Press release

# PEP-Therapy receives FDA Orphan Drug Designation for PEP-010 for treatment of Pancreatic Cancer

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**Paris (France), March 17, 2025 – PEP-Therapy, a clinical-stage biotechnology company developing first-in-class peptides as targeted therapies in oncology, announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug designation to its flagship product, PEP-010, for the treatment of pancreatic cancer.**

PEP-010, PEP-Therapy's first-in-class bifunctional therapeutic peptide is being studied in an ongoing Phase Ib clinical trial evaluating safety, tolerability, pharmacokinetics and preliminary anti-tumor activity, in combination with chemotherapy (paclitaxel or gemcitabine), in particular in patients with pancreatic cancer.

"Obtaining Orphan Drug Designation for PEP-010 marks a significant milestone in our efforts to develop our drug candidate for pancreatic cancer and highlights the significant unmet medical need that exists for these patients" said **Hatem Azim, MD, PhD, Chief Medical Officer of PEP-Therapy**. "They currently have limited treatment options, and the growing incidence and mortality of pancreatic cancer underscores the urgency for new therapies. ODD along with encouraging initial data from our ongoing Phase I study strengthen the advancement of PEP-010 as a potential novel alternative."

**Antoine Prestat, CEO and co-founder of PEP-Therapy**, added: "Receiving FDA's ODD is an important milestone for PEP-Therapy. It will support our objective to accelerate the development of PEP-010 toward delivering an innovative solution for challenging-to-treat cancers. We look forward to reporting updated clinical data."

The FDA Office of Orphan Products Development grants Orphan Drug Designation to an investigational drug or biological product intended to prevent, diagnose or treat a rare disease or condition that affects less than 200,000 people in the United States. The designation provides development and commercial incentives such as tax credits for qualified clinical trials, exemptions from significant regulatory fees and the potential for up to seven years of market exclusivity following drug approval.

PEP-Therapy will be present at BioSpring from March 17 to 19, where Antoine Prestat will be available to discuss the company's latest advancements.

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**About PEP-Therapy**

PEP-Therapy is a Paris-based clinical-stage biotechnology company developing first-in-class peptides as targeted therapies in oncology. PEP-010, lead drug candidate, is a pro-apoptotic agent currently evaluated in a Phase Ib clinical trial. The company also develops a pipeline of peptide-based products in oncology.
Founded in 2014, PEP-Therapy builds on research from Sorbonne University and Institut Curie, and is backed by international investors: Seventure Partners (Quadrivium 1 Seed Fund), CapHorn, i&i Prague, Italian Angels for Growth (IAG), Doorway, Magna Capital Partners (MCP), Business Angels des Grandes Ecoles (BADGE),

and Jérôme Majoie (former Managing Director of Laboratoires Fournier affiliates (USA, UK, Sweden, Japan), CEO of La Fondation Fournier-Majoie).
For more information, please visit www.pep-therapy.com.

**About PEP-010**

PEP-010 is a first-in-class bifunctional peptide that penetrates cells and specifically disrupts the interaction between Caspase-9 and PP2A, two key proteins involved in the apoptotic pathway. PP2A and Caspase-9, when released, restore normal apoptosis in cancer cells.
PEP-010 is a pro-apoptotic agent which has demonstrated a good safety and tolerability profile and first signals of efficacy in the CLEVer-PEPtide Phase Ia dose escalation clinical trial. It is currently evaluated in a Phase Ib clinical trial in patients with metastatic pancreatic ductal adenocarcinoma and advanced or metastatic ovarian cancer. Four sites in France are currently recruiting: Institut Curie, Gustave Roussy, Centre François Baclesse and Institut de Cancérologie de l'Ouest.

**About Pancreatic Cancer in the U.S.**

Pancreatic cancer is the fourth leading cause of cancer-related death in the United States, with around 66,000 people diagnosed and 51,000 people dying every year. Unlike many other cancers, mortality rates are rising, with cancer-related death projected to rise by almost 80% from 2018 to 2040.
Surgical resection is the only potentially curative technique. However, more than 80% of patients present with disease that is not amenable to resection. Even after primary resection and adjuvant therapy, median survival ranges from 3-4 years. In advanced or metastatic patients, outcome is dismal with only 3% of patients surviving their disease, and median survival hardly reaching the 1-year mark.
Systemic management of advanced pancreatic cancer has witnessed very limited progress over the past 2 to 3 decades. The introduction of novel class of agents has shown limited promise in pancreatic cancer, albeit could be beneficial in very small subsets of patients. Thus, chemotherapeutic agents remain the main stay of treatment, underscoring the importance of identifying novel treatment options that could improve survival without compromising quality of life.

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