Avidity Biosciences Reports Second Quarter 2024 Financial Results and Recent Highlights

Avidity reports positive del-zota (AOC 1044) data; demonstrated 25% increase in dystrophin production and reduction of creatine kinase levels to near normal in people living with DMD44 in Phase 1/2 EXPLORE44™ trial

Initiated global Phase 3 HARBOR™ trial and began administration of del-desiran in people living with DM1; del-desiran received FDA Breakthrough Therapy designation

Avidity plans to accelerate initiation of del-brax (AOC 1020) registrational cohorts after reporting unprecedented and consistent reductions in DUX4 regulated genes, trends of functional improvement and favorable safety and tolerability in people living with FSHD in Phase 1/2 FORTITUDE™ trial

Announcement of our lead precision cardiology program target planned for Q4 2024

Cash on hand of approximately $1.3 billion

SAN DIEGO, Aug. 9, 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 second quarter ended June 30, 2024, and highlighted recent corporate progress.

"The last few months have been incredible for Avidity, but more importantly, for people living with DM1, FSHD and DMD. We received breakthrough designation for del-desiran, initiated the global Phase 3 HARBOR™ trial, reported unprecedented data from the del-brax FORTITUDE™ trial and earlier today, shared data from del-zota's Phase 1/2 EXPLORE44™ trial demonstrating significant increases in dystrophin production and exon 44 skipping for people living with DMD44," said Sarah Boyce, president and chief executive officer at Avidity. "All of this progress is due to our AOC platform which has proven we can target a range of genetic diseases. We plan to expand beyond rare neuromuscular diseases as we share the first target from our precision cardiology pipeline in Q4 2024. These achievements, accomplished in conjunction with our patient communities, accelerate our vision of profoundly improving people's lives by revolutionizing RNA delivery."

"We successfully closed an upsized public offering in June, our second successful equity raise this year. Our cash position of ~$1.3 billion at the end of the second quarter, allows us to progress our current clinical trials and expand our DMD franchise to include multiple potential treatments for people living with Duchenne Muscular Dystrophy beyond DMD44," said Mike MacLean, chief financial officer and chief business officer at Avidity.

Recent Highlights

Del-zota (AOC 1044)

- In August, Avidity reported positive initial del-zota 5mg/kg patient data from the Phase 1/2 EXPLORE44™ trial in people living with DMD44 demonstrating unsurpassed delivery in skeletal muscle, unprecedented increase in dystrophin production, robust exon 44 skipping, and a profound reduction in creatine kinase
- In the Phase 1/2 EXPLORE44 trial, del-zota data from 10 participants at the four-month time period after three doses of 5mg/kg del-zota (PMO dose), or placebo every six weeks, demonstrated:
    - Unsurpassed delivery of PMO of 200 nM in skeletal muscle
    - Statistically significant 37% increase in exon 44 skipping and up to 66% exon 44 skipping compared to baseline
    - Statistically significant increase of 25% of normal in dystrophin production and restored total dystrophin up to 54% of normal
    - Profound reduction in creatine kinase as levels were reduced to near normal with greater than 80% reduction compared to baseline
- Safety and tolerability for 25 participants was assessed across two dose levels (5 mg/kg and 10 mg/kg) from the Phase 1/2 EXPLORE44 trial. Del-zota demonstrated favorable safety and tolerability with most treatment emergent adverse events (AEs) mild or moderate in participants with DMD44
- Enrollment is now complete for the Phase 1/2 EXPLORE44 trial. Avidity plans to enroll additional patients in the EXPLORE44 Open-label Extension study (OLE)
- Avidity introduced delpacibart zotadirsen as the approved international nonproprietary name of AOC 1044, abbreviated as del-zota

Del-desiran (AOC 1001)

- In June, Avidity initiated and began administration of del-desiran in the global Phase 3 HARBOR™ trial in people living

with myotonic dystrophy type 1 (DM1)
- The FDA granted del-desiran Breakthrough Therapy designation in May 2024 for DM1

Del-brax (AOC 1020)

- Avidity reported positive initial del-brax data from the Phase 1/2 FORTITUDE™ trial demonstrating unprecedented and consistent reductions of greater than 50% in DUX4 regulated genes, trends of functional improvement, and favorable safety and tolerability in people living with facioscapulohumeral muscular dystrophy (FSHD)
- In the Phase 1/2 FORTITUDE trial, del-brax 2 mg/kg data from 12 participants at the four-month time period demonstrated:
    - Greater than 50% mean reductions in DUX4 regulated genes across multiple panels for DUX4 regulated gene expression in muscle
    - All participants treated with del-brax showed reductions greater than 20% in DUX4 regulated genes
    - Mean reductions of 25% or greater in a novel circulating biomarker and creatine kinase
    - Trends of functional improvements including increased strength in upper and lower limb muscles, and muscle function as measured by reachable workspace (RWS) compared to placebo and the ReSolve natural history study
    - Trends of improvement in patient and clinician reported outcomes
- A four-month look at the safety and tolerability for all 39 participants across two dose levels (2 mg/kg and 4 mg/kg) from the Phase 1/2 FORTITUDE trial demonstrated favorable safety and tolerability with all adverse events (AEs) mild or moderate, no serious adverse events and no discontinuations
- Completed enrollment for the Phase 1/2 FORTITUDE trial. Avidity plans to accelerate the initiation of registrational cohorts with the biomarker cohort planned in 2H 2024 and functional cohort planned in 1H 2025.
- Avidity introduced delpacibart braxlosiran as the approved international nonproprietary name of AOC 1020, abbreviated as del-brax

Pipeline Advancements

- Avidity plans to announce its lead precision cardiology program target in Q4 2024

Organizational Highlights

- Announced the appointment of Simona Skerjanec, M.Pharm, MBA to its board of directors in May 2024
- Announced the appointment of John B. Moriarty, Jr., J.D., as Chief Legal Officer and Corporate Secretary in August 2024

Upcoming Milestones

- Upcoming anticipated milestones include:
    - In Q4 2024, announcing lead precision cardiology program target
    - Accelerating the initiation of registrational cohorts in FORTITUDE™ trial:
        - Biomarker cohort planned for 2H 2024
        - Functional cohort planned for 1H 2025

Second Quarter 2024 Financial Results

- **Cash, Cash Equivalents and Marketable Securities:** Cash, cash equivalents and marketable securities totaled $1.3 billion as of June 30, 2024, which reflects a gross $461 million raise from a public offering.
- **Collaboration Revenue:** Collaboration revenues of $2.0 million for the second quarter of 2024 and $5.6 million for the first six months of 2024 primarily relates to Avidity's research collaboration and license partnership with Bristol Myers Squibb. Collaboration revenues of $2.3 for the second quarter of 2023 and $4.5 million for the first six months of 2023 primarily related to Avidity's research collaboration and license partnership with Eli Lilly and Company. There was no revenue related to the partnership with Bristol Myers Squibb in 2023.
- **Research and Development (R&D) Expenses**: R&D expenses include external and internal costs associated with research and development activities. These expenses were $63.9 million for the second quarter of 2024 compared with $42.6 million for the second quarter of 2023, and $130.8 million for the first six months of 2024 compared with $90.4 million for the first six 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 $20.7 million for the second quarter of 2024 compared with $12.3 million for the second quarter of 2023, and $34.6 million for the first six months of 2024 compared with $24.3 million for the first six 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 focused on 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: the anticipated announcement of a lead precision cardiology program target, including the timing thereof; the potential to develop first-in-class and best-in-class treatments; plans to accelerate the initiation of registrational cohorts in the FORTITUDE™ trial and the timing thereof; the characterization of data associated with del-brax from the FORTITUDE study and del-zota with the EXPLORE44™ study, and the impact of such data on the advancement of the respective product candidates; Avidity's DMD franchise; Avidity's plans to become an integrated global biopharmaceutical company; Avidity's platform, planned operations and programs; and Avidity's financial position, cash balance and expected cash 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 planned additional 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; Avidity may not be able to resolve the partial clinical hold related to del-desiran; 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; 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.

Investor Contact:
Mike MacLean
(619) 837-5014
investors@aviditybio.com

Media Contact:
Navjot Rai
(619) 837-5016
media@aviditybio.com

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

Statements of Operations	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023

Collaboration revenue	$ 2,045	$ 2,316	$ 5,588	$ 4,549
Operating expenses:				
Research and development	63,940	42,672	130,772	90,437
General and administrative	20,731	12,278	34,629	24,342
Total operating expenses	84,671	54,950	165,401	114,779
Loss from operations	(82,626)	(52,634)	(159,813)	(110,230)
Other income, net	11,833	5,609	20,165	10,811
Net loss	$ (70,793)	$ (47,025)	$ (139,648)	$ (99,419)
Net loss per share, basic and diluted	$ (0.65)	$ (0.66)	$ (1.44)	$ (1.40)
Weighted-average shares outstanding, basic and diluted	106,928	71,390	97,070	70,914

Balance Sheets	**June 30, 2024**	**December 31, 2023**
Assets		
Current assets:		
Cash, cash equivalents and marketable securities	$ 1,299,046	$ 595,351
Prepaid and other assets	29,755	15,956
Total current assets	1,328,801	611,307
Property and equipment, net	8,498	8,381
Restricted cash	2,795	295
Right-of-use assets	6,967	8,271
Other assets	364	301
Total assets	$ 1,347,425	$ 628,555
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable and other liabilities	$ 57,515	$ 52,315
Deferred revenue, current portion	26,697	28,365
Total current liabilities	84,212	80,680
Lease liabilities, net of current portion	4,617	6,213
Deferred revenue, net of current portion	37,560	40,898
Total liabilities	126,389	127,791
Stockholders' equity	1,221,036	500,764
Total liabilities and stockholders' equity	$ 1,347,425	$ 628,555

SOURCE Avidity Biosciences, Inc.

---

https://investors.aviditybiosciences.com/2024-08-09-Avidity-Biosciences-Reports-Second-Quarter-2024-Financial-Results-and-Recent-Highlights