Press Release

FDA grants Orphan Drug Designation to APR Applied Pharma Research's Investigational Drug for the Treatment of Epidermolysis Bullosa

Balerna, 4th December 2019 – APR Applied Pharma Research sa ("APR"), the Swiss pharma company focused in niche and rare therapeutic areas, announces that the US Food and Drug Administration (the "FDA") has granted Orphan Drug Designation for its investigational drug code-named APR-TD011 for treatment of Epidermolysis Bullosa ("EB").

APR-TD011 is a hypotonic acid-oxidizing solution containing hypochlorous acid in a sprayable form for topical application, specifically developed for EB. Thanks to its unique physico-chemical profile, APR-TD011 could be able to prevent or reduce infections as well as inflammation through modulation of the wound microenvironment, thus accelerating the physiological wound healing.

"The granting of Orphan Drug Designation in the US highlights the significant need for a drug that could improve the treatment of EB, a debilitating disease that affects the life of half a million people in the US. We believe that APR-TD011 holds great promises for EB patients and their families and the Orphan status will help APR to find expedited pathways to fruition" said Paolo Galfetti, CEO of APR. "This designation is also an important milestone and step forward in APR's evolution as we advance our pipeline targeting rare diseases."

EB market is estimated to range between $ 1,0 - 1.3 billion worldwide. EB imposes a major burden for global health care: the cost of treatment of a severely affected patients in the United States can exceed $300,000 per year, whereas wound care supplies for those with Dystrophic EB could require more than $10,000 per month.

The Orphan Drug Designation program provides incentives and support for the development of drugs for patients with rare diseases, specifically to drugs that are intended for the treatment of diseases or conditions that affect fewer than 200,000 people in the United States. Orphan Drug Designation provides certain benefits, including seven years of market exclusivity upon regulatory approval for the designated indication, exemption from FDA application user fees, FDA assistance in clinical trial design as well as tax credits (up to 25%) for qualified clinical trial expenses.

About APR-TD011

APR-TD011 is a hypotonic acidic oxidizing solution, containing hypochlorous acid, obtained through the company patented nanotechnology platform, Tehclo ™.

The solution is featured by an exclusive combination of three physico-chemical properties - highly pure HClO, hypotonic low pH and high ORP, which are believed to support a faster physiological healing of EB wounds by creating a favourable wound microenvironment. In particular, hypochlorous acid is well known as a broad-spectrum, fast acting antimicrobial agent, which reinforced by low pH and high ORP contributes to prevent and treat skin infections. APR-TD011 could also reduce inflammation by inhibiting the NF-kB pro-inflammatory pathway and by inactivating the Matrix metalloproteases, known to play a key role in the inflammatory process of wounds. APR-TD011 could reasonably ameliorate the quality of life of EB patients by supporting faster wound healing and by reducing itching

and pain, linked to infections and inflammation. Available in an easy-to-use spray formulation, the solution allows wounds to be treated while avoiding skin contact and cross-contamination risk.

For more information please visit: https://www.apr.ch/apr-pharma-products/medical-prescription/apr-td011-epidermolysis-bullosa/

About Epidermolysis Bullosa

Epidermolysis Bullosa (EB) is a group of rare, genetic, life-threatening connective tissue disorders characterized by skin blistering throughout the body as well as severe impact to internal organs. There are many genetic variations of EB with common symptom of fragile skin that blisters and tears. It is estimated that EB affects 1 out of 20'000 births and approximately half a million patients in the US with limited life expectancy.

EB is always painful, often pervasive and debilitating: itching, pain and scars reduce significantly the quality of life while persistent skin inflammation and infections are correlated to tumor incidence and early death. No approved treatments or cure are available. The standard of care is currently supportive treatments such as pain and wound management in order to prevent infections and sustain wound healing for the entire life. Wound care management remains a complex procedure affecting daily EB patients and caregivers. The goal is to obtain a faster and physiological healing of the wounds, avoiding or limiting infections (with the consequent use of antibiotics), as well as wounds chronicization.

About APR Applied Pharma Research s.a.

APR is a Swiss independent pharma company focused on development and commercialization of innovative, research-driven products designed to address unmet needs in niche and rare therapeutic areas. APR combines pharmaceutical development expertise with proprietary drug delivery technologies to develop solutions that meaningfully improve the lives of people with rare diseases. A diverse and balanced portfolio of revenue-generating products in all major markets is complemented by a robust pipeline of innovative products at different stages of development for the treatment of recessive metabolic disorders, as well as rare dermatological and ocular diseases. Products are commercialized by APR with its sales and marketing teams in selected countries of Europe and by a growing network of commercial partners. For more information, please visit: https://www.apr.ch/

Forward-looking statements

The contents of this announcement include statements that are, or may be deemed to be, "forward-looking statements". These forward-looking statements can be identified by the use of forward-looking terminology, including the words "believes", "estimates," "expects", "intends", "may", "will", "plans", "continue", "ongoing", "potential", "project", "seek" or "should", and include statements the Company makes concerning the intended results of its strategy. By their nature, forward-looking statements involve risks and uncertainties and readers are cautioned that any such forward-looking statements are not guarantees of future performance. The Company's actual results may differ materially from those predicted by the forward-looking statements. The Company undertakes no obligation to publicly update or revise forward-looking statements, except as may be required by law.

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