



AMRYT RECEIVES POSITIVE FEEDBACK FROM THE FDA ON THE PATH FORWARD FOR MYALEPT® (METRELEPTIN) INDICATION IN PARTIAL LIPODYSTROPHY

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DUBLIN, Ireland, and Boston MA, March 23, 2021, Amryt (Nasdaq: AMYT, AIM: AMYT), a global, commercial-stage biopharmaceutical company focussed on acquiring, developing and commercializing novel treatments for rare diseases, today provides an update on its engagement with the FDA on Myalept® and its proposed development plan and study design to support an indication for patients with partial lipodystrophy (PL).

Myalept® / Myalepta® (metreleptin) is approved in the US (under the trade name Myalept®) as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy (GL) and in the EU (under the trade name Myalepta®) as an adjunct to diet for the treatment of leptin deficiency in patients with congenital or acquired GL in adults and children two years of age and above and familial or acquired PL in adults and children 12 years of age and above for whom standard treatments have failed to achieve adequate metabolic control.

The prevalence of PL patients severely affected by their disease is thought to be similar to GL in the US. Therefore, if approved for treatment of this patient sub-group, this represents approximately double the current US market opportunity.

Building on previous FDA interactions, Amryt has recently received additional feedback via a Type C written response on the proposed development plan and study design to support an indication for patients with PL. The FDA confirmed that they are willing to consider an efficacy supplement based on 6-months efficacy and safety data from a randomized, placebo-controlled trial in PL patients. Safety data will continue to be collected up to the completion of the 12-month treatment period and the overall assessment of the benefit-risk ratio for PL patients will take into consideration anti-leptin neutralizing activity.

Agreement was also received on Amryt's responses addressing PL study design comments from

previous FDA discussions, which have been incorporated into the study protocol (APG-20). The 12-month randomized, placebo-controlled Phase 3 trial to evaluate the safety and efficacy of daily subcutaneous metreleptin treatment in patients with PL will enroll approximately 80 patients globally. The study will enroll patients with severe metabolic consequences of their disease as reflected by blood glucose control and/or triglyceride levels on optimal background treatment.

Amryt has completed feasibility assessments to identify investigators and sites with potentially eligible PL patients. It is anticipated that the study will be initiated by the end of 2021.

Dr Joe Wiley, CEO of Amryt Pharma, commented today: *“Today’s news represents further progress as we grow the reach of our commercial products across both territories and indications. We now have a very clear path forward for our global clinical study of metreleptin in PL which, if successful, could offer the potential to address a broader population of patients in need.”*

About Amryt

Amryt is a global commercial-stage biopharmaceutical company focused on acquiring, developing and commercializing innovative treatments to help improve the lives of patients with rare and orphan diseases. Amryt comprises a strong and growing portfolio of commercial and development assets.

Amryt’s commercial business comprises two orphan disease products - metreleptin (Myalept®/ Myalepta®) and lomitapide (Juxtapid®/ Lojuxta®).

Myalept® / Myalepta® (metreleptin) is approved in the US (under the trade name Myalept®) as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy (GL) and in the EU (under the trade name Myalepta®) as an adjunct to diet for the treatment of leptin deficiency in patients with congenital or acquired GL in adults and children two years of age and above and familial or acquired partial lipodystrophy (PL) in adults and children 12 years of age and above for whom standard treatments have failed to achieve adequate metabolic control. For additional information, please follow this [link](#).

Juxtapid®/ Lojuxta® (lomitapide) is approved as an adjunct to a low-fat diet and other lipid-lowering medicinal products for adults with the rare cholesterol disorder, Homozygous Familial Hypercholesterolaemia ("HoFH") in the US, Canada, Columbia, Argentina and Japan (under the trade name Juxtapid®) and in the EU, Israel and Brazil (under the trade name Lojuxta®). For additional information, please follow this [link](#).

Amryt's lead development candidate, Filsuvez® (Oleogel-S10) is a potential treatment for the cutaneous manifestations of Junctional and Dystrophic Epidermolysis Bullosa (“EB”), a rare and distressing genetic skin disorder affecting young children and adults for which there is currently no approved treatment. Filsuvez® has been selected as the brand name for Oleogel-S10. The product does not currently have regulatory approval to treat EB.

Amryt’s pre-clinical gene therapy platform, AP103, offers a potential treatment for patients with Dystrophic EB, and is also potentially relevant to other genetic disorders. For more information on Amryt, including products, please visit www.amrytpharma.com.

The person making this notification on behalf of Amryt is Rory Nealon, CFO/COO and Company Secretary.

Financial Advisors

Shore Capital (Edward Mansfield, Daniel Bush, John More) are NOMAD and Joint Broker to Amryt in the UK. Stifel (Ben Maddison) are Joint Broker to the company in the UK.

Forward-Looking Statements

This press release may contain forward-looking statements containing the words "expect", "anticipate", "intends", "plan", "estimate", "aim", "forecast", "project" and similar expressions (or their negative) identify certain of these forward-looking statements. The forward-looking statements in this announcement are based on numerous assumptions and Amryt's present and future business strategies and the environment in which Amryt expects to operate in the future. Forward-looking statements involve inherent known and unknown risks, uncertainties and contingencies because they relate to events and depend on circumstances that may or may not occur in the future and may cause the actual results, performance or achievements to be materially different from those expressed or implied by such forward-looking statements. These statements are not guarantees of future performance or the ability to identify and consummate investments. Many of these risks and uncertainties relate to factors that are beyond each of Amryt's ability to control or estimate precisely, such as future market conditions, the course of the COVID-19 pandemic, currency fluctuations, the behaviour of other market participants, the outcome of clinical trials, the actions of regulators and other factors such as Amryt's ability to obtain financing, changes in the political, social and regulatory framework in which Amryt operates or in economic, technological or consumer trends or conditions. Past performance should not be taken as an indication or guarantee of future results, and no representation or warranty, express or implied, is made regarding future performance. No person is under any obligation to update or keep current the information contained in this announcement or to provide the recipient of it with access to any additional relevant information that may arise in connection with it. Such forward-looking statements reflect the Company's current beliefs and assumptions and are based on information currently available to management.

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