



## **CytRx Corporation Highlights Updated Arimoclomol Milestone Guidance Provided by Orphazyme A/S in Niemann-Pick Disease**

*CytRx Eligible to Receive up to \$120 Million in Future Milestones, Plus Single and Double-Digit Royalties, From the Sales of Arimoclomol*

**LOS ANGELES – April 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 (ORPH.CO) has provided updated information regarding its projected filing of a New Drug Application (NDA) for arimoclomol in Niemann-Pick disease (NPC) and its preparation for getting arimoclomol to patients.

Orphazyme has indicated they are still on track to file their NDA in the first half of 2020, having a COVID-19 mitigation plan in place. They also confirmed their Early Access Program (EAP) for NPC is now operational with a goal of treating their first patient in May 2020, having already established approximately 45 patients under consideration for EAP before the selected sites have even opened. The 10 sites selected include Children’s Hospitals in Boston, Philadelphia, Pittsburgh, Cincinnati, and Orange County. Also, NYU, Emory, Rush, Mayo Clinic, and UCSF Benioff Children’s Oakland. Orphazyme updated their corporate presentation to highlight they have expanded their U.S. commercial team to build out their Medical Affairs and MSL teams (Marketing, Sales and Logistics) to be prepared for their U.S. launch and a lean go-to-market rollout in 2020.

In the European Union (EU) Orphazyme is focused on key EU markets, especially Germany and France. They are building a lean in-country and cluster organization in Europe. In addition, in the rest of the world Orphazyme indicated they are finalizing partnering for key countries, including Japan, Canada and Turkey.

“This updated outlook from Orphazyme A/S on arimoclomol at this time with the challenges the world is facing during this COVID-19 crisis is so encouraging, since there are no approved drugs available on the market in the U.S. for NPC,” said Steven A. Kriegsmann, CytRx’s Chairman and CEO. CytRx would be eligible to receive future milestone and royalty payments from our arimoclomol agreement.

CytRx would receive milestone payments of \$6 million in the U.S., \$4 million in Europe and \$2 million in Japan upon approval of arimoclomol in Orphazyme’s first non-ALS indication, plus royalties.

Orphazyme also announced they are still on target for the filing of a Marketing Authorization Applications Submission (MAA) with the European Medicines Agency (EMA) in the second half of 2020, as well as continuing to meet their timelines for their clinical trials using arimoclomol in Amyotrophic Lateral Sclerosis (ALS), Sporadic Inclusion Body Myositis (sIBM) and Gaucher disease.

### **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 and approximately 3,000 individuals worldwide. Diagnostic challenges may affect the number of potential patients. However, a treatment option could also increase awareness of the disease and assist in identifying more cases.

### **About ALS**

The rare neuromuscular disease Amyotrophic Lateral Sclerosis (ALS), also called Lou Gehrig's disease, is rapidly progressive and fatal, usually within two to five years. The disease attacks the neurons responsible for controlling muscles leading to paralysis of all skeletal muscles, eventually also affecting breathing, speaking, and swallowing. The cause of damage to the neurons includes protein misfolding and aggregation. Arimoclomol has so far been tested in two Phase II ALS trials, one dose-ranging trial in sporadic ALS, and one trial in ALS caused by SOD1 mutations. It is estimated that the number of potential ALS patients in the United States and in the EU is up to 50,000 individuals in total.

### **About Sporadic Inclusion Body Myositis (sIBM)**

Sporadic Inclusion Body Myositis (sIBM) is a progressively debilitating muscle-wasting disease. sIBM is characterized by a build-up of protein aggregates and atrophy of muscle cells, which leads to weakness and over time severe disability. The estimated prevalence of sIBM is 24.8-45.6 per million or up to 40,000 patients in the USA and Europe. There are no approved treatments for sIBM. Arimoclomol has been granted Orphan Drug Designation (EU and USA) for the treatment of sIBM.

### **About Gaucher Disease**

Gaucher disease is an inherited metabolic disorder caused by mutations in a protein called glucocerebrosidase, which leads to the accumulation of certain sugar-containing lipids. The usual symptoms of Gaucher disease include an abnormally enlarged liver and/or spleen (hepatosplenomegaly), low levels of circulating red blood cells (anemia), blood cells promoting clotting (thrombocytopenia), and skeletal abnormalities. Disease of the nervous system is observed in a significant subpopulation of Gaucher disease (neuropathic Gaucher). There are three types of Gaucher disease, type II being the most severe.

### **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 (ALS).

### **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 most advanced drug conjugate, aldoxorubicin, is an improved version of the widely used anti-cancer drug doxorubicin and has been out-licensed to ImmunityBio, Inc. In addition, CytRx's other drug candidate, arimoclomol has been sold to Orphazyme A/S (Nasdaq Copenhagen exchange: ORPHA.CO). 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. Their 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).

### **About ImmunityBio**

ImmunityBio, Inc. is a privately held immunotherapy company with one of the broadest portfolios of biological molecules spanning albumin-linked chemotherapeutics, peptides, fusion proteins, cytokine, monoclonal antibodies, adenovirus and yeast vaccine therapies.

### **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, such as the risks and uncertainties relating to the ability of Orphazyme A/S to obtain regulatory approval for its products that use arimoclomol; the ability of Orphazyme A/S to manufacture and commercialize products or therapies that use arimoclomol; the amount, if any, of future milestone and royalty payments that we may receive from Orphazyme A/S; the ability of ImmunityBio, Inc., to obtain regulatory approval for its products that use aldoxorubicin; the ability of ImmunityBio, Inc., to manufacture and commercialize products or therapies that use aldoxorubicin; the amount, if any, of future milestone and royalty payments that we may receive from ImmunityBio, Inc.; 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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