

31. Genetic Engineering in Stem Cell Therapy

1 unit, Inbal Caspi, November 18, 2025

Lesson Outline: Genetic Engineering in Stem Cell Therapy

1. Evaluating a Genetic Disorder as a Candidate for Gene Therapy

- Assess disease features that make it suitable for stem cell-based correction.
- Use *LCA10* as a case study for monogenic, cell-replaceable disorders.

2. Designing the Genetic Correction

- Compare and select editing tools.
- Plan a correction strategy.

3. Validating and Differentiating Cells

- Confirm successful gene correction and restoration of normal function.

4. From Bench to Therapy

- Test functional rescue in animal models.
- Discuss translational, safety, and regulatory steps toward clinical application.