

# CytRx Highlights Orphazyme's Acceleration of Pre-Launch Activities for Arimoclomol

***Applauds Orphazyme for Investing in Commercial and Other Pre-Launch Activities in Preparation of Potential FDA Approval of Arimoclomol to Treat Niemann-Pick Disease Type C***

***Arimoclomol for Niemann-Pick Disease Type C is Under the FDA's Priority Review With a Target Action Date of March 17, 2021***

LOS ANGELES – October 27, 2020 – (BUSINESS WIRE) – CytRx Corporation (OTCQB: CYTR) (“CytRx” or the “Company”), a specialized biopharmaceutical company focused on research and development for the oncology and neurodegenerative disease categories, today highlighted that Orphazyme A/S (ORPHA.CO) (NASDAQ:ORPH) (“Orphazyme”) has announced new plans to accelerate commercial and other pre-launch activities during the fourth quarter of 2020 in preparation for potential approval of arimoclomol in Niemann-Pick disease Type C (“NPC”), which is currently under Priority Review by the U.S. Food and Drug Administration (“FDA”) with a target action date of March 17, 2021. CytRx has an agreement with Orphazyme that can yield potential milestone payments and future royalties paid on sales of arimoclomol.

According to Orphazyme, the organization's reinforced financial position following a successful global stock offering has enabled it to commit additional resources to efforts pertaining to arimoclomol. Orphazyme stated that it will further invest in its Early Access Programs, additional API manufacturing, regulatory affairs and clinical safety activities. Orphazyme also expects continued costs through year-end for the ongoing trials in sporadic Inclusion Body Myositis and amyotrophic lateral sclerosis in order to provide home nursing and direct to patient distribution due to the COVID-19 pandemic. Orphazyme has stated that it wants to be well-situated to commercialize and launch arimoclomol for NPC if it receives FDA approval in 2021.

Orphazyme's Chief Executive Officer, issued the following statement last week:

“There is real momentum here at Orphazyme as we move closer to potential approval of arimoclomol in the U.S. in its first indication of NPC and accelerating our preparatory efforts now will help ensure a smooth launch. There are currently no approved products for NPC in the U.S. and arimoclomol has the potential to make a significant difference to patients with this devastating disease, so our team is working expeditiously to ensure we are optimally positioned for a successful launch if approved.”

In September 2020, the FDA accepted Orphazyme's New Drug Application (“NDA”) for arimoclomol for NPC, with priority review. The FDA set a target action date of March 17, 2021 under the Prescription Drug User Fee Act, or PDUFA, for completion of its review of the NDA. Orphazyme continues to expect to submit a Marketing Authorization Application, or MAA, to the European Medicines Agency in the second half of 2020.

Steven Kriegsman, Chairman and Chief Executive Officer of CytRx, commented:

“We applaud Orphazyme for investing in a strong foundation to underpin arimoclomol's commercialization and distribution for NPC if it receives FDA approval in the first quarter of 2021. It is also noteworthy that during the first half of 2021, Orphazyme will be reporting Phase 3 data on arimoclomol for sporadic Inclusion Body Myositis and for amyotrophic lateral sclerosis. Arimoclomol is a promising drug that can hopefully be a gamechanger for patients afflicted with NPC and other neurodegenerative diseases. We remain very optimistic about the drug's long-term potential.”

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

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## **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 four indications including amyotrophic lateral sclerosis ("ALS"), Niemann-Pick disease Type C ("NPC"), Gaucher disease and sporadic Inclusion Body Myositis ("sIBM"). 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 for four orphan diseases: Niemann-Pick disease Type C, Gaucher disease, sporadic Inclusion Body Myositis, and Amyotrophic Lateral Sclerosis. Orphazyme is headquartered in Denmark and has operations in the U.S. and Switzerland. Orphazyme shares are listed on Nasdaq Copenhagen (ORPHA.CO). For more information, please visit [www.orphazyme.com](http://www.orphazyme.com).

## **About NPC**

Niemann-Pick disease Type C (NPC) is a rare, genetic and progressive disease that impairs the ability of the body to move cholesterol and other fatty substances (lipids) inside the cells. The result is an accumulation of lipids within the body's tissue, including the brain tissue, causing damage to the affected 2 areas. The symptoms upon onset of NPC vary from fatality during the first months after birth to a progressive disorder not diagnosed until adulthood. The disease affects neurologic and psychiatric functions as well as various internal organs. Systemic symptoms of NPC are more common in infancy or childhood and the rate of progression is usually much slower in individuals with onset of symptoms during adulthood. NPC is usually fatal and the majority of individuals with the disease die before the age of 20. NPC has been granted Orphan Drug Designation (EU and U.S.) for the treatment of NPC. It is estimated the incidence of NPC to be one in 100,000 live births and the number of NPC patients in the United States and in Europe to be approximately 1,800 individuals. There are no approved treatments for NPC in the U.S. and only one approved product in Europe called miglustat.

## **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, Gaucher disease, sIBM and ALS. Arimoclomol has received orphan drug designation for NPC, sIBM and ALS in the US and EU, as well as fast-track designation from the US Food and Drug Administration (FDA) for NPC, sIBM 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 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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