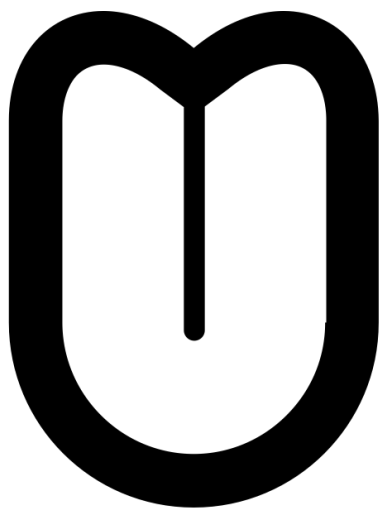
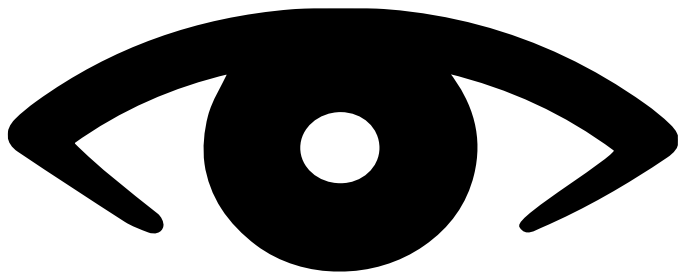


Therapeutic Approaches in Sensory Disorders

Summary of Day 1 Scientific Presentations

Casey Trimmer, PhD





A large, light gray, stylized diagram of a cell with a nucleus and various organelles, positioned in the upper left background.

Stem cell therapy

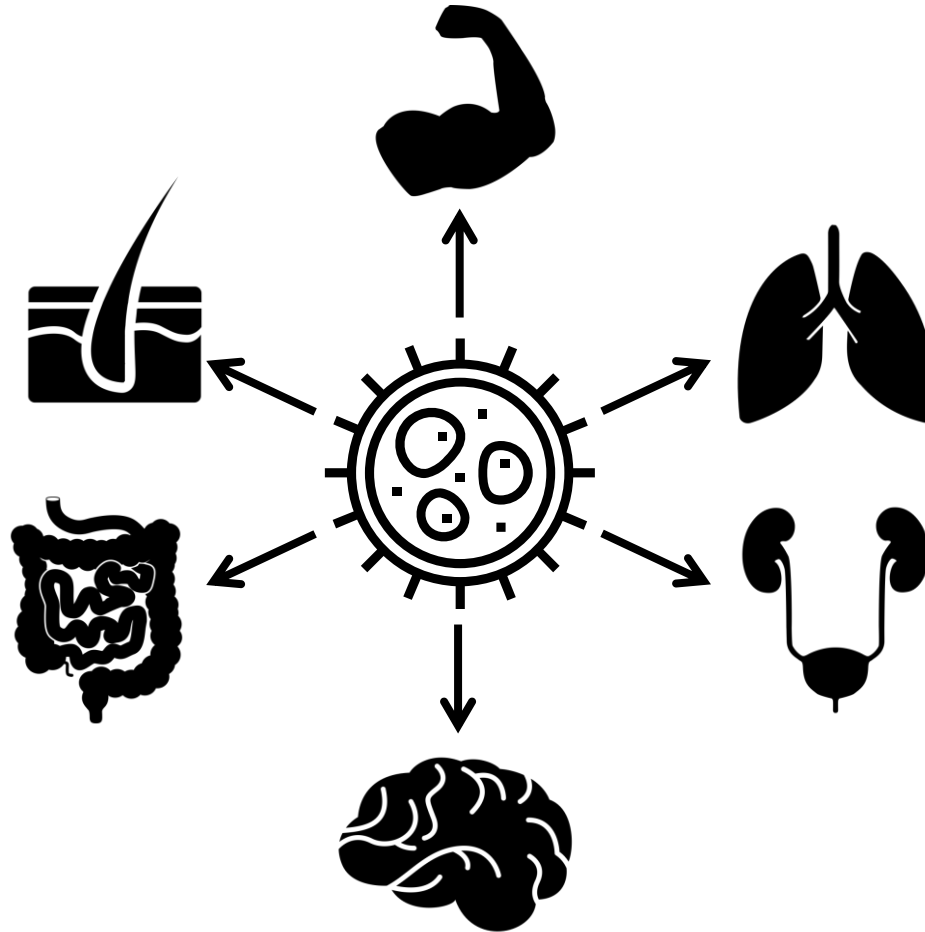
- Replaces **non-functional cells** in the body with healthy cells

Gene therapy

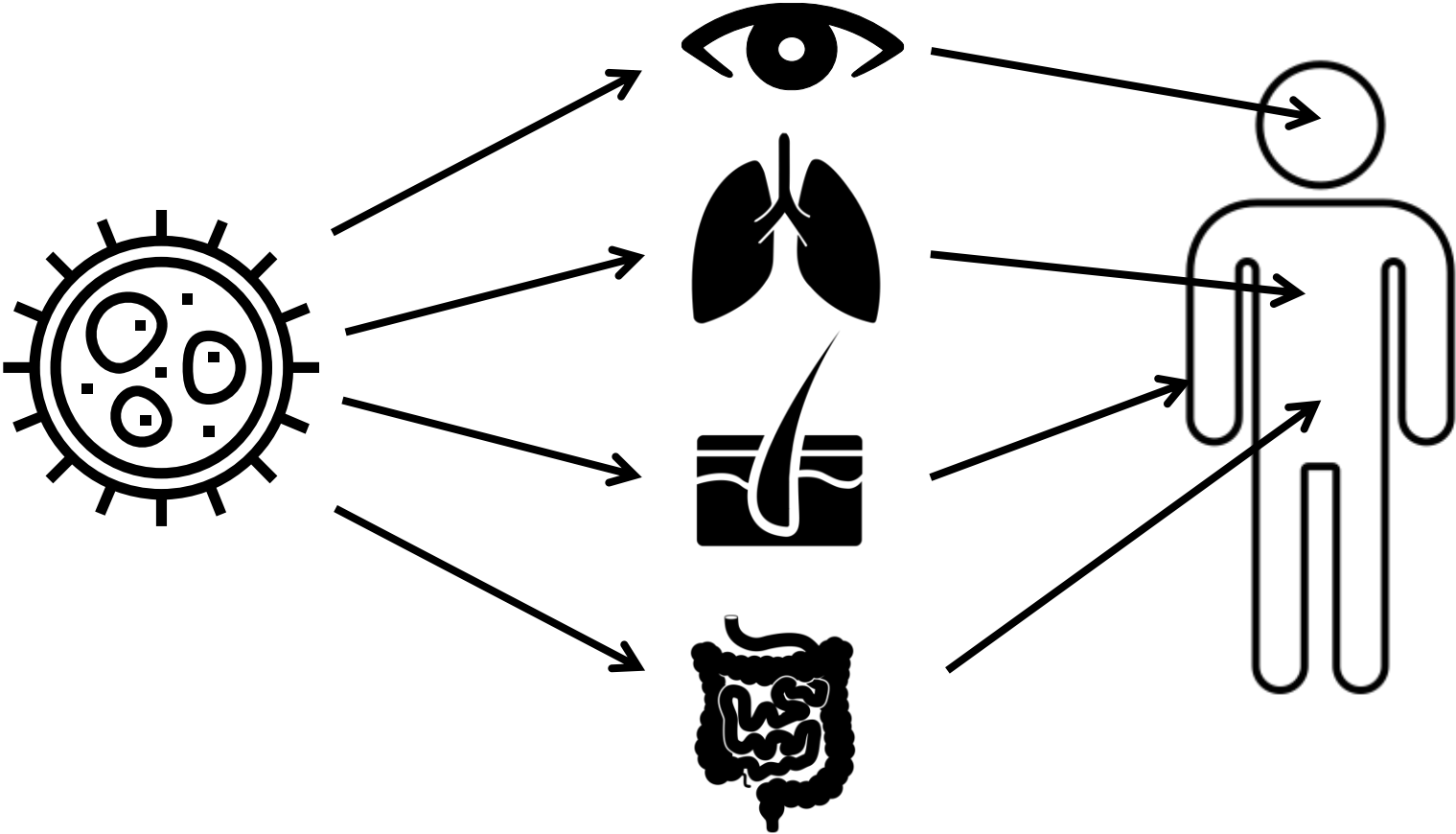
Replaces or fixes a **faulty gene** with a working gene



Stem cells are the precursors for all the cells of the body



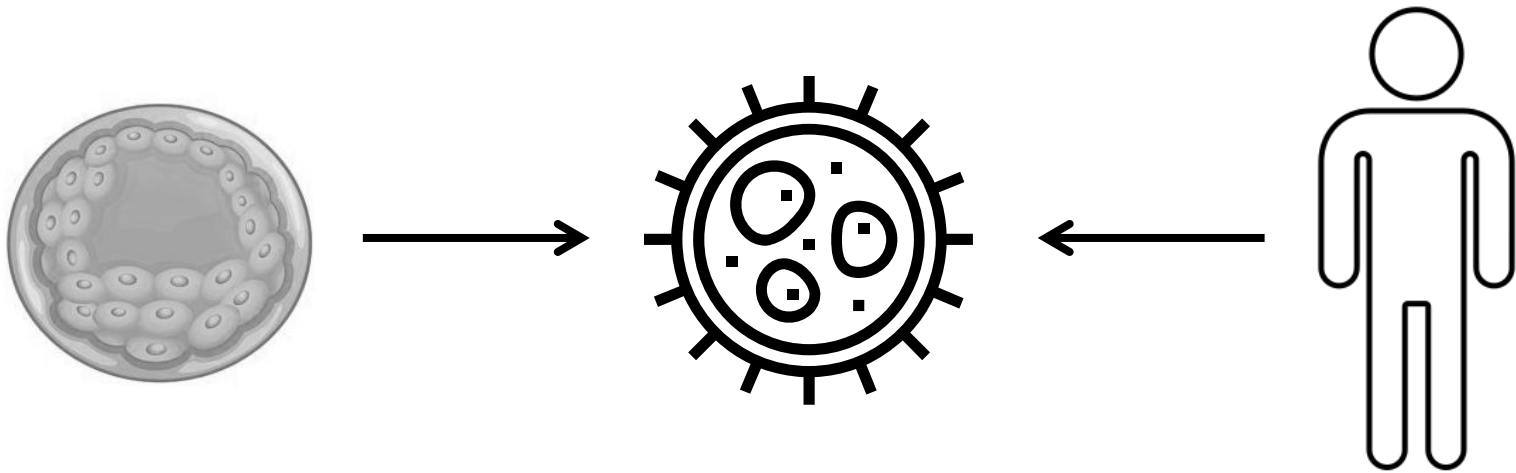
Stem cell therapy replaces non-functional cells with healthy cells



We have different sources for stem cells

Embryonic stem cells

Isolated from the mass of cells formed
soon after fertilization
Made from any adult cell in the body!



A large, light gray, stylized diagram of a cell with a nucleus and various organelles, positioned in the upper left background.

Stem cell therapy

- Replaces **non-functional cells** in the body with healthy cells

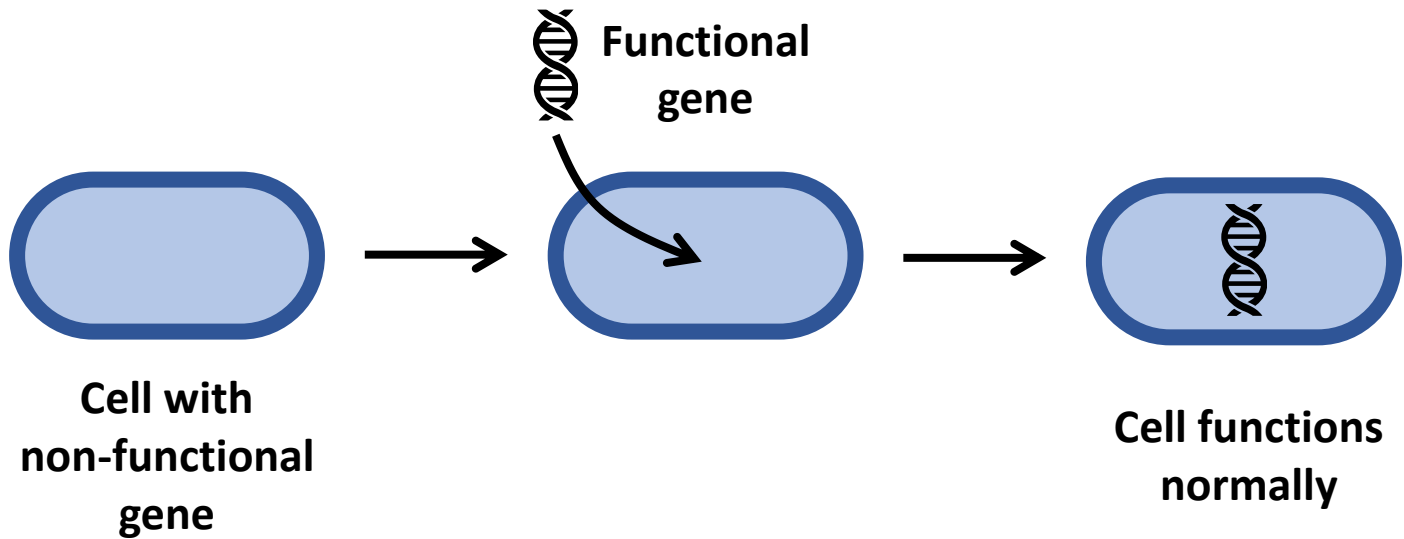
Gene therapy

Replaces or fixes a **faulty gene** with a working gene



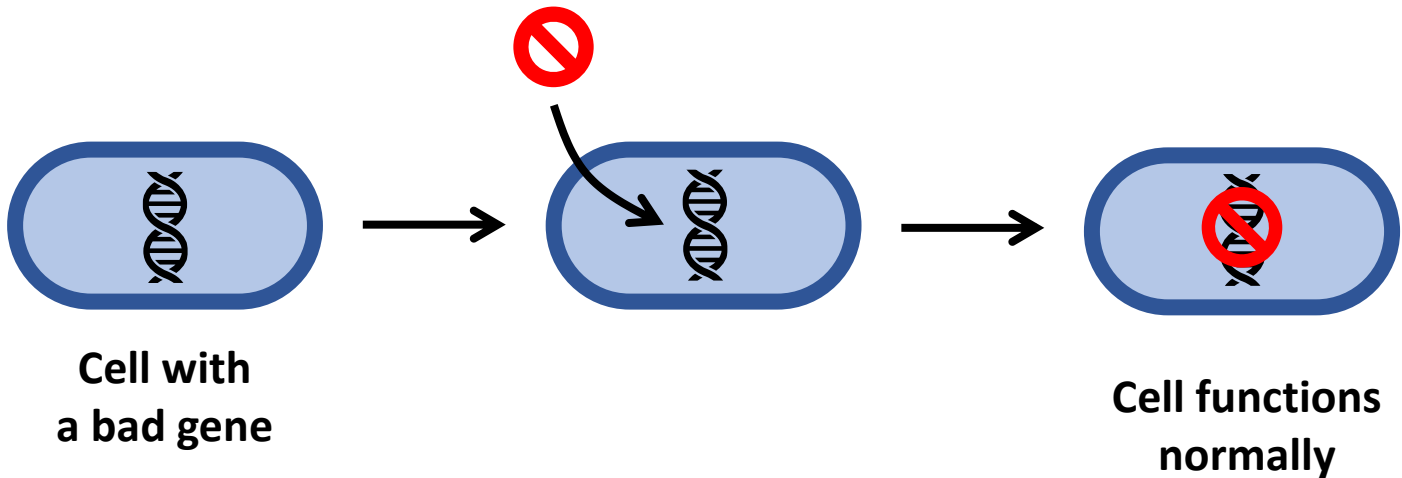
Gene therapy corrects a faulty gene

Add a new gene to replace a non-functional gene



Gene therapy corrects a faulty gene

Block a bad gene



A large, light gray, stylized diagram of a cell with a nucleus and various organelles, positioned in the upper left background.

Stem cell therapy

- Replaces **non-functional cells** in the body with healthy cells

Gene therapy

Replaces or fixes a **faulty gene** with a working gene



How science becomes medicine

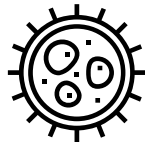
Basic research

Examines how living organisms work and what can go wrong



Clinical research

Tests the safety and efficacy of potential treatments in patients



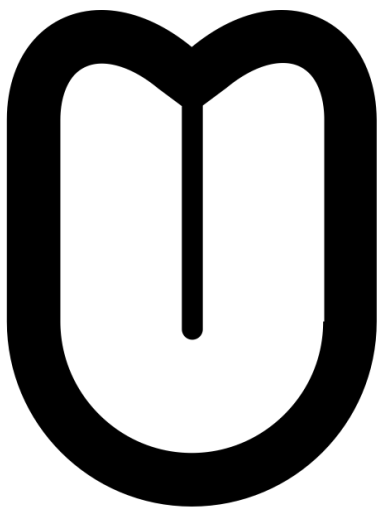
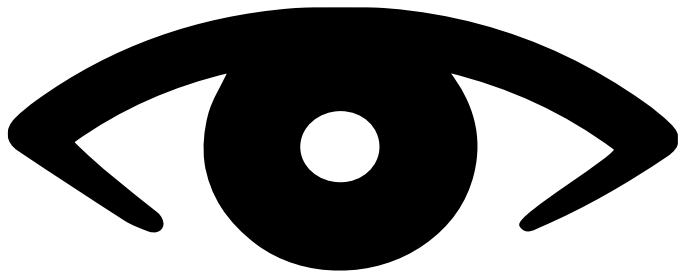
Preclinical research

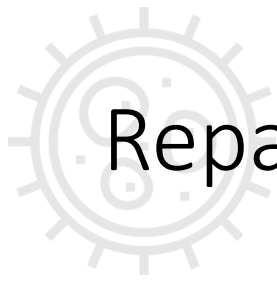
Translates the findings of basic research into potential treatments tested in animals and human tissues



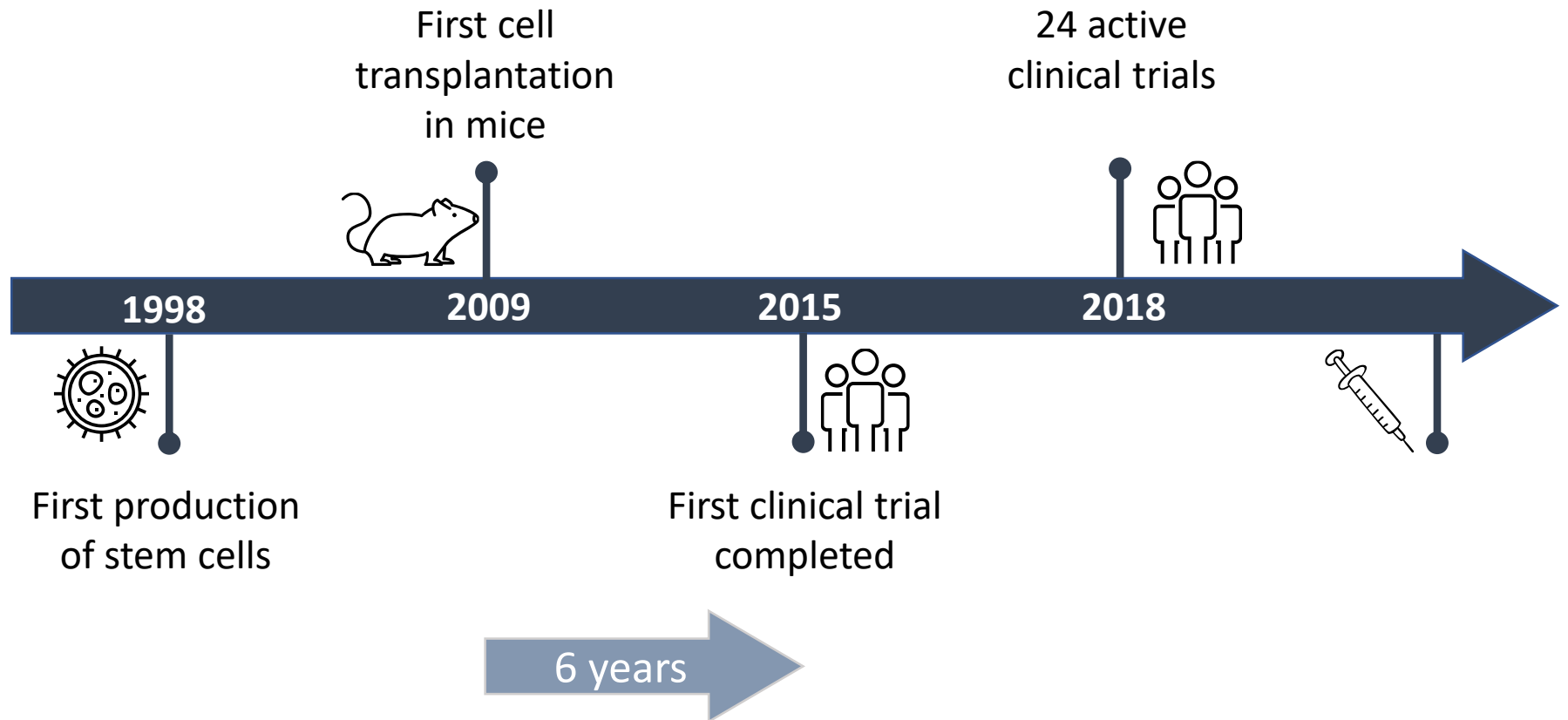
FDA approval

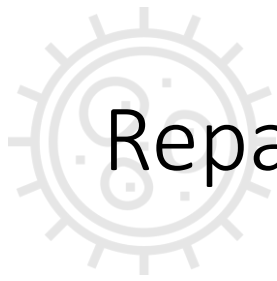
Available for treatment





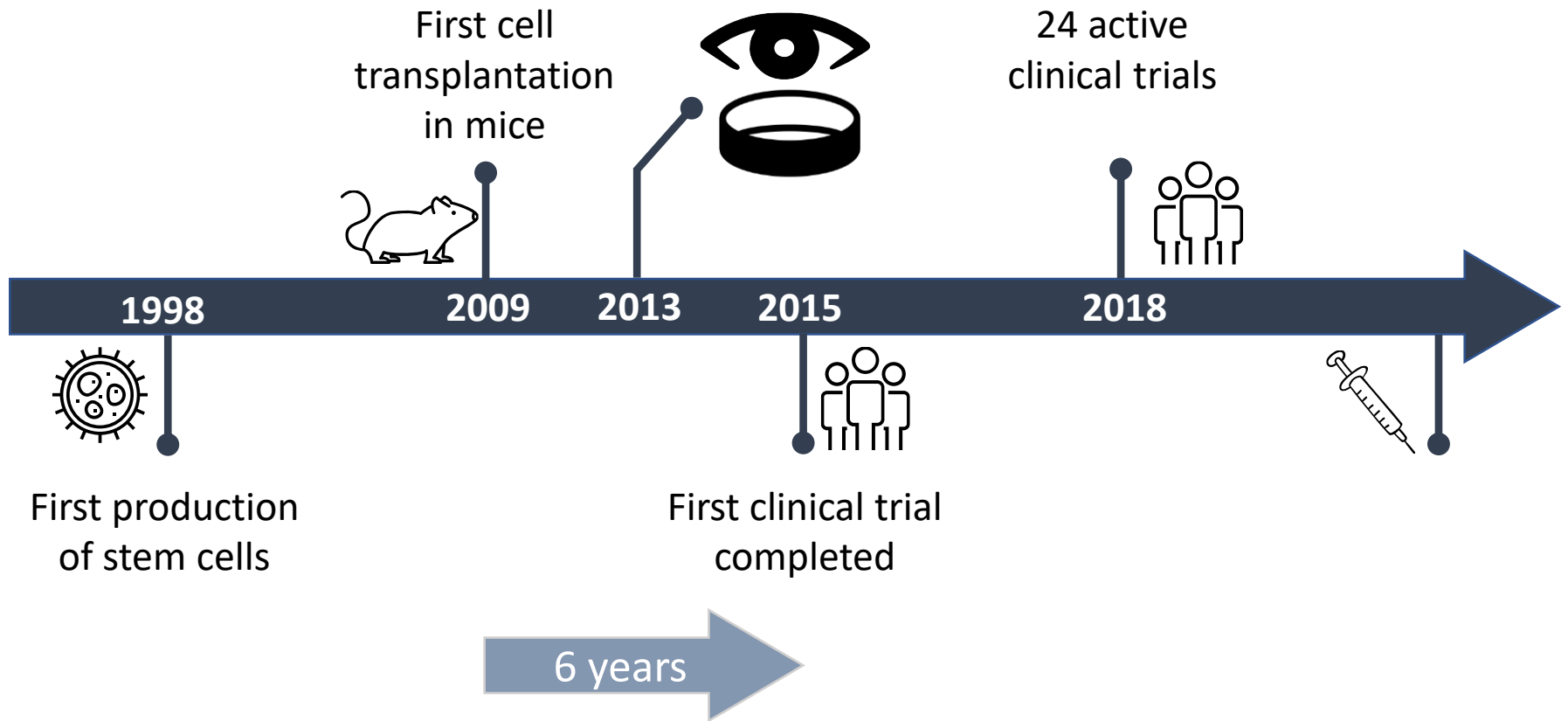
Repairing vision loss with stem cells





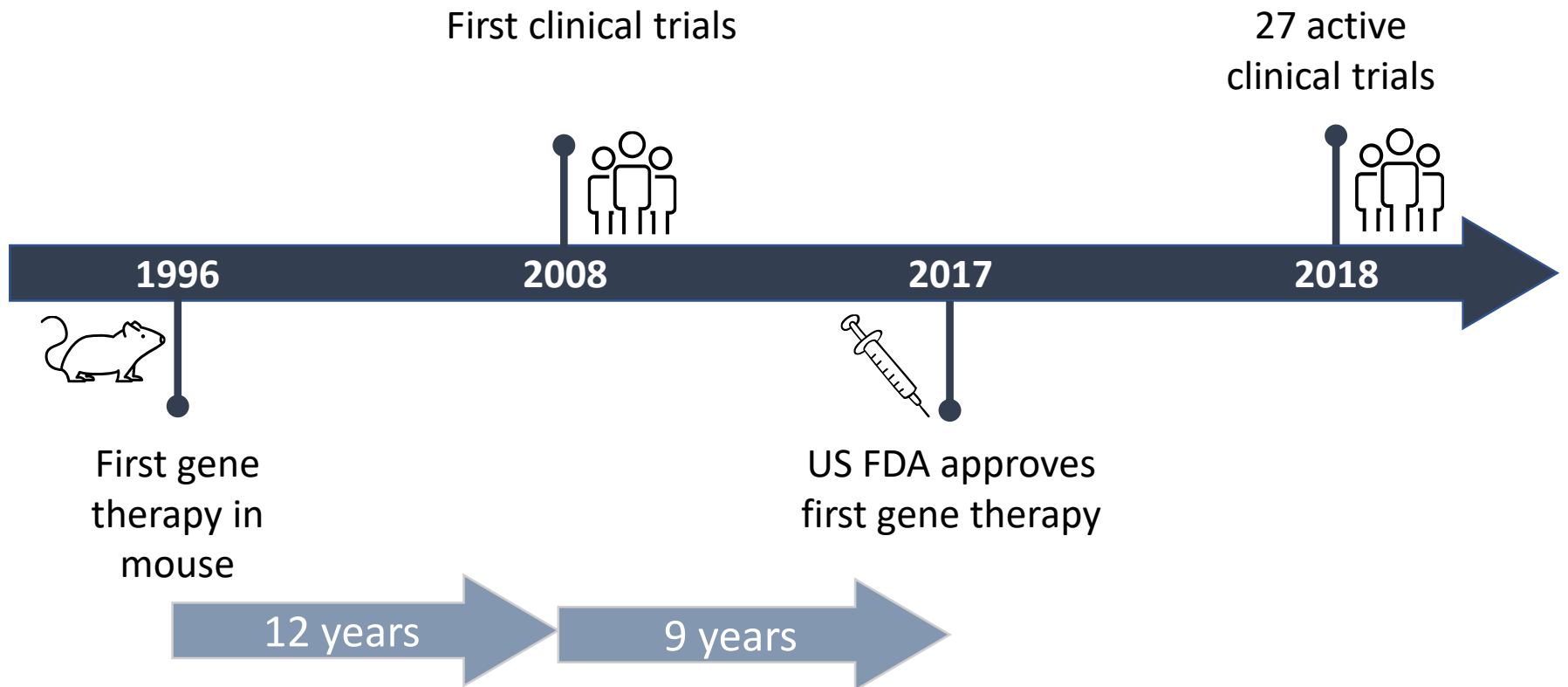
Repairing vision loss with stem cells

M. Natalia Vergara, PhD





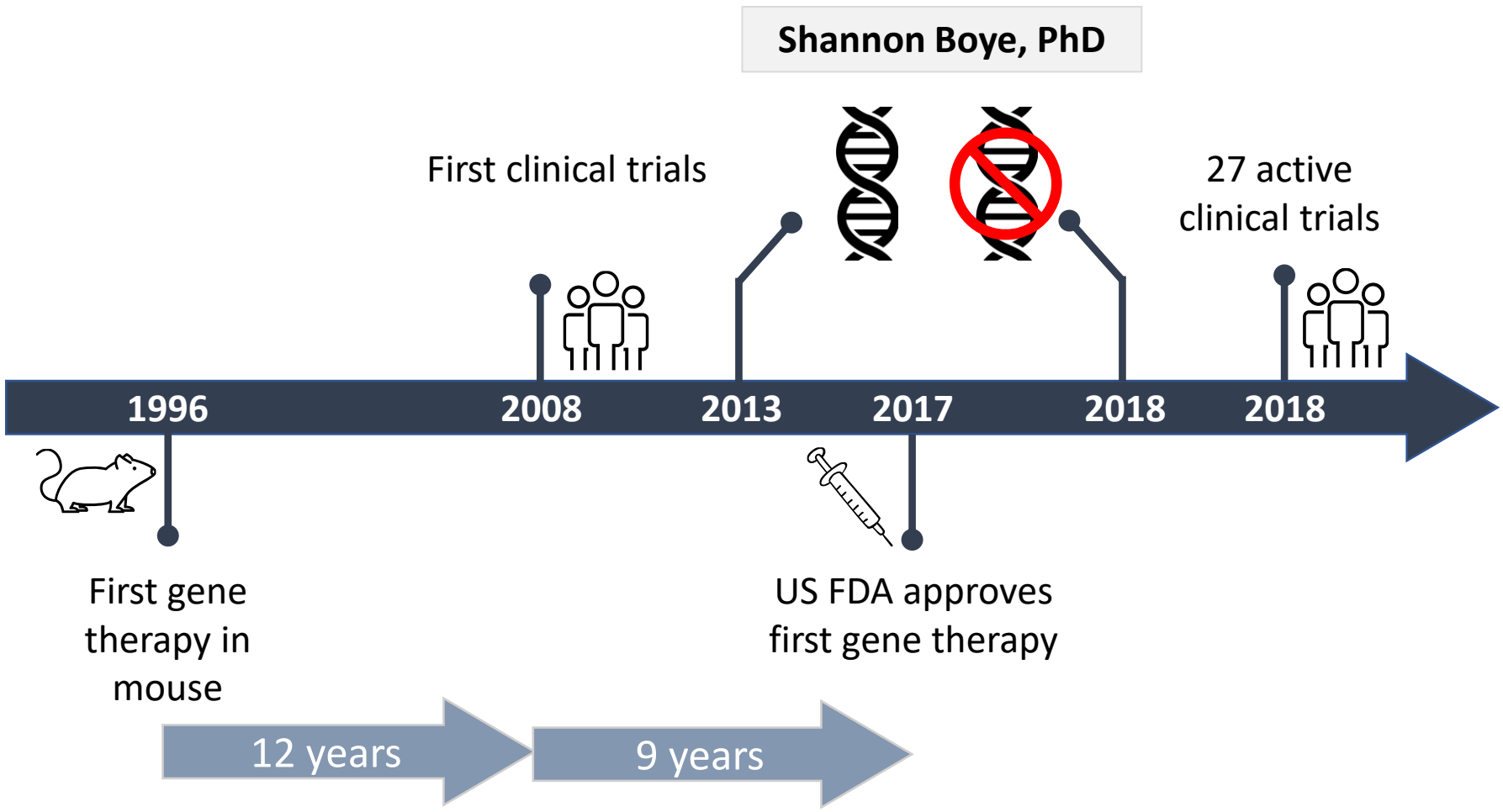
Repairing vision loss with gene therapy: The example of Leiber Congenital Amaurosis (LCA)

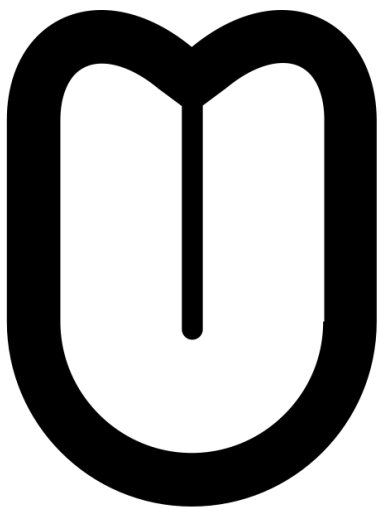
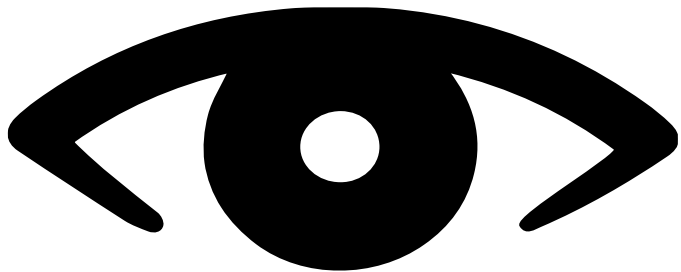


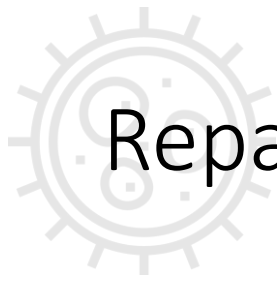


Repairing vision loss with gene therapy: The example of Leiber Congenital Amaurosis (LCA)

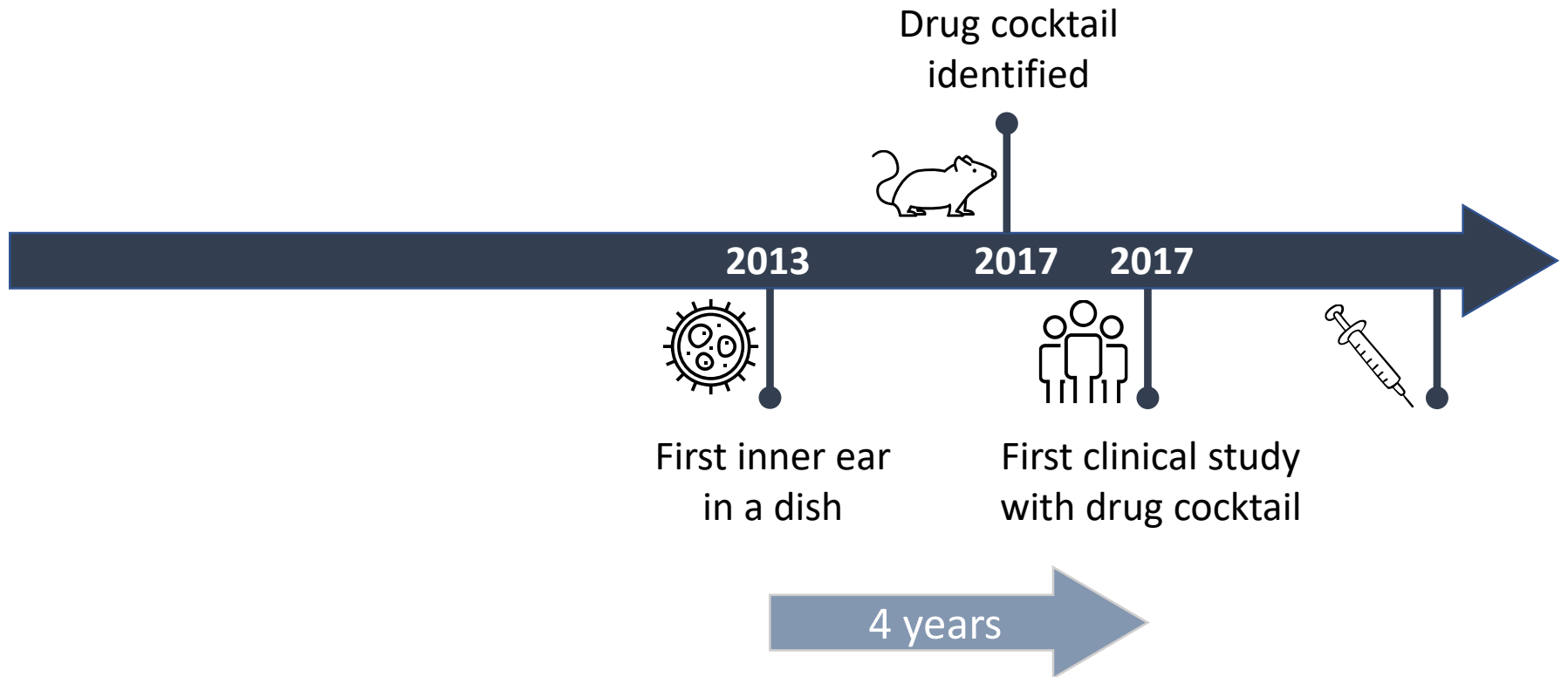
Shannon Boye, PhD

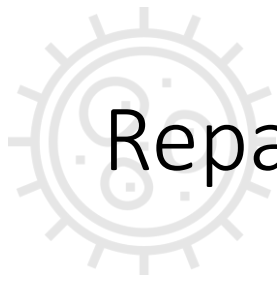






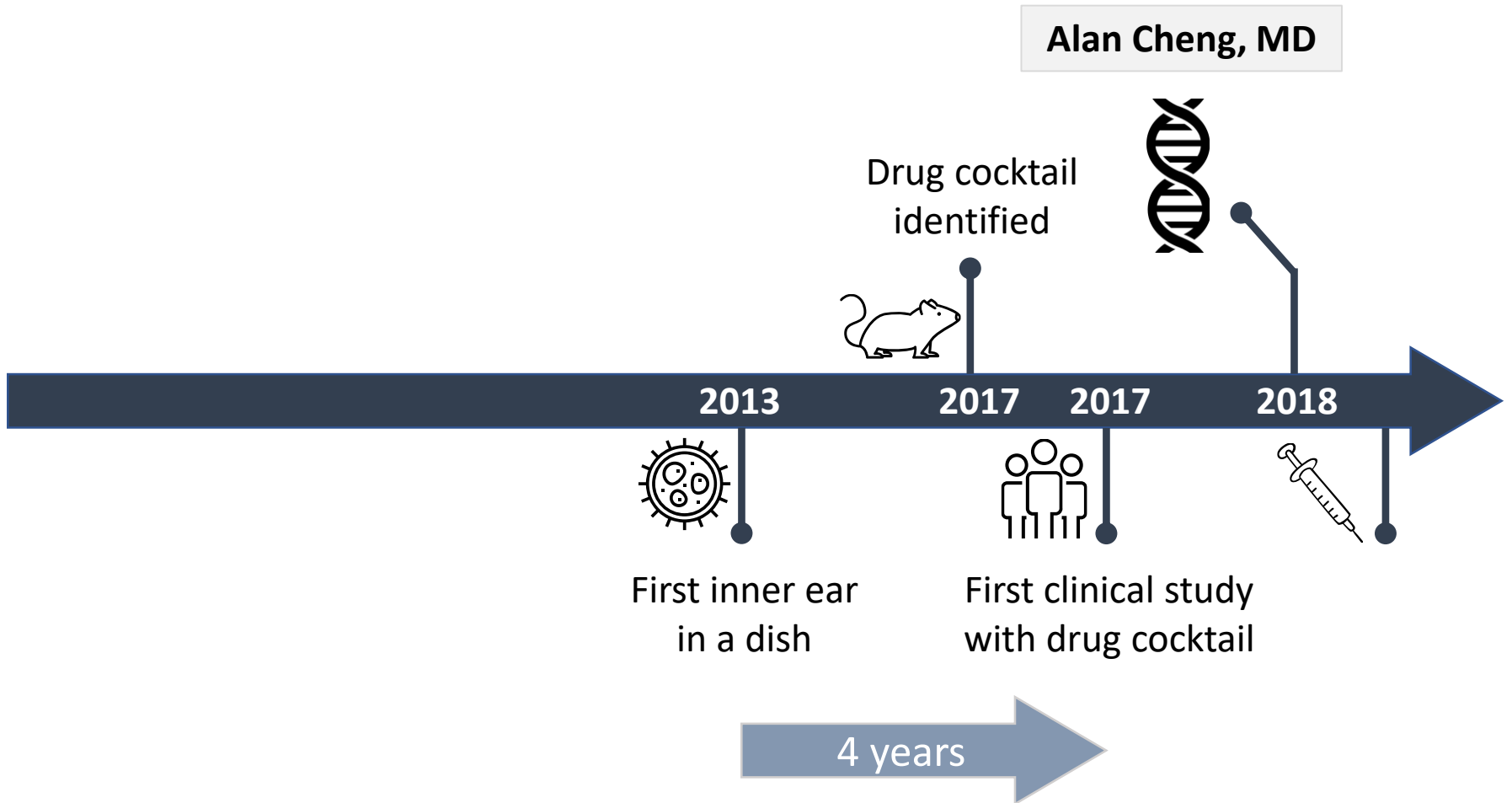
Repairing hearing loss with stem cells





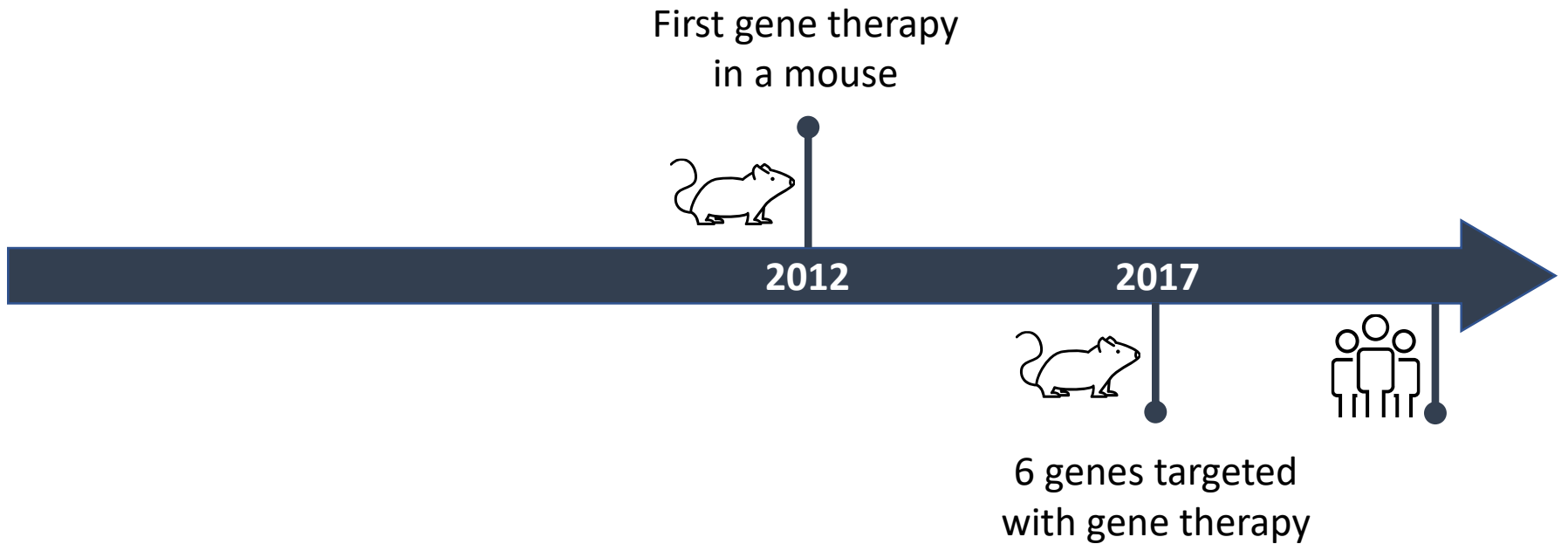
Repairing hearing loss with stem cells

Alan Cheng, MD





Repairing hearing loss with gene therapy





Repairing hearing loss with gene therapy

Jeffrey Holt, PhD

First gene therapy
in a mouse



2012



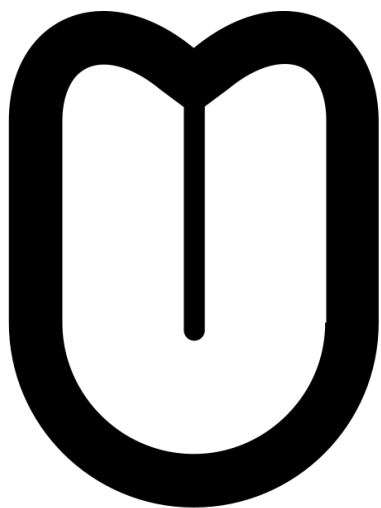
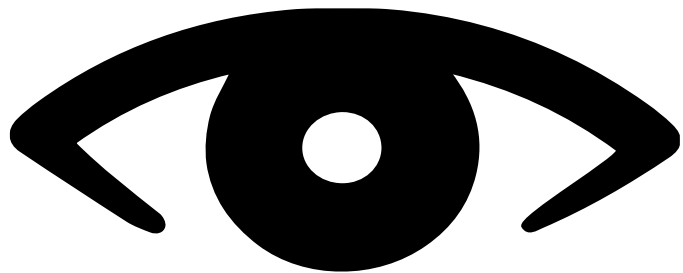
2017

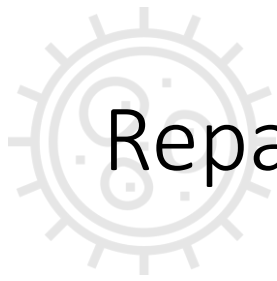
2018



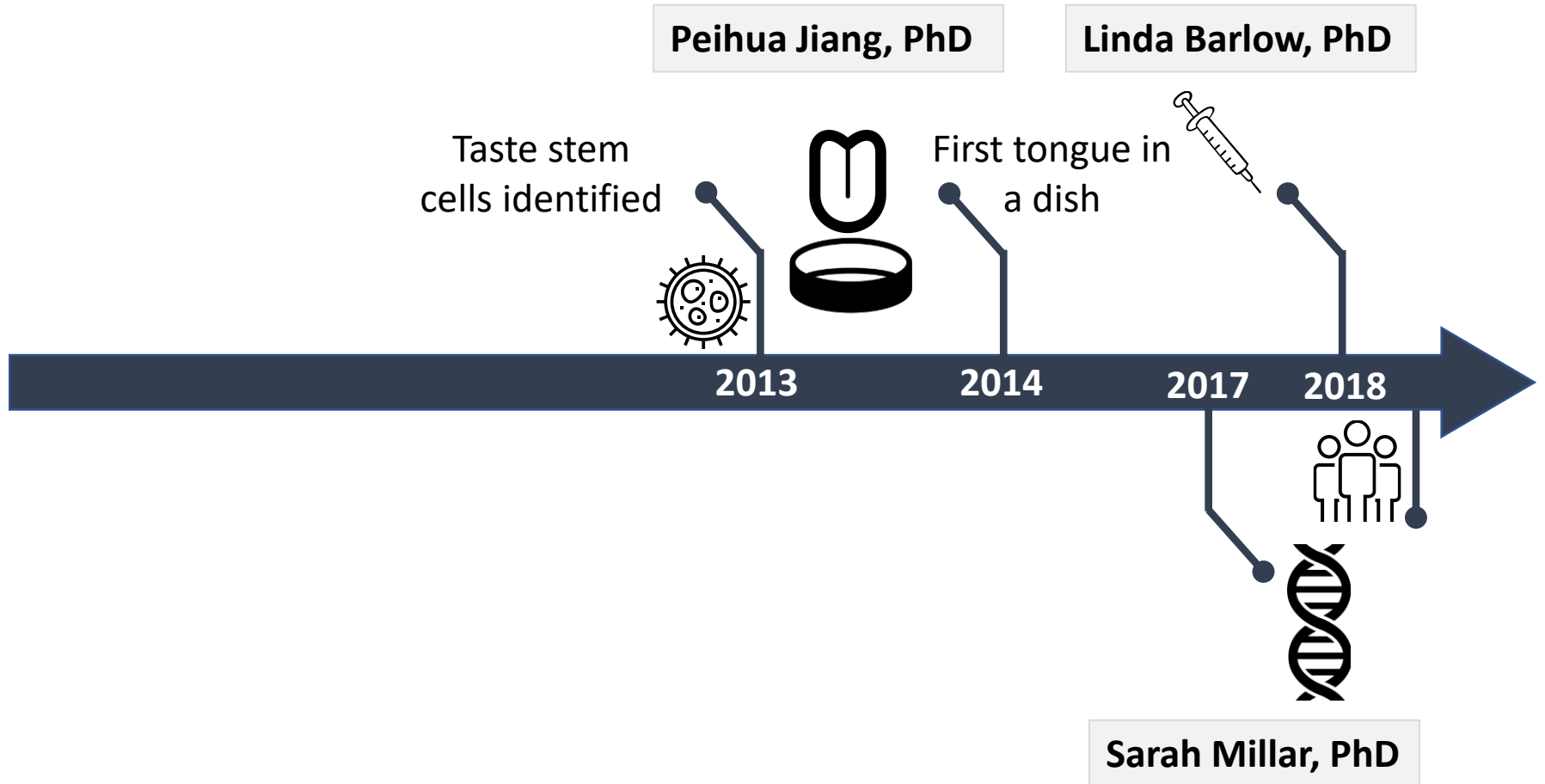
6 genes targeted
with gene therapy

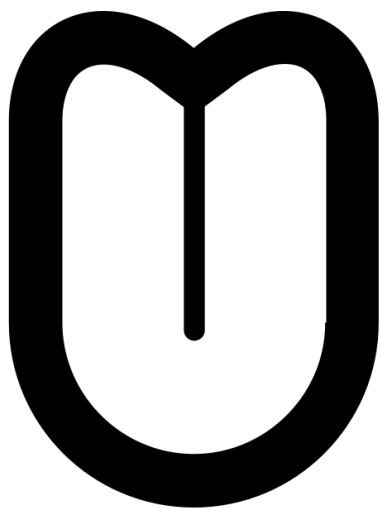
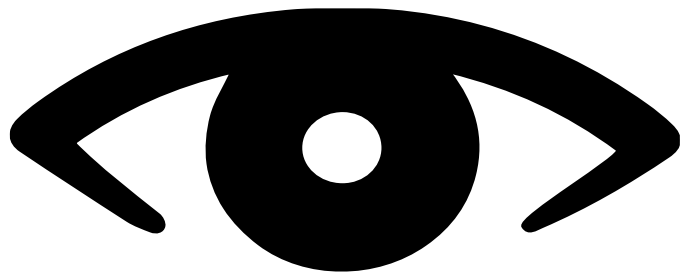


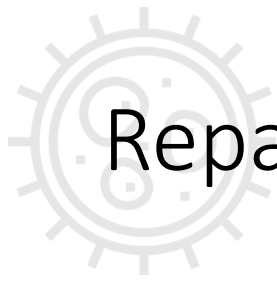




Repairing taste loss with stem cells







Repairing smell loss with stem cells

James Schwob, MD/PhD



2016

2017



Bradley Goldstein, MD/PhD





Repairing smell loss with gene therapy: An example with ciliopathies

Jeffrey Martens, PhD

First gene therapy
in a mouse



2012



2017



