

A Study to Investigate the Safety, Tolerability, and Efficacy of SAR446268, an Adeno-associated Viral Vector-mediated Gene Therapy in Participants Aged 10 to 50 Years of Age With Non-congenital Myotonic Dystrophy Type 1

Status: RECRUITING

Eligibility Criteria

Age: 10 years to 50 years old

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

Participants are eligible to be included in the study only if all of the following criteria apply: * For Part A, participants must be 18 to 50 years of age inclusive, at the time of signing the informed consent. * For Part B, participants must be as follows: * 10 to 17 years of age inclusive, at the time of signing the informed consent or, * 18 to 50 years of age inclusive, at the time of signing the informed consent. * Participants with non-congenital onset DM1 * Participants presenting with signs of DM1 including myotonia and muscle weakness, as diagnosed previously by a clinician based on medical history. * Participants with genetic diagnosis of DM1 [cytosine-thymine-guanine (CTG) repeat length ≥ 50 in one allele from medical history] * Participants who can walk independently for at least 10 meters at screening (orthoses and ankle braces allowed). * Participants who have been classified according to cardiac risk by the Investigator as: * Moderate risk participants with pacemaker and/or implantable cardioverter-defibrillator (ICD) for Part A * Low, moderate, or high cardiac risk for Part B

Exclusion Criteria:

Participants are excluded from the study if any of the following criteria apply: * Participants with neutralizing antibodies against the AAV.SAN011 capsid * Participants with left ventricular ejection fraction (LVEF) $< 50\%$ * Participants with liver or biliary disease defined as having at least one of the following: * Alanine aminotransferase (ALT) $> 2 \times$ ULN and aspartate aminotransferase (AST) $> 2 \times$ ULN * Alkaline phosphatase $> 2 \times$ ULN * Total bilirubin $> 1.5 \times$ ULN (unless has a genetically confirmed diagnosis of Gilbert's syndrome) * Direct bilirubin $\geq 1.5 \times$ ULN * Participants with International normalized ratio > 1.5 * Participants with renal disease defined as: • Serum creatinine $> 1.5 \times$ ULN and/or estimated glomerular filtration rate < 60 mL/min/1.73 m² as determined by Chronic Kidney Disease Epidemiology Collaboration (CKD-EPI) (2021) for those age ≥ 18 years and Bedside Schwartz Equation for those < 18 years * Participants with chronic respiratory insufficiency and on non-invasive ventilatory support, nighttime ventilatory support or full-time ventilation. * Participants with contraindication to corticosteroid or with conditions that could worsen in the presence of corticosteroids, as determined by the Investigator. * Participants with active hepatitis B or C infection; HBsAg (+), or HCV RNA (+), or current antiviral therapy for either. * Participants with HBcAb (+) who are not amenable for prophylactic anti-HBV therapy or pre-emptive therapy guided by serial HBV DNA monitoring during the corticosteroids therapy. * Participants at high risk for tuberculosis reactivation during the corticosteroids therapy as determined by the Investigator. * Participants with a known HIV infection * Participants with serious intercurrent illness that, in the opinion of the Investigator, would preclude participation in the study or potentially decrease survival. * Participants with recent history of or current drug or alcohol abuse in the past 12 months prior to screening. * Participants with history of tibialis anterior biopsy within 12 weeks from Day 1 or planning to undergo tibialis anterior biopsies during the duration of this clinical trial. * Participants with significant developmental delay, intellectual disability, or behavioral neuropsychiatric manifestations as determined by the Investigator. * Participants with previous systemic corticosteroids treatment at doses of > 5 mg/day within 15 days of Day 1 * Participants with previous treatment with anti-myotonic medication within 15 days of Day 1 * Participants not suitable for participation, whatever the reason, as judged by the Investigator, including medical or clinical conditions, or participants potentially at risk of noncompliance to study procedures. The above information is not intended to contain all considerations relevant to a participant's potential participation in a clinical trial.

Conditions & Interventions

Interventions:

BIOLOGICAL: SAR446268

Conditions:

Myotonic Dystrophy

More Information

Contact(s): Trial Transparency email recommended (Toll free for US & Canada) - contact-us@sanofi.com

Principal Investigator:

IRB

Number:

System ID: NCT06844214

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