Correction of Myotonic dystrophy Type 1 in Induced Pluripotent Stem Cells

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Presentation outline

• Motivation for research
• Research strategy
• Data
• What we hope to achieve via our research
How can we study DM in the lab?

**Problem:** we do not have a good human DM *cell model* to study the functional problems of DM-affected *tissues* such as heart or brain that also can be used to develop new DM treatments.

**Solution:** Create two versions of cells from the same patient: one that contains the mutation and one which has had the mutation corrected.
Cellular reprogramming and genome editing

Genome editing

Normal
Normal
Normal

CTG(x5)
CTG(x5)

Antibiotic

Antibiotic

Donor DNA

Expanded
Confirming Targeting by Sequencing

CTG Repeat

Normal Allele

Antibiotic

Modified Allele
What we hope to achieve via our research

- Compare the uncorrected DM heart cells with the corrected cells to define disease-specific characteristics.
- Use the corrected and uncorrected heart cells to screen for drugs with therapeutic effects.
- **Long term goal:** Utilize the corrected heart cells for “customized” tissue repair.
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