Givinostat in Duchenne's Muscular Dystrophy Long-term Safety and Tolerability Study

Status: RECRUITING

Eligibility Criteria

Age: 7 years and over

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

1. Must have participated in one of the previous studies with GIVINOSTAT in DMD and have attended the End of Study Visit or must have been screened in study DSC/14/2357/48 and met: * all the inclusion criteria and none of the exclusion criteria, * had a baseline vastus lateralis muscle fat fraction (VL MFF) assessed by MRS in the range ≤5% or \>30%, i.e. included in"off-target" group, * never been randomized because, the enrollment in the off target group was completed. 2. Aged ≥6 years old; 3. Are able to give informed assent and/or consent in writing signed by the subject and/or parent/legal guardian (according to localregulations); 4. Subjects must be willing to use adequate contraception: * Contraceptive methods must since the previous GIVINOSTAT study through 3 months after the last dose of study drug, and include the following: * True abstinence (absence of any sexual intercourse), when in line with the preferred and usual lifestyle of the subject. * Periodic abstinence (e.g. calendar, ovulation, symptothermal, postovulation methods) and withdrawal are not acceptable methods of contraception. * Condom with spermicide and the female partner must use an acceptable method of contraception, such as an oral, * transdermal, injectable or implanted steroid-basedcontraceptive, or a diaphragm or a barrier method of contraception in conjunction with spermicidal jelly such asfor example cervical cap with spermicide jelly.

Exclusion Criteria:

1. Use of any pharmacologic treatment, other than corticosteroids, that might have had an effect on muscle strength or function within 3 months prior to be enrolled in this study (e.g., growth hormone); Vitamin D, calcium, and any other supplements will be allowed; 2. Use of any current investigational drug other than Givinostat; 3. Have presence of other clinically significant disease, which, in the Investigator's opinion, could adversely affect the safety of the subject, making it unlikely that the course of treatment or follow-up would be completed, or could impair the assessment of study results; 4. Have a diagnosis of other uncontrolled neurological diseases or presence of relevant uncontrolled somatic disorders that are not related to DMD; 5. Have platelets count, White Blood Cell and Hemoglobin at screening \< Lower Limit of Normal (LLN)* (for abnormal screening laboratory test results (\ 300 mg/dL (3.42 mmol/L) in fasting condition at screening visit* (for abnormal screening laboratory test results (\>300 mg/dL), the triglycerides will be repeated once; if the repeat test result is still \>300 mg/dL, then exclusionary); 7. Have inadequate renal function, as defined by serum Cystatin C \>2 x the upper limit of normal (ULN) at screening visit*. If the value is \>2 x ULN, the serum Cystatin C will be repeated once; if the repeated test result is still \>2 x ULN, the subject should be excluded); 8. Have heart failure (New York Heart Association Class III or IV) 9. Have a current liver disease or impairment, including but not limited to an elevated total bilirubin* (i.e. \> 1.5 x ULN), unless secondary to Gilbert disease or pattern consistent with Gilbert's; 10. Have a baseline QTcF \>450 msec, (as the mean of 3 consecutive readings 5 minutes apart) or history of additional risk factors for torsades de pointes (e.g., heart failure, hypokalemia, or family history of long QT syndrome); 11. Have a psychiatric illness/social situation rendering the potential subject unable to understand and comply with the muscle function tests and/or with the study protocol procedures. 12. Have any hypersensitivity to the components of study medication; 13. Have a sorbitol intolerance or sorbitol malabsorption or have the hereditary form of fructose intolerance. * the Investigators to evaluate these exclusion criteria can use the laboratory results obtained within 5 months from V1, to allow the continuity of the treatment. It is worth noting, as soon as the site will receive the laboratory results done in screening/baseline (Visit 1) visit they will check the GIVINOSTAT dose and modify it as per protocol safety rules and/or dosage modifications rules.

Conditions & Interventions

Interventions:
DRUG: Givinostat
Conditions:
Duchenne Muscular Dystrophy

More Information

Contact(s): Reference Study ID Number: DSC/14/2357/51 - patientadvocacy@italfarmaco.com
Principal Investigator: Harper, Amy
IRB Number: HM20018448
System ID: NCT03373968

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