



CytRx Highlights Orphazyme's Receipt of FDA Acceptance and Priority Review of its New Drug Application for arimoclomol for Niemann-Pick disease Type C

FDA's Priority Review Shortens Assessment Period to 6 Months, Resulting in a Target Action Date of March 17, 2021 Under PDUFA

LOS ANGELES – September 17, 2020 – CytRx Corporation (OTCQB: CYTR), a biopharmaceutical research and development company specializing principally in oncology and neurodegenerative diseases, today highlighted that Orphazyme A/S (“Orphazyme”) announced that the U.S. Food and Drug Administration (“FDA”) has accepted, with Priority Review, the company’s New Drug Application (“NDA”) for arimoclomol for the treatment of Niemann-Pick disease Type C (“NPC”).

The FDA grants Priority Review to applications for potential therapies that, if approved, could offer a significant improvement in safety, effectiveness, diagnosis or prevention of serious conditions. This designation shortens the review period from the standard 10 months to 6 months from the acceptance of the NDA. The FDA has set a target action date of March 17, 2021 under the Prescription Drug User Fee Act (“PDUFA”) and has indicated that it does not currently plan to hold an advisory committee meeting to discuss the application.

Arimoclomol was previously granted FDA Fast Track and Breakthrough Therapy Designations for NPC, as well as Orphan Drug and Rare Pediatric Disease Designations. Orphazyme expects to file a Marketing Authorisation Application with the European Medicines Agency for arimoclomol in NPC in H2 2020.

Kim Stratton, Chief Executive Officer of Orphazyme, commented in yesterday’s announcement: “The filing acceptance marks a significant milestone in our journey towards our first potential approval of arimoclomol for NPC, a devastating and often fatal disease for which there is no approved therapy in the U.S. We look forward to collaborating with the FDA as they complete their review of this NDA to address the unmet medical need in NPC, and meanwhile are working to expand our U.S. activities in preparation for potential commercial availability next year.”

Joslyn Crowe, Executive Director of the National Niemann-Pick Disease Foundation, commented in yesterday’s announcement: “Acceptance of the arimoclomol filing by the FDA is another major step forward in the effort to bring a treatment to people affected by Niemann-Pick disease Type C and is a reflection of the commitment of the entire community coming together to support promising research.”

Steven A. Kriegsman, Chairman and Chief Executive Officer of CytRx, concluded: “If approved, arimoclomol will become the first approved therapy in the U.S. for people with Niemann-Pick disease Type C. In line with our strategy, we intend to prudently manage CytRx’s operations and balance sheet as we await prospective developments related to Orphazyme’s commercialization of arimoclomol and our other high-potential assets. We are hopeful that our stockholders will begin to realize the value of our legacy research and development efforts over the next year.”

To learn more about CytRx's go-forward strategy and priorities, review the presentation shared at the Company's 2020 Annual Meeting of Stockholders [here](#).

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. 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.

About Orphazyme

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.

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 estimated the incidence of NPC to be one in 100,000 live births and the number of NPC patients in the United States and in Europe to be approximately 1,800 individuals. 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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