



## **CytRx Corporation Highlights Arimoclomol Licensee Orphazyme A/S Prepares for Regulatory Filing of Arimoclomol in U.S. for Niemann-Pick Disease Type C**

*Orphazyme to File both the New Drug Application in the U.S. and Marketing Authorization Application in Europe in the First Half of 2020*

**LOS ANGELES – July 30, 2019** – CytRx Corporation (OTCQB: CYTR), a biopharmaceutical research and development company specializing in oncology and rare diseases, today highlighted that following a positive meeting with the U.S. Food and Drug Administration (FDA), arimoclomol licensee Orphazyme A/S (ORPH.CO) remains on track to submit a New Drug Application (NDA) for arimoclomol in Niemann-Pick Disease Type C (NPC) in the first half of 2020. Orphazyme also announced plans to introduce an Early Access Program for NPC in the fall of 2019. As previously announced, Orphazyme intends to file a Marketing Authorization Application (MAA) in Europe in the first half of 2020.

“We are very pleased to see that our partner Orphazyme’s diligent work and commitment to the clinical development of arimoclomol is culminating in productive and positive discussions with regulatory authorities, in an effort to bring this innovative drug candidate to a patient population that desperately needs help,” commented Eric Curtis, CytRx’s President and Chief Operating Officer. “There are currently no approved treatments for NPC in the U.S., and only one approved product in Europe. We are proud that arimoclomol may be able to be prescribed by physicians to improve clinical outcomes for patients in both the U.S. and Europe.”

In 2011, CytRx sold the rights to arimoclomol to Orphazyme A/S (formerly Orphazyme ApS) in exchange for a one-time, upfront payment of \$150,000 (USD) and the right to receive up to a total of \$120 million (USD) in milestone payments upon the achievement of certain pre-specified regulatory and business milestones, as well as royalty payments based on a specified percentage of any net sales of products derived from arimoclomol. Orphazyme, a public company trading on the Nasdaq Copenhagen exchange, is testing arimoclomol in several indications beyond NPC, including amyotrophic lateral sclerosis (ALS), Gaucher disease, and sporadic Inclusion Body Myositis (sIBM).

### **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 and three Phase 2 clinical trials. Arimoclomol is in clinical development at Orphazyme for the treatment of Niemann-Pick disease Type C, Gaucher disease, sporadic Inclusion Body Myositis, and amyotrophic lateral sclerosis.

### **About Niemann-Pick Disease Type C**

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



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 conservatively estimated that the number of potential NPC patients in the United States and in the EU is between 1,000 and 2,000 individuals in total. There are no approved treatments for NPC in the U.S. and only one approved product in Europe called miglustat.

### **About CytRx Corporation**

CytRx Corporation (OTCQB: CYTR) is a biopharmaceutical company with expertise in discovering and developing new therapeutics to treat patients with high unmet needs. CytRx's most advanced drug conjugate, aldoxorubicin, is an improved version of the widely used anti-cancer drug doxorubicin and has been out-licensed to NantCell, Inc. In addition, CytRx's other drug candidate, arimoclomol, has been out-licensed to Orphazyme A/S (Nasdaq Copenhagen exchange: ORPHA). Orphazyme is testing arimoclomol in four indications including amyotrophic lateral sclerosis (ALS), Niemann-Pick disease Type C (NPC), Gaucher disease and sporadic Inclusion Body Myositis (sIBM). CytRx Corporation's website is [www.cytrx.com](http://www.cytrx.com).

### **About Orphazyme A/S**

Orphazyme is a biopharmaceutical company focused on bringing novel treatments to patients living with life-threatening or debilitating rare diseases. This research focuses on developing therapies for diseases caused by misfolding of proteins and lysosomal dysfunction. 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. The Denmark-based company is listed on Nasdaq Copenhagen (ORPHA.CO). For more information, please visit [www.orphazyme.com](http://www.orphazyme.com).

### **Forward-Looking Statements**

This press release contains forward-looking statements. 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 A/S to obtain regulatory approval for, manufacture and commercialize its products and therapies that use arimoclomol; the results of future clinical trials involving arimoclomol; the amount, if any, of future milestone and royalty payments that we may receive from Orphazyme A/S; and other risks and uncertainties described in the most recent annual and quarterly reports filed by CytRx with the Securities and Exchange Commission and current reports filed since the date of CytRx's most recent annual report. All forward-looking statements are based upon information available to CytRx on the date the statements are first published. CytRx 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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