PepGen Announces Clinical Hold in the U.S. on IND Application to Initiate CONNECT2-EDO51 Phase 2 Study of PGN-EDO51 for Duchenne Muscular Dystrophy

December 16, 2024

-Company continues to advance PGN-EDO51 in CONNECT1-EDO51, with the 10 mg/kg cohort now fully enrolled-

BOSTON--(BUSINESS WIRE)--Dec. 16, 2024-- 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 announced that the Company received a clinical hold notice from the U.S. Food and Drug Administration (FDA) regarding its Investigational New Drug (IND) application to initiate the CONNECT2-EDO51 clinical trial in patients with Duchenne muscular dystrophy (DMD). The FDA indicated they will provide an official clinical hold letter to the Company within 30 days.

CONNECT2 is PepGen's Phase 2 multinational, double-blind placebo-controlled, multiple ascending dose, 25-week clinical trial of PGN-EDO51 in patients with DMD. The study is open in the United Kingdom.

"We intend to work closely with the FDA to address their questions on our application to initiate CONNECT2 as expeditiously as possible," said Paul Streck, MD, MBA, Head of R&D of PepGen. "Our open-label CONNECT1-EDO51 multiple ascending dose study of PGN-EDO51 in boys and young men living with DMD continues as planned in Canada. We have completed enrollment of the 10 mg/kg dose cohort; all four patients in this cohort have received at least one dose."

About PGN-EDO51

PGN-EDO51, PepGen's clinical candidate for the treatment of DMD, utilizes the Company's proprietary Enhanced Delivery Oligonucleotide (EDO) technology to deliver a therapeutic phosphorodiamidate morpholino oligomer (PMO) 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 Enhanced Delivery Oligonucleotide (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. 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 expected interactions with the FDA regarding our IND for the CONNECT2-EDO51 study of PGN-EDO51 and enrollment in the ongoing CONNECT1-EDO51 study.

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; our ability to enroll patients in our clinical trials, including CONNECT1 and CONNECT2; 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; our product candidates, including PGN-EDO51, 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 and CONNECT2 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, an investigational therapy that has 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 or any other investigational therapy will successfully complete clinical development or gain regulatory authority approval.

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