Metagenomi Therapeutics Announces FDA Clearance of Investigational New Drug Application for MGX-001 and Reports Third Quarter 2026 Financial Results

11.10.26

FDA clearance enables initiation of a first-in-human Phase 1/2 trial of MGX-001 in patients with hemophilia A

MGX-001 is designed to deliver durable, endogenous Factor VIII (FVIII) activity from a one-time treatment for adults and children

Company plans to initiate the INTEGR8 clinical trial in the first half of 2027 and provide initial clinical data in the second half of 2027

$101.7 million in cash, cash equivalents, and available-for-sale marketable securities as of September 30, 2026, with runway anticipated to support operations into 2028

EMERYVILLE, Calif., Nov. 10, 2026 (GLOBE NEWSWIRE) -- Metagenomi Therapeutics, Inc. (Nasdaq: MGX) (the "Company"), an in vivo genome editing company capitalizing on its proprietary technologies to create potentially curative genetic medicines for patients, today announced that the U.S. Food and Drug Administration (FDA) has cleared the Company's Investigational New Drug (IND) application for MGX-001, its lead, wholly owned program for the treatment of hemophilia A. The Company also reported financial results for the third quarter ended September 30, 2026, and provided business updates.

"We are thrilled to reach this pivotal milestone with the FDA clearance of our IND for MGX-001, our lead, wholly owned program in hemophilia A," said Jian Irish, Ph.D., M.B.A., President and Chief Executive Officer of Metagenomi Therapeutics. "MGX-001 is designed to leverage our proprietary genome editing platform to integrate a Factor VIII (FVIII) gene site-specifically into the genome, harnessing the power of the body's native albumin promoter to drive durable, endogenous FVIII production. This represents an exciting departure from prior approved gene therapy for hemophilia A that relied on episomal FVIII expression. The MGX-001 preclinical data demonstrated FVIII activity in the normal physiologic range and durable expression in non-human primates and no detectable off-target editing. In a landscape with no cure for hemophilia A, we believe MGX-001 has the potential to provide long-term benefit to both adult and pediatric patients. We plan to initiate the INTEGR8 global clinical trial in the first half of 2027 and provide initial clinical data, including safety, tolerability, and FVIII functional activity data, in the second half of 2027."

Third Quarter 2026 and Recent Updates

MGX-001 – Hemophilia A Program

- IND clearance enables initiation of the INTEGR8 trial in the U.S. The Company also intends to expand the INTEGR8 trial to hemophilia treatment centers beyond the U.S., subject to clinical clearances in additional jurisdictions.
- The INTEGR8 Phase 1/2 trial of MGX-001 is designed to evaluate the safety and tolerability of MGX-001, and to identify a dose that achieves target FVIII activity levels in the normal physiologic range. The clinical trial is expected to be a global, multi-center, open-label, single-arm, dose escalation followed by dose expansion trial in adult patients with moderately severe to severe hemophilia A.
- Additional MGX-001 preclinical data, including the most recent efficacy and safety results, will be presented in an oral presentation (Abstract 952) at the 68th American Society of Hematology (ASH) Annual Meeting on Monday, December 14, 2026 in New Orleans.

Site-Specific Large Gene Integration for Other Protein Deficiency Diseases

- The Company continues to evaluate additional disease indications which may be treated by protein replacement via gene insertion leveraging the same site-specific large gene integration at the albumin locus as used in MGX-001. The potential for application in other diseases was previously demonstrated by the Company in non-human primates in another secreted protein disorder.

Third Quarter 2026 Financial Results

Cash Position: Cash, cash equivalents and available-for-sale marketable securities were $101.7 million as of September 30, 2026. The Company anticipates that the runway will support operations into 2028.

R&D Expenses: Research and development (R&D) expenses were $17.5 million for the quarter ended September 30, 2026, compared to $25.3 million for the comparable period in 2025.

G&A Expenses: General and administrative (G&A) expenses were $6.9 million for the quarter ended September 30, 2026 including a $1.2 million one-time net litigation settlement expense, compared to $6.2 million for the comparable period in 2025.

About MGX-001

MGX-001 is an investigational, one-time genome editing treatment designed to provide life-long protection from bleeding events and joint damage in adults and children with hemophilia A. MGX-001 is designed to site-specifically integrate the FVIII gene in a "safe harbor" location within an intron of the albumin gene, leveraging the strong native albumin promoter to drive endogenous FVIII expression. MGX-001 consists of an adeno-associated virus (AAV) vector that delivers the promoterless FVIII gene cassette as donor DNA, and a lipid nanoparticle (LNP) that delivers mRNA encoding the Company's proprietary Type V CRISPR nuclease, MG29-1, together with a guide RNA targeting the albumin gene. This represents a departure from prior approved gene therapy for hemophilia A that relied on episomal FVIII expression.

In preclinical studies in non-human primates (NHPs), MGX-001 demonstrated FVIII activity in the normal physiologic range, with clear dose dependency across both the AAV and LNP components, and, in an earlier study, durable FVIII activity over approximately 19 months. MGX-001 has also shown no detectable off-target editing in a series of orthogonal assays.

About Hemophilia A

Hemophilia A is the most common X-linked inherited and de novo bleeding disorder, largely affecting males, and is caused by a variety of mutations in the FVIII gene that all lead to loss of functional FVIII protein. Hemophilia A affects approximately 25,000 patients in the United States and an estimated 500,000 worldwide. Current standard-of-care treatments, including FVIII replacement therapy and bispecific antibody therapy, require lifelong, repeated dosing, are not curative and carry a risk of breakthrough bleeding.

About Metagenomi Therapeutics

Metagenomi Therapeutics, Inc. is an in vivo genome editing company capitalizing on its proprietary technologies to create potentially curative genetic medicines for patients. The Company was founded on the science of metagenomics, the study of genetic materials recovered from the natural environment, to discover and develop a suite of novel CRISPR gene-editing tools potentially capable of correcting any type of genetic mutation found anywhere in the human genome. The Company focuses on high value programs in disease indications with well-understood biology and clearly defined clinical development and regulatory pathways. Going forward, the Company intends to continue to expand its pipeline by leveraging its proprietary genetic editing capabilities in site-specific deletion, insertion and correction.

MGX-001, the Company's lead, wholly owned development program in hemophilia A, has demonstrated a preclinical profile with best-in-class treatment potential, including targeted genome editing and durable gene expression in a one-time treatment. The FDA has cleared the Company's Investigational New Drug (IND) application for MGX-001, and the Company is advancing MGX-001 into the clinic in its global Phase 1/2 INTEGR8 trial. MGX-001 is designed to provide curative, life-long protection from bleeding events and joint damage in adults and children, potentially enabling a new standard of care for the treatment of hemophilia A. The Company is also currently pursuing indications leveraging the same site-specific large gene integration at the albumin locus as used in MGX-001 and partnered assets targeting cardiometabolic diseases. For more information, please visit https://metagenomi.co/.

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 product development programs, including the timing of and our ability to make regulatory filings such as INDs, the design and timing of the INTEGR8 trial and initial clinical data, expectations regarding MGX-001 including the preclinical profile with best-in-class treatment potential, statements regarding the Company's plans to prioritize its preclinical pipeline and potential for value creation and sustainable growth, statements regarding upcoming milestones, statements concerning the potential of therapies and product candidates, statements concerning the impact of the organizational restructuring, 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 IND submissions and starting and completing clinical trials; the risk that the FDA or other regulatory authorities may place a clinical hold on our clinical trials or that initiation of our clinical trials may be delayed; 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 and the current regulatory environment; patent and intellectual property matters; competition; the volatility of capital markets and other adverse macroeconomic factors; as well as other risks described in "Risk Factors," in our most recent Form 10-K and Form 10-Q 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.

Investor Contact:
Brian Ritchie
LifeSci Advisors
britchie@lifesciadvisors.com

Media Contact:
Michael Fitzhugh
LifeSci Communications
mfitzhugh@lifescicomms.com

Condensed Financial Statements
Condensed Balance Sheet Data
(Unaudited)

(In thousands)	September 30, 2026	December 31, 2025
Cash, cash equivalents and available-for-sale marketable securities	$ 101,674	$ 160,799
Total assets	$ 157,388	$ 221,103
Total liabilities	$ 64,200	$ 62,507
Total stockholders' equity	$ 93,188	$ 158,596
Total liabilities and stockholders' equity	$ 157,388	$ 221,103

Condensed Statements of Operations
(Unaudited)

	Three Months Ended September 30,	Nine Months Ended September 30,

(In thousands, except share and per share data)	2026	2025	2026	2025
Collaboration revenue	$ 2,184	$ 8,659	$ 3,175	$ 21,299
Operating expenses:				
Research and development	17,532	25,283	59,344	72,932
General and administrative	6,878	6,215	19,417	20,013
Total operating expenses	24,410	31,498	78,761	92,945
Loss from operations	(22,226)	(22,839)	(75,586)	(71,646)
Other income (expense):				
Interest income	1,047	2,214	3,842	7,586
Change in fair value of long-term investments	—	—	—	(1,292)
Other income (expense), net	53	(2)	87	(80)
Total other income (expense), net	1,100	2,212	3,929	6,214
Net loss before provision for income taxes	(21,126)	(20,627)	(71,657)	(65,432)
Benefit from (provision for) income taxes	—	234	(10)	92
Net loss	$ (21,126)	$ (20,393)	$ (71,667)	$ (65,340)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.56)	$ (0.55)	$ (1.90)	$ (1.76)
Weighted average common shares outstanding, basic and diluted	37,775,051	37,349,449	37,672,176	37,176,361