Safety and Efficacy of Tideglusib in Congenital or Childhood Onset Myotonic Dystrophy

NCT05004129

Status	RECRUITING
Phase	Phase 2, Phase 3
Sponsor	AMO Pharma Limited
Enrollment	76 participants

Key Eligibility Criteria

Inclusion (13)

- Subjects who do not enter this study directly from completing the AMO-02-MD-2-003 study (i.e. subjects who did not complete AMO-02-MD-2-003, subjects who completed AMO-02-MD-2-003 but did not directly rollover or subjects who are re-entering AMO-02-MD-2-004), will not be considered eligible for the study without meeting all of the criteria below:
- Subjects under study must be individuals with a diagnosis of Congenital or Childhood Onset DM1.
- Diagnosis must be genetically confirmed
- Subjects must be male or female aged e6 years to d45 years at Screening
- Subjects must have a Clinical Global Impression - Severity (CGI-S) score of 3 or greater at Screening (V-1)

... and 8 more (see full listing online)

Exclusion (7)

- Body mass index (BMI) less than 13.5 kg/m² or greater than 40 kg/m²
- New or change in medications/therapies within 4 weeks prior to Eligibility/Baseline Visit
- Use within 4 weeks prior to Eligibility/Baseline Visit of strong CYP3A4 inhibitors (eg.clarithromycin, telithromycin, ketoconazole, itraconazole, posaconazole, nefazodone, idinavir and ritonavir)
- Concurrent use of drugs metabolized by CYP3A4 with a narrow therapeutic window (e.g. warfarin and digitoxin)
- Current enrollment in a clinical trial of an investigational drug or enrollment in a clinical trial of an investigational drug in the last 6 months other than the AMO-02- MD-2-003 study

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

Locations (14 total)

Arkansas Children's Hospital, Little Rock, Arkansas, United States
University of California, Los Angeles (UCLA), Los Angeles, California, United States
Stanford University, Palo Alto, California, United States
... and 11 more locations