COMP Adopts Positive Opinion on Orphan Designation for Mycapssa® for the Treatment of Carcinoid Syndrome Associated with Neuroendocrine Tumors

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DUBLIN, Ireland, and Boston MA, December 19, 2022 Amryt (Nasdaq: AMYT), a global, commercial-stage biopharmaceutical company dedicated to acquiring, developing and commercializing novel treatments for rare diseases, today announces that the European Medicines Agency's (EMA) Committee for Orphan Medicinal Products (COMP) has adopted a positive opinion for orphan designation for the use of Mycapssa® in the treatment of carcinoid syndrome associated with neuroendocrine tumors (NET).

Orphan designation in the European Union (EU) is granted by the European Commission (EC) within 30 days of a positive opinion being issued by the COMP. This designation provides certain regulatory and financial incentives including but not limited to product market exclusivity for ten years in the EU following regulatory approval. Orphan designation is available to companies developing products intended to treat a life-threatening or chronically debilitating conditions affecting no more than five in 10,000 persons in the EU, and where the treatment provides a significant benefit to those affected by the condition compared to available treatment or where no satisfactory treatment is available.

The granting of the orphan designation in the treatment of carcinoid syndrome follows the COMP positive opinion in October 2022 recommending that the orphan designation of Mycapssa® in the treatment of acromegaly in the EU is maintained following approval of the marketing authorisation for the product. The Committee for Medicinal Products for Human Use (CHMP) issued a positive opinion on September 16, 2022, recommending the approval of Mycapssa® in the EU for the maintenance treatment of acromegaly in patients who have responded to and tolerated treatment with octreotide or lanreotide, with the EC approval subsequently being granted on December 2, 2022.

Dr Joe Wiley, CEO of Amryt Pharma, commented: *"The COMP positive opinion on the recommendation to grant orphan designation for Mycapssa® in the treatment of carcinoid syndrome represents a significant development for patients with carcinoid syndrome associated with neuroendocrine tumors in Europe. Amryt will continue the development of Mycapssa® to show the potential for significant benefit for patients over the available injectable forms of somatostatin analogues."*

Mycapssa®- NET Opportunity
Amryt's TPE® platform enables the oral delivery of the octreotide molecule which is otherwise delivered as an injectable. Mycapssa® (oral octreotide) is approved by the FDA and the EC for long-term maintenance treatment in acromegaly patients who have responded to and tolerated injectable treatment with octreotide or lanreotide (i.e. somatostatin analogs (SSAs)).

Injectable SSAs are also approved and are the pharmaceutical standard of care in the treatment of carcinoid syndrome associated with NET and their utilization in NET accounts for an estimated $1.9bn* globally and approximately $1.0bn* in the US. The potential addressable patient population on SSAs in the US is estimated at 24,000**. Compared to acromegaly, patients with NET are known to require higher average doses of injectable SSAs to achieve adequate symptom control. Pharmacokinetic studies with Mycapssa® showed dose linearity from 20mg to 80mg (40mg/day to 160mg/day), hence support dosing requirements for the planned Phase 3 study in patients with carcinoid syndrome, planned to be initiated early in 2023.

About Neuroendocrine Tumors (NET)
NETs arise from neuroendocrine cells throughout the body, most commonly in the gastrointestinal tract, lung, and rarely, the pancreas. While well differentiated neuroendocrine tumors are known to be slow growing, they are often asymptomatic in early stages leading to a substantial number of patients being diagnosed when the tumors have already spread regionally or distantly. Capable of secreting hormones and bioactive amines, approximately 19% of patients have carcinoid syndrome characterized by secretory diarrhea and flushing.

* Based on management estimates
** National Cancer Institute SEER Database; Halperin et al. 2017 https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6066284/

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 four orphan disease products – metreleptin (Myalept®/ Myalepta®); oral octreotide (Mycapssa®); lomitapide (Juxtapid®/ Lojuxta®); and Oleogel-S10 (Filsuvez®).

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.

Mycapssa® (octreotide capsules) is approved in the US and the EU for long-term maintenance therapy in acromegaly patients who have responded to and tolerated treatment with octreotide or lanreotide. Mycapssa® is the first and only oral somatostatin analog approved by the FDA and the EC. 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, Colombia, Argentina and Japan (under the trade name Juxtapid®) and in the EU, Israel, Saudi Arabia and Brazil (under the trade name Lojuxta®). For additional information, please follow this link.

Filsuvez® is approved in the EU and Great Britain for the treatment of partial thickness wounds associated with junctional and dystrophic Epidermolysis Bullosa in patients 6 months and older.

Amryt's pre-clinical gene therapy candidate, AP103, offers a potential treatment for patients with Dystrophic EB, and the polymer-based delivery platform has the potential to be developed for the treatment of other genetic disorders.

For more information on Amryt, including products, please visit www.amrytpharma.com.

Forward-Looking Statements

This announcement may contain forward-looking statements and 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 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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