

European Commission Approves Filsuvez® for the treatment of Dystrophic and Junctional EB

Released : 23 Jun 2022



European Commission Approves Filsuvez® for the treatment of Dystrophic and Junctional EB

Filsuvez® will be the first and only approved treatment for EB Patients

DUBLIN, Ireland, and Boston MA, June 23, 2022, Amryt (Nasdaq: AMYT), a global, commercial-stage biopharmaceutical company focussed on acquiring, developing and commercializing novel treatments for rare diseases, is pleased to announce the European Commission (EC) approval of Filsuvez® in the European Union (EU) for the treatment of partial thickness wounds associated with dystrophic and junctional Epidermolysis Bullosa (EB) in patients 6 months and older. EB is a rare and distressing genetic skin disorder affecting young children and adults for which, until now, there has been no approved treatment in any market.

The centralised marketing authorisation will be valid in all EU Member States as well as in Iceland, Liechtenstein, and Norway. The authorization of Filsuvez® in the EU provides a regulatory core dossier which may form the basis for future regulatory submissions in LATAM and the Middle East.

The EC approval of Filsuvez® is supported by Phase 3 data from the EASE trial which was the largest ever global trial conducted in patients with EB, performed across 58 sites in 28 countries.

Dr Joe Wiley, CEO of Amryt Pharma, commented: *“The EC approval of Filsuvez® in Europe is a major positive development for European patients who suffer from this debilitating condition. Filsuvez® will be our fourth commercial product for rare diseases. We have in place the team, financial flexibility and global infrastructure to bring it to market swiftly and to execute our significant growth plans. We are very excited to begin delivering Filsuvez® to treat European patients as soon as possible.”*

About Epidermolysis Bullosa

Epidermolysis Bullosa (EB) is a rare and devastating group of hereditary disorders of the skin, mucous membranes, and internal epithelial linings characterized by extreme skin fragility and blister development. Patients with severe forms of EB suffer from severe, chronic blistering, ulceration and scarring of the skin, mutilating scarring of the hands and feet, joint contractures, strictures of the esophagus and mucous membranes, a high risk of developing aggressive squamous cell carcinomas, infections and risk of premature death. The global market opportunity for EB is estimated by the Company to be in excess of \$1.0 billion.

About EASE

The EASE trial ([NCT03068780](#)) is the largest ever global Phase 3 trial conducted in patients with EB, performed across 58 sites in 28 countries. It comprises a 3-month double-blind randomised controlled phase followed by a 24-month open-label, single-arm phase. Patients with dystrophic and junctional EB target wounds of between 10 and 50cm² in size that were present for > 21 days and < 9 months were randomized in the double-blind phase to study treatment in a 1:1 ratio and wound dressings applied according to standard of care. 223 patients were enrolled into the trial including 156 pediatric patients. Of those that completed the double-blind phase, 100% entered the open label safety follow up phase.

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 three orphan disease products – metreleptin (Myalept®/ Myalepta®); oral octreotide (Mycapssa®); 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](#).

Mycapssa® (octreotide capsules) is approved in the US 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. Mycapssa® has also been submitted to the EMA and is not yet approved in Europe. 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](#).

Amryt's lead development candidate, 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. Filsuvez® has been selected as the brand name for Oleogel-S10.

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.

Amryt also intends to develop oral medications that are currently only available as injectable therapies through its Transient Permeability Enhancer (TPE®) technology platform. 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.

Contacts

Joe Wiley, CEO / Rory Nealon, CFO/COO, +353 (1) 518 0200, ir@amrytpharma.com

Tim McCarthy, LifeSci Advisors, LLC, +1 (212) 915 2564, tim@lifesciadvisors.com



Source: Amryt Pharma plc