Metagenomi Reports Business Updates and Full Year 2024 Financial Results

03.17.25

Hemophilia A presentation at American Society of Hematology (ASH) 66th Annual Meeting demonstrated sustained Factor VIII (FVIII) activity in nonhuman primate (NHP) study for more than 16 months

Leveraged hemophilia A albumin platform to achieve in vivo proof-of-concept in multiple secreted protein deficiencies to support wholly-owned follow-on program

Progressed four Wave 1 Ionis targets to lead optimization with plans to declare one to two development candidates (DCs) in 2025

Well capitalized with $248.3 million in cash, cash equivalents and available-for-sale marketable securities at the end of Q4 2024; Cash runway anticipated to support operating plans into 2027

EMERYVILLE, Calif., March 17, 2025 (GLOBE NEWSWIRE) -- Metagenomi, Inc. (Nasdaq: MGX), a precision genetic medicines company committed to developing curative therapeutics for patients using its proprietary gene editing toolbox, today reported financial results for the full year ended December 31, 2024, and provided business updates.

“Our diverse and modular AI-driven metagenomics platform is designed to precisely target any site in the human genome, yielding the potential to address the full spectrum of genetic diseases,” said Brian C. Thomas, PhD, CEO and founder of Metagenomi. “In 2024, we made significant progress toward our goal of developing curative genetic medicines for patients. We progressed MGX-001, our potentially transformative treatment for hemophilia A, and leveraged the MGX-001 platform to advance an additional wholly-owned program for an undisclosed secreted protein deficiency disorder. We advanced Wave 1 of our Ionis collaboration, progressing all four targets in cardiometabolic indications. We defined our goals for 2025 and 2026, and remain on track to submit our first IND in hemophilia A in 2026.”

2024 Pipeline Achievements

Hemophilia A Program

- Declared development candidate for wholly-owned program in hemophilia A, MGX-001
- Oral presentation at ASH demonstrated sustained FVIII activity in an NHP study for more than 16 months, supporting the durability of our gene editing therapy
- Nominated a development candidate MGX-001 that includes a bioengineered FVIII construct with higher FVIII activity levels vs wild type construct
- Completed initial regulatory engagement with FDA and initiated GxP manufacturing activities

Secreted Protein Deficiencies

- Identified targets for wholly-owned therapeutic programs leveraging the gene integration approach used in MGX-001 and achieved in vivo proof-of-concept in rodents across three secreted protein targets

Cardiometabolic Indications

- Advanced four Wave 1 Ionis collaboration programs to lead optimization, including transthyretin (TTR) for transthyretin amyloidosis and angiotensinogen (AGT) for refractory hypertension, and achieved in vivo proof-of-concept in rodents across all four programs
- Demonstrated 95% protein knockdown in spontaneous hypertensive rats, a widely used preclinical model for refractory hypertension, which represents an example of the progress across the collaboration

Key 2024 Technology Achievements

- Used artificial intelligence (AI), ancestral state reconstruction, and structural biology to enhance our gene editing systems
- Presented compact SMall Arginine-Rich sysTems (SMART) nucleases demonstrating robust in vitro genome editing activity at multiple therapeutically relevant loci
- Presented novel adenine base editors (ABEs) achieving over 95% knockdown of three target proteins in primary T-cells via simultaneous triplex editing with high specificity and post-editing cell health

2025 - 2026 Anticipated Milestones

Hemophilia A

- Plan to release final FVIII durability and related preclinical study data from NHP durability study in the first half of 2025
- On track for Pre-IND and ex-US regulatory meetings in 2025
- Plan to file IND/ CTA submissions in 2026 to advance MGX-001 into first-in-human studies

Secreted Protein Deficiencies

- Plan to demonstrate NHP proof-of-concept for lead secreted protein deficiency target in 2025 and nominate DC for lead secreted protein deficiency in 2026

Cardiometabolic Indications

- On track to nominate one to two DCs from the four Wave 1 Ionis collaboration development programs and disclose remaining therapeutic indications in large cardiometabolic indications in 2025
- Plan to initiate IND-enabling activities for DCs nominated in 2025 and nominate additional DCs from the remaining Wave 1 targets in 2026

Other Business Updates

- Eric Bjerkholt, CFO of Mirum Pharmaceuticals, Inc., joined Metagenomi's Board of Directors, serving on Metagenomi's Audit and Compensation committees
- Publication in *Nature Communications* describing novel, compact CRISPR-associated transposases (CAST) demonstrated integration of a large, therapeutically relevant gene into the genome of human cells using CAST systems

Full Year 2024 Financial Results

Cash Position: Cash, cash equivalents, and available-for-sale marketable securities were $248.3 million as of December 31, 2024.

R&D Expenses: Research and development (R&D) expenses were $109.2 million for the full year ended December 31, 2024, compared to $94.4 million for the full year ended December 31, 2023.

G&A Expenses: General and administrative (G&A) expenses were $32.0 million for the full year ended December 31, 2024, compared to $28.8 million for the full year ended December 31, 2023.

About Hemophilia A

Hemophilia A is the most common X-linked inherited bleeding disorder, caused by a large variety of mutations in the FVIII gene leading to a loss of functional FVIII protein. Intracranial bleeding is of greatest concern as this can lead to major morbidity and mortality. Bleeding into joints leads to cumulative joint damage and is a major cause of morbidity. Diagnosis of severe disease typically occurs in infancy due to exaggerated bleeding in response to minor injury or routine medical procedures. Prevalence is estimated to be up to 26,500 patients in the US and more than 500,000 patients globally according to the World Federation of Hemophilia, with the vast majority of patients being male.

About Metagenomi

Metagenomi is a precision genetic medicines company committed to developing curative therapeutics for patients using its AI-driven metagenomics platform. 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 (CAST)). Metagenomi believes its proprietary, 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.

Metagenomi intends to use the Investor Relations section of its website as a means of disclosing material nonpublic information and for complying with its disclosure obligations under Regulation FD. Accordingly, investors should monitor Metagenomi's website in addition to following its press releases, SEC filings, public conference calls, presentations, and webcasts.

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 expressions 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 our anticipated cash runway, 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 other risk factors set forth from time to time in our filings with the Securities and Exchange Commission made pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934, as amended. 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)	December 31, 2024	December 31, 2023
Cash, cash equivalents and available-for-sale marketable securities	$ 248,307	$ 271,182
Total assets	$ 324,599	$ 364,842
Total liabilities	$ 89,742	$ 149,668

Redeemable convertible preferred stock	$ —	$ 350,758
Total stockholders' equity (deficit)	$ 234,857	$ (135,584)
Total liabilities, redeemable convertible preferred stock and stockholders' equity (deficit)	$ 324,599	$ 364,842

Condensed Consolidated Statements of Operations
(Unaudited)

(in thousands, except share and per share data)	Years Ended December 31, 2024	2023
Collaboration revenue	$ 52,295	$ 44,756
Operating expenses:		
Research and development	109,179	94,403
General and administrative	32,017	28,845
Total operating expenses	141,196	123,248
Loss from operations	(88,901)	(78,492)
Other income (expense):		
Interest income	14,722	15,468
Change in fair value of long-term investments	(9,185)	2,870
Other expense, net	(207)	(74)
Total other income, net	5,330	18,264
Net loss before benefit (provision) for income taxes	(83,571)	(60,228)
Benefit (provision) for income taxes	5,513	(8,027)
Net loss	$ (78,058)	$ (68,255)
Net loss per share attributable to common stockholders, basic and diluted	$ (2.36)	$ (20.05)
Weighted average common shares outstanding, basic and diluted	33,027,889	3,404,585