

CytRx Comments on Orphazyme's Promising 24-Month Interim Trial Results of Arimoclomol for Niemann-Pick Disease Type C

LOS ANGELES – JUNE 29, 2021 – CytRx Corporation (OTCQB: CYTR) ("CytRx"), 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") recently announced 24-month interim results of an open-label extension (OLE) trial of arimoclomol for the treatment of Niemann-Pick disease type C (NPC).

Orphazyme announced 24-month interim results of an OLE trial, providing efficacy and safety data for its investigational treatment arimoclomol in NPC for up to 36 months. The data are featured in a presentation as part of the [Parseghian Scientific Conference](#) for NPC Research. The results demonstrate that arimoclomol provided a sustained benefit to study participants by reducing NPC progression as measured by the 5-domain NPC Clinical Severity Scale (5D-NPCCSS).

"Following on the outcomes from the 12-month double-blind phase, which indicated a clinically meaningful effect on disease progression, these longer-term data provide an encouraging picture that arimoclomol could deliver a sustained benefit and consistent safety profile over time," said Marc Patterson, MD, Professor of Neurology, Pediatrics and Medical Genetics, Mayo Clinic Children's Center in Rochester, MN.

Orphazyme stated arimoclomol demonstrated a consistent safety profile throughout the 36-month treatment period. Adverse events observed during the open label extension phase were similar to those observed in the double-blind phase. A total of 41 patients joined the OLE following the double-blind period; 33 have now completed up to 36 months of treatment.

Orphazyme also indicated data from the 36-month period support the findings from the 12-month double-blind period, which showed a clinically meaningful difference on the 5-domain NPCCSS, with a significant p-value of 0.046 (previously calculated at $p=0.0537$).

Orphazyme CEO Christophe Bourdon stated: "These data provide further evidence of the clinical profile of arimoclomol to treat this population and may support our efforts to pursue regulatory approval to deliver a much-needed option for the NPC community. We continue to evaluate our path forward in the U.S. following the recent FDA response, and our application remains under active review in the European Union."

Steven A. Kriegsman, Chairman and Chief Executive Officer of CytRx, stated: "We are optimistic about the direction Orphazyme is headed in and look forward to continued developments on the path to regulatory approval for arimoclomol for NPC in Europe and the U.S. Additionally, we are excited by the interim results of the open-label extension trial, which indicated that arimoclomol had a clinically meaningful effect on reducing NPC progression in participants. The data further demonstrates the significance of what arimoclomol can do for society and highlights the importance of focusing on its advancement. We recognize that progress in the biopharmaceutical industry is not always linear, and we believe the actions Orphazyme is taking through its announced restructuring plan will only serve to strengthen the company and streamline its efforts on its drug pipeline progress."

Orphazyme disclosed that its financial outlook remains unchanged for 2021.

Orphazyme noted it will provide an update and further information in connection with the publication of its interim report for the first half of 2021, due for release August 24, 2021.

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.

Contacts

Greg Marose / Bela Kirpalani
cytrx@profileadvisors.com