2024 Financial Results and Recent Highlights

Building on success across its three clinical programs, Avidity is leading in rare neuromuscular diseases with a strong balance sheet to execute on a transformational 2025

Major milestones anticipated for each rare neuromuscular program in 2025,including preparing for Avidity's first BLA submission

Commercial preparations well underway in anticipation of three potential successive product launches for DMD, DM1 and FSHD starting in 2026

Phase 1/2 EXPLORE44® top-line del-zota data to be presented at the 2025 Muscular Dystrophy Association (MDA) Clinical and Scientific Conference in Dallas, Texas

SAN DIEGO, Feb. 27, 2025 /PRNewswire/ -- Avidity Biosciences, Inc. (Nasdaq: RNA), a biopharmaceutical company committed to delivering a new class of RNA therapeutics called Antibody Oligonucleotide Conjugates (AOCs™) to profoundly improve people's lives, today reported financial results for the fourth quarter ended December 31, 2024, highlighting recent progress and reiterating 2025 catalysts for its three clinical programs.

"Successful readouts from our three clinical-stage programs in 2024 demonstrate the consistent and reproducible data of our AOC platform. We are extending our leadership position in the rare neuromuscular space as we plan to submit our first BLA for an AOC and prepare for three potential successive product launches to provide therapies for people living with rare neuromuscular diseases with limited or no treatment options," said Sarah Boyce, president and chief executive officer at Avidity. "We have now completed enrollment in the EXPLORE44-OLE study which, together with the Phase 1/2 EXPLORE44 study data, will form the basis of our BLA submission planned for year-end 2025. We are also rapidly progressing del-desiran in DM1 and del-brax in FSHD – both are on track to potentially be the first globally approved drugs for people living with these serious, rare diseases. We are committed to executing on our broad pipeline and strategic initiatives to bring forward these important therapeutics as quickly as possible for patients who are waiting."

"As we move into 2025, our strong balance sheet with approximately$1.5 billion at the end of 2024 allows us to continue to expedite our global commercial infrastructure development and expand our team of experienced industry professionals across all areas. We are transitioning to the next stage as the company continues to advance its AOC technology in rare neuromuscular and precision cardiology, and next-generation innovations," said Mike MacLean, chief financial officer at Avidity.

Recent Highlights

Avidity will be reporting top-line del-zota data from the completed Phase 1/2 EXPLORE44® trial for people living with Duchenne muscular dystrophy amenable to exon 44 skipping (DMD44) at the 2025 Muscular Dystrophy Association (MDA) Clinical and Scientific Conference being held in Dallas, Texas, on March 19, 2025.

The company has now completed enrollment in the EXPLORE44 Open-label Extension (OLE) study for people living with DMD44. The data from the Phase 1/2 EXPLORE44 and EXPLORE44-OLE™ studies will support the company's first BLA submission anticipated at year end 2025.

Avidity reported its 2025 outlook, including upcoming clinical and regulatory highlights, and recent organization appointments to execute on the full range of strategic initiatives and growth anticipated in 2025 and beyond. Updates include:

- **Delpacibart zotadirsen (del-zota) for the treatment of DMD44:**
    - Planned BLA submission year end 2025
        - The U.S. Food and Drug Administration (FDA) confirmed the accelerated approval path is available for del-zota and that the clinical data package from the EXPLORE44® program could support a BLA filing
    - Presentation of topline data from the EXPLORE44 trial (Q1)
    - Presentation of topline data from the ongoing EXPLORE44-OLE trial (Q4)
- **Delpacibart etedesiran (del-desiran) for the treatment of myotonic dystrophy type 1 (DM1):**
    - Completion of enrollment of the ongoing Phase 3 HARBOR™ trial (mid-2025)
    - Update from the ongoing MARINA-OLE™ trial including long-term 4mg/kg and safety data (Q4)
    - Publication of data analyses from the completed Phase 1/2 MARINA® trial (2025)

- Planned marketing application submissions in 2026, including in the U.S. and European Union

- **Delpacibart braxlosiran (del-brax) for the treatment of facioscapulohumeral muscular dystrophy (FSHD):**

  - Regulatory alignment on a global Phase 3 trial design (Q2)
  - Alignment on a potential accelerated approval path for the ongoing FORTITUDE™ biomarker cohort (Q2)
  - Completion of enrollment of the FORTITUDE biomarker cohort (Q2)
  - Presentation of topline data from the FORTITUDE trial (Q2)
  - Initiation of a global, potentially registrational trial in FSHD (Q2)

Full Year 2024 Highlights

Del-zota 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.
- In addition to the participants rolling over from the Phase 1/2 EXPLORE44 trial, Avidity announced it was enrolling additional participants in the EXPLORE44 Open-label Extension (OLE) study to support the BLA submission anticipated at year end 2025. Enrollment in the EXPLORE44-OLE study is now complete.
- In February, Avidity announced the FDA granted Rare Pediatric Disease Designation for del-zota for the treatment of DMD44.

Del-desiran for DM1

- Enrollment for the global Phase 3 HARBOR™ trial is ongoing and on track for completion in mid-2025.
- In May, Avidity announced the FDA granted breakthrough therapy designation for del-desiran for the treatment of DM1.
- Achieved global regulatory alignment with FDA, EMA and other global regulatory authorities on the design of the del-desiran Phase 3 HARBOR study in March 2024.
- In March, Avidity reported positive del-desiran long-term 4 mg/kg data from the MARINA-OLE™ study showing reversal of disease progression in people living with DM1 across multiple endpoints, including vHOT, muscle strength and activities of daily living when compared to END-DM1 natural history data.

Del-brax 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, 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.

Pipeline Advancements

- In November, Avidity announced the expansion of its pipeline into precision cardiology, including two wholly-owned candidates for PRKAG2 syndrome and PLN cardiomyopathy. In addition, Avidity shared details of its next-generation technology innovations with up to 30-fold improvements in delivery observed in preclinical studies.
- 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.

Fourth Quarter and Year End 2024 Financial Results

- **Cash, Cash Equivalents and Marketable Securities:** Cash, cash equivalents and marketable securities totaled approximately $1.5 billion as of December 31, 2024.
- **Collaboration Revenue:** Collaboration revenues of $3.0 million for the fourth quarter of 2024 and $10.9 million for the year ended 2024 primarily relate to Avidity's research collaboration and license partnership with Bristol Myers Squibb. Collaboration revenues of $2.2 million for the fourth quarter of 2023 and $9.6 million for the year ended 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 $95.6 million for the fourth quarter of 2024 compared with $52.8 million for the fourth quarter of 2023, and $303.6 million for the year ended 2024 compared with $191.0 million for the year ended 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 $28.3 million for the fourth quarter of 2024 compared with $16.1 million for the fourth quarter of 2023, and $86.2 million for the year ended 2024 compared with $54.2 million for the year ended 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: Avidity's plans for three potential successive product launches; Avidity's plans for a BLA submission for del-zota and the timing thereof; the status of three of Avidity's programs as potentially registrational; the status of Avidity's ongoing clinical trials and cohorts therein, including but not limited to initiation, enrollment, design and goals; the ability for del-zota and del-brax to achieve accelerated approval; the presentation of additional data, analyses and other updates from Avidity's ongoing clinical programs and the timing thereof; planned marketing applications for del-desiran in the U.S. and European Union and the timing thereof; Avidity's plans to become a global commercial organization and the status of its commercialization efforts; Avidity's precision cardiology candidates and next-generational technology innovations; plans for the advancement of DMD programs beyond DMD44; the characterization of data associated with Avidity's product candidates in their respective clinical trials and preclinical studies, the conclusions drawn therefrom, the impact of such data on the advancement of the respective product candidates and their abilities to treat their intended disease targets; 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: the data and results produced in Avidity's ongoing clinical trials as of the most recent respective cutoff dates may not be indicative of final results, may not support BLA submissions or accelerated approvals, may not be satisfactory to the FDA and other regulators, and new analyses of existing data and results may produce different conclusions than established as of the date hereof; even if approved, Avidity may not be able to execute any successful product launches; Avidity's efforts to build a global commercial organization may be unsuccessful; 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; 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, 2024 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 December 31,		Twelve Months Ended December 31,	
	2024	2023	2024	2023
Collaboration revenue	$ 2,973	$ 2,193	$ 10,897	$ 9,560
Operating expenses:				
Research and development	95,625	52,817	303,593	190,968
General and administrative	28,338	16,119	86,240	54,190
Total operating expenses	123,963	68,936	389,833	245,158
Loss from operations	(120,990)	(66,743)	(378,936)	(235,598)
Other income, net	18,733	6,300	56,634	23,378
Net loss	$ (102,257)	$ (60,443)	$ (322,302)	$ (212,220)
Net loss per share, basic and diluted	$ (0.80)	$ (0.79)	$ (2.89)	$ (2.91)
Weighted-average shares outstanding, basic and diluted	128,497	76,052	111,582	73,012

Balance Sheets	December 31, 2024	December 31, 2023
Assets		
Current assets:		
Cash, cash equivalents and marketable securities	$ 1,501,497	$ 595,351
Prepaid and other assets	40,793	15,956
Total current assets	1,542,290	611,307
Property and equipment, net	12,670	8,381
Restricted cash	2,795	295
Right-of-use assets	5,619	8,271
Other assets	521	301
Total assets	$ 1,563,895	$ 628,555
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and other liabilities	$ 77,031	$ 52,315
Deferred revenue, current portion	20,987	28,365
Total current liabilities	98,018	80,680
Lease liabilities, net of current portion	2,957	6,213
Deferred revenue, net of current portion	37,961	40,898
Total liabilities	138,936	127,791
Stockholders' equity	1,424,959	500,764
Total liabilities and stockholders' equity	$ 1,563,895	$ 628,555

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