PRESS RELEASE

APEPTICO announces top-line results in phase IIa clinical study of AP301 in treatment of pulmonary permeability oedema in mechanically ventilated patients

Vienna, Austria, April 9th, 2014: APEPTICO, a privately held biotechnology company developing peptide drugs, today announced that the phase IIa clinical study of AP301 delivered top-line results in the treatment of pulmonary permeability oedema in mechanically ventilated patients suffering from Acute Respiratory Distress Syndrome.

Pulmonary oedema occurs when fluid leaks from the pulmonary capillary network into the lung interstitium and alveoli. There are many possible causes of lung oedema, such as sepsis, trauma, pneumonia, aspiration, cardiac failure, and other. Massive pulmonary permeability oedema is a major characteristic of Acute Respiratory Distress Syndrome (ARDS) too, a life-threatening condition having a mortality rate of around 35-45%, despite modern day care. Currently, there is no effective pharmacotherapy available for treatment of pulmonary permeability oedema and patients having ARDS.

AP301 is a small peptide designed to activate pulmonary oedema clearance in a variety of patients, including mechanically ventilated patients. AP301 opens up the pulmonary epithelial sodium ion channel (ENaC), blunts protein kinase C-α activation and MLC phosphorylation, and reduces reactive oxygen species generation in lung tissue. All this leads to lung tissue repair and pulmonary oedema clearance.

The proof-of-concept phase IIa clinical study was conducted at the Division of General Anaesthesia and Intensive Care Medicine of the Medical University of Vienna. It was an interventional, randomized, double blind, placebo-controlled, parallel-group study. Patients were randomized in a 1:1 ratio. The primary objective of this study was to assess the treatment-associated changes of extra-vascular lung water (EVLW) upon oral inhalation of AP301 in comparison to placebo. Patients were evaluated every 12 hours for 7 days.

Results from this study showed that oral inhalation of AP301 led to an earlier onset and more pronounced activation of pulmonary oedema clearance compared to placebo. Subgroup analysis revealed that oral inhalation of AP301 was statistically significant and more effective in pulmonary oedema clearance in patients with elevated Sequential Organ Failure Assessment (SOFA) score compared to placebo. AP301 was equally effective in patients with direct and indirect lung injury and patients with initial very low P/F-ratio. In addition to oedema clearance, upon AP301 inhalation, critical patient's parameters, such as oxygenation index and Murray Lung Injury score, improved.

Dr. Bernhard Fischer, CEO of APEPTICO, stated: "We are very proud to have achieved this significant clinical goal. I am convinced that AP301 will have the potential to play a key role in the management of various forms of pulmonary oedema. This major success would not have happened without the steady support by Professor Rudolf Lucas from the Medical College of Georgia, Professor Rosa Lemmens-Gruber from the Institute of Pharmacology and Toxicology of the University of Vienna, and the clinical teams of Professor Roman Ullrich and Professor Klaus Markstaller from the Division of General Anaesthesia and Intensive Care Medicine of the Medical University Vienna". "Our excellent scientific results will establish partnering process with interested global and specialised pharmaceutical and biotech companies," Dr. Fischer added.

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About APEPTICO GmbH (www.apeptico.com)

APEPTICO is a privately-held biotechnology company based in Austria, developing peptide-based products targeting chronic and life-threatening diseases. The peptide molecules correspond to validated, pharmacodynamic active structures and domains of well-known proteins and biopharmaceuticals. By concentrating on synthetically produced protein structures APEPTICO avoids general risks associated with gene- and cell-technologies. APEPTICO makes use of its technology platforms PEPBASE$^{(TM)}$ and PEPSCREEN$^{(TM)}$ to significantly reduce cost and to shorten time to market.

About the AP301 peptide family

AP301 and derived peptides are synthetic molecules whose structures are based on structural elements of human proteins. AP301 peptide is water soluble and can be administered into the lung by oral inhalation. Formulated AP301 is easily nebulised and the resulting aerosol is composed of peptide/water droplets of diameter 4 μm or less. AP301 and derived peptides are designed for activation of the pulmonary epithelial sodium channel (ENaC). Activation of ENaC by AP301 results an accelerated lung oedema clearance in the airspace.

Comprehensive research and development conducted by APEPTICO has demonstrated that AP301 peptides are effective in various forms of pulmonary oedema, such as pulmonary permeability oedema, hydrostatic oedema, high altitude pulmonary oedema (HAPE), pulmonary oedema associated with acute lung injury (ALI) and acute respiratory distress syndrome (ARDS), pulmonary oedema resulting from pneumonia, sepsis and lung transplantation (primary graft dysfunction).

APEPTICO's AP301 has been granted orphan drug status (i) for treatment of pulmonary permeability oedema in ALI/ARDS, (iii) for treatment of primary graft dysfunction following lung, and (iii) for treatment of high altitude pulmonary oedema by the European Commission and European Medicines Agency (EMA) and by the Food and Drug Agency (FDA).

About pulmonary oedema

Pulmonary oedema occurs when fluid leaks from the pulmonary capillary network into the lung interstitium and alveoli. There are many possible causes of lung oedema, such as heart failure (cardiac/hydrostatic lung oedema); inhaling high concentrations of smoke, toxins, or oxygen; severe burns; blood infections / sepsis; infection of the lung / pneumonia; aspirations, cerebral damage or trauma to other parts of the body and lung transplantation. Lungs contain alveoli, which are tiny air sacs where the oxygen is passed into the blood. During lung oedema, blood and fluid begin to leak into the alveoli. When this happens, oxygen cannot enter the alveoli, which means oxygen no longer passes into the blood. Because the lungs are inflamed and filled with fluid, the patient finds it increasingly difficult to breathe. The mortality rate of patients with pulmonary oedema in ALI/ARDS is 35% to 45% within two to four weeks. Currently, no specific drug treatment exists for patients suffering from pulmonary permeability oedema and patients having ARDS. ARDS is also a major economic burden to hospitals and health care budgets. It is estimated that due to a long ICU and hospital stay the cost of every saved live from ARDS is approximately $70,000 USD.

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