



KemPharm Remains Committed to the Niemann-Pick Community

KemPharm recognizes the importance of the International Niemann-Pick Disease Alliance (INPDA) and other patient organizations dedicated in not only raising awareness of Niemann-Pick Disease but also in providing advice, information, and education to those living with NPC, their families, other caregivers, and all involved in the management of this disease. Global Niemann-Pick Awareness Day provides an opportunity for KemPharm to support and unite with these groups in advocating for those affected by NPC and their families and in seeking new therapeutic options for NPC where no satisfactory alternative treatments currently exist.

“NPC is a devastating neurodegenerative disease that affects people of all ages and leads to progressive loss of mobility, cognition, speech, and swallowing, often resulting in premature death,” said Christal Mickle, Senior Vice President, Operations and Product Development at KemPharm, Inc. “Unfortunately, there are limited treatment options, and diagnosis is challenging due to the wide range of possible disease presentations and variability in the timing of onset. This diagnostic complexity results in the disease frequently being unrecognized and misdiagnosed with diagnostic delays averaging four to five years in the

U.S. For these reasons, KemPharm has become an active participant in the NPC community, and we applaud the tireless work conducted by patient advocacy organizations to offer support for those living with NPC, their families and caregivers.”

We are grateful for the opportunity to have recently sponsored and attended both the National Niemann-Pick Disease Foundation (NNPDF) and Niemann-Pick UK (NP-UK) annual conferences and meetings. These provide us with deeper insights into the challenges and experiences of those affected by NPC. We have quickly recognized the meaningful work of the INPDA and are pleased to also sponsor these efforts.

Our key priorities are simple: (i) continuing and full support of the compassionate use of arimoclomol for the treatment of NPC via our Early Access Programs in countries where this is possible, (ii) resubmission

of the arimoclomol New Drug Application (“NDA”) to the FDA, and (iii) evaluation and identification of a regulatory pathway to approval for arimoclomol with the European Medicines Agency (“EMA”).

KemPharm understands the importance of retaining access to arimoclomol through our Expanded Access Programs in the U.S. and Europe, given the continuing unmet need in the treatment of NPC. 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.

The NPC community’s engagement is one of the most important elements to continue building awareness of the disease and to help guide policymakers toward the approval of an effective treatment for NPC. We are excited to continue our collaboration and remain committed to bring much needed solutions to those living with NPC.

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