

# CytRx Corporation Highlights Orphazyme's Receipt of FDA Fast Track Designation for the Development of Arimoclomol in Amyotrophic Lateral Sclerosis (ALS)

**LOS ANGELES – May 26, 2020 –** CytRx Corporation (OTCQB: CYTR), a biopharmaceutical research and development company specializing principally in oncology and neurodegenerative diseases, today highlighted that Orphayzme A/S announced they have received Fast Track Designation from the U.S. Food and Drug Administration (FDA) for the development of arimoclomol for the treatment of amyotrophic lateral sclerosis (ALS).

In July 2019, Orphazyme A/S had announced they had completed enrollment in their Phase 3 clinical trial evaluating arimoclomol for the treatment of ALS ahead of schedule. Orphazyme A/S anticipates announcing top-line results from its Phase 3 clinical trial in the first half of 2021.

Arimoclomol has also received Fast Track status from the FDA for the treatment of Niemann-Pick disease Type C (NPC) and sporadic Inclusion Body Myositis (sIBM).

Fast Track is a designation by the FDA of an investigational drug for expedited review to facilitate development of drugs which treat a serious or life-threatening condition and fill an unmet medical need. Fast Track status entails eligibility for Accelerated Approval and Priority Review if certain criteria are met, as well as Rolling Review, which means that a company can submit completed sections of its Biologic License Application (BLA) or New Drug Application (NDA) for review by the FDA, rather than waiting until every section is completed before the entire application can be reviewed.

"Orphazyme continues to make significant progress in treating neurodegenerative diseases with arimoclomol. We are very pleased that the Company has received Fast Track Designation," said Steven A. Kriegsman, CytRx's Chairman and CEO.

## **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. In addition, one of CytRx's drug candidates, arimoclomol, was sold to Orphazyme A/S in exchange for milestone payments and royalties. Orphazyme is testing arimoclomol in four indications including amyotrophic lateral sclerosis (ALS), Niemann-Pick disease Type C (NPC), Gaucher disease and sporadic Inclusion Body Myositis (sIBM). CytRx Corporation's website is <a href="https://www.cytrx.com">www.cytrx.com</a>.

## About Orphazyme A/S

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 and lysosomal dysfunction. 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. The Denmark-based company is listed on Nasdaq Copenhagen (ORPHA.CO). For more information, please visit <a href="https://www.orphazyme.com">www.orphazyme.com</a>.

#### **About ALS**

The rare neuromuscular disease Amyotrophic Lateral Sclerosis (ALS), also called Lou Gehrig's disease, is rapidly progressive and fatal, usually within two to five years. The disease attacks the neurons responsible for controlling muscles leading to paralysis of all skeletal muscles, eventually also affecting breathing, speaking, and swallowing. The cause of damage to the neurons includes protein misfolding and aggregation. Arimoclomol has so far been tested in two Phase II ALS trials, one dose-ranging trial in sporadic ALS, and one trial in ALS caused by SOD1 mutations. It is estimated that the number of potential ALS patients in the United States and in the EU is up to 50,000 individuals in total.

### **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 2 function of lysosomes. Arimoclomol is administered orally, crosses the blood brain barrier, and has been studied in seven Phase 1 and three Phase 2 clinical trials. Arimoclomol is in clinical development at Orphazyme for the treatment of NPC, Gaucher disease, sIBM, and ALS.

## **Forward-Looking Statements**

This press release contains forward-looking statements. 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, such as the risks and uncertainties relating to the ability of Orphazyme to obtain regulatory approval for its products that use arimoclomol; the ability of Orphazyme A/S to manufacture and commercialize products or therapies that use arimoclomol; the amount, if any, of future milestone and royalty payments that we may receive from Orphazyme A/S; and other risks and uncertainties described in the most recent annual and quarterly reports filed by CytRx with the Securities and Exchange Commission and current reports filed since the date of CytRx's most recent annual report. All forward-looking statements are based upon information available to CytRx on the date the statements are first published. 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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