TrialNet TOPPLE Study

Tolerance using plasmid in people with T1D

Type 1 Diabetes TrialNet

About the Study

This is a Phase 1 study, which means this is the first time the treatment, NNC0361-0041, is being tested for safety in people. The study will enroll 48 adults diagnosed with type 1 diabetes (T1D) in the past 48 months. If this study results in no safety concerns, we plan to conduct a larger study to see if this same treatment can slow down or stop T1D in people at high risk, before clinical diagnosis.

Stages of Clinical Research Development

About the Study Treatment

TrialNet is testing the safety of a new treatment, NNC0361-0041, in adults with T1D. The treatment is a plasmid vector designed to transfer DNA into cells, where it can communicate with the immune system. Earlier studies in the lab show this treatment might retrain the immune system to stop its attack on insulin-producing beta cells.

While plasmids are small circular pieces of DNA, they do not change your DNA. They are currently being studied in many clinical trials for other conditions.

As with any medical intervention, there are risks and benefits to participating in this study. Before you decide to participate, a member of our research team will explain all potential risks and benefits and answer any questions you may have.

For more information, contact your local TrialNet site:
Lucia Alfano, RN at Naomi Berrie Diabetes Center, phone **212.851.5449** or email **lja2141@cumc.columbia.edu.**

Quick Facts

Eligibility Requirements*

- Age 18-45.
- T1D diagnosis in past 48 months.

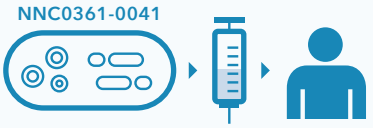
Plasmid Therapy

- This is the first time this treatment is being tested for safety in people. The treatment, NNC0361-0041, is a plasmid therapy.

What's a plasmid?

A plasmid is a circular piece of DNA. A plasmid vector is a delivery agent for the plasmid. Like a truck shipping packages to various locations, plasmid vectors are used to transfer DNA into cells, where it can communicate with the immune system.

- This study is using a plasmid encoding four human proteins.



- Earlier studies in the lab show this treatment working together might retrain the immune system to stop attacking insulin-producing beta cells.
- The plasmid is delivered via injection (like a shot).
- Plasmids are currently being studied in many clinical trials for other conditions.

** Select criteria; please see participant handbook for full list.*

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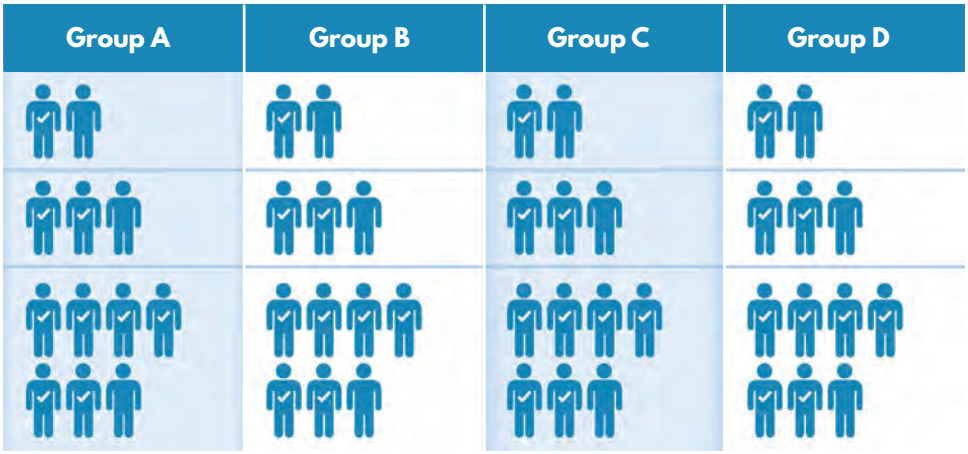
Enrollment Process

Because this is the first time this treatment is being tested in people, it requires a staggered enrollment process that allows TrialNet to evaluate the safety of the treatment at each dose before enrolling additional participants.



Step 1: Interest and Availability

Let us know you are interested and available! Your assigned TrialNet site will work closely with you to make sure you understand the study and time commitment, as well as answer any questions you have.

Step 2: Screening Visit to Confirm Eligibility

Participants will go to a TrialNet site for a screening visit. The results of the screening visit will determine who is eligible to join the study. Because of the unique enrollment process for this study, there may be times that we screen more people than we will need.

Step 3: Enrollment

In each group, participants will enroll and begin treatment in a staggered fashion. We will first enroll 2 participants and evaluate for safety, then 3 and evaluate for safety, then the remaining 7.

- Because a limited number of "slots" are available in each group, it is important to communicate your availability. Your study team will work closely with you and notify you when a slot is available. You can decide at that time if you're ready to continue in the study.
- If we don't hear back from you, or the timing doesn't work for you, we'll move to the next participant in line. Also, depending how long it's been since you had your study screening visit, you may need additional tests to be sure you're still eligible.

Treatment & Follow up

This study has a 12-week treatment phase, followed by 9 months of follow up.

Treatment Phase

FIRST VISIT	VISITS 2-12
48 hours	3-7 hours

- In the treatment phase, you will need to visit a TrialNet clinical research site once a week for 12 weeks.
- At each visit you will receive an injection (treatment or placebo), have blood tests, and be closely monitored.
- For the first visit, you will need to stay at the clinical research site as an inpatient for 48 hours of observation.
- The remaining visits will be outpatient visits. Length of the visit will depend on how long you need to be observed after receiving treatment and which tests are needed.

Follow Up Phase

VISIT	VISIT	VISIT	VISIT
3 months ✓	4 months ✓	6 months ✓	12 months ✓

- In the follow up phase, you will need to make study visits at months 3, 4, 6 and 12. These visits will last 1-4 hours, depending on which tests are needed.

Please refer to the Informed Consent document for complete details.