



## **CytRx Corporation Highlights Orphazyme’s Completion of Rolling Submission of its New Drug Application with the U.S. FDA for Arimoclomol in Niemann-Pick Disease Type-C**

*Orphazyme expects to submit MAA for arimoclomol for NPC to European Medicines Agency in the second half of 2020*

*Arimoclomol progressing through registrational trials in two additional indications - ALS and sIBM*

**LOS ANGELES – July 21, 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 they have completed their rolling submission of their New Drug Application (NDA) with the U.S. Food and Drug Administration (FDA) for arimoclomol for the treatment of Niemann-Pick Disease Type-C (NPC).

Arimoclomol has received Fast Track and Breakthrough Therapy Designations for the treatment of NPC, in addition to Orphan Drug and Rare Pediatric Disease Designations. The FDA has up to 60 days to determine whether to accept the application for review.

A spokesperson from the Mayo Clinic Children’s Center in Rochester, MN commented *“Data from the randomized, controlled, clinical trial of arimoclomol for Niemann-Pick disease Type C support the positive effect of this agent in stabilizing neurologic progression of the disease, specifically in subgroups of patients over four years of age, and in those also taking miglustat. The data show clear evidence of target engagement, specifically an elevation of Heat-Shock Protein levels, with encouraging changes in biomarkers of excess lipid storage. These data support the role of enhanced Heat-Shock Protein 70 expression in Niemann-Pick disease Type C and may have applications in other lysosomal disorders.”*

A spokesperson from the National Niemann-Pick Disease Foundation (NNPDF) commented *“The NPC patient community’s need for disease-modifying therapy could not be more urgent as there are no FDA-approved treatments for this malicious disease. On behalf of the NNPDF and the Niemann-Pick community, we would like to extend our heartfelt thanks to Orphazyme for their tireless commitment to improving the lives of NPC patients and congratulate them in their completed NDA submission for arimoclomol to the FDA as a treatment for NPC.”*

Orphazyme also re-affirmed they expect to submit a Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) for arimoclomol in NPC in the second half of this year.

A spokesperson from Orphazyme stated *“The rapid completion of the rolling submission of arimoclomol in NPC brings us one step closer to the potential approval of a new treatment option that can address a substantial unmet need for patients with NPC. We look forward to working together with the FDA as they review our application. If approved, arimoclomol would be the only FDA-approved treatment for NPC in the U.S. This exciting step adds to the momentum at Orphazyme, as we make progress in our commercial preparations in the U.S. and other major markets ahead of potential approval in NPC, and as we progress arimoclomol through clinical trials in additional indications with unmet medical needs – Amyotrophic Lateral Sclerosis (ALS), sporadic Inclusion Body Myositis (sIBM), and Gaucher disease.”*

“We are extremely pleased that Orphazyme has now submitted their complete NDA for arimoclomol in NPC and look forward to a potential successful launch in the U.S.” said Steven A. Kriegsman, CytRx’s Chairman and CEO.

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.

#### **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 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 and three Phase 2 clinical trials. Arimoclomol is in clinical development at Orphazyme for the treatment of NPC, Gaucher disease, sIBM, and ALS.

### **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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