

A phase I trial of DT-818 in healthy volunteers and adults with myotonic dystrophy type 1

Submission date 05/11/2025	Recruitment status Recruiting	☒ Prospectively registered ☐ Protocol
Registration date 07/11/2025	Overall study status Ongoing	☐ Statistical analysis plan ☐ Results
Last Edited 27/07/2026	Condition category Nervous System Diseases	☐ Individual participant data ☒ Record updated in last year

Plain English summary of protocol

Background and study aims

This is a phase I, open-label, multiple dose-escalation study to assess the safety, tolerability, and pharmacokinetics (PK; how a drug moves through the body) of DT-818 in adult healthy participants, and in adult patients with myotonic dystrophy

Who can participate?

Healthy adult volunteers and adult patients with genetically confirmed myotonic dystrophy Type 1 (DM1).

What does the study involve?

The trial includes subcutaneous (SC) infusion and intravenous (IV) infusion administration routes, with multiple ascending dose cohorts. Assessments include adverse events, vital signs, ECGs, labs, and PK sampling.

What are the possible benefits and risks of participating?

Benefits and risks not provided at registration

Where is the study run from?

Design Therapeutics, Inc.

When is the study starting and how long is it expected to run for?

September 2025 to December 2027

Who is funding the study?

Design Therapeutics, Inc.

Who is the main contact?

Study Manager, info@designtx.com

Contact information

Type(s)

Principal investigator

Contact name

Dr Thomas Brankin-Frisby

Contact details

Level 4, 10 St Andrews Place
East Melbourne
Australia
3121
+61 (03) 9970 4200
tfrisby@dohertyclinicaltrials.com

Type(s)

Public, Scientific

Contact name

None Design Therapeutics Study Manager

Contact details

6005 Hidden Valley Road
Suite 110
Carlsbad
United States of America
92011
+1 858-293-4900
info@designtx.com

Additional identifiers

Protocol serial number

DTX-818-101

Study information

Scientific Title

A phase 1, open-label trial to assess the safety and pharmacokinetics of multiple ascending doses of DT-818 in healthy volunteers and participants with myotonic dystrophy type 1

Study objectives

To assess the safety, tolerability, and pharmacokinetics (PK) of DT-818

Ethics approval required

Ethics approval required

Ethics approval(s)

1. Approved 29/10/2025, Bellberry Human Research Ethic Committee (Level 1, 196 Greenhill Road Eastwood, Adelaide, 5063, Australia; +61 08 8361 3222; bellberry@bellberry.com.au), ref: DTX-818-101

2. Approved 18/02/2026, Ministry of Health (133 Molesworth Street PO Box 5013, Wellington, 6011, New Zealand; Not provided at registration; hdec@health.govt.nz), ref: 2025 FULL 24545

3. Approved 23/01/2026, Advarra CANADA Office (125 Don Hillock Drive, Unit 18, Aurora, Ontario, L4G 0H8, Canada; Not provided at registration; cirbi@advarra.com), ref: Pro000092586

Primary study design

Interventional

Study design

Open-label non-randomized interventional phase I multicenter study

Study type(s)

Safety, Other

Health condition(s) or problem(s) studied

Myotonic dystrophy (DM1)

Interventions

DT-818 will be administered in an open-label fashion to healthy participants (Cohort 1) and then to participants with myotonic dystrophy type 1 (DM1, Cohorts 2-6). Each sequential Cohort will receive weekly administrations of DT-818, intravenously or subcutaneously, in a 28-day cycle. The first and 5th doses will require an overnight stay for full PK analysis. Other doses will be administered as an outpatient in the research unit. The dose and subsequent dose escalations will be determined by a safety review committee. DM1 participants will undergo a muscle biopsy before and at the end of treatment to look for the impact of DT-818 on splicing. Total study participation duration is estimated to be approximately four months.

Intervention Type

Drug

Phase

Phase I

Drug/device/biological/vaccine name(s)

DT-818

Primary outcome(s)

Safety of DT-818 measured using data collected from electronic Case Report Forms (CRF), including incidence of Treatment Emergent Adverse Events (TEAEs), including Serious Adverse Events (SAEs), from enrollment to end of study

Key secondary outcome(s)

Characterization of the PK profile of DT-818 measured using plasma PK parameters, including but not limited to AUClast, AUCinf, Cmax, t1/2, from first dose to end of study, from enrollment to end of study

Completion date

01/12/2027

Eligibility

Key inclusion criteria

Healthy Volunteers (Cohort 1)

1. Male or female participants, 18–55 years of age, inclusive.
2. Medically healthy with no clinically significant abnormalities based on medical history, physical exam, vital signs, ECG, and clinical laboratory tests at screening.
3. Negative tests for drugs of abuse, alcohol, hepatitis B surface antigen, hepatitis C antibody, and HIV.
4. Willing and able to comply with study restrictions and provide written informed consent.
5. Women of childbearing potential must use highly effective contraception from screening through the end of the study; men must use contraception and refrain from sperm donation for the same period.

DM1 Participants (Cohort 2 and beyond)

1. Adults 18–65 years of age with genetically confirmed Myotonic Dystrophy Type 1 (DM1).
2. Stable clinical status and medications for ≥ 1 month prior to screening.
3. Able and willing to comply with study visits, assessments, and treatment requirements.
4. Provides written informed consent (and caregiver consent, if applicable).

Participant type(s)

Healthy volunteer, Patient

Healthy volunteers allowed

No

Age group

Mixed

Lower age limit

18 Years

Upper age limit

65 Years

Sex

All

Total final enrolment

0

Key exclusion criteria

1. History or presence of any clinically significant cardiovascular, hepatic, renal, gastrointestinal, respiratory, hematologic, or neurologic disease (other than DM1 for patient cohorts).
2. Clinically significant ECG findings per protocol defined limits
3. Abnormal laboratory values at screening or per protocol-defined laboratory limits.
4. Participation in another investigational drug or device study within 90 days prior to dosing.
5. Positive test for alcohol or drugs of abuse at screening or admission.

6. Pregnant or breastfeeding women.

7. Any condition that, in the opinion of the investigator, would make the participant unsuitable for the study.

Date of first enrolment

12/11/2025

Date of final enrolment

01/09/2027

Locations

Countries of recruitment

Australia

Canada

New Zealand

Study participating centre

Doherty Clinical Trials

Melbourne

Australia

3002

Study participating centre

Scientia Clinical Research

Sydney

Australia

2031

Study participating centre

Momentum Clinical Research

New Zealand

0740

Study participating centre

BioPharma Services Inc.

Canada

M9L 3A2

Sponsor information

Organisation

Design Therapeutics, Inc.

Funder(s)

Funder type

Industry

Funder Name

Design Therapeutics, Inc.

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary

Not expected to be made available