

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

<b>Submission date</b> 05/11/2025	<b>Recruitment status</b> Recruiting	<input checked="" type="checkbox"/> Prospectively registered <input type="checkbox"/> Protocol
<b>Registration date</b> 07/11/2025	<b>Overall study status</b> Ongoing	<input type="checkbox"/> Statistical analysis plan <input type="checkbox"/> Results
<b>Last Edited</b> 18/11/2025	<b>Condition category</b> Nervous System Diseases	<input type="checkbox"/> Individual participant data <input checked="" type="checkbox"/> 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](mailto:info@designtx.com)

## Contact information

Type(s)

Principal investigator

**Contact name**

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## **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**

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**Ethics approval(s)**

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

## Study design

Open-label non-randomized interventional phase I multicenter study

## Primary study design

Interventional

## 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  $\geq 1$  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

### Study participating centre

#### Doherty Clinical Trials

Melbourne

Australia

3002

### Study participating centre

#### Scientia Clinical Research

Sydney

Australia

2031

## 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

