

# CytRx Notes Topline Data From Trial of Arimoclomol in Amyotrophic Lateral Sclerosis

CytRx Corporation (OTCQB: CYTR) ("CytRx"), a specialized biopharmaceutical company focused on research and development for the oncology and neurodegenerative disease categories, today noted that Orphazyme A/S (NASDAQ: ORPH) ("Orphazyme") announced that the ORARIALS-01 pivotal trial for arimoclomol in Amyotrophic Lateral Sclerosis ("ALS") did not meet primary and secondary endpoints to show benefit in people living with the disease. According to Orphazyme, no important safety signals were reported in the trial. Topline data will be presented at the upcoming virtual European Network to Cure ALS meeting and complete data from the study will be published later this year.

Orphazyme's announcement disclosed that the randomized, placebo-controlled Phase 3 trial was conducted among 245 patients at 29 sites in 12 countries in North America and Europe. Participants were randomized (2:1 ratio) to receive either arimoclomol (248 mg three times daily) or placebo for up to 76 weeks. The primary endpoint was to determine the efficacy of chronic treatment with arimoclomol compared to placebo in participants with ALS as assessed by the combined assessment of function and survival (CAFS). This endpoint was selected to illustrate the overall treatment effect based on survival and the change in the ALS Functional Rating Scale-Revised (ALSFRS-R) score. Secondary endpoints included survival, change in ALSFRS-R, and slow vital capacity (SVC).

Notably, Orphazyme's applications for arimoclomol (to be branded MIPLYFFA™) for Niemann-Pick disease type C are under priority review with the U.S. Food and Drug Administration, with an expected PDUFA action date of June 17, 2021, and with the European Medicines Agency, an opinion from their Committee for Medicinal Products for Human Use is expected later this year.

CytRx will continue to provide updates that are relevant to our agreement with Orphazyme.

## **About CytRx Corporation**

CytRx Corporation (OTCQB: CYTR) is a biopharmaceutical company with expertise in discovering and developing new therapeutics principally to treat patients with cancer and neurodegenerative diseases. CytRx's drug candidate, arimoclomol, was sold to Orphazyme A/S (Nasdaq Copenhagen exchange: ORPHA.CO) in exchange for milestone payments and royalties. Orphazyme is developing arimoclomol in Niemann-Pick disease Type C ("NPC") and Gaucher disease. Learn more at [www.cytrx.com](http://www.cytrx.com).

## **About Orphazyme**

Orphazyme is a biopharmaceutical company focused on bringing novel treatments to patients living with life threatening or debilitating rare diseases. Their research focuses on developing therapies for diseases caused by misfolding of proteins including lysosomal storage diseases. Arimoclomol, the company's lead candidate, is in clinical development in Niemann-Pick disease Type C and Gaucher disease. Orphazyme is headquartered in Denmark and has operations in the U.S. and Switzerland. Orphazyme shares are listed on Nasdaq (ORPH). For more information, please visit [www.orphazyme.com](http://www.orphazyme.com).

## **About Arimoclomol**

Arimoclomol is an investigational drug candidate that amplifies the production of heat-shock proteins (HSPs). HSPs can rescue defective misfolded proteins, clear protein aggregates, and improve the function of lysosomes. Arimoclomol is administered orally, crosses the blood brain barrier, and has been studied in seven Phase 1, four Phase 2 and one pivotal Phase 2/3 clinical trial. Arimoclomol is

in clinical development at Orphazyme for the treatment of NPC and Gaucher disease. Arimoclomol has received orphan drug designation for NPC, IBM and ALS in the US and EU, as well as fast-track designation from the US Food and Drug Administration (FDA) for NPC, IBM and ALS. In addition, arimoclomol has received breakthrough therapy designation and rare-pediatric disease designation from the FDA for NPC.

### **Forward-Looking Statements**

This press release contains forward-looking statements, including statements relating to the potential receipt of EMA and FDA approval of arimoclomol, the Company's potential receipt of future milestone and royalty payments from Orphazyme and the achievement of long-term value for the Company's stockholders. Such statements involve risks and uncertainties that could cause actual events or results to differ materially from the events or results described in the forward-looking statements, including risks and uncertainties relating to the ability of Orphazyme to obtain regulatory approval for, manufacture and commercialize its products and therapies that use arimoclomol; the results of clinical trials involving arimoclomol; the amount, if any, of future milestone and royalty payments that we may receive from Orphazyme; and other risks and uncertainties described in the most recent annual and quarterly reports filed by the Company with the SEC and current reports filed since the date of the Company's most recent annual report. All forward-looking statements are based upon information available to the Company on the date the statements are first published. The Company undertakes no obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

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