



Press release

A global market research shows relevant unmet needs in Phenylketonuria management: APR is ready to take up the challenge

Balerna (Switzerland), 31st May, 2017 – APR Applied Pharma Research s.a. (“APR”), the Swiss independent developer of science driven and patent protected healthcare products, announces today the preliminary results of a **global market research project** commissioned to **IDR Medical**, a leading international healthcare market research organization. The study gathered insights from over 100 interviews conducted in seven countries: U.S.A., U.K., Germany, France, Italy, Spain and Portugal. Main objective of the research was to validate the most relevant **drivers for decisions** and **unmet needs** of Phenylketonuria (PKU) patients, families and healthcare professionals. The results highlighted that **dietary compliance** is still a key issue in PKU management. The survey showed that **physicians and dieticians’ concerns** mainly focus on securing and supporting patient adherence to the prescribed dietary regimen, thus achieving a **balance between nutritional intake and metabolic management** and allowing **an appropriate control of PHE levels**. Most of the patients and HCPs reported that palatability (taste and odor) of aminoacids (AA) formulations still represents a significant obstacle to obtain better and long-term patient compliance to the dietary treatment as well as it has a significant impact on quality of life.

“As part of our patient-centric approach, we start all our research and development projects deeply analysing if there are patients and/or healthcare professionals’ needs still unmet. – states Paolo Galfetti, CEO of Applied Pharma Research – IDR Medical market research confirmed APR’s intuitions and perspectives in the development of a new medical food designed to obtain, through the application of an innovative pharmaceutical technology, patients’ acceptance of daily dietary restrictions. We are confident that APR’s innovative Medical Food will make the difference in PKU patients’ life, enabling them to exploit their current and future potential.”

PKU is a rare, **genetic, recessive metabolic disorder** affecting about 50’000 people worldwide (on average 1:10’000 new born) and characterized by the lack or the malfunctioning of a liver enzyme needed to process phenylalanine (“PHE”), an essential amino acid. There is no cure for PKU, it can only be treated through a stringent, life-long, low-protein (low-PHE) dietetic treatment combined with a daily assumption of Medical Foods which provide PHE-free amino acids and other important micronutrients, needed due to the dietary restrictions of PKU patients. The ideal management of a PKU dietary regimen aims at maintaining the best possible balance between nutritional intake and metabolic management, assuring compliance through a strict low protein diet and an AA mix supplementation.



APR developed a patented **new technology** to provide PKU patients with an **innovative PHE-free Medical Food**, engineered to allow a **physiological absorption of amino acids**, mimicking that of dietary proteins. This advanced formulation, has the potential to contribute to the **maintenance of PHE levels within the recommended ranges**, with less prominent fluctuations of PHE levels over time, thus meeting one of the most significant healthcare professionals' concerns. The applied patented technology, on the other hand, is able to mask taste and odor of free amino acids, by creating a product that can be easily accepted by patients in order to obtain a better and lasting compliance to the dietary treatment, thus fulfilling one of the first unmet needs of PKU patients.

APR will take part as sponsor in some forthcoming international events on inherited metabolic disorders. During the 13th ICIEM (International Congress of Inborn Errors of Metabolism) in Rio de Janeiro (September 5th-8th) scientific data about the patented technology at the basis of this innovation will be presented. On October, 5th-8th 2017, the 31st E.S. PKU Conference (European Society for Phenylketonuria and Allied Disorders Treated as Phenylketonuria Conference), which will be held in Hell (Norway) will be a great occasion to deepen APR familiarity with patients' needs and clinicians' expectations.

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.

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

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