

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

NCT06844214

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	Sanofi
Enrollment	32 participants

Key Eligibility Criteria

Inclusion (11)

- 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:
 - to 17 years of age inclusive, at the time of signing the informed consent or,
 - to 50 years of age inclusive, at the time of signing the informed consent.
 - Participants with non-congenital onset DM1
- ... and 6 more (see full listing online)

Exclusion (24)

- Participants with neutralizing antibodies against the AAV.SAN011 capsid
 - Participants with left ventricular ejection fraction (LVEF) $\lt 50\%$
 - Participants with liver or biliary disease defined as having at least one of the following:
 - Alanine aminotransferase (ALT) $\gt 2$ x ULN and aspartate aminotransferase (AST) $\gt 2$ x ULN
 - Alkaline phosphatase $\gt 2$ x ULN
- ... and 19 more (see full listing online)

Locations (8 total)

University of Florida, 2004 Mowry Road - Site Number: 8400005, Gainesville, Florida, United States
University of South Florida - Neuromuscular Research, 13330 USF Laurel Drive - Site Number: 8400001, Tampa, Florida, United States
Columbia University Medical Center - Neurological Institute, 710 W. 168th, 2nd floor, suite 204 - Site Number : 8400003, New York, New York, United States
... and 5 more locations

<https://clinicaltrials.gov/study/NCT06844214>

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