Not approved outside of the United States; investigational in the EU

September 20, 2024

Dear Global NPC Community,

We are thrilled to share with you a milestone we have all been eagerly awaiting – today, the U.S. Food and Drug Administration (FDA) has approved MIPLYFFA™ (arimoclomol) capsules, for oral use, for use in combination with miglustat for the treatment of neurological manifestations of Niemann-Pick disease type C (NPC) in adult and pediatric patients 2 years of age and older. MIPLYFFA is the first therapy approved by the FDA for the treatment of NPC. You can read the full news release here Zevra Therapeutics' MIPLYFFA™ (arimoclomol) Receives U.S. FDA Approval as Treatment for Niemann-Pick Disease Type C.

We want to express our deepest gratitude and appreciation to the patients, families, researchers and clinicians in the US and Europe who have participated in the arimoclomol clinical studies and the expanded access program. We are sincerely grateful for the unwavering support of both Zevra and the global NPC community. Your dedication and partnership throughout our journey have been vital in achieving this groundbreaking approval to bring a much-needed treatment option to those affected by NPC.

We are incredibly proud to be a part of the ongoing efforts to positively impact the lives of those living with rare diseases such as NPC patients. In accordance with our current EAP policies, the Expanded Access Program will remain open for current patients and new enrollments in countries throughout Europe, which are currently active. Given the recent FDA approval, Zevra will now evaluate the regulatory pathway for resubmitting arimoclomol to EMA and explore opportunities for expanding access to arimoclomol in other countries worldwide.

If you have any questions or need further information, please don't hesitate to reach out to us at patientadvocacy@zevra.com.

Kind regards,

The Zevra Team

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