CytRx Notes Orphazyme's Regulatory Update from the FDA on Arimoclomol for Niemann-Pick Disease Type C

LOS ANGELES – JUNE 18, 2021 – CytRx Corporation (OTCQB: CYTR) ("CytRx"), a specialized biopharmaceutical company focused on research and development for the oncology and neurodegenerative disease categories, today noted that Orphazyme A/S (NASDAQ: ORPH) ("Orphazyme") announced it has received a Complete Response Letter ("CRL") from the U.S. Food and Drug Administration ("FDA") following its review of the new drug application for arimoclomol, a heat shock protein amplifier intended for the treatment of Niemann-Pick disease type C ("NPC").

Orphazyme's announcement disclosed that the FDA issued the CRL based on needing additional qualitative and quantitative evidence to further substantiate the validity and interpretation of the 5-domain NPC Clinical Severity Scale ("NPCCSS") and, in particular, the swallow domain. Further, the FDA noted in the CRL that additional data are needed to bolster confirmatory evidence beyond the single phase 2/3 clinical trial to support the benefit-risk assessment of the NDA.

A primary endpoint of the phase 2/3 clinical trial was progression in disease severity as measured by the 5-domain NPCCSS. This is a disease-specific measure of disease progression consisting of the five clinically most relevant domains to patients with NPC, caregivers and physicians.

Orphazyme CEO Christophe Bourdon stated: "We are disheartened by the outcome of the FDA's review, given the urgent need for a new therapeutic option for NPC, but we remain committed to working with the regulators, with the goal of delivering arimoclomol to families managing this challenging disease. We will focus our efforts on pursuing the European regulatory approval, with CHMP opinion expected in Q4 2021 and potential Marketing Authorization in Q1 2022. We are assessing the potential path forward in the U.S. in partnership with the FDA. In the short-term, we will need to reduce our costs substantially and freeze all company efforts not related to clinical and regulatory activities to support approval for NPC."

Orphazyme disclosed that as stated in its Annual Report 2020, initial outlook for the year was subject to various risks and uncertainties, including but not limited to the timing of regulatory decisions, the success of Orphazyme's commercial efforts and development activities. The outcome of the FDA decision has significant influence on Orphazyme's outlook for full-year 2021. Orphazyme's cash position at year-end 2021 is now expected to be approximately $8 million (previously $56 million).

Orphazyme noted it will provide an update and further information in the coming weeks.

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 drug candidate, arimoclomol, was sold to Orphazyme A/S (Nasdaq Copenhagen exchange: ORPHA.CO) in exchange for milestone payments and royalties. Orphazyme is developing arimoclomol in Niemann-Pick disease Type C ("NPC") and Gaucher disease. Learn more at 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 in Niemann-Pick disease Type C and Gaucher disease. Orphazyme is headquartered in Denmark and has operations in the U.S. and Switzerland. Orphazyme shares are listed on NASDAQ: ORPH. For more information, please visit www.orphazyme.com.

About Niemann-Pick disease type C

Niemann-Pick disease type C (NPC) is a rare, genetic, progressively debilitating, and often fatal neurovisceral disease. It belongs to a family known as lysosomal storage diseases and is caused by mutations leading to defective NPC protein. As a consequence, lipids that are normally cleared by the lysosome accumulate in tissues and organs, including the brain, and drive the disease pathology. We estimate 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.

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 and Gaucher disease. Arimoclomol has received orphan drug designation for NPC in the US and EU, as well as fast-track designation from the US Food and Drug Administration (FDA) for NPC. 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, including statements relating to the potential receipt of EMA and FDA approval of arimoclomol, the Company's potential receipt of future milestone and royalty payments from Orphazyme and the achievement of long-term value for the Company's stockholders. 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 to obtain regulatory approval for, manufacture and commercialize its products and therapies that use arimoclomol; the results of clinical trials involving arimoclomol; the amount, if any, of future milestone and royalty payments that we may receive from Orphazyme; and other risks and uncertainties described in the most recent annual and quarterly reports filed by the Company with the SEC and current reports filed since the date of the Company's most recent annual report. All forward-looking statements are based upon information available to the Company on the date the statements are first published. The Company 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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