

PRESS RELEASE by APEPTICO

Vienna, Austria, January 2nd 2015

Vienna, Austria, 2nd January 2015: APEPTICO, a privately-held biotechnology company developing synthetic protein structures, today announced that it has been awarded a research grant by the Wellcome Trust under the Pathfinder Award scheme to further explore the role of APEPTICO's compound 'Enaritide' for the treatment of Pseudohypoaldosteronism type 1b (PHA type 1b).

Based on its discovery work on interactions of the pulmonary epithelial sodium ion channel (ENaC) and various pharmacodynamically active protein structures, APEPTICO has been encouraged by the European Medicines Agency (EMA, London) to apply for a Pathfinder Award of the Wellcome Trust. The Wellcome Trust is an internationally leading charitable foundation dedicated to achieving extraordinary improvements in health by supporting the brightest minds. The Pathfinder Scheme offers pilot funding for discrete projects from partnerships between academia and industry to catalyse innovative early-stage applied research and development projects in areas of unmet medical need.

APEPTICO's Pathfinder Award "Effect of a synthetic peptide on PHA type 1b causing mutations in the amiloride-sensitive epithelial sodium channel (ENaC)" addresses the orphan disease Pseudohypoaldosteronism type 1b (PHA type 1b), a life-threatening condition in which the sodium ion channel, ENaC, found in kidneys, colon, lungs, salivary and sweat glands has either reduced or no functionality.

APEPTICO successfully develops various synthetic protein structures for treatment of various unmet pulmonary diseases. Since it was founded in 2009, APEPTICO has become a champion in pulmonary delivery of biologic macromolecules to patients with life-threatening lung diseases.

Bernhard Fischer, CEO of APEPTICO commented: "I am very proud that the Wellcome Trust has awarded the Pathfinder Grant to APEPTICO. Based on the funding scheme, we have initiated a scientific research collaboration with the Department of Pharmacology and Toxicology of the University Vienna." "In parallel with *in vitro* testing of the Enaritide-peptide in cells heterologously expressing human ENaC carrying mutations known to cause PHA type 1b, we will develop a specific dry powder formulation of the synthetic macromolecule, composed of micrometre small solid particles. This will enable APEPTICO to deliver the bioactive protein structure into the patient's lung via a dry powder inhaler" he added.

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To Editors

About the Wellcome Trust

The Wellcome Trust's vision is to achieve extraordinary improvements in human and animal health. In pursuit of this, the Wellcome Trust supports the brightest minds in biomedical research and the medical humanities. In 2013, the Wellcome Trust funded grants to more than 385 different organisations; more than 90 per cent of funding is awarded to organisations in the United Kingdom.

About APEPTICO GmbH

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 Pseudohypoaldosteronism type 1b

Pseudohypoaldosteronism type 1b (PHA type 1b) is a life-threatening condition in which the sodium ion channel, ENaC, found in kidneys, colon, lungs, salivary and sweat glands has either reduced or no functionality. Non-function of ENaC results in loss of sodium in the urine and faeces and severe salt imbalance in the body. Characteristic features are low levels of sodium (hyponatremia) and high levels of potassium (hyperkalemia) in the blood. The disease usually presents in newborns who fail to thrive and suffer from severe dehydration; other symptoms are abnormal heartbeat or shock due to salt imbalance and recurrent lung infections due to accumulated fluid. The condition does not improve with age and patients require life-long salt supplements and special treatment to remove potassium. If APEPTICO's synthetic protein structure activates the defective ENaC, then it could be used to treat lung ailments of PHA type 1b patients.

About the APEPTICO's synthetic protein structures

APEPTICO's molecules are synthetically manufactured structural equivalents to domains of the human Tumour Necrosis Factor α . The protein structures are water-soluble and can be administered into the lung by inhalation of liquid aerosol droplets of diameter 4 μm or less. Most recently, APEPTICO has successfully completed two phase II clinical trials with orally inhaled peptides for treatment of pulmonary permeability oedema and treatment of primary graft dysfunction following lung transplantation. APEPTICO's synthetic molecules have been granted orphan drug status various life-threatening condition by the European Medicines Agency (EMA) and by the Food and Drug Agency (FDA).

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