



PepGen Reports Fourth Quarter and Year-End 2024 Financial Results and Recent Corporate Highlights

February 24, 2025

– The Company today reported positive initial results from the ongoing FREEDOM-DM1 trial in patients with DM1; mean splicing correction of 12.3% and 29.1% in 5 and 10 mg/kg cohorts, respectively –

– FREEDOM-DM1 15 mg/kg cohort is dosing, with results expected in second half of 2025 –

– CONNECT1-EDO51 10 mg/kg cohort is fully enrolled, with results expected in third quarter of 2025 –

BOSTON--(BUSINESS WIRE)--Feb. 24, 2025-- PepGen Inc. (Nasdaq: PEPG), a clinical-stage biotechnology company advancing the next generation of oligonucleotide therapies with the goal of transforming the treatment of severe neuromuscular and neurological diseases, today reported financial results for the quarter and year-ended December 31, 2024, and recent corporate highlights.

“Today, we reported initial results from FREEDOM-DM1, which showed robust splicing correction in patients with DM1 following a single dose of PGN-EDODM1. We believe these data contribute to the growing evidence of our novel EDO platform’s potential to deliver the drug to the nucleus, the site of action,” said James McArthur, PhD, President and CEO of PepGen. “We remain dedicated to our mission of developing life-changing therapeutics for patients with neuromuscular diseases and are committed to demonstrating the ability of our investigational candidates to address the root cause of disease for improved patient outcomes. We have numerous key data milestones expected this year and look forward to announcing clinical results from the 15 mg/kg cohort of FREEDOM and 10 mg/kg cohort of CONNECT1-EDO51.”

Recent Program Updates

PGN-EDODM1: Myotonic Dystrophy Type 1 (DM1)

- **Phase 1 FREEDOM-DM1 Single Ascending Dose (SAD) Randomized, Placebo-Controlled Clinical Trial of PGN-EDODM1:**
 - The Company today reported positive initial clinical data, including mean splicing correction of 12.3% and 29.1% from evaluable participants in the 5 mg/kg (n=6) and 10 mg/kg (n=4)¹ dose cohorts, respectively, and a favorable emerging safety profile in the ongoing FREEDOM study. The Company issued a separate press release this morning announcing these initial results and will host a [webcast](#) with a live question and answer session today at 8:00 a.m. ET.
 - The Company expects to report results from the 15 mg/kg cohort in the second half of 2025.
- **Phase 2 FREEDOM2-DM1 Multiple Ascending Dose (MAD) Randomized, Placebo-Controlled Clinical Trial of PGN-EDODM1:**
 - The Company has opened the FREEDOM2 study and has started dosing participants in the 5 mg/kg dose cohort. The Company expects to report results from the 5 mg/kg cohort in the first quarter of 2026.

PGN-EDO51: Duchenne Muscular Dystrophy (DMD)

- **Phase 2 CONNECT1-EDO51 Open-Label MAD Clinical Trial of PGN-EDO51:**
 - The 10 mg/kg cohort is fully enrolled (n=4) and participants in the 5 mg/kg cohort (n=3) are continuing in the long-term extension phase of the study. The Company expects to report clinical data from the 10 mg/kg cohort in the third quarter of 2025.
 - As previously reported on January 29, 2025, Health Canada has allowed continued dosing of participants in the 5 and 10 mg/kg cohorts with additional information requested to address its safety concerns before further dose escalation or enrollment of any additional participants at the current dose levels. The Company is working with Health Canada to address its questions.

- **Phase 2 CONNECT2-EDO51 MAD Clinical Trial of PGN-EDO51:**

- CONNECT2 is open in the United Kingdom. As previously reported, the Company received a clinical hold notice from the U.S. Food and Drug Administration (FDA) in December 2024 regarding its investigational new drug (IND) application to initiate the CONNECT2 study in the U.S. and is working to address its questions regarding supportive data for the dosing levels planned for the patient population.

Financial Results for the Three and Twelve Months Ended December 31, 2024

- **Cash, Cash Equivalents and Marketable Securities** were \$120.2 million as of December 31, 2024, which is anticipated to fund currently planned operations into 2026.
- **Research and Development Expenses** were \$19.0 million for the three months ended December 31, 2024, compared to \$16.3 million for the same period in 2023. Research and development expenses were \$76.5 million for the year ended December 31, 2024, compared to \$68.1 million for the same period in 2023.
- **General and Administrative Expenses** were \$5.4 million for the three months ended December 31, 2024, compared to \$4.5 million for the same period in 2023. General and administrative expenses were \$21.3 million for the year ended December 31, 2024, compared to \$16.6 million for the same period in 2023.
- **Net Loss** was \$22.2 million, or \$(0.68) basic and diluted net loss per share, for the three months ended December 31, 2024, compared to \$19.5 million, or \$(0.82) basic and diluted net loss per share, for the same period in 2023. Net loss was \$90.0 million, or \$(2.85) basic and diluted net loss per share, for the year ended December 31, 2024, compared to \$78.6 million, or \$(3.30) basic and diluted net loss per share, for the same period in 2023. PepGen had approximately 32.6 million shares outstanding on December 31, 2024.

About PGN-EDODM1

PGN-EDODM1, PepGen's investigational candidate in development for the treatment of DM1, utilizes the Company's proprietary EDO technology to deliver a therapeutic oligonucleotide that is designed to restore the normal splicing function of MBNL1, a key RNA splicing protein. PGN-EDODM1 is designed to directly address the deleterious effects of cytosine-uracil-guanine (CUG) repeat expansion in the *DMPK* transcripts which sequester MBNL1, by binding to the pathogenic CUG trinucleotide repeat expansion present in the *DMPK* transcripts, disrupting the binding between the CUG repeat expansion and MBNL1. We believe this mechanism will allow the *DMPK* transcripts to continue performing its normal function within the cell, while also liberating MBNL1 to correct downstream mis-splicing events. We believe that this innovative therapeutic approach has considerable advantages over oligonucleotide modalities that rely on knockdown or degradation of the *DMPK* transcripts. We believe that this therapeutic strategy positions us to potentially provide clinically meaningful benefits for DM1 patients while mitigating the risk of potential deleterious outcomes. The FDA has granted PGN-EDODM1 both Orphan Drug and Fast Track Designations for the treatment of patients with DM1.

About PGN-EDO51

PGN-EDO51, PepGen's investigational candidate in development for the treatment of DMD, utilizes the Company's proprietary EDO technology to deliver a therapeutic oligonucleotide that is designed to target the root cause of this devastating disease. PGN-EDO51 is designed to skip exon 51 of the dystrophin transcript, an established therapeutic target for approximately 13% of DMD patients, thereby aiming to restore the open reading frame and enabling the production of a truncated, yet functional dystrophin protein. The FDA has granted PGN-EDO51 both Orphan Drug and Rare Pediatric Disease Designations for the treatment of patients with DMD amenable to an exon-51 skipping approach.

About PepGen

PepGen is a clinical-stage biotechnology company advancing the next generation of oligonucleotide therapies with the goal of transforming the treatment of severe neuromuscular and neurological diseases. PepGen's EDO platform is founded on over a decade of research and development and leverages cell-penetrating peptides to improve the uptake and activity of conjugated oligonucleotide therapeutics. Using these EDO peptides, we are generating a pipeline of oligonucleotide therapeutic candidates designed to target the root cause of serious diseases.

For more information, please visit [PepGen.com](https://www.pep-gen.com). Follow PepGen on [LinkedIn](#) and [X](#).

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. These statements may be identified by words such as "aims," "anticipates," "believes," "could," "estimates," "expects," "forecasts," "goal," "intends," "may," "plans," "possible," "potential," "seeks," "will," and variations of these words or similar expressions that are intended to identify forward-looking statements. Any such statements in this press release that are not statements of historical fact may be deemed to be forward-looking statements. These forward-looking statements include, without limitation, statements regarding the therapeutic potential and safety profile of our product candidates, including, based on early data, PGN-EDO51 and PGN-EDODM1, the potential of our EDO platform to deliver higher levels of oligonucleotide to the nuclei, the design, initiation and conduct of clinical trials, including expected timelines for our CONNECT1 and CONNECT2 Phase 2 trials, our FREEDOM Phase 1 trial and our FREEDOM2 Phase 2 trial, the expected timing for additional data reports from our CONNECT1

Phase 2 trial, and our FREEDOM Phase 1 trial, ongoing and planned regulatory interactions, and our financial resources and expected cash runway.

Any forward-looking statements in this press release are based on current expectations, estimates and projections only as of the date of this release and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to risks related to: delays or failure to successfully initiate or complete our ongoing and planned development activities for our product candidates, including PGN-EDO51 and PGN-EDODM1; our ability to enroll patients in our clinical trials, including CONNECT1, CONNECT2, FREEDOM and FREEDOM2; that our interpretation of clinical and preclinical study results may be incorrect, or that we may not observe the levels of therapeutic activity in clinical testing that we anticipate based on prior clinical or preclinical results, including for PGN-EDO51 and PGN-EDODM1; our product candidates, including PGN-EDO51 and PGN-EDODM1, may not be safe and effective or otherwise demonstrate safety and efficacy in our clinical trials; adverse outcomes from our regulatory interactions, including delays in regulatory review, clearance to proceed or approval by regulatory authorities with respect to our programs, including clearance to commence planned clinical studies of our product candidates, or other regulatory feedback requiring modifications to our development programs, including in each case with respect to our CONNECT1, CONNECT2, FREEDOM and FREEDOM2 clinical trials; changes in regulatory framework that are out of our control; unexpected increases in the expenses associated with our development activities or other events that adversely impact our financial resources and cash runway; and our dependence on third parties for some or all aspects of our product manufacturing, research and preclinical and clinical testing. Additional risks concerning PepGen's programs and operations are described in our most recent annual report on Form 10-K and quarterly report on Form 10-Q that are filed with the SEC. PepGen explicitly disclaims any obligation to update any forward-looking statements except to the extent required by law.

This release discusses PGN-EDO51 and PGN-EDODM1, investigational therapies that have not been approved for use in any country and is not intended to convey conclusions about their efficacy or safety. There is no guarantee that PGN-EDO51, PGN-EDODM1 or any other investigational therapy will successfully complete clinical development or gain regulatory authority approval.

Condensed Consolidated Statements of Operations
(unaudited, in thousands)

	Twelve Months Ended December 31,		Three Months Ended December 31,	
	2024	2023	2024	2023
Operating expenses:				
Research and development	\$ 76,478	\$68,126	\$18,961	\$ 16,300
General and administrative	21,261	16,640	5,384	4,511
Total operating expenses	<u>\$ 97,739</u>	<u>\$84,766</u>	<u>\$24,345</u>	<u>\$ 20,811</u>
Operating loss	<u>\$(97,379)</u>	<u>\$(84,766)</u>	<u>\$(24,345)</u>	<u>\$(20,811)</u>
Other income (expense)				
Interest income	7,142	6,400	1,460	1,346
Other income (expense), net	(1)	(187)	26	43
Total other income, net	<u>7,141</u>	<u>6,213</u>	<u>1,486</u>	<u>1,389</u>
Net loss before income tax	<u>\$(90,598)</u>	<u>\$(78,553)</u>	<u>\$(22,859)</u>	<u>\$(19,422)</u>
Income tax (expense) benefit	617	(73)	617	(73)
Net loss	<u>\$(89,981)</u>	<u>\$(78,626)</u>	<u>\$(22,242)</u>	<u>\$(19,495)</u>
Net loss per share, basic and diluted	<u>\$(2.85)</u>	<u>\$(3.30)</u>	<u>\$(0.68)</u>	<u>\$(0.82)</u>
Weighted-average common shares outstanding, basic and diluted	<u>31,583,073</u>	<u>23,796,000</u>	<u>32,602,981</u>	<u>23,816,919</u>

Condensed Consolidated Balance Sheets
(unaudited, in thousands)

	December 31,	
	2024	2023
Assets		
Cash, cash equivalents and marketable securities	\$ 120,191	\$ 110,407
Other assets	30,692	32,645
Total assets	<u>\$ 150,883</u>	<u>\$ 143,052</u>
Liabilities and stockholders' equity		
Liabilities	\$ 32,263	\$ 34,631
Stockholders' equity	118,620	108,421
Total liabilities and stockholders' equity	<u>\$ 150,883</u>	<u>\$ 143,052</u>

1. In the 10 mg/kg cohort, one participant's biopsy was not collected at day 28 due to pseudoaneurysm in connection with the biopsy procedure and one participant's sample showed a splicing index outside the pre-specified assay range at both baseline and day 28 (no detectable mis-splicing) and was excluded from the analysis.

Investor

Dave Borah, CFA

SVP, Investor Relations and Corporate Communications

dborah@pepgen.com

Media

Julia Deutsch

Lyra Strategic Advisory

jdeutsch@lyraadvisory.com

Source: PepGen Inc.