Amryt Announces Positive Top Line Results from Phase 3 Pediatric Trial of Lomitapide in HoFH

Released : 05 Jan 2023

Amryt Announces Positive Top Line Results from Phase 3 Pediatric Trial of Lomitapide in HoFH

DUBLIN, Ireland, and Boston MA, January 5, 2023, Amryt (Nasdaq: AMYT), a global, commercial-stage biopharmaceutical company dedicated to acquiring, developing and commercializing novel treatments for rare diseases, is pleased to announce positive results from its APH-19 Phase 3 trial of lomitapide for the treatment of Homozygous Familial Hypercholesterolemia (HoFH) in children aged 5-17 years.

Dr Joe Wiley, CEO of Amryt Pharma, commented: *"We are encouraged by these important additional results regarding the efficacy of lomitapide in treating children with HoFH. This is good news for patients and their families."*

Top Line Results
A total of 46 pediatric patients were enrolled, with 43 patients completing the run-in phase and entering the efficacy phase. 20 patients were between 5 and 10 years of age. 23 patients were between 11 and 17 years of age. 88% had genetic confirmation of HoFH. The mean low density lipoprotein cholesterol (LDL-C) at baseline was 436 mg/dl (SD 189).

Primary Endpoint
The study met its primary endpoint with a clinically and statistically meaningful result. Percent change in LDL-C at Week 24 compared to baseline: mean reduction 54%, p <0.0001 (95% CL: -62% to -45%, p<0.0001) from baseline to week 24 in LDL-C, for the overall group of patients (n=43 patients).

This was consistent between the two pre-specified age groups:

- Patients aged 5 to 10 years (n=20): a mean reduction of 57% (95% CI: -68% to -45%) from baseline to week 24 in LDL-C
- Patients aged 11 to 17 years (n=23): a mean reduction of 51% (95% CI: -62% to -40%) from baseline to week 24 in LDL-C

Key Secondary Endpoints
Statistically meaningful reduction in key lipid parameters: Percent change at week 24 compared to baseline in the following lipid parameters:

- Mean reduction in non-high density lipoprotein cholesterol (non-HDL-C) : 54% (95% CI -62, -45%), p <0.0001
- Mean reduction in total cholesterol (TC) : 50% (95% CI -58, -42%), p <0.0001
- Mean reduction in very low-density lipoprotein cholesterol (VLDL-C) : 50% (95% CI -59, -41%), p <0.0001

Safety was consistent with the known profile of lomitapide, no new signal identified

- 41 patients completed the 24-week efficacy phase. 2 patients withdrew due to adverse events
- 5 patients had serious adverse events, with only 1 considered to be related to lomitapide

About APH-19 Trial
The Phase 3 study was a single-arm, open-label, international, multi-center study to evaluate the efficacy and safety of lomitapide in pediatric patients with Homozygous Familial Hypercholesterolemia (HoFH) on stable lipid-lowering therapy. Approximately 45 pediatric patients with HoFH were to be treated with lomitapide, added to their current stable lipid lowering therapy (LLT) (including lipoprotein apheresis (LA), when applicable) that was established during the Run-in Period. After the stabilization of the patient at their Maximum Tolerated Dose (MTD) of LLT (including LA, when applicable) during the 6-week Run-in Period, treatment with lomitapide was started as an add-on therapy on Day zero of the Efficacy Phase. Dosing of lomitapide was initiated at the recommended starting dose and escalated to the maximum dose applicable to their age group based upon safety and tolerability in addition to LDL-C values. During the 24-week Efficacy Phase, patients were required to remain on their stable LLT regimen (including LA, when applicable) established during the 6-week Run-in Period. Enrolment was stratified to ensure an adequate number of patients were enrolled in the following age groups: 5 to 10 years and 11 to ≤17 years.

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 Hypercholesterolemia ("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.

Contacts

Joe Wiley, CEO / Rory Nealon, CFO/COO, +353 (1) 518 0200, ir@amrytpharma.com
Tim McCarthy, LifeSci Advisors, LLC, +1 (917) 679 9282, tim@lifesciadvisors.com