A First in Human Study to Assess the Safety, Tolerability, and Pharmacokinetics of EDK060 in Adults With CMT1A.

NCT07140614

Status	RECRUITING
Phase	Phase 1
Sponsor	Novartis Pharmaceuticals
Enrollment	28 participants

Key Eligibility Criteria

Inclusion (6)

- Provide written informed consent before any assessment is performed.
- Be male or female and 18 to 60 (inclusive) years of age at the time of screening.
- Participant must have a clinical diagnosis of Charcot-Marie-Tooth Disease Type 1A (CMT1A) including verified documentation of genetic testing showing duplication of the PMP22 gene by an accredited/certified laboratory (according to local regulations)
- Have detectable upper extremity nerve conduction velocities (sensory and/or motor) in at least one extremity at screening.
- CMTNSv2R score \>2 and d20 in at least one assessment, confirmed either at the baseline visit or documented in the medical record within the 6 months prior to baseline.

... and 1 more (see full listing online)

Exclusion (7)

- Unable to communicate well with the investigator, to understand and comply with the visits and procedures of the study.
- History of cardiac, renal, liver, hematological, immune system disorders.
- Pregnant/nursing female participants. Women of child-bearing potential, defined as all women physiologically capable of becoming pregnant, unless they are using highly effective methods of contraception during dosing and for the duration of the follow-up period. Sexually active males unless they use a condom during intercourse.
- Inability or unwillingness to provide serial skin biopsy samples.
- Inability or unwillingness to undergo repeated venipuncture or in the opinion of the investigator, participant would be at an increased risk for adverse events related to these procedures.

... and 2 more (see full listing online)

Locations (3 total)

Novartis Investigative Site, Ottawa, Ontario, Canada
Montreal Neurological Institute, Montreal, Quebec, Canada
CIUSS de l´Estrie-CHUS- Hôpital Fleurimont, Sherbrooke, Quebec, Canada