CytRx Issues Statement Regarding U.S. Regulatory Review of Arimoclomol for Niemann-Pick Disease Type C

LOS ANGELES--(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 provided an update on the U.S. Food and Drug Administration's ("FDA") review of Orphazyme A/S's (NASDAQ: ORPH) ("Orphazyme") New Drug Application ("NDA") for arimoclomol to treat Niemann-Pick Disease Type C ("NPC"). According to Orphazyme, the FDA has extended the review period with a standard extension of three months in order to complete the NDA review. The updated Prescription Drug User Fee Act ("PDUFA") target action date is June 17, 2021.

Orphazyme disclosed that the FDA has confirmed that the NDA remains under Priority Review. The FDA grants Priority Review to applications for potential therapies that, if approved, could offer a significant improvement in safety or effectiveness, diagnosis, or prevention of serious conditions. Notably, the FDA has confirmed that the extension does not impede eligibility for a Pediatric Rare Disease Priority Review Voucher.

Arimoclomol previously received the FDA's Fast-Track and Breakthrough Therapy Designations for NPC as well as Orphan Drug and Rare Pediatric Disease Designations. If approved in the U.S., arimoclomol will be the first and only approved medicine for NPC. In November 2020, Orphazyme also submitted a Marketing Authorisation Application to the European Medicines Agency for arimoclomol to treat NPC.

Orphazyme's U.S. President issued the following statement on Monday:

"Orphazyme is working closely with the FDA to support the final review of the new drug application for arimoclomol. There is significant unmet medical need for the treatment of NPC, and we are committed to bringing arimoclomol to patients in the U.S. and Europe as soon as possible."

Orphazyme's Chief Medical Officer added:

"We have responded to all FDA information requests and submitted all outstanding information regarding the arimoclomol NDA for NPC. The Phase 3 trials for Amyotrophic Lateral Sclerosis and Inclusion Body Myositis remain on track for read-out in the first half of 2021 and we look forward to providing an update on our progress."

Steven A. Kriegsman, Chairman and Chief Executive Officer of CytRx, commented:

"We believe Orphazyme has taken a number of important steps in 2020 ahead of potential commercialization of arimoclomol for NPC upon prospective FDA approval during the first half of 2021. Orphazyme has strengthened its financial position through a successful capital raise and subsequently established a strong U.S. footprint with new headquarters in Chicago and the addition of more than 30 employees. In our view, Orphazyme is well positioned for future distribution and expanded engagement with providers, patients, regulators and the clinical testing community in 2021. It is also noteworthy that next year, Orphazyme may receive a response to its submission for regulatory approval in Europe for arimoclomol to treat NPC."

We 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 (ORPHA). For more information, please visit www.orphazyme.com.

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.

Contacts

For Investors:

Greg Marose / Charlotte Kiaie
cytrx@profileadvisors.com

###