CytRx Highlights Orphazyme's Update on Productive Meeting with FDA Regarding Arimoclomol in Niemann-Pick Disease Type C

FDA Recommends Orphazyme Provides Supplemental Information and Analyses

FDA Offers to have Further Interactions with Orphazyme to Identify Path to Resubmission for Arimoclomol in NPC

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 commented on Orphazyme A/S's (NASDAQ: ORPH) ("Orphazyme") encouraging regulatory update following its recently held Type A meeting with the U.S. Food and Drug Administration ("FDA") on arimoclomol, a heat shock protein amplifier intended for the treatment of Niemann-Pick disease Type C (“NPC”). Arimoclomol, CytRx's drug candidate, was sold to Orphazyme in exchange for milestone payments and royalties.

In June 2021, Orphazyme announced it had received a Complete Response Letter ("CRL") from the FDA following the regulatory agency's review of the new drug application ("NDA") for arimoclomol. Orphazyme disclosed that the FDA issued the CRL based on needing additional evidence to further substantiate the validity and interpretation of the 5-domain NPC Clinical Severity Scale and, in particular, the swallow domain. Further, the FDA noted in the CRL that additional data is needed to bolster confirmatory evidence beyond the single phase 2/3 clinical trial to support the benefit-risk assessment of the NDA.

Orphazyme's recent Type A meeting to discuss the CRL resulted in the following takeaways:

- The FDA recommended that Orphazyme submit additional data, information and analyses to address certain topics in the CRL and engage in further interactions with the FDA to identify a pathway to resubmission.
- The FDA concurred with Orphazyme's proposal to remove the cognition domain from the NPCCSS endpoint, with the result that the primary endpoint is permitted to be recalculated using the 4-domain NPCCSS, subject to the submission of additional requested information which Orphazyme intends to provide. To bolster the confirmatory evidence already submitted, the FDA affirmed that it would require additional in vivo or pharmacodynamic (PD)/pharmacokinetic (PK) data; Orphazyme is considering the optimal path forward to address the FDA’s requests.

Christophe Bourdon, Chief Executive Officer of Orphazyme A/S, commented:

“We are pleased to have gained a greater understanding from the FDA on the information that could address topics in the CRL. This is good progress. We are encouraged by the FDA’s request to submit additional information and the invitation to further engage to discuss our approach and potential path forward. While we have not yet established a path to resubmission, our team will now work on putting a plan in place to discuss with the FDA during our next interactions and we will share more details about our strategy as and when appropriate. We firmly believe in the establishment of a positive benefit-risk balance for arimoclomol and will continue to support the NPC community and our early access programs.”

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

"We are highly encouraged by the outcome of Orphazyme's recent meeting with the FDA on arimoclomol in Niemann-Pick disease Type C. It's especially impactful to hear that the FDA welcomed the submission of additional information and invited Orphazyme to engage further with the agency in order to identify a path toward resubmission of an NDA. We look forward to monitoring additional developments in the U.S. as well as Orphazyme's pursuit of European regulatory approval for arimoclomol in Q1 2022."

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 most recent advanced drug conjugate, aldoxorubicin, is an improved version of the widely used anti-cancer drug doxorubicin and has been out-licensed to ImmunityBio, Inc. In addition, CytRx's drug candidate, arimoclomol, was sold to Orphazyme A/S in exchange for milestone payments and royalties. Orphazyme is developing arimoclomol in two indications, including Niemann-Pick disease Type C (NPC), and Gaucher disease. CytRx Corporation's website is www.cytrx.com.

Forward-Looking Statements

This press release contains forward-looking statements. These statements are not historical facts, but instead represent only CytRx's belief regarding future events, many of which, by their nature, are inherently uncertain and outside of CytRx's control. Forward-looking statements include statements relating to the potential receipt of EMA and FDA approval of arimoclomol and the CytRx's potential receipt of future milestone and royalty payments from Orphazyme. 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 CytRx with the SEC, including disclosures under the heading "Risk Factors", and current reports filed since the date of the CytRx's most recent annual report. All forward-looking statements are based upon information available to the CytRx on the date the statements are first published. The 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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