



## **CytRx Corporation Highlights Updated Arimoclomol Milestone Guidance Provided by Licensee Orphazyme A/S**

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

*CytRx Net Operating Losses Will Shelter at Least \$250 Million of Potential Future Pre-Tax Profits*

**LOS ANGELES – February 4, 2020** – CytRx Corporation (OTCQB: CYTR), a biopharmaceutical research and development company specializing principally in oncology and neurodegenerative diseases, today highlighted that arimoclomol licensee Orphazyme A/S (ORPH.CO) has provided updated clinical and regulatory guidance on arimoclomol milestones through 2021 in its four indications, amyotrophic lateral sclerosis (ALS), sporadic Inclusion Body Myositis (sIBM), Niemann-Pick disease Type C (NPC) and Gaucher Disease. Pre-clinical work has also commenced in Parkinson’s Disease.

“The updated outlook from our arimoclomol licensee Orphazyme A/S addressing these debilitating neurodegenerative diseases are potential game-changers,” said Eric Curtis, CytRx’s President and Chief Operating Officer. “Orphazyme’s announcement last month of the launch of its Early Access Program in NPC is just the start of rewarding patients worldwide who suffer from these diseases. CytRx would be eligible to receive future milestone and royalty payments from our arimoclomol licensing agreement. In addition, these revenue payments will be sheltered by approximately \$250 million in carryforward net operating losses.”

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

The following are Orphazyme’s expected pipeline milestones and projections:

### Niemann-Pick disease Type C (NPC):

- Announced Early Access Program launch in January 2020
- Announce Pre-NDA meeting with FDA Q1 2020
- Regulatory filing with the FDA in H1 2020
- Regulatory filing with EMA in H2 2020
- Go to market in US H1 2021 and EU/RoW H2 2021
- Expected price range is \$300,000 - \$600,000 per patient
- Total worldwide patients number approximately 3,000
- There is currently no approved drug in the U.S. for this neurodegenerative disease

### Amyotrophic Lateral Sclerosis (ALS):

- Enrollment completed in Phase 3 trial of ALS **last July 2019**
- Announcement of Phase 3 results in ALS – H1 2021



- Regulatory submission of arimoclomol in ALS – H2 2021

#### Sporadic Inclusion Body Myositis (sIBM):

- Sporadic inclusion body myositis (sIBM) Announced – P2/3 trial fully enrolled last May 2019
- Announcement of Phase 2/3 results in sIBM – H1 2021
- Regulatory submission of arimoclomol in sIBM – H2 2021

#### Gaucher Disease

- Announcement of results of Phase 2 trial in Gaucher disease – H1 2020

#### Parkinson's Disease

- Commenced pre-clinical work with arimoclomol

#### **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, aldorubicin, 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 out-licensed 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).

### **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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