Correction of Myotonic dystrophy Type 1 in Induced Pluripotent Stem Cells

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Presentation outline

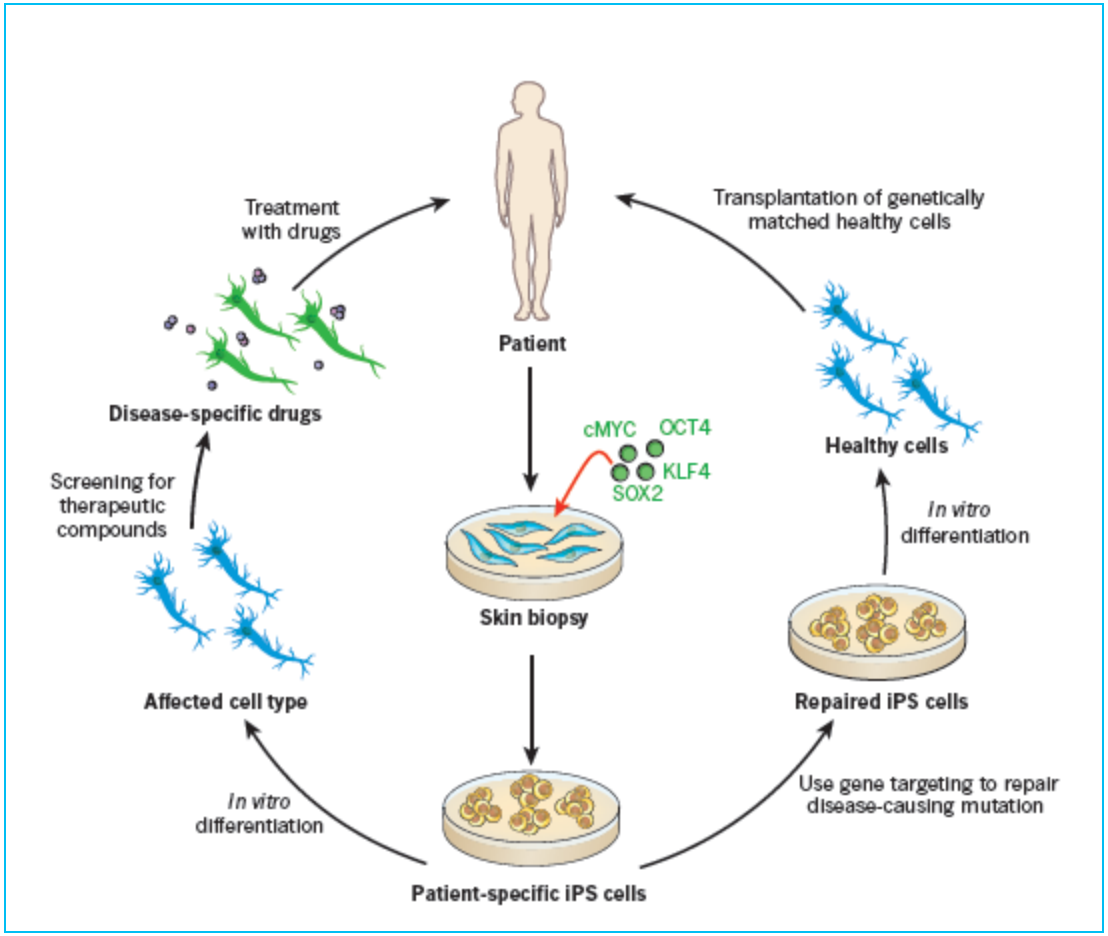
- Motivation for research
- Research strategy
- Data
- What we hope to achieve via our research

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.

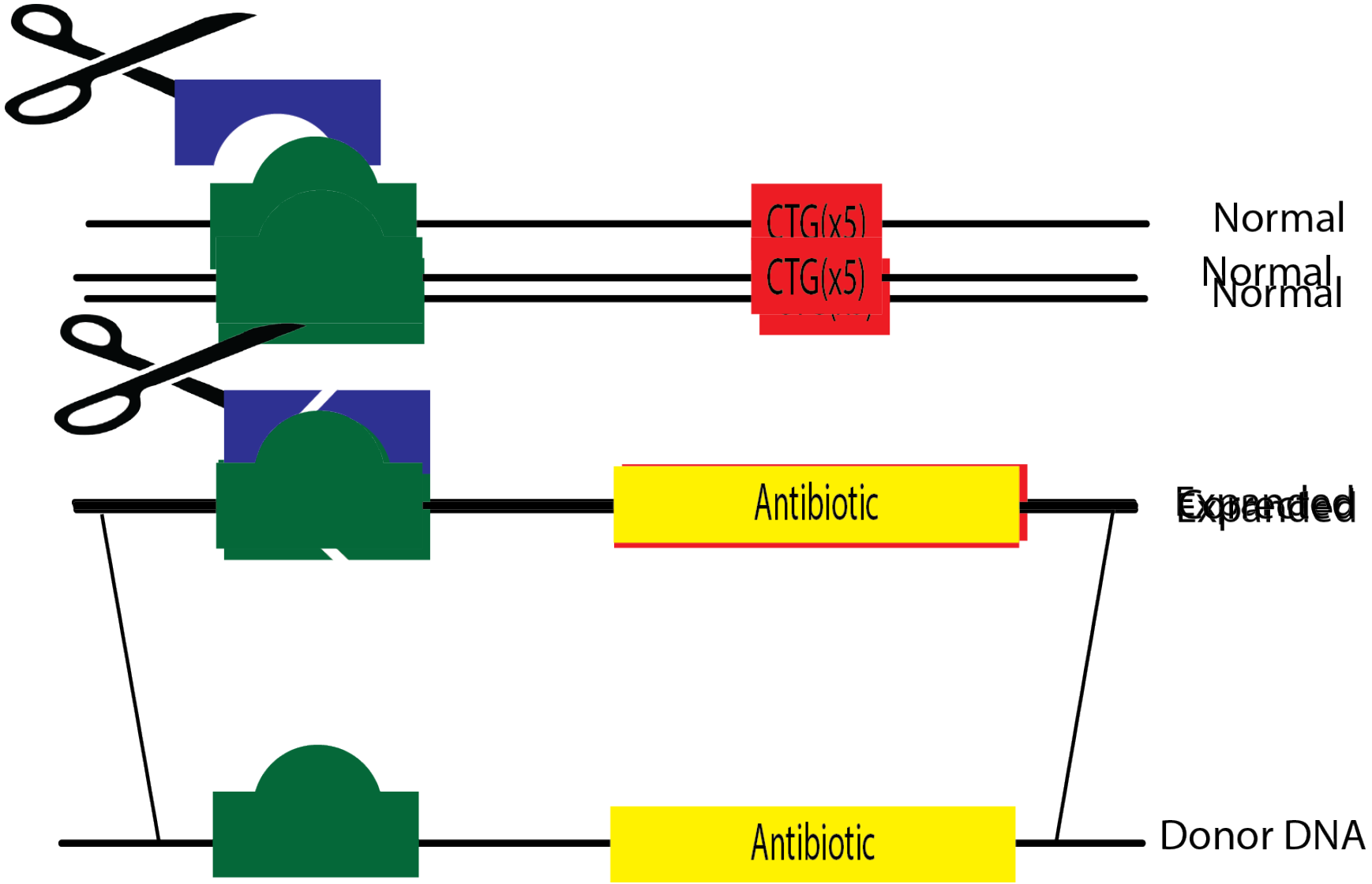
Solution: Create two versions of cells from the same patient: one that contains the mutation and one which has had the mutation corrected.

Cellular reprogramming and genome editing

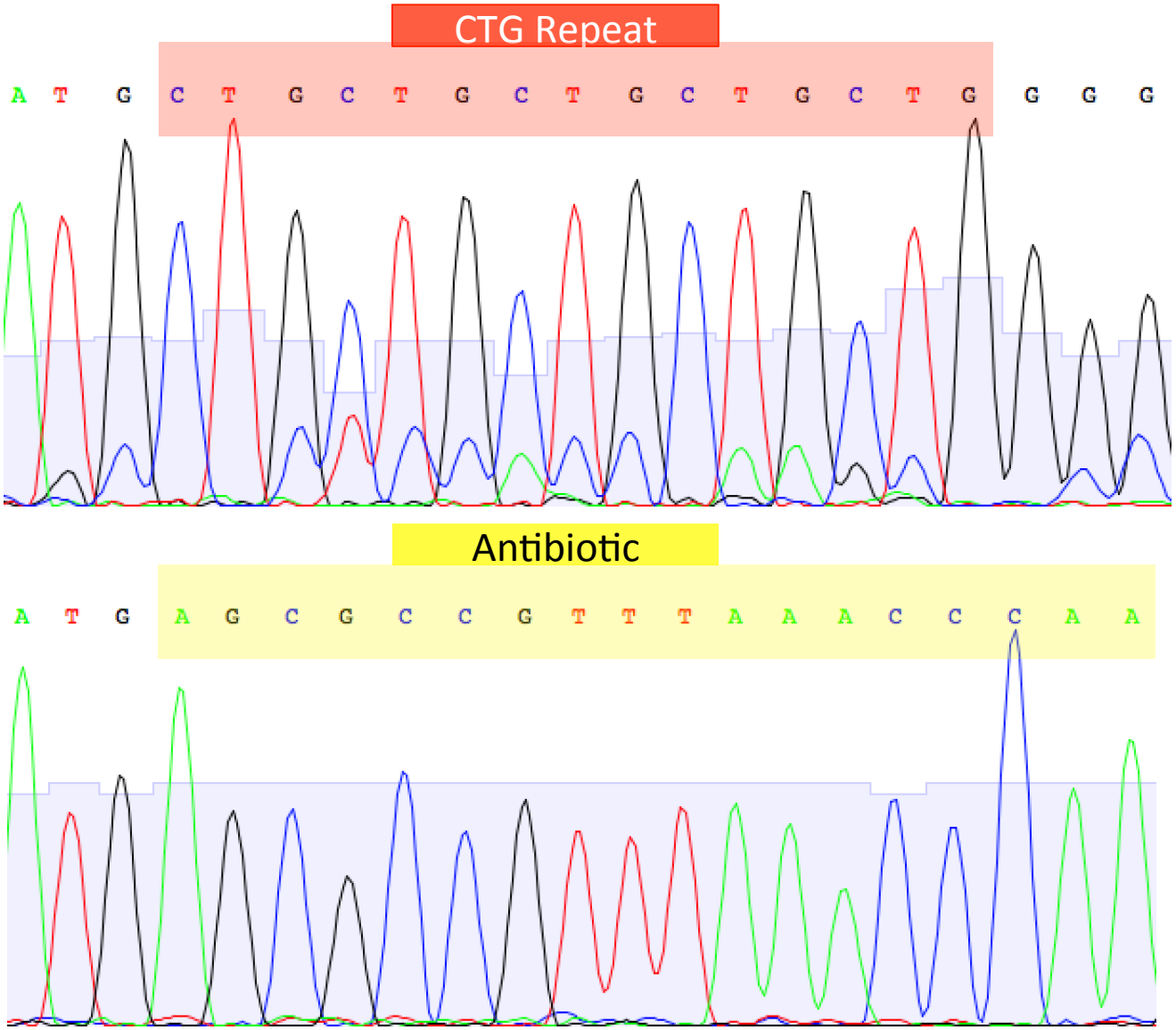


Robinton DA, Daley GQ. Nature. 2012 Jan 18;481(7381):295-305.

Genome editing



Confirming Targeting by Sequencing



Normal Allele

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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