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Amryt Pharma plc
("Amryt" or the "Company")

FDA grants Pediatric Rare Disease designation for AP101 for patients with Epidermolysis Bullosa

Amryt, a biopharmaceutical company focused on rare and orphan diseases, today announces that the US Food and Drug Administration ("FDA") has granted a Rare Pediatric Disease designation for AP101, the Company's lead development asset, for the treatment of Epidermolysis Bullosa ("EB").

Rare Pediatric Disease designations are granted for diseases that predominantly affect children aged 18 or younger and which affect a total of fewer than 200,000 people in the US.[1] The designation means if a New Drug Application ("NDA") for AP101 is approved, Amryt will be eligible to receive a priority review voucher that can be used, sold or transferred.

EB is a rare genetic skin disorder that can cause skin to blister and tear from the slightest friction or trauma and can, in some cases, cause blistering and erosion of the epithelial lining of internal organs. EB is chronic, potentially disfiguring and in some cases fatal. There are approximately 500,000 people living with EB worldwide and there are currently no approved treatments.[2]

AP101 is currently in a Phase III clinical trial (EASE), the largest ever global Phase III study for EB. An interim efficacy data readout is due later this year and the top-line data readout is expected in Q2 2019.

Joe Wiley, CEO of Amryt Pharma, commented: *"We are pleased to have been granted this Pediatric Rare Disease designation by the FDA, which recognises the rare and serious nature of EB and its impact on the lives of children suffering with this condition. With the interim efficacy readout for our Phase III trial of AP101 in EB due later this year, we look forward to continuing to demonstrate progress with our lead development asset."*

[1] https://www.fda.gov/downloads/RegulatoryInformation/Guidances/UCM423325.pdf
[2] https://www.debra.org.uk/what-is-eb/what-is-eb

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About Amryt

Amryt is a biopharmaceutical company focused on developing and delivering innovative new treatments to help improve the lives of patients with rare or orphan diseases.

Lojuxta is an approved treatment for adult patients with the rare cholesterol disorder - Homozygous Familial Hypercholesterolaemia ("HoFH"). This disorder impairs the body's ability to remove low density lipoprotein ("LDL") cholesterol ("bad" cholesterol) from the blood, typically leading to abnormally high blood LDL cholesterol levels in the body from before birth – often ten times more than people without HoFH – and subsequent aggressive and premature narrowing and blocking of blood vessels, heart attacks and strokes, even at a very young age if not properly diagnosed or receiving adequate treatment. Lojuxta is indicated as an adjunct to a low-fat diet and other lipid-lowering medicinal products with or without LDL apheresis in adult patients with HoFH.

Amryt holds an exclusive licence to sell Lojuxta (lomitapide) across the European Economic Area, Middle East and North Africa, Switzerland, Turkey, Israel, Russia, the Commonwealth of Independent States and the non-EU Balkan states.

Amryt's lead drug candidate, AP101, is a potential treatment for Epidermolysis Bullosa ("EB"), a rare and distressing genetic skin disorder affecting young children for which there is currently no treatment. It is currently in Phase III clinical trials. The European and US market opportunity for EB is estimated to be in excess of €1 billion.

Amryt's earlier stage product, AP102, is focused on developing novel, next generation somatostatin analogue ("SSA") peptide medicines for patients with rare neuroendocrine diseases, where there is a high unmet medical need, including Acromegaly and Cushing's disease.

In March 2018, Amryt in-licenced a pre-clinical gene-therapy platform technology, AP103, which offers a potential treatment for patients with Recessive Dystrophic Epidermolysis Bullosa, a subset of EB, and is also potentially relevant to other genetic disorders.

For more information on Amryt, please visit amrytpharma.com