



Press release

PKU: APR Applied Pharma Research presents new data from a global research at the 40th SIMD Annual Meeting

Balerna (Switzerland), 12th March 2018 – APR Applied Pharma Research s.a. (“APR”), the Swiss independent developer of science driven and patent protected healthcare products, participates in the 40th edition of the **annual meeting** promoted by **SIMD (Society for Inherited Metabolic Diseases)**, which is held on **March 11th-14th, in San Diego, California**. Within the congress, APR presents the results of a global market research project on **PKU (Phenylketonuria)**, a rare, genetic, recessive **metabolic disorder**. This qualitative research has been conducted by **IDR Medical**, a leading international healthcare market research organization, in the **United States and five European Countries (France, Germany, Spain/Portugal, Italy, UK)** in order to evaluate how PKU is currently managed, with the aim of identifying areas for improvement. A total of **96 interviews** with **pediatricians, dieticians and PKU care-givers or patients** provided insights to unfold many aspects of **PKU management**, including patient flows, stakeholders’ influence and **decision criteria for medical food adoption**.

The final results and conclusions of the survey indicate that **patients’ adherence** to the currently prescribed amino acid (AA) supplements is still a **key issue** and that **it is mainly driven by taste fatigue**. **Dieticians and pediatricians** have still to make great efforts to reach a **good long-term patient compliance** to the recommended dietary regimen, which is pivotal to achieve the desired balance between adequate nutritional intake and good metabolic control with PHE levels within the ranges. These difficulties are also confirmed by patients, who highlighted how the most highly ranked aspects in terms of importance in the choice of an AA supplement are taste, good control of phenylalanine (Phe) levels and **the contribution to personal wellbeing**.

The research supports the relevance of **an innovative supplementation** addressing some of these needs and thus significantly contributing **to ameliorate PKU management**.

APR developed a patented new technology (known as Physiomimic Technology™), with the precise objective of providing PKU patients with **an innovative Phe-free AA supplement**, which can concomitantly help patients remain adherent to the prescription of amino acid supplementation as well as improve their quality of life.

In particular, **the technology is able to remarkably mask the naturally unpleasant taste and the odor of some amino acids, offering a considerably palatable medical food**, which can be easily accepted by patients, helping them to obtain a rewarding and lasting compliance to the dietary treatment, positively impacting their quality of life.



On the other side, the supplementation, by **providing amino acids in a physiological manner**, has the very encouraging potential of increasing the rate of amino acid assimilation into protein and **balancing the steep blood fluctuations typical of the free amino acids**, aspects that are being studied for their expected benefits on patients' health.

*"We are very pleased of the results of this extensive research, now validated by the poster "Phenylketonuria: not all problems are solved", as they confirm product palatability as one of the main open issues in the management of PKU, resulting in poor long-term compliance. - states **Paolo Galfetti**, CEO of Applied Pharma Research-. We are confident that APR's innovative Medical Food will deliver tangible and significant benefits to PKU patients, by making easier to accept life-long daily dietary restrictions and thus contributing to their wellbeing."*

Further results and advancements will be presented by APR at the **56th Annual Symposium of the SSIEM (Society for the Study of Inborn Errors of Metabolism)** on **September 4th-7th** in **Athens**, which will be a great occasion for clinicians to get to know APR innovation and the patented technology behind it.

About APR Applied Pharma Research s.a.

APR is a Swiss, independent developer of science driven, patent protected Healthcare products. The Company identifies, develops and licenses science driven, value added products designed to address patient or consumer needs in selected therapeutic areas on a global basis. In particular, APR is currently focused on 2 (two) areas: (i) internally developed and financed (alone or together with our co-development partners) proprietary, value added products to be licensed to healthcare companies for their commercialization, and (ii) support to third party projects by offering added value R&D services under contract and fee for service arrangements. APR has a balanced pipeline of revenue generating branded products marketed in all major markets combined with a compelling pipeline of products at different stage of development. APR has entered into licensing and partnership agreements with pharmaceutical companies, in over 70 countries with international sales on a worldwide basis.

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. If not properly treated, PKU leads to severe, non-recoverable mental retardation and major cognitive disabilities. 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.

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

For press releases and other company information visit: www.apr.ch

Contacts:

APR Applied Pharma Research s.a.,
Paolo Galfetti; CEO
T: +41 91 6957020 or email to paolo.galfetti@apr.ch

Havas PR Milan - Press Office
Sara Bernabovi
T: +39 02 85457032 or email to sara.bernabovi@havaspr.com