



## **CytRx Corporation Highlights Recently Published Data of its Licensee Orphazyme A/S Phase 2 Trial of Arimoclomol in the Treatment of Sporadic Inclusion Body Myositis (sIBM)**

*Orphazyme A/S Also Completed a Major New Capital Raise of \$110 Million to Cover its Expansion*

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

**LOS ANGELES – February 11, 2020** – CytRx Corporation (OTCQB: CYTR), a biopharmaceutical research and development company specializing principally in oncology and neurodegenerative diseases, today highlighted Orphazyme’s recently published data from its double-blinded Phase 2 trial of arimoclomol for the treatment of Sporadic Inclusion Body Myositis (sIBM). This was a 24-patient randomized pilot trial (16 patients on arimoclomol and 8 on placebo) where 83% of arimoclomol treated patients were stabilized versus 25% on placebo. Four months of continuous treatment resulted in a 60% reduction in progression, and at 8 months (4 months after treatment discontinuation), there was a 75% reduction in progression.

Their fully-enrolled Phase 2/3 trial of arimoclomol for the treatment of sIBM is a 152-patient, 20-month, 1:1 randomized, double-blind, placebo-controlled trial in 11 centers in the United States and one in the United Kingdom. Orphazyme expects to conduct an interim analysis in the first half of 2020 and to complete the study by the end of 2020, with results anticipated to be announced in the first half of 2021 and regulatory submission in the second half of 2021.

“We are extremely encouraged by the results of this Phase 2 clinical trial using arimoclomol for the treatment of sIBM. It is estimated there are up to 40,000 patients in the USA and Europe who suffer from this debilitating disease,” said Eric Curtis, CytRx’s President and Chief Operating Officer. “There are currently no effective-disease modifying drugs for this huge market potential to treat sIBM.”

Orphazyme is currently developing arimoclomol in four different indications, including amyotrophic lateral sclerosis (ALS), Niemann-Pick disease Type C (NPC), Gaucher disease and Sporadic Inclusion Body Myositis (sIBM). Pre-clinical work has also commenced in Parkinson’s Disease.

CytRx also highlighted that arimoclomol licensee Orphazyme A/S (CPH: ORPHA) completed a major new capital raise of approximately \$110 million for expansion. Orphazyme indicated the funding will support the completion of arimoclomol’s clinical development in sIBM and to prepare regulatory filings in Europe and potentially in the U.S.

The funding also provides significant cash for Orphazyme to progress arimoclomol towards commercialization in NPC. The proceeds are expected to cover clinical development plans well into 2021. This will support their imminent U.S. and European filings of arimoclomol for NPC and preparation for commercial launch.



Orphazyme also intends to use the proceeds to cover completion of arimoclomol's clinical development in ALS and to prepare regulatory filings in Europe and potentially in the U.S.

CytRx is eligible to receive up to \$120 million in future milestones, plus royalties, from its arimoclomol licensing agreement. These amounts, if received, would be sheltered by at least \$250 million in net operating losses.

### **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 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 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 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 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; changes in Orphazyme's strategy and approach for the development of arimoclomol from those described in this press release; 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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