



CytRx Corporation Highlights Arimoclomol Licensee Orphazyme A/S Completes Enrollment in its Phase 3 Clinical Trial of Arimoclomol in Amyotrophic Lateral Sclerosis

Enrollment Completed Ahead of Schedule

CytRx Eligible to Receive up to \$120 Million in Future Milestones, Plus Royalties, From Arimoclomol Licensing Agreement

LOS ANGELES – July 22, 2019 – CytRx Corporation (OTCQB: CYTR), a biopharmaceutical research and development company specializing in oncology, today highlighted that arimoclomol licensee Orphazyme A/S (ORPH.CO) has completed enrollment in its Phase 3 clinical trial evaluating arimoclomol for the treatment of amyotrophic lateral sclerosis (ALS) ahead of schedule.

The Phase 3 trial of arimoclomol for ALS is a 76-week, randomized, placebo-controlled trial being conducted at 30 centers of excellence in North America and Europe. A total of 213 patients have been randomized 2:1 to receive arimoclomol or placebo and up to an additional 18 individuals on stable treatment with edaravone may participate in the U.S. Patients completing the trial will be offered participation in an open-label extension trial. Orphazyme anticipates announcing top-line results from the Phase 3 trial in the first half of 2021.

“We continue to be impressed with the progress our partner Orphazyme is making with the clinical development of arimoclomol,” commented Eric Curtis, CytRx’s President and Chief Operating Officer. “ALS is a devastating disease with few treatment options currently available, and we remain hopeful that arimoclomol could become a life-changing drug for these patients. We thank Orphazyme for their commitment to the arimoclomol program and their dedication to this patient population.”

In 2011, CytRx sold the rights to arimoclomol to Orphazyme A/S (formerly Orphazyme ApS) in exchange for a one-time, upfront payment of \$150,000 (USD) and the right to receive up to a total of \$120 million (USD) in milestone payments upon the achievement of certain pre-specified regulatory and business milestones, as well as royalty payments based on a specified percentage of any net sales of products derived from arimoclomol. Orphazyme, a public company trading on the Nasdaq Copenhagen exchange, is testing arimoclomol in three additional indications beyond ALS, including Niemann-Pick disease Type C (NPC), Gaucher disease and sporadic Inclusion Body Myositis (sIBM).

In addition, CytRx recently announced that Orphazyme issued positive news on its EMEA meeting on arimoclomol in Niemann-Pick disease type C. Orphazyme is planning to submit its Marketing Authorization Application in the first half of 2020.

CytRx is eligible to receive \$6 million upon approval of arimoclomol in the U.S. and \$4 million upon approval in Europe in Orphazyme’s first non-ALS indication, plus royalties on net sales in both territories.



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 and three Phase 2 clinical trials. Arimoclomol is in clinical development at Orphazyme for the treatment of Niemann-Pick disease Type C, Gaucher disease, sporadic Inclusion Body Myositis, and amyotrophic lateral sclerosis.

About ALS

Amyotrophic Lateral Sclerosis (ALS) is a rare, rapidly progressive, and always fatal neurodegenerative disease. Protein misfolding and aggregation in motor neurons are important contributors to the disease process, which ultimately leads to paralysis of skeletal muscles as well as the muscles that enable breathing. The patient population in Europe and the United States is estimated to be approximately 50,000. Currently, there are only limited treatment options available. Arimoclomol has been granted Orphan Drug Designation (EU and USA) for the treatment of ALS.

About CytRx Corporation

CytRx Corporation (OTCQB: CYTR) is a biopharmaceutical company with expertise in discovering and developing new therapeutics to treat patients with high unmet needs. CytRx's most advanced drug conjugate, aldoxorubicin, is an improved version of the widely used anti-cancer drug doxorubicin and has been out-licensed to NantCell, Inc. In addition, CytRx's other drug candidate, arimoclomol, has been out-licensed to Orphazyme A/S (Nasdaq Copenhagen exchange: ORPHA). Orphazyme is currently 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 www.cytrx.com.

About Orphazyme A/S

Orphazyme is a biopharmaceutical company focused on bringing novel treatments to patients living with life-threatening or debilitating rare diseases. This 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 www.orphazyme.com.

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, including risks and uncertainties relating to the potential for aldoxorubicin to transform cancer treatment; the appeal of the reconstituted formula for patients and physicians; the ability of NantCell, Inc., to obtain regulatory approval for its products that use aldoxorubicin; the ability of



NantCell, Inc., to manufacture and commercialize products or therapies that use aldoxorubicin; the amount, if any, of future milestone and royalty payments that we may receive from NantCell, Inc.; risks and uncertainties relating to the ability of Orphazyme A/S 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; Centurion BioPharma Corporation's ability to develop and finance new ultra-high potency drug candidates based on its LADR™ technology platform; our ability to attract potential licensees; 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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