ZEVRA THERAPEUTICS

ARIMOCLOMOL

Arimoclomol is an orally delivered, first-in-class investigational product candidate for the treatment of Niemann-Pick Disease Type C (NPC) being developed by Zevra Therapeutics.

Arimoclomol was granted Orphan Drug Designation for NPC by the FDA and European Medicines Agency. Arimoclomol was also granted Breakthrough Therapy Designation, Fast Track Designation, and Rare Pediatric Disease.

> *Zevra continues its ongoing dialogue with the FDA and seeks FDA approval for arimoclomol in 2024 with a PDUFA action date set for June 21, 2024.*

HOW DOES ARIMOCLOMOL WORK?

The exact mechanism of action of arimoclomol is unknown. Evidence indicates that arimoclomol acts on multiple fronts to help clear lipid build-up in cells which improves lysosomal function.

WHAT IS NPC?

- NPC is an ultra-rare, genetic, relentlessly progressive neurodegenerative disease caused by mutations in the NPC1 or NPC2 genes which are responsible for making proteins in the membrane of cells called lysosomes.
- The lysosomes job is to recycle and clear out build-up of lipids (or fats) in cells, such as in the brain, spleen and liver. In NPC, the NPC1 or NPC2 gene are not working properly so lipids are building up in the body's organs.
- NPC is an inherited disorder that affects both children and adults with varying age of onset, rate of progression and presents differently in each person.
- NPC is characterized by visceral (internal organs) manifestations, including enlarged liver and spleen, neurological and psychiatric manifestations.
- Those living with NPC lose independence due to physical and cognitive limitations. Key neurological impairments are in speech, cognition, swallowing, ambulation, and fine motor skills.
- Disease progression is irreversible and can be fatal within months or take years to diagnose and advance

The incidence of NPC is estimated to be between 1 in 100,000 live births.

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ADMINISTRATION

Administration of arimoclomol can be flexible, with a capsule that can be swallowed whole or opened to mix with soft foods and/or liquids or delivered through a gastric feeding tube.

RESEARCH

- In NPC, arimoclomol has been investigated in one Phase 2/3 double-blind, placebo-controlled trial (CT-ORZY-NPC-002) including 50 patients.
- A sub-study of the completed Phase 2/3 CT-ORZY-NPC-002 trial is ongoing, investigating the safety of arimoclomol in children 6-24 months of age with NPC.
- More information can be found at ***Clinicaltrials.gov (NCT02612129)***

PATIENT ADVOCACY COLLABORATION

Zevra is working closely with key patient advocacy groups to raise awareness, provide education and support research for NPC. Through these collaborations our goal is to support and empower those affected by NPC and to address their urgent unmet treatment need.

ZEVRA'S COMMITMENT

- We are committed to making promising rare diseases therapies available to patients with the aim of improving their quality of life.
- Zevra currently offers an Expanded Access Program. To learn more about the program, visit our ***Expanded Access Policy*** page.
- Requests for expanded access to arimoclomol should only be made by qualified and licensed physicians. For more information, please contact medicalaffairs@zevra.com or ***ClinicalTrials.Gov (NCT04316637)***

Zevra Patients and Providers Expanded Access Policy

RESOURCES FOR INDIVIDUALS AND FAMILIES AFFECTED BY NPC

nnpdf NATIONAL NIEMANN PICK DISEASE FOUNDATION — *nnpdf.org*

inpdr — *inpdr.org*

Ara Parseghian Medical Research Fund, University of Notre Dame — *parseghianfund.nd.edu*

np uk — *npuk.org*

Firefly Fund — *fireflyfund.org*

Hope for Marian — *hopeformarian.org*

Zevra Therapeutics is a rare disease company driven by science, data, and patients' unmet needs to create transformational therapies for diseases with limited or no treatment options.

More information is available at *Zevra.com*