# CytRx Highlights Orphazyme's Momentum Ahead of Potential Regulatory Approval of Arimoclomol for Niemann-Pick Disease Type C

***Orphazyme Announces the Global Brand Name for Arimoclomol – MIPLYFFA – Ahead of Prospective FDA and EMA Approvals***

***Expanded its Early Access Program for NPC in the U.S. and Europe as Well as Expectation of European Regulatory Decision by Q4 2021***

LOS ANGELES--(BUSINESS WIRE)--CytRx Corporation (OTCQB: CYTR) ("CytRx"), a specialized biopharmaceutical company focused on research and development for the oncology and neurodegenerative disease categories, today highlighted that Orphazyme A/S (NASDAQ: ORPH) ("Orphazyme") has released a new investor presentation that outlines its momentum ahead of potential U.S. and European approval of arimoclomol for the treatment of Niemann-Pick disease Type C ("NPC"). The March 2nd presentation can be found under the "Events & Presentations" section of www.orphazyme.com. CytRx has an agreement with Orphazyme that can yield potential milestone payments and future single and double-digit royalties paid on sales of arimoclomol.

Items noted in the presentation include:

- Arimoclomol expected to be first to market in NPC in the U.S.
- In anticipation of U.S. Food and Drug Administration ("FDA") and European Medicine Agency ("EMA") approval, Orphazyme announced MIPLYFFA as the global brand name for arimoclomol.
- Orphazyme has expanded its NPC Early Access Program (EAP) in the U.S. and opened similar programs in France and Germany.
- Orphazyme now expects to receive a decision on European regulatory approval for arimoclomol for NPC by Q4 2021.
- Arimoclomol for NPC remains under the FDA's priority review with a Prescription Drug User Fee Act target action date of June 17, 2021.
- Orphazyme has expanded its commercial organization in the U.S. and EU while extending its footprint to support launch readiness.
- Goal is to position arimoclomol as first-line in NPC globally.
- Orphazyme projects arimoclomol sales for NPC this year of up to $20 million.

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

"We are very excited to see Orphazyme announce a global brand name for arimoclomol ahead of potential regulatory approvals in the U.S. and Europe. We are equally enthusiastic about the company's commitment to expanding EAPs and fortifying its global operations in anticipation of commercializing MIPLYFFA. In our view, Orphazyme is taking the right steps to commence future distribution and enhance its engagement with providers, patients and regulators. CytRx looks forward to monitoring Orphazyme's progress as it works to make MIPLYFFA available for NPC and three other rare diseases over time."

We will continue to provide updates that are relevant to our agreement with Orphazyme. As noted, Orphazyme's March 2nd presentation can be found under the "Events & Presentations" section of www.orphazyme.com.

**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 Inclusion Body Myositis ("IBM"). 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, 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 (ORPH). For more information, please visit www.orphazyme.com.

**About NPC**

Niemann-Pick disease Type C (NPC) is a rare, genetic and progressive disease that impairs the ability of the body to move cholesterol and other fatty substances (lipids) inside the cells. The result is an accumulation of lipids within the body's tissue, including the brain tissue, causing damage to the affected 2 areas. The symptoms upon onset of NPC vary from fatality during the first months after birth to a progressive disorder not diagnosed until adulthood. The disease affects neurologic and psychiatric functions as well as various internal organs. Systemic symptoms of NPC are more common in infancy or childhood and the rate of progression is usually much slower in individuals with onset of symptoms during adulthood. NPC is usually fatal and the majority of individuals with the disease die before the age of 20. NPC has been granted Orphan Drug Designation (EU and U.S.) for the treatment of NPC. It is estimated 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. and only one approved product in Europe called miglustat.

**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, Gaucher disease, IBM and ALS. Arimoclomol has received orphan drug designation for NPC, IBM and ALS in the US and EU, as well as fast-track designation from the US Food and Drug Administration (FDA) for NPC, IBM and ALS. 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.

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