A Phase 3 Global Study to Evaluate AOC 1001 for the Treatment of Myotonic Dystrophy Type 1

Estado	Tipo de Participantes	Rangos de Edad
EC Finalizado	Sujetos incapaces de otorgar consentimiento	Menores de 18 , Adultos (18-64 años)
Género	**Fases**	**Participantes esperados**
Ambos	Fase III	111
Resultados	**Bajo nivel intervención**	**Enfermedad rara**
Sin resultados	No	Si
Cobertura geográfica	**Ámbitos del ensayo**	**Terapia avanzada**
Sin determinar	seguridad, eficacia, farmacocinética	NO

Información

Identificador
2024-511378-60-00 (CTIS)

Cod. Protocolo
AOC 1001-CS3

Área terapéutica
Enfermedades [C] - Enfermedades musculoesqueléticas [C05]|

Enfermedad investigada
Myotonic Dystrophy Type 1

Título Científico
A Phase 3 Randomized, Double-Blind, Placebo-Controlled, Global Study to Evaluate the Efficacy and Safety of Intravenous AOC 1001 for the Treatment of Myotonic Dystrophy Type 1

Justificación

No aportado

Objetivo Principal

To evaluate the efficacy of AOC 1001 on hand function.

Variables de Evaluación Primaria

Change from baseline to Week 54 in video Hand Opening Time (vHOT).

Momentos temporales de evaluación primaria

No aportado

Objetivo Secundario

To evaluate the efficacy of AOC 1001 on measures of mobility, muscle strength, muscle function, and patient-reported outcomes.

Variables de Evaluación Secundaria

1. Change from baseline to Week 54 in: Hand grip strength. QMT total composite score. DM1-ActivC. 10MWRT
2. Change from baseline to Week 54 in: QMT upper extremity composite score. QMT lower extremity composite score. QMT individual muscle group scores. PGI-S. EQ-5D-5L. PGI-C at Week 54

Momentos temporales de evaluación secundaria

No aportado

Criterios de Inclusión

1. Age: 16 to 65. Age: 18-65 (Denmark, Germany)
2. Clinical and genetic diagnosis (CTG repeat length 100) of DM1.
3. Ability to walk independently (orthoses and ankle braces allowed) for at least 10 meters at screening.

Criterios de Exclusión

1. Breastfeeding, pregnancy, or intent to become pregnant during the study. 2. Unwilling or unable to comply with contraceptive requirements. 3. Abnormal lab values, conditions or diseases that would make the participant unsuitable for the study. 4. Diabetes that is not adequately controlled. 5. History of decompensated heart failure within 3 months of screening. Participants with preexisting pacemaker/ICD are not excluded. 6. Body Mass Index > 35 kg/m2 at Screening. 7. Recently treated with an investigational drug or biological agent. 8. Treatment with anti-myotonic medication within 5 half-lives or 14 days of baseline, whichever is longer, prior to baseline. 9. Note: Additional protocol defined inclusion and exclusion criteria apply.

Calendario

(Última actualización: 13/08/2026)

Autorización	Inicio de Ensayo	Inclusión Primer Paciente	Interrumpido	Reiniciado
08/08/2024	28/03/2025	10/04/2025	No aportado	No aportado

Fin de reclutamiento	Fin prematuro (España)	Fin prematuro (Global)	Fin del ensayo en España	Fin del ensayo global
28/07/2025	No aportado	No aportado	13/07/2026	29/07/2026

Promotor

Avidity Biosciences Inc. United States

3020 Callan Road

Contact Person

Avidity Biosciences Inc.

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Monetary support: N/A

Tipo de promotor: Comercial

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Centros

Activo (28/03/2025)

Hospital Universitario Donostia

Donostia

GUIPÚZCOA

Neurology

Medicamentos

AOC 1001
SOLUTION FOR INFUSION
INTRAVENOUS

-

Principios Activos: N/A

Huérfano **Experimental**

AOC 1001
SOLUTION FOR INFUSION
INTRAVENOUS

-

Principios Activos: N/A

Huérfano **Experimental**

Sin resultados

A Phase 3 Global Study to Evaluate AOC 1001 for the Treatment of Myotonic Dystrophy Type 1

State	Type of participants	Age Ranges
Finished CT	Incapable subjects of giving consent	Less than 18 , Adults (18-64 years)
Gender	**Phases**	**Expected Participants**
Both	Phase III	111
Results	**Low level of intervention**	**Rare disease**
No results	No	Yes
Geographic coverage	**Areas of the study**	**Advanced therapy**
Undetermined	safety, effectiveness, pharmacokinetics	NO

Information

Identifier
2024-511378-60-00 (CTIS)

Protocol Code
AOC 1001-CS3

Therapeutic area
Diseases [C] - Musculoskeletal Diseases [C05]|

Investigated Disease
Myotonic Dystrophy Type 1

Scientific Title
A Phase 3 Randomized, Double-Blind, Placebo-Controlled, Global Study to Evaluate the Efficacy and Safety of Intravenous AOC 1001 for the Treatment of Myotonic Dystrophy Type 1

Rationale

Not provided

Main Objective

To evaluate the efficacy of AOC 1001 on hand function.

Primary Endpoints

Change from baseline to Week 54 in video Hand Opening Time (vHOT).

Temporary moments of secondary assessment

Not provided

Secondary Objective

To evaluate the efficacy of AOC 1001 on measures of mobility, muscle strength, muscle function, and patient-reported outcomes.

Secondary Endpoints

1. Change from baseline to Week 54 in: Hand grip strength. QMT total composite score. DM1-ActivC. 10MWRT
2. Change from baseline to Week 54 in: QMT upper extremity composite score. QMT lower extremity composite score. QMT individual muscle group scores. PGI-S. EQ-5D-5L. PGI-C at Week 54

Temporary moments of secondary assessment

Not provided

Inclusion criteria

1. Age: 16 to 65. Age: 18-65 (Denmark, Germany)
2. Clinical and genetic diagnosis (CTG repeat length 100) of DM1.
3. Ability to walk independently (orthoses and ankle braces allowed) for at least 10 meters at screening.

Exclusion criteria

1. Breastfeeding, pregnancy, or intent to become pregnant during the study. 2. Unwilling or unable to comply with contraceptive requirements. 3. Abnormal lab values, conditions or diseases that would make the participant unsuitable for the study. 4. Diabetes that is not adequately controlled. 5. History of decompensated heart failure within 3 months of screening. Participants with preexisting pacemaker/ICD are not excluded. 6. Body Mass Index > 35 kg/m2 at Screening. 7. Recently treated with an investigational drug or biological agent. 8. Treatment with anti-myotonic medication within 5 half-lives or 14 days of baseline, whichever is longer, prior to baseline. 9. Note: Additional protocol defined inclusion and exclusion criteria apply.

Calendar

(Last Update: 13/08/2026)

Authorization	Start of Trial	First patient inclusion	Halted	Restarted
08/08/2024	28/03/2025	10/04/2025	Not aported	Not aported

End of recruitment	Premature end (Spain)	Premature End (Global)	Trial end (Spain)	Trial end (Global)
28/07/2025	Not aported	Not aported	13/07/2026	29/07/2026

Sponsor

Avidity Biosciences Inc. United States

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Contact Person

Avidity Biosciences Inc.

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Monetary support: N/A

Sponsor type: Commercial

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Sites

Active (28/03/2025)

Hospital Universitario Donostia

Donostia

GUIPÚZCOA

Neurology

Medication

AOC 1001
SOLUTION FOR INFUSION
INTRAVENOUS

-

Active Principles: N/A

Orphan **Experimental**

AOC 1001
SOLUTION FOR INFUSION
INTRAVENOUS

-

Active Principles: N/A

Orphan **Experimental**

No results