



## **CytRx Corporation Highlights Orphazyme's Announcement of its Phase 2 Study Results with Arimoclomol in Gaucher Disease**

*Arimoclomol demonstrated a marked and clinically meaningful dose-dependent reduction in liver and spleen size*

*Data supports Orphazyme's intention to proceed with pivotal stage clinical development in Gaucher disease*

**LOS ANGELES – June 29, 2020** – CytRx Corporation (OTCQB: CYTR), a biopharmaceutical research and development company specializing principally in oncology and neurodegenerative diseases, today highlighted that Orphazyme A/S announced topline results of their Phase 2 dose-finding study with arimoclomol in Gaucher disease. The data showed a dose-dependent effect of arimoclomol on certain disease-relevant clinical secondary endpoints, such as liver and spleen size. Furthermore, the data demonstrated sustained levels of arimoclomol in the cerebrospinal fluid (CSF), providing further evidence of arimoclomol's ability to cross the brain-blood barrier.

Orphazyme reported that arimoclomol demonstrated a relative reduction in serum chitotriosidase activity from baseline to six months, the primary endpoint, across all dosages compared to placebo ranging from -12% to -29%, although statistical significance was not achieved ( $p=0.4$ ). However, a statistically significant and clinically meaningful dose-dependent reduction in liver size ranging from -15% to -20% relative to placebo was observed (dose trend analysis  $p<0.05$ ). In addition, a clinically meaningful dose-dependent reduction in spleen size ranging from -5% to -21% relative to placebo was observed, although statistical significance was not achieved likely due to a small sample size (dose trend analysis  $p<0.10$ ). The company indicated their open-label extension of their phase 2 trial is ongoing across the three weight-adjusted dose levels and they will continue to evaluate clinical outcomes, monitor safety, and further explore relevant biomarkers.

A representative of Orphazyme said "We are very encouraged by the data from this exploratory study which show a clear dose-dependent effect of arimoclomol on liver and spleen size as early as 6 months. Although some aspects of Gaucher disease are well-managed by existing drugs, these therapies do not readily cross the blood-brain barrier, leaving an urgent need for new products that can address the debilitating neurological symptoms of this disease. With its oral administration, ability to cross the blood-brain barrier and the overall body of evidence we have gathered, we are encouraged by the potential for arimoclomol to both address an unmet need in Gaucher disease as well as a range of additional neurodegenerative orphan diseases."

Orphazyme announced plans to proceed with pivotal stage clinical development in Gaucher disease and its plans to discuss the data, along with results from the open-label extension, with Gaucher disease experts and regulators.

“The clinical data of arimoclomol in fighting Gaucher disease, combined with the previously announced results of arimoclomol in Orphazyme’s pivotal trial results for Niemann-Pick Disease Type C (NPC) is encouraging, since these lysosomal storage and neurodegenerative disorders have such high unmet needs,” said Steven A. Kriegsman, CytRx’s Chairman and CEO.

Orphazyme recently initiated a rolling New Drug Application (NDA) to the US Federal Drug Administration for arimoclomol in NPC, and announced plans to submit a Marketing Authorisation Application (MAA) for NPC in Europe in the second half of this year.

### **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. In addition, one of CytRx's drug candidates, arimoclomol, was sold to Orphazyme A/S 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). 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. 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. The Denmark-based company is listed on Nasdaq Copenhagen (ORPHA.CO). For more information, please visit [www.orphazyme.com](http://www.orphazyme.com).

### **About Gaucher disease**

Gaucher disease is a rare, inherited metabolic disorder causing certain sugar containing fats to abnormally accumulate in the lysosomes of cells, especially within cells of the blood system and nerve cells, thereby affecting organs such as the brain, bone marrow, spleen and liver. The typical systemic symptoms of Gaucher disease, which can appear at any age, include an abnormally enlarged liver and/or spleen and low levels of circulating red blood cells and platelets. These systemic symptoms can be treated by existing enzyme replacement therapy (ERT), and substrate reduction therapy (SRT). The neurological symptoms, although heterogenous, may include muscle rigidity, loss of movement, seizures, cognitive impairment and vision problems and are unable to be treated by these therapies, given their inability to cross the blood brain barrier (BBB). Gaucher disease is the most common lysosomal storage disorder (LSD) with an estimated incidence of 1:40,000 to 1:60,000, and affecting approximately 15,000 individuals in the United States and Europe combined.

### **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 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 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. 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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