Metagenomi Reports Business Updates and Second Quarter 2024 Financial Results

08.14.24

Declared development candidate MGX-001 for the treatment of hemophilia A; plans to present 12-month NHP durability study data in September 2024

All Wave 1 Ionis collaboration programs advancing in lead optimization, unlocking potential for multiple development candidate nominations in 2025

Achieved milestone from partner Affini-T related to Metagenomi licensed technology

Well capitalized with $299.9M in cash, cash equivalents and available-for-sale marketable securities at the end of Q2 2024; cash runway anticipated to support operating plans into 2027

EMERYVILLE, Calif., Aug. 14, 2024 (GLOBE NEWSWIRE) -- Metagenomi, Inc. (Nasdaq: MGX) (Metagenomi), a precision genetic medicines company committed to developing curative therapeutics for patients using its proprietary, comprehensive metagenomics-derived gene editing toolbox, today provided a business update and reported second quarter 2024 financial results.

“We are progressing our pipeline while remaining laser focused on our vision to create curative genetic medicines by harnessing the power of our AI-driven metagenomics platform,” said Brian C. Thomas, PhD, CEO and Founder. “We have significantly advanced our wholly-owned program in hemophilia A, including recent positive regulatory interactions with Food and Drug Administration (FDA) and the declaration of MGX-001 as our lead development candidate. Our team has worked diligently to allow us to accelerate the data release from our 12-month nonhuman primate (NHP) durability study from the end of 2024 into September 2024. This study is an important step towards advancing MGX-001 as a potentially life-long curative treatment for both adults and children with hemophilia A, and demonstrating our ability to potentially achieve large targeted gene integration in patients using our metagenomics toolbox.”

Dr. Thomas continued, “We are also progressing our partnered programs and advancing our metagenomics-based toolbox. The foundation of our Ionis collaboration was strengthened by the addition of two high-value targets associated with cardiometabolic disease, and all collaboration programs have progressed into the lead optimization phase of development. We achieved a development milestone with Affini-T, where our team provided support for Affini-T’s regulatory filing related to Metagenomi licensed technology and current good manufacturing practices (cGMP) material supply. We continue to advance a broad range of technologies to support our goal of being able to address any disease by enabling any therapeutic genome edit in the human genome. Going forward, we remain well capitalized to execute our strategic priorities, with a cash runway into 2027.”

Second Quarter 2024 Business Updates and Key 2024-2025 Anticipated Milestones

Therapeutic Pipeline Updates

- Metagenomi continues to advance its wholly-owned lead program in hemophilia A, designed as a one-time curative treatment for both adults and children.
    - The company engaged with FDA in the second quarter to discuss key aspects of the planned Investigational New Drug (IND) submission.
    - The company declared a lead development candidate (DC), MGX-001, meeting previously announced guidance for the timing of a DC nomination.
    - The company plans to accelerate the release of data from the 12-month NHP durability study for the lead hemophilia A program from late 2024 into September 2024. The 12-month NHP durability study was designed to show therapeutically relevant, durable expression of Factor VIII in NHPs, addressing a key concern which has impacted other genetic medicines programs in hemophilia A that have experienced loss of Factor VIII expression over time.
    - The company plans to initiate cGMP manufacturing and related IND enabling activities in 2024 and continue IND enabling activities in 2025.
    - As previously disclosed, the company remains on track to file an IND in hemophilia A in 2026.
- Building on the recent success of the hemophilia A program, Metagenomi plans to advance additional wholly-owned therapeutic candidates targeting secreted protein disorders, leveraging the MGX-001 editing system with the goal of achieving targeted and durable gene expression.

    - This editing system consists of two components: a highly efficient and specific nuclease creates a precise cut at the albumin safe harbor gene locus after delivery by LNP; and an AAV vector delivered FVIII DNA template is inserted into the nuclease cut site by a naturally occurring DNA repair process.

- Following selection of the remaining Wave 1 targets in the Ionis collaboration in the first quarter of 2024, all four therapeutic programs successfully advanced into the lead optimization phase in the second quarter of 2024.

    - All four therapeutic targets in Wave 1 collaboration focus on cardiometabolic diseases; the company announced Refractory Hypertension as the initial disease target leveraging the angiotensinogen pathway.
    - The company aims to demonstrate *in vivo* proof-of-concept in 2024, and advance NHP studies supporting DC nominations thereafter.
    - The company plans to nominate one to two DCs for cardiometabolic programs in 2025.

- The company achieved a milestone in connection with its Affini-T collaboration, establishing cGMP gene editing
  reagents for cell therapy and filing related Drug Master Files with FDA to support an IND for Affini-T's T-cell receptor-based therapy.

    - Metagenomi received 933,650 shares of Affini-T common stock upon achievement of this regulatory milestone.

Technology Platform Updates

- Metagenomi achieved *in vitro* proof-of-concept for an undisclosed neuromuscular target with its ultra small editing system, a key milestone, opening up therapeutic potential for extrahepatic disease targets.
- Metagenomi achieved *in vitro* proof-of-concept using its RNA-mediated integration system (RIGS) for undisclosed liver targets.
- Metagenomi achieved multiplex base editing proof-of-concept and plans to present this data at a scientific conference in the second half of 2024.
- Metagenomi achieved *in vitro* proof-of-concept in human cells for large gene integration using its potentially industry leading, compact CRISPR-associated transposase (CAST) technology, with a publication planned.
- As previously disclosed, the company remains on track to demonstrate *in vivo* proof-of-concept for a large gene integration in 2026.

Other Business Updates

- The company continues to exercise fiscal responsibility in prioritizing certain internal development programs while looking for collaboration partners to leverage other development opportunities.

    - Metagenomi is looking for a partner or licensee for further development of its program for primary hyperoxaluria type 1 (PH1). Previously disclosed data demonstrated preclinical proof-of-concept in an accepted disease model of PH1.
    - The company is continuing to focus its internal efforts on *in vivo* gene editing therapeutic approaches and pursue technology out-licensing for *ex vivo* cell therapy, where next generation gene editing systems are an important enabler of novel therapies.

- Going forward, the company is not pursuing amyotrophic lateral sclerosis (ALS) based upon recent peer company clinical data regarding the lack of efficacy of Ataxin-2 as a therapeutic target for ALS.
- The company is revising its pipeline design to consolidate earlier stage programs and make it easier to identify our near term pipeline priorities focused on in vivo gene editing and liver indications.

- As a result of the acceleration of all four Wave 1 Ionis targets into lead optimization, as well as the company's decision to deprioritize PH1, Metagenomi is revising to provide pipeline guidance only through year-end 2025, including the next one to two DC nominations in 2025. The company is maintaining its guidance for the planned IND filing for hemophilia A in 2026. The updated milestones are available in the corporate presentation on the company website.

Upcoming Events
Metagenomi plans to participate in the following events during the third quarter of 2024:

- Wells Fargo Healthcare Conference, September 4-6, 2024, Everett, Massachusetts
- H.C. Wainwright 26th Annual Global Investment Conference, September 9-11, 2024, New York City
- Chardan 8th Annual Genetics Medicines Conference, September 30-October 1, 2024, New York City

Second Quarter 2024 Financial Results

- **Cash Position:** Cash, cash equivalents, and available-for-sale marketable securities were $299.9 million as of June 30, 2024.
- **R&D Expenses:** Research and development (R&D) expenses were $28.3 million for the three months ended June 30, 2024, compared to $22.7 million for the three months ended June 30, 2023.
- **G&A Expenses:** General and administrative (G&A) expenses were $8.6 million for the three months ended June 30, 2024, compared to $6.6 million for the three months ended June 30, 2023.

About Metagenomi

Metagenomi is a precision genetic medicines company committed to developing curative therapeutics for patients using its proprietary, comprehensive metagenomics-derived toolbox. Metagenomi is harnessing the power of metagenomics, the study of genetic material recovered from the natural environment, to unlock four billion years of microbial evolution to discover and develop a suite of novel editing tools capable of correcting any type of genetic mutation found anywhere in the genome. Its comprehensive genome editing toolbox includes programmable nucleases, base editors, and RNA and DNA-mediated integration systems (including prime editing systems and clustered regularly interspaced short palindromic repeat associated transposases). Metagenomi believes its diverse and modular toolbox positions the company to access the entire genome and select the optimal tool to unlock the full potential of genome editing for patients. For more information, please visit https://metagenomi.c.

Cautionary Note Regarding Forward- Looking Statements

This press release contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934, each as amended. Such statements, which are often indicated by terms such as "anticipate," "believe," "could," "estimate," "expect," "goal," "intend," "look forward to," "may," "plan," "potential," "predict," "project," "should," "will," "would" and similar e include, but are not limited to, any statements relating to our growth strategy and product development programs, including the timing of and our ability to conduct IND-enabling studies, make regulatory filings such as INDs, statements concerning the potential of therapies and product candidates, statements concerning the timing of data presentations and publications, and any other statements that are not historical facts. Forward looking statements are based on management's current expectations and are subject to risks and uncertainties that could negatively affect our business, operating results, financial condition, and stock value. Factors that could cause actual results to differ materially from those currently anticipated include: risks relating to our growth strategy; our ability to obtain, perform under, and maintain financing and strategic agreements and relationships; risks relating to the results of research and development activities; risks relating to the timing of starting and completing clinical trials; uncertainties relating to preclinical and clinical testing; our dependence on third party suppliers; our ability to attract, integrate and retain key personnel; the early stage of products under development; our need for substantial additional funds; government regulation; patent and intellectual property matters; competition; as well as other risks described in "Risk Factors," in our most recent Form 10-K and our most recent 10-Qs on file with the Securities and Exchange Commission. We expressly disclaim any obligation or undertaking to release publicly any updates or revisions to any forward-looking statements contained herein to reflect any change in our expectations or any changes in events, conditions or circumstances on which any such statement is based, except as required by law, and we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995.

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Condensed Financial Statements
Condensed Consolidated Balance Sheet Data
(Unaudited)

(in thousands)	June 30, 2024	December 31, 2023
Cash, cash equivalents and available-for-sale marketable securities	$ 299,921	$ 271,182
Total assets	$ 385,905	$ 364,842
Total liabilities	$ 116,486	$ 149,668
Redeemable convertible preferred stock	$ —	$ 350,758
Total stockholders' equity (deficit)	$ 269,419	$ (135,584)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 385,905	$ 364,842

Condensed Consolidated Statements of Operations
(Unaudited)

(In thousands, except share and per share data)	Three Months Ended June 30, 2024	Three Months Ended June 30, 2023	Six Months Ended June 30, 2024	Six Months Ended June 30, 2023
Collaboration revenue	$ 20,008	$ 11,337	$ 31,167	$ 19,994
Operating expenses:				
Research and development	28,320	22,681	59,759	42,811
General and administrative	8,551	6,619	17,303	13,084
Total operating expenses	36,871	29,300	77,062	55,895
Loss from operations	(16,863)	(17,963)	(45,895)	(35,901)
Other income (expense):				
Interest income	3,976	3,967	7,910	7,970
Change in fair value of long-term investments	—	2,870	—	2,870
Other income (expense), net	(51)	16	(101)	15
Total other income, net	3,925	6,853	7,809	10,855
Net loss before benefit (provision) for income taxes	(12,938)	(11,110)	(38,086)	(25,046)
Benefit (provision) for income taxes	2,199	(1,898)	2,199	(4,095)
Net loss	$ (10,739)	$ (13,008)	$ (35,887)	$ (29,141)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.29)	$ (3.82)	$ (1.24)	$ (8.56)
Weighted average common shares outstanding, basic and diluted	36,625,291	3,404,585	28,901,399	3,404,585