

Press release

APR Applied Pharma Research wants to make the difference for PKU people thanks to an innovative and patented technology

Balerna (Switzerland), 6th April, 2017 – APR Applied Pharma Research s.a. ("APR"), the Swiss independent developer of science driven and patent protected healthcare products, announces today to enter the inherited metabolic disorders therapeutic area by developing an **innovative food for special medical purposes (Medical Food)**, intended to offer a new treatment option to **Phenylketonuria (PKU) patients, families and Healthcare Professionals (HCPs)**.

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. Excessive amounts of PHE in the bloodstream are mirrored in 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 impairments. There is no cure for PKU, it can only be treated through a strict, life-long, low-protein (low-PHE) dietetic treatment combined with a daily assumption of Medical Food which provides PHE-free amino acids and other important nutrients, needed due to the dietary restrictions of PKU patients.

APR developed and patented a **technology** to provide PKU patients with an **innovative PHEfree Medical Food**, engineered to allow a **physiological absorption of amino acids**, mimicking that of dietary proteins. This advanced formulation has the potential of contributing to **maintain PHE levels within the recommended ranges**, with less prominent fluctuations of PHE levels over time. Furthermore, with the applied patented technology, the product results odor, taste and aftertaste free, for a better and lasting patient compliance to the dietary treatment, fulfilling one of the needs of PKU patients.

"Consistently with our mission, with this patented technology we mean to set the pace for PKU people, who, in addition to specific nutritional needs, have to deal also with the clinical implications of a life-long treatment and dietary restrictions. Having this in mind, we leveraged our extensive know-how in pharmaceutical technologies to develop an innovative medical food, which we are confident will deliver tangible and significant benefits to PKU patients, enabling them to completely exploit their current and future potential. Our dream is to make a real difference for those who live and deal with PKU" – Paolo Galfetti, CEO of Applied Pharma Research, states.



Scientific literature and clinical experience strongly recommend to PKU patients a strict dietetic treatment in order to control PHE levels in the blood, thus maintaining the plasma PHE levels within the appropriate range, that vary according to different stages of children growth. Dietary compliance is still a key issue in PKU management; moreover, despite the diet, some neurological or psychosocial issues may occur.

"Starting from these scientific and clinical evidences we began our research and for the first time, the application of a pharmaceutical technology to a Medical Food brings a real change in the spectrum of the amino acid mixes, currently the main tool able to support PKU patients in their life-long necessity of controlling blood PHE levels. As a result, our solution, which aims at allowing patients to absorb a nutritionally adequate mix of amino acids in a more physiological manner, has the potential to modulate those unwanted – and sometimes hidden – disabilities expressed by some PKU patients, with plausible benefits on the quality of their lives." Giorgio Reiner, Corporate Director R&D of Applied Pharma Research, states.

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: <u>www.apr.ch</u>

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