Press Release

Rare Diseases: Expected Benefits of a Physiological Absorption of Amino Acids in PKU management. PKU GOLIKE® by APR can play a role

Balerna (Switzerland), 6th November 2018- The recent article "Optimising amino acid absorption: essential to improve nitrogen balance and metabolic control in phenylketonuria", published on the last issue of "Nutrition Research Reviews", sheds the light on PKU unmet needs, opening up new interesting avenues in PKU dietary management.

The paper, by four renowned experts in the field of inherited metabolic diseases (Anita MacDonald, Rani H. Singh, Júlio C. Rocha and Francjan J. van Spronsen), highlights for the first time the expected important benefits for PKU patients of an amino acids (AA) formulation providing a physiological absorption profile of AA, similar to that of intact natural proteins [(1)].

The authors start from the consequences of the absorption profile of free AAs: as they are ready for absorption and bypass the digestive phase, free AAs intake is associated to a less efficient AA utilization, compared with intact protein sources, as well as excessive blood AA fluctuations.

Experts agree that **protein substitutes with the ability to prolong absorption of AAs, mimicking physiological absorption kinetics of intact natural proteins, may allow a more efficient AA utilization,** and thus contribute to support effectively normal growth and an overall healthy body composition.

Such **prolonged-release formulations could play an important role in ameliorating the dietary management of PKU** patients and provide them significant benefits. In particular, they could ideally simplify treatment by reducing the number of AA mixture administrations, and thus relieve the PKU burden. According to the conclusions of the article, positive consequences are also plausible on neurocognitive performance via a better balance in brain AA concentrations.

APR Applied Pharma Research s.a. ("APR") is confident that **PKU GOLIKE®**, the innovative Phenylalanine (Phe)-free medical food formula for Phenylketonuria (PKU), **could offer** a **new dietary treatment option to the benefit of patients and HCPs.**

Leveraging on its extensive pharma development expertise, **APR applied for the first time a pharmaceutical technology to a medical food -** the patented **Physiomimic Technology™**, which **can effectively prolong the release of the AAs and support their physiological absorption**, mimicking that of intact proteins.

As showed in pre-clinical data generated independently by APR, the kinetic profile of the AAs engineered with the Physiomimic™ Technology resembles that of casein - a reference food protein known to have a prolonged absorption profile. Recent clinical data in human subjects confirmed the same kinetic absorption pattern of AAs with PKU GOLIKE® in favor of an improved efficiency in the use of AAs.

Further pre-clinical and clinical evidences will be generated by APR in the coming future to support the benefits of AAs physiological absorption associated with PKU GOLIKE® and the patented pharmaceutical Physiomimic Technology ™ behind it. Besides PKU GOLIKE®, other formulations are currently in the pipeline to convey similar benefits to other rare inherited metabolic disorders by applying the same pharmaceutical technology platform.

(1) Optimising amino acid absorption: essential to improve nitrogen balance and metabolic control in phenylketonuria, Anita MacDonald, Rani H. Singh, Júlio César Rocha and Francjan J. van Spronsen - *Nutrition Research Reviews 10/2018*

About Phenylketonuria or PKU

Phenylketonuria or PKU is a rare, genetic, recessive metabolic disorder affecting about 50.000 people worldwide. PKU is characterized by the deficiency or the malfunctioning of a liver enzyme needed to process phenylalanine ("Phe"), an essential amino acid found in most protein-containing food. Excessive amounts of Phe in the bloodstream become toxic to the brain, impairing the normal development of the central nervous system. PKU can only be treated through a strict, life-long, low-protein (low-Phe) dietetic treatment combined with a daily assumption of low-protein modified foods and medical food, which provides Phe-free amino acids and other important nutrients, needed due to the dietary restrictions of PKU patients.

About PKU GOLIKE®

PKU GOLIKE is an innovative food for special medical purposes (FSMP) consisting of a Phe-free amino acid mixture. For the first time a pharmaceutical technology has been applied to a FSMP and the innovative PKU GOLIKE, engineered with Physiomimic technology, provides a prolonged-released of amino acids and grants a remarkable taste and odor masking while preventing aftertaste. This prolonged release leads to a physiological absorption of amino acids, similar to dietary proteins, to favor a more efficient amino acid utilization and health benefits. On the other hand, the improvement of free-amino acids' organoleptic features aims to support compliance to the nutritional management of the diet for a better quality of life.

The product line is formed by: ***PKU GOLIKE PLUS 3-16*** & ***PKU GOLIKE PLUS 16+*** with amino acids, vitamins & minerals, and ***PKU GOLIKE PURE 3+*** with only amino acids.

For more information about PKU GOLIKE® and the Physiomimic™ Technology, please visit: www.apr.ch/apr-pharma-products/medical-prescription/genetic-metabolic-disease/

About APR Applied Pharma Research s.a.

APR is a Swiss independent pharma company focused on development and commercialization of innovative, science-driven products designed to address unmet needs in niche or rare therapeutic areas on a global basis. APR combines drug development expertise with proprietary drug delivery technologies to deliver to patients suffering from rare diseases with solutions meaningfully improving their life and empowering caregivers with better ways to manage such rare diseases. APR has a balanced portfolio of revenue generating products marketed in all major markets, combined with a compelling pipeline of innovative products at different stages of development, specifically in the treatment of rare recessive metabolic disorders, rare dermatological and ocular diseases. APR's products are commercialized by APR directly through its own sale and marketing teams in selected countries of Europe as well as through a solid global network of commercial partners.

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