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

Status: RECRUITING

Eligibility Criteria

Age: 6 years to 45 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

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: 1. Subjects under study must be individuals with a diagnosis of Congenital or Childhood Onset DM1. 2. Diagnosis must be genetically confirmed 3. Subjects must be male or female aged ≥6 years to ≤45 years at Screening 4. Subjects must have a Clinical Global Impression

•Severity (CGI-S) score of 3 or greater at Screening (V-1) 5. Written, voluntary informed consent must be obtained before any study related procedures are conducted. Where a parent or legally authorized representative (LAR) provides consent, there must also be assent from the subject (as required by local regulations) 6. Subject's caregiver must be willing and able to support participation for duration of study 7. Subject must be willing and able to comply with the required food intake restrictions as outlined per protocol Subjects entering directly from completing the antecedent AMO-02-MD-2-003 study will not be considered eligible for the study without meeting all of the criteria below: 1. Subjects who have completed the antecedent AMO-02-MD-2-003 study through V11 2. Written, voluntary informed consent must be obtained before any study related procedures are conducted. Where a parent or LAR provides consent, there must also be assent from the subject (as required by local regulations) 3. Subject's caregiver must be willing and able to support participation for duration of study 4. Subject must be willing and able to comply with the required food intake restrictions as outlined per protocol Key

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Exclusion Criteria:

1. Body mass index (BMI) less than 13.5 kg/m² or greater than 40 kg/m² 2. New or change in medications/therapies within 4 weeks prior to Eligibility/Baseline Visit 3. Use within 4 weeks prior to Eligibility/Baseline Visit of strong CYP3A4 inhibitors (eg.clarithromycin, telithromycin, ketoconazole, itraconazole, posaconazole, nefazodone, idinavir and ritonavir) 4. Concurrent use of drugs metabolized by CYP3A4 with a narrow therapeutic window (e.g. warfarin and digitoxin) 5. 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 6. Existing or historical medical conditions or complications (eg. neurological, cardiovascular, renal, hepatic, gastrointestinal, endocrine or respiratory disease) that may impact the interpretability of the study results 7. Hypersensitivity to tideglusib or any components of its formulation including allergy to strawberry

Conditions & Interventions

Interventions:
DRUG: Tideglusib
Conditions:
Congenital Myotonic Dystrophy
Keywords:
Tideglusib, AMO-02-MD-2-004, Congenital Myotonic Dystrophy, Myotonic Dystrophy, Dystrophia Myotonica, Myotonia Atrophica, Myotonia Dystrophica, Myotonic Dystrophy, Congenital, Steinert Disease, Steinert Myotonic Dystrophy, Steinert's Disease

More Information

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