



DECEMBER 27, 2020

ORPHAZYME PROVIDES REGULATORY UPDATE ON ARIMOCLOMOL FOR NPC

Copenhagen, Denmark – December 27, 2020 – Orphazyme A/S (ORPHA.CO; ORPH), a late-stage biopharmaceutical company pioneering the Heat-Shock Protein response for the treatment of neurodegenerative orphan diseases, announced today the U.S. Food and Drug Administration (FDA) has extended the review period of the New Drug Application (NDA) for arimoclomol for the treatment of Niemann-Pick Disease Type C (NPC) by a standard extension period of three months. This extension is necessary for the FDA to complete its review. The updated Prescription Drug User Fee Act (PDUFA) target action date is June 17, 2021.

The FDA has confirmed the NDA remains under Priority Review, and the extension does not impede eligibility for a Pediatric Rare Disease Priority Review Voucher. The FDA grants Priority Review to applications for potential therapies that, if approved, could offer a significant improvement in safety or effectiveness, diagnosis, or prevention of serious conditions.

“Orphazyme is working closely with the FDA to support the final review of the new drug application for arimoclomol,” said Molly Painter, US President, Orphazyme. *“There is significant unmet medical need for the treatment of NPC, and we are committed to bringing arimoclomol to patients in the U.S. and Europe as soon as possible.”*

“We have responded to all FDA information requests and submitted all outstanding information regarding the arimoclomol NDA for NPC,” said Thomas Blaettler, Chief Medical Officer, Orphazyme. *“The Phase 3 trials for Amyotrophic Lateral Sclerosis and Inclusion Body Myositis remain on track for read-out in the first half of 2021 and we look forward to providing an update on our progress.”*

Arimoclomol has received FDA Fast-Track and Breakthrough Therapy Designations for NPC, as well as Orphan Drug and Rare Pediatric Disease Designations. If approved in the US, arimoclomol will be the first and only approved medicine for NPC, a rare, relentlessly progressive, neurodegenerative disease with an estimated incidence of one in 100,000 live births. In November 2020, the company also submitted a Marketing Authorisation Application to the European Medicines Agency for arimoclomol in NPC.



ORPHAZYME A/S

Phone: (+45) 39 17 82 72

E-mail: contact@orphazyme.com

Ole Maaløes Vej 3

DK-2200 Copenhagen N

Denmark

ORPHAZYME US, INC.

Phone: +1-773-770-6888

E-mail: contact@orphazyme.com

180 North LaSalle Street, Suite 3475

Chicago, Illinois 60601

USA

For inquiries from US HCPs and patients, please contact Orphazyme, Inc. at usmedicalaffairs@orphazyme.com