Avidity Biosciences Reports Second Quarter 2025 Financial Results and Recent Highlights

Aligned with FDA on del-brax accelerated and full approval pathways for FSHD, reported positive topline data from Phase 1/2 FORTITUDE™ trial, and initiated global confirmatory Phase 3 study

Planned del-zota BLA submission at year end 2025 for DMD44 on track to be Avidity's first BLA submission

On track for three potential BLA submissions over a 12-month period

Strong balance sheet and cash runway to mid-2027 enabling global commercial launch readiness; first potential commercial launch in U.S. in 2026

SAN DIEGO, Aug. 7, 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 second quarter ended June 30, 2025 and highlighted recent progress.

"Avidity continues to deliver on its leadership in RNA therapeutics as we prepare for three potential BLA submissions in a 12-month period with strong clinical data, regulatory progress, and operational execution," said Sarah Boyce, president and chief executive officer of Avidity. "Our programs in FSHD, DMD44 and DM1 each made meaningful advances during the second quarter of 2025, and we are encouraged by the consistent and reproducible data across these late-stage neuromuscular programs. We are keenly aware of the challenges facing patients and families living with these conditions and are preparing for potential commercialization with great thoughtfulness and urgency."

"Avidity continues to operate from a position of financial strength," saidMike MacLean, chief financial officer at Avidity. "Avidity's recent topline data for del-brax continues to demonstrate the consistency of the platform. As we move quickly toward potentially three successive launches starting in 2026, we continue to make meaningful progress in building our global infrastructure."

Company Highlights

- **Delpacibart zotadirsen (del-zota) for the treatment of people living with Duchenne muscular dystrophy with mutations amenable to exon 44 skipping (DMD44):**
  - In July 2025, Avidity announced the U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy designation to del-zota
  - Remain on track for planned BLA submission at year end 2025, which will be Avidity's first BLA
  - Plan to present topline and functional data from the ongoing EXPLORE44-OLE™ trial in the fourth quarter of 2025
- **Delpacibart etedesiran (del-desiran) for the treatment of myotonic dystrophy type 1 (DM1):**
  - In July 2025, Avidity announced completion of enrollment in the ongoing Phase 3 HARBOR™ trial
  - On track to share updates from the ongoing MARINA-OLE™ trial including long-term 4 mg/kg efficacy and safety data in the fourth quarter 2025
  - Expected publication of data analyses from the completed Phase 1/2 MARINA® trial (2025)
  - Topline data readout from HARBOR™ study anticipated in the second quarter of 2026
  - Marketing application submissions for del-desiran including in U.S., E.U. andJapan anticipated to start in the second half of 2026
- **Delpacibart braxlosiran (del-brax) for the treatment of facioscapulohumeral muscular dystrophy(FSHD):**
  - In June 2025, Avidity announced multiple milestone updates for del-brax including:
    - Alignment with FDA on accelerated and full approval pathways for del-brax;
    - Positive topline Phase 1/2 FORTITUDE™ data from the dose escalation cohorts, demonstrating consistent improvement compared to placebo on multiple functional and quality of life measures, rapid and significant reductions in levels of KHDC1L or cDUX, a DUX4-regulated biomarker, and creatine kinase, a biomarker of muscle damage, and favorable long-term safety and tolerability;
    - Initiated global, confirmatory FORTITUDE-3™ (formerly known as FORWARD™) study of del-brax 2 mg/kg every six weeks, intended to support Avidity's global approval strategy for del-brax
  - Topline data from FORTITUDE biomarker cohort anticipated in the second quarter of 2026
  - Planned BLA submission for accelerated approval in the second half of 2026, using data from the ongoing, fully enrolled FORTITUDE biomarker cohort

Second Quarter 2025 Financial Results

- Cash, cash equivalents and marketable securities totaled approximately $1.2 billion as of June 30, 2025
    - Following the end of the second quarter of 2025, the Company received net proceeds of $185.5 million from the sale of stock through its at-the-market offering program
    - The Company expects that its cash, cash equivalents and marketable securities as of June 30, 2025, together with the net proceeds from the at-the-market offering, will be sufficient to fund its operations to mid-2027
- Collaboration revenues were $3.8 million for the second quarter of 2025, compared to $2.0 million for the same period of 2024, and primarily relate to the recognition of revenues under Avidity's research collaboration and license partnership with Bristol Myers Squibb. Collaboration revenues were $5.4 million for the six months ended June 30, 2025, compared to $5.6 million for the same period of 2024, and primarily relate to Avidity's research collaboration and license partnership with Bristol Myers Squibb
- Research and development expenses for the second quarter of 2025 were $138.1 million, compared to $63.9 million for the same period of 2024. Research and development expenses for the six months ended June 30, 2025 were $237.6 million, compared to $130.8 million for the same period of 2024. 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 expenses for the second quarter of 2025 were $36.9 million, compared to $20.7 million for the same period of 2024. General and administrative expenses for the six months ended June 30, 2025 were $70.5 million, compared to $34.6 million for the same period of 2024. The increases were primarily due to higher personnel and commercial infrastructure 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 also advancing two wholly-owned precision cardiology development candidates addressing rare genetic cardiomyopathies. In addition, Avidity is broadening the reach of AOCs with its advancing and expanding pipeline including programs in cardiology and immunology through 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 product launches; Avidity's plans for BLA submissions for each of its product candidates and the timing thereof; the status of Avidity's ongoing clinical trials and cohorts therein, including but not limited to initiation, enrollment, design and goals; the ability for any of Avidity's product candidates to achieve accelerated approval; plans to present 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., European Union and Japan, and the timing thereof; the regulatory status of each of Avidity's clinical programs; Avidity's plans to become a global commercial organization and the status of its commercialization efforts; the characterization of data associated with Avidity's product candidates, the conclusions drawn therefrom, the reproducibility of such data, the impact of such data on the advancement of Avidity's product candidates and their abilities to treat their respective disease indication; 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 clinical trials; Avidity's dependence on third parties in connection

with clinical testing and product manufacturing; 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.

Investor Contact:
Kat Lange
(619) 837-5014
investors@aviditybio.com

Media Contact:
Kristina Coppola
(619) 837-5016
media@aviditybio.com

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

Statements of Operations	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Collaboration revenue	$ 3,847	$ 2,045	$ 5,420	$ 5,588
Operating expenses:				
Research and development	138,125	63,940	237,615	130,772
General and administrative	36,864	20,731	70,464	34,629
Total operating expenses	174,989	84,671	308,079	165,401
Loss from operations	(171,142)	(82,626)	(302,659)	(159,813)
Other income, net	13,827	11,833	29,571	20,165
Net loss	$ (157,315)	$ (70,793)	$ (273,088)	$ (139,648)
Net loss per share, basic and diluted	$ (1.21)	$ (0.65)	$ (2.11)	$ (1.44)
Weighted-average shares outstanding, basic and diluted	129,622	106,928	129,428	97,070

Balance Sheets	June 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash, cash equivalents and marketable securities	$ 1,183,144	$ 1,501,497
Prepaid and other current assets	67,416	40,793
Total current assets	1,250,560	1,542,290
Property and equipment, net	20,535	12,670
Restricted cash	2,798	2,795
Right-of-use assets	4,227	5,619
Other assets	90,806	521
Total assets	$ 1,368,926	$ 1,563,895
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and other liabilities	$ 121,524	$ 77,031
Deferred revenue, current portion	13,537	20,987
Total current liabilities	135,061	98,018
Lease liabilities, net of current portion	1,210	2,957
Deferred revenue, net of current portion	39,991	37,961

Total liabilities	176,262	138,936
Stockholders' equity	1,192,664	1,424,959
Total liabilities and stockholders' equity	$ 1,368,926	$ 1,563,895

SOURCE Avidity Biosciences, Inc.

---

https://investors.aviditybiosciences.com/2025-08-07-Avidity-Biosciences-Reports-Second-Quarter-2025-Financial-Results-and-Recent-Highlights