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KemPharm Joins with NPC Community

Following KemPharm Inc.'s ("KemPharm") recent purchase of substantially all of the assets of Orphazyme A/S (in restructuring), including the product candidate arimoclomol intended for the treatment of Niemann-Pick Disease Type C (NPC), the company has confirmed its commitment to seek regulatory approval of the treatment, as well as to engage with the NPC community.

"Our regulatory team has carefully evaluated the progress made with arimoclomol to date following the Complete Response Letter (CRL) issued by the U.S. Food and Drug Administration (FDA) in June 2021, as well as the minutes from the subsequent meeting held with the FDA in October 2021," said KemPharm's President and CEO, Travis Mickle, Ph.D. "Based upon the data we have reviewed so far, we believe that arimoclomol has the potential to offer clinical benefit to those living with NPC. We have committed our resources to working with the FDA to obtain approval for the resubmission of the NDA, as well as to finding a regulatory path forward with the European Medicines Agency (EMA), which is part of our efforts to support the global NPC community."

Since the purchase was first announced in mid-May 2022, KemPharm has focused on three key areas for arimoclomol: (1) the resubmission to the FDA, which is targeted to be filed as early as the first quarter of 2023, (2) supporting the continuation of the Expanded Access Programs in the various countries where it has been available thus far, and (3) identifying a regulatory path forward with the EMA.

"KemPharm has significant experience with challenging regulatory situations, having successfully led or participated in three FDA product approvals, two of which followed an initial CRL," said Christal Mickle, KemPharm's Vice President of Operations and Product Development. "We also have a keen interest in the advancement of novel treatments that address rare CNS conditions, and particularly, neurodegenerative and other rare orphan disease indications, which makes NPC and arimoclomol a natural addition to our product candidate pipeline."

The European Marketing Authorisation Application (MAA) for arimoclomol for the treatment of NPC with the EMA was withdrawn by Orphazyme ahead of a final vote and opinion by the Committee for Medicinal Products for Human Use (CHMP). Once there is a clearer path to an NDA resubmission with the FDA, KemPharm has committed to further exploring the best regulatory path forward in collaboration with the EMA authorities.

KemPharm has also made it clear that the voice of the NPC community is important. "We have heard from many of you how important it is to retain access to arimoclomol through our Expanded Access Programs in the U.S. and Europe, given the continuing unmet need in the treatment of NPC," said Dr. Mickle. "KemPharm is committed to providing continued access to arimoclomol for eligible patients through the available compassionate use programs while the regulatory process progresses within each respective jurisdiction."

As part of its commitment to the NPC community, KemPharm has also announced the recent appointment of Tara Greene as Senior Director, U.S. Medical Operations and Patient Advocacy. Other former employees of Orphazyme have also now joined the KemPharm team in an effort by the company to maintain continuity in both the regulatory processes and the Early Access Programs.

“Guided by its data-driven and scientific approach, KemPharm is a company with a history of success in overcoming regulatory challenges,” said Ms. Greene. “I am excited to join the global KemPharm team as part of our continued pursuit to make effective treatments available for patients affected by NPC. The combined experience in rare disease, drug development and engagement with the NPC community adds new energy and resources to these vital efforts.”

The NPC community’s engagement is one of the most important elements to continue building awareness of the disease, the unmet medical needs of those affected by NPC, and to help guide policymakers toward the approval of an effective treatment for NPC. KemPharm executives have expressed their intention to attend and engage with the NPC community via the upcoming NNPDF and the INPDA Family Support and Medical Conference and Meeting, which will provide an excellent mix of high-quality advocacy-focused educational sessions, clinical trial updates, and family-services oriented sessions.
