CytRx Highlights Orphazyme’s Submission of European Marketing Authorisation for Arimoclomol to Treat Niemann-Pick Disease Type C

Applauds Orphazyme’s Efforts to Pursue Approval in Europe for Arimoclomol in the Treatment of Niemann-Pick Disease Type C

Notes That Arimoclomol for Niemann-Pick Disease Type C is Under the FDA’s Priority Review With a Target Action Date of March 17, 2021

LOS ANGELES – November 11, 2020 – (BUSINESS WIRE) – CytRx Corporation (OTCQB: CYTR) (“CytRx” or the “Company”), a specialized biopharmaceutical company focused on research and development for the oncology and neurodegenerative disease categories, today highlighted that Orphazyme A/S (ORPHA) (NASDAQ: ORPH) (“Orphazyme”) has submitted a Marketing Authorisation Application (“MAA”) to the European Medicines Agency (“EMA”) for arimoclomol in the treatment of Niemann-Pick disease Type C (“NPC”). CytRx has an agreement with Orphazyme that can yield potential milestone payments and future royalties paid on sales of arimoclomol.

This submission of an MAA to the EMA comes on the heels of Orphazyme filing a New Drug Application (“NDA”) in the United States for arimoclomol in the treatment of NPC. The NDA is currently under Priority Review with the U.S. Food and Drug Administration (“FDA”) with a Prescription Drug User Fee Act (“PDUFA”) target action date of March 17, 2021. It is also important to note that arimoclomol was previously granted Orphan Drug Designation in Europe and the U.S. and it received the FDA’s Fast Track and Breakthrough Therapy Designations for NPC as well as Rare Pediatric Disease Designations in the U.S.

With respect to the submission of an MAA to the EMA, Orphazyme’s Chief Executive Officer delivered the following statement this week:

“This filing in Europe is a significant milestone for Orphazyme as we work toward our first potential approvals of arimoclomol in major markets. There are few options today that can address the devastating effects of NPC, and we are hopeful we can address an important need for this community. We look forward to working with EMA as they complete their review of our application.”

Steven Kriegsman, Chairman and Chief Executive Officer of CytRx, added:

“We are pleased that Orphazyme is building momentum and making strong progress in its pursuit of potential approvals for arimoclomol to treat NPC with both the FDA and EMA. Given the lack of treatment options for individuals afflicted with NPC, arimoclomol represents a possible breakthrough for patients with this brutal disease. We are encouraged that Orphazyme has publicly disclosed that it is investing in a strong foundation to underpin arimoclomol’s prospective commercialization and distribution for NPC if it receives requisite approvals in March 2021.”

Mr. Kriegsman concluded:

“It is also noteworthy that during the first half of 2021, Orphazyme will be reporting Phase 2/3 data on arimoclomol for sporadic Inclusion Body Myositis and Phase 3 data for amyotrophic lateral sclerosis. As we have said in the past, arimoclomol is a promising drug that can be a game changer for patients afflicted with NPC and other neurodegenerative diseases.”

CytRx will continue to provide updates that are relevant to our agreement with Orphazyme.

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 four indications including amyotrophic lateral sclerosis (“ALS”), Niemann-Pick disease Type C (“NPC”), Gaucher disease and sporadic Inclusion Body Myositis (“sIBM”). 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 for four orphan diseases: Niemann-Pick disease Type C, Gaucher disease, sporadic Inclusion Body Myositis, and Amyotrophic Lateral Sclerosis. Orphazyme is headquartered in Denmark and has operations in the U.S. and Switzerland. Orphazyme shares are listed on Nasdaq U.S. (ORPH) and Nasdaq Copenhagen (ORPHA). 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 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, 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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