Avidity Biosciences Reports First Quarter 2025 Financial Results and Recent Highlights

On track to deliver key regulatory updates for del-brax and topline data from dose escalation cohorts in the FORTITUDE™ trial in the second quarter

Positive topline del-zota data further supports first BLA submission at year end 2025 – continues to highlight reproducibility and consistency across three late-stage clinical trials for DMD44, DM1 and FSHD

Executing on global commercial infrastructure development and on track with preparations for first potential commercial launch in U.S. in 2026

Strong balance sheet supports execution across three late-stage clinical programs and commercial launch preparations with cash runway into mid-2027

SAN DIEGO, May 8, 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 first quarter ended March 31, 2025, highlighting recent progress.

"We continue to build on the consistent and reproducible data readouts from our platform across all three of our clinical programs for DMD44, DM1 and FSHD, and are executing on our transition to become a global commercial organization. In March 2025, we shared positive topline EXPLORE44® Phase 1/2 del-zota data demonstrating remarkable and consistent improvements across multiple measures including a substantial increase in dystrophin production and reduction in creatine kinase levels to near normal, which will support our planned BLA submission at year end 2025 and reinforces del-zota's potential to become a groundbreaking treatment for people living with DMD44," said Sarah Boyce, president and chief executive officer at Avidity. "We look forward to delivering on multiple milestones this year and remain on track to share several key regulatory updates for del-brax in the second quarter that include a potential accelerated approval path in U.S., alignment on the design of the global Phase 3 trial and initiation of the trial. We are also planning to share topline del-brax data from the FORTITUDE™ dose escalation cohorts in the second quarter."

"2025 is a pivotal year for Avidity as we build out our global commercial infrastructure to support three potential product launches for del-zota, del-desiran and del-brax. With a strong balance sheet and a cash balance of approximately $1.4 billion at the end of the first quarter, we are well positioned to execute across all of our late-stage clinical trials as we begin to set the stage for our planned first commercial launch in 2026," said Mike MacLean, chief financial officer at Avidity.

Company Highlights

- **Delpacibart zotadirsen (del-zota) for the treatment of DMD44:**
  - In March 2025, Avidity announced positive topline data from the completed Phase 1/2 EXPLORE44® trial for people living with Duchenne muscular dystrophy amenable to exon 44 skipping (DMD44) demonstrating statistically significant increases in exon skipping, a substantial increase in dystrophin production, a significant reduction in creatine kinase levels to near normal and consistent favorable safety and tolerability results across both dose cohorts. Based on the consistent data between the 5 mg/kg every six weeks and the 10 mg/kg every eight weeks groups across all parameters, Avidity has selected the dose of 5 mg/kg every six weeks of del-zota for the Biologics License Application (BLA) submission and future clinical studies
  - In February 2025, Avidity announced completion of enrollment for the ongoing EXPLORE44-OLE™ study for 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 April 2025, Avidity announced the Japan Ministry of Health, Labour and Welfare (MHLW) granted Orphan Drug designation (ODD) for del-desiran
  - Completion of enrollment for the ongoing Phase 3 HARBOR™ trial is on track for mid-2025
  - Plan to provide an update from the ongoing MARINA-OLE™ trial including long-term 4 mg/kg and safety data in the fourth quarter 2025
  - Publication of data analyses from the completed Phase 1/2 MARINA® trial (2025)
  - Planned marketing application submissions starting in 2026, including in the U.S., European Union and Japan
- **Delpacibart braxlosiran (del-brax) for the treatment of facioscapulohumeral muscular dystrophy (FSHD):**

- In March 2025, Avidity announced completion of enrollment for the FORTITUDE™ biomarker cohort for del-brax with 51 total participants enrolled – biomarker cohort supports pathway to potential accelerated approval for del-brax. Enrollment was completed in the first quarter of 2025, ahead of original second quarter guidance.
- In the second quarter, Avidity plans to share multiple updates from the del-brax program:
    - Regulatory alignment on a potential accelerated approval path in the U.S.;
    - Regulatory alignment on the design of the global Phase 3 trial as well as initiation of the trial; and
    - Topline data from the dose escalation cohorts in the FORTITUDE trial.

First Quarter 2025 Financial Results

- Cash, cash equivalents and marketable securities totaled approximately $1.4 billion as of March 31, 2025.
- Collaboration revenues were $1.6 million for the first quarter of 2025, compared to $3.5 million for the same period of 2024, and primarily relate to Avidity's research collaboration and license partnership with Bristol Myers Squibb. The decrease was primarily due to the recognition of revenues under Avidity's research collaboration and license partnership with Eli Lilly and Company in the prior year for which there were no revenues recognized in the current year.
- Research and development expenses for the first quarter of 2025 were $99.5 million, compared to $66.8 million for the same period of 2024. The increase was 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 first quarter of 2025 were $33.6 million, compared to $13.9 million for the same period of 2024. The increase was 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 neuromuscular 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 a BLA submission for del-zota 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; 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., European Union and Japan, and the timing thereof; actual or prospective regulatory alignment related to 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 del-zota, the conclusions drawn therefrom, the reproducibility of such data, the impact of such data on the advancement of del-zota and its ability to treat DMD44; 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; 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:
(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 March 31,**	
	2025	**2024**
Collaboration revenue	$ 1,573	$ 3,543
Operating expenses:		
Research and development	99,490	66,832
General and administrative	33,600	13,898
Total operating expenses	133,090	80,730
Loss from operations	(131,517)	(77,187)
Other income, net	15,744	8,332
Net loss	$ (115,773)	$ (68,855)
Net loss per share, basic and diluted	$ (0.90)	$ (0.79)
Weighted-average shares outstanding, basic and diluted	129,232	87,212

Balance Sheets	**March 31, 2025**	**December 31, 2024**
Assets		
Current assets:		
Cash, cash equivalents and marketable securities	$ 1,379,877	$ 1,501,497
Prepaid and other assets	55,098	40,793
Total current assets	1,434,975	1,542,290
Property and equipment, net	15,637	12,670
Restricted cash	2,795	2,795
Right-of-use assets	4,929	5,619
Other assets	739	521
Total assets	$ 1,459,075	$ 1,563,895
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and other liabilities	$ 70,887	$ 77,031
Deferred revenue, current portion	13,978	20,987
Total current liabilities	84,865	98,018
Lease liabilities, net of current portion	2,090	2,957
Deferred revenue, net of current portion	43,397	37,961
Total liabilities	130,352	138,936
Stockholders' equity	1,328,723	1,424,959
Total liabilities and stockholders' equity	$ 1,459,075	$ 1,563,895

SOURCE Avidity Biosciences, Inc.

https://investors.aviditybiosciences.com/2025-05-08-Avidity-Biosciences-Reports-First-Quarter-2025-Financial-Results-and-Recent-Highlights