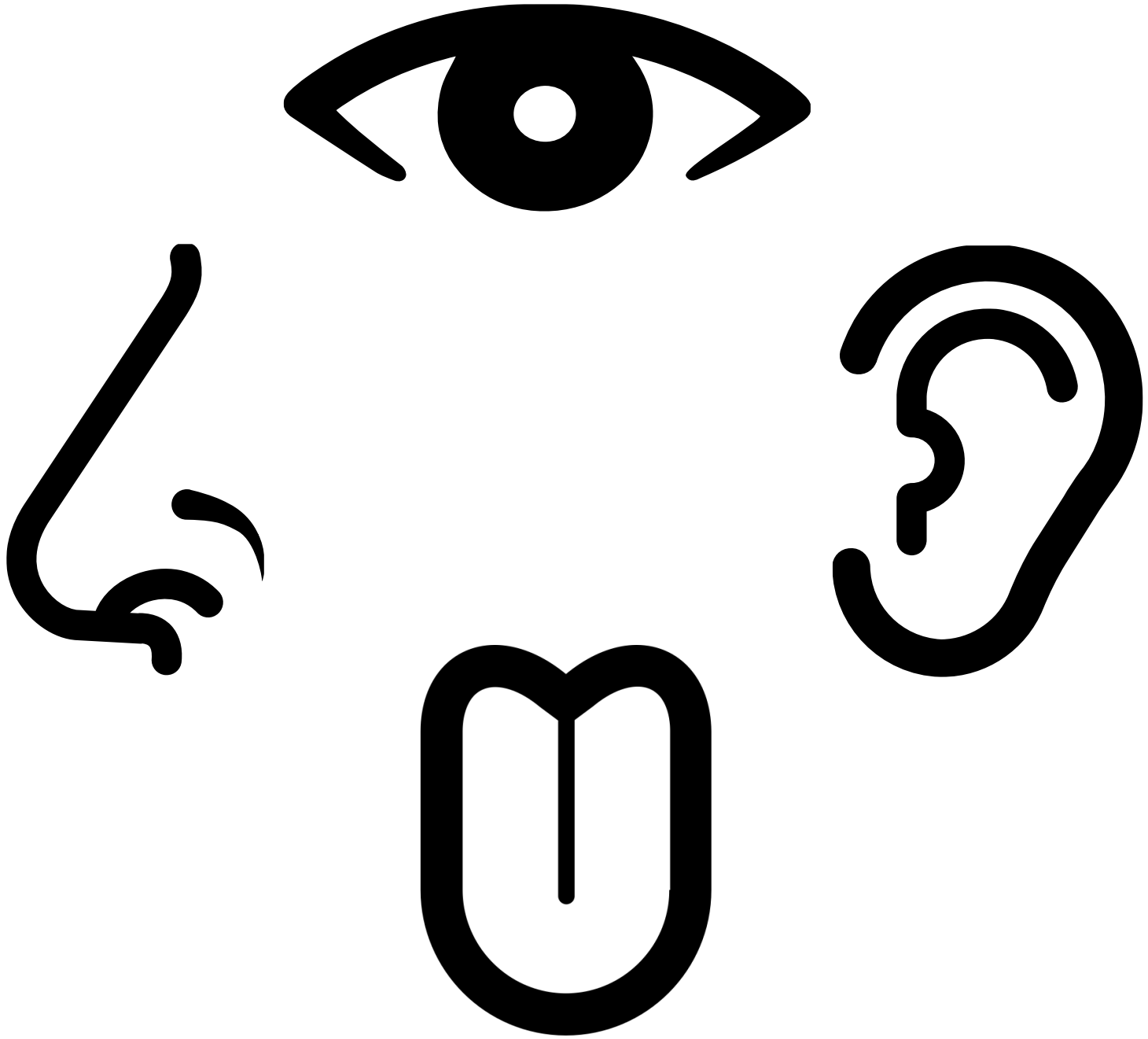
Therapeutic Approaches in Sensory Disorders

Summary of Day 1 Scientific Presentations

Casey Trimmer, PhD



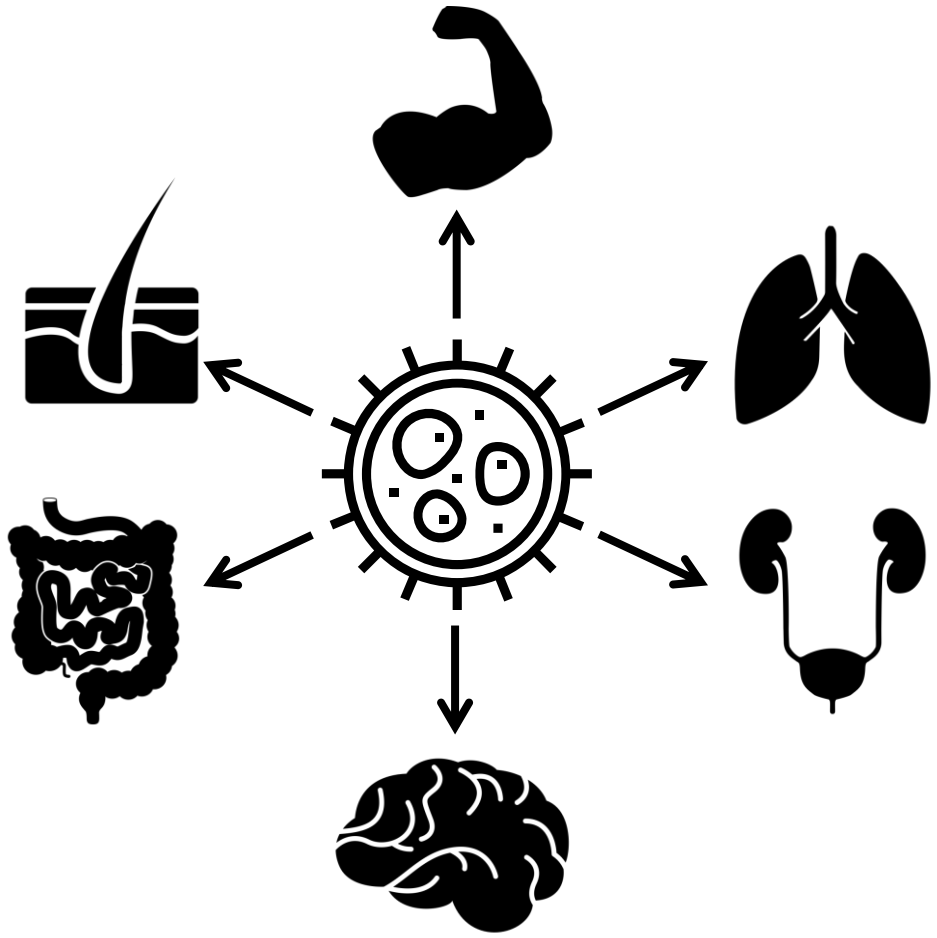
Stem cell therapy

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



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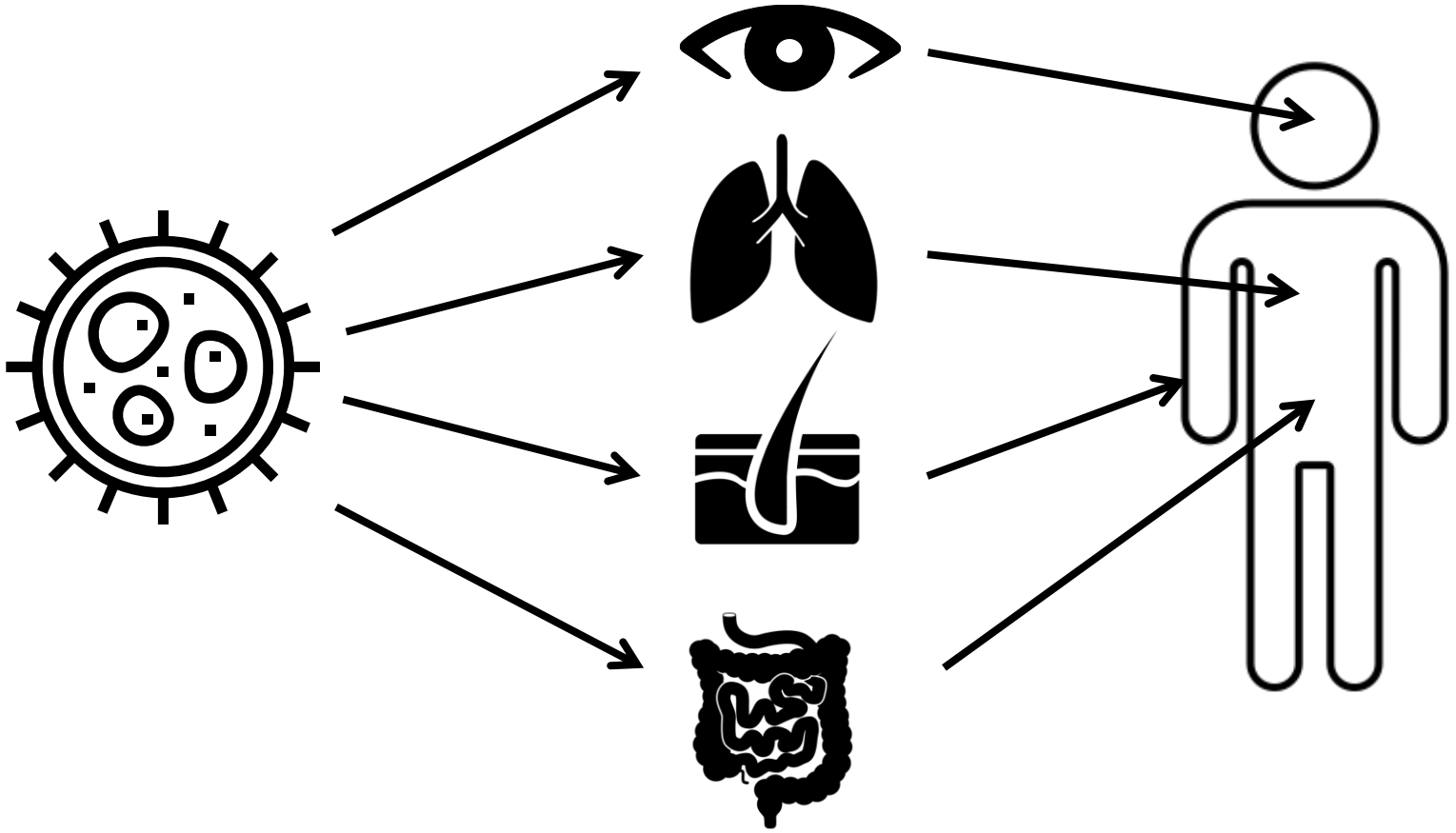


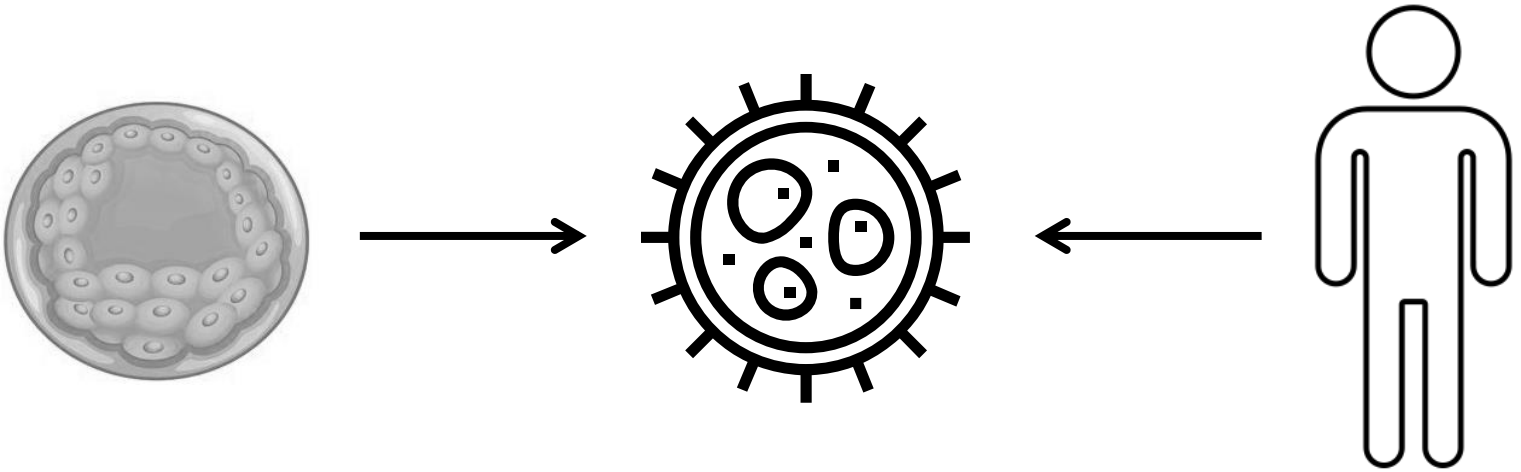
We have different sources for stem cells

Embryonic stem cells

Isolated from the mass of cells formed soon after fertilization

Induced stem cells

Made from any adult cell in the body!



Stem cell therapy

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

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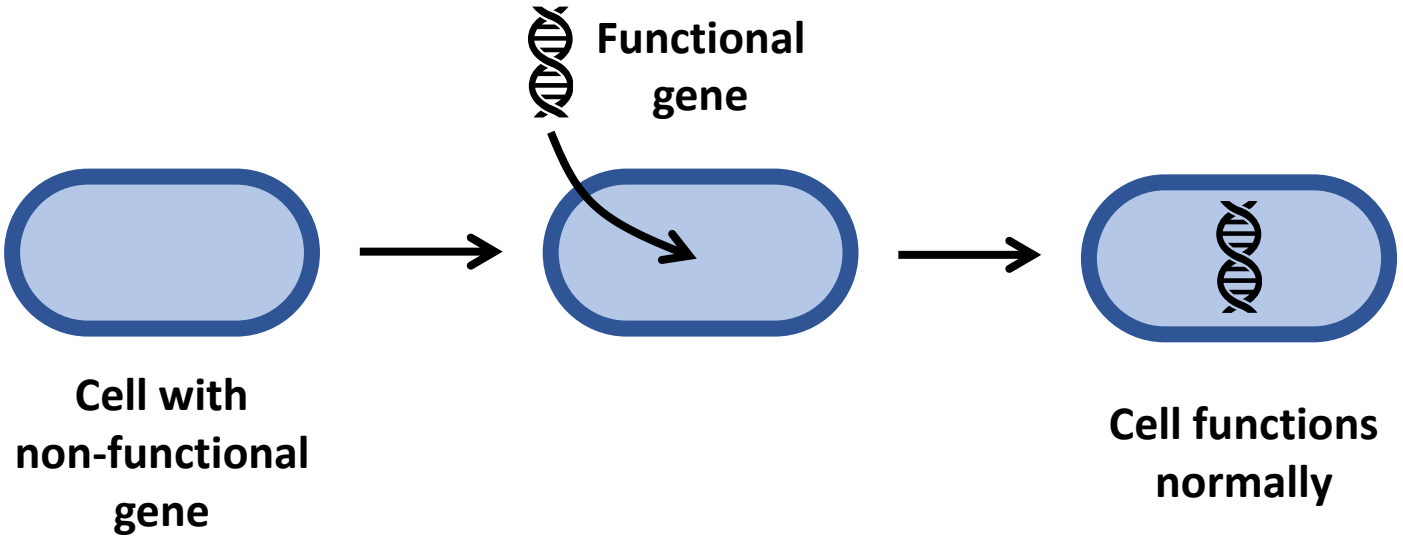
Replaces or fixes a **faulty gene** with a working gene

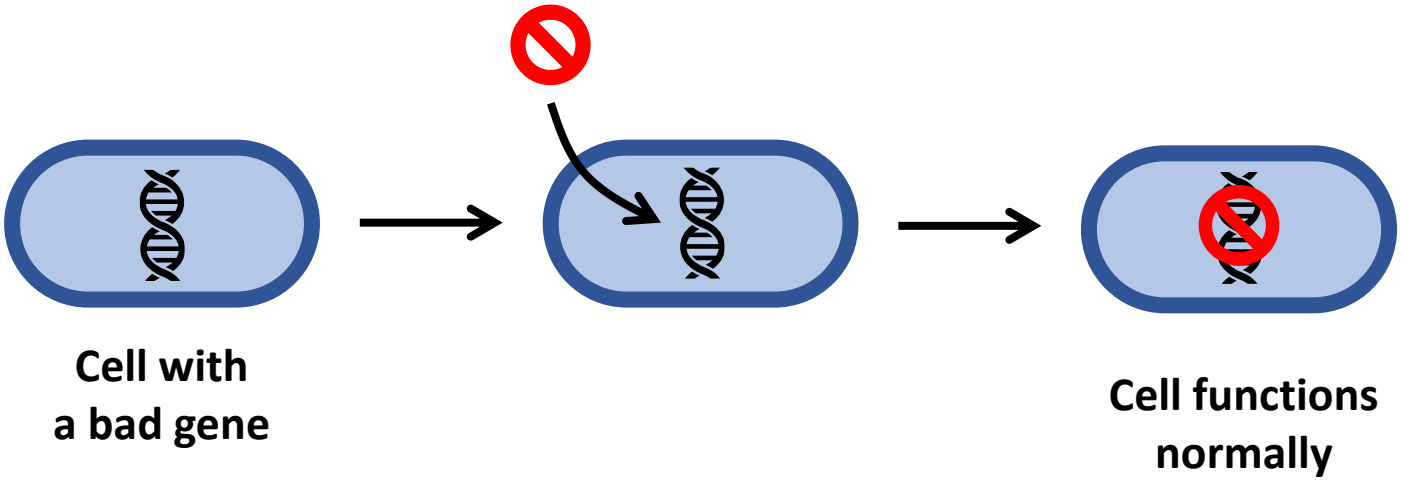
Gene therapy corrects a faulty gene

Add a new gene to replace a non-functional gene



Gene therapy corrects a faulty gene

Block a bad gene



Stem cell therapy

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

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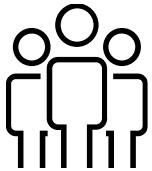
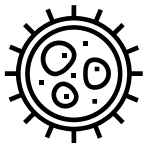
How science becomes medicine

Basic research
Examines how living organisms work and what can go wrong

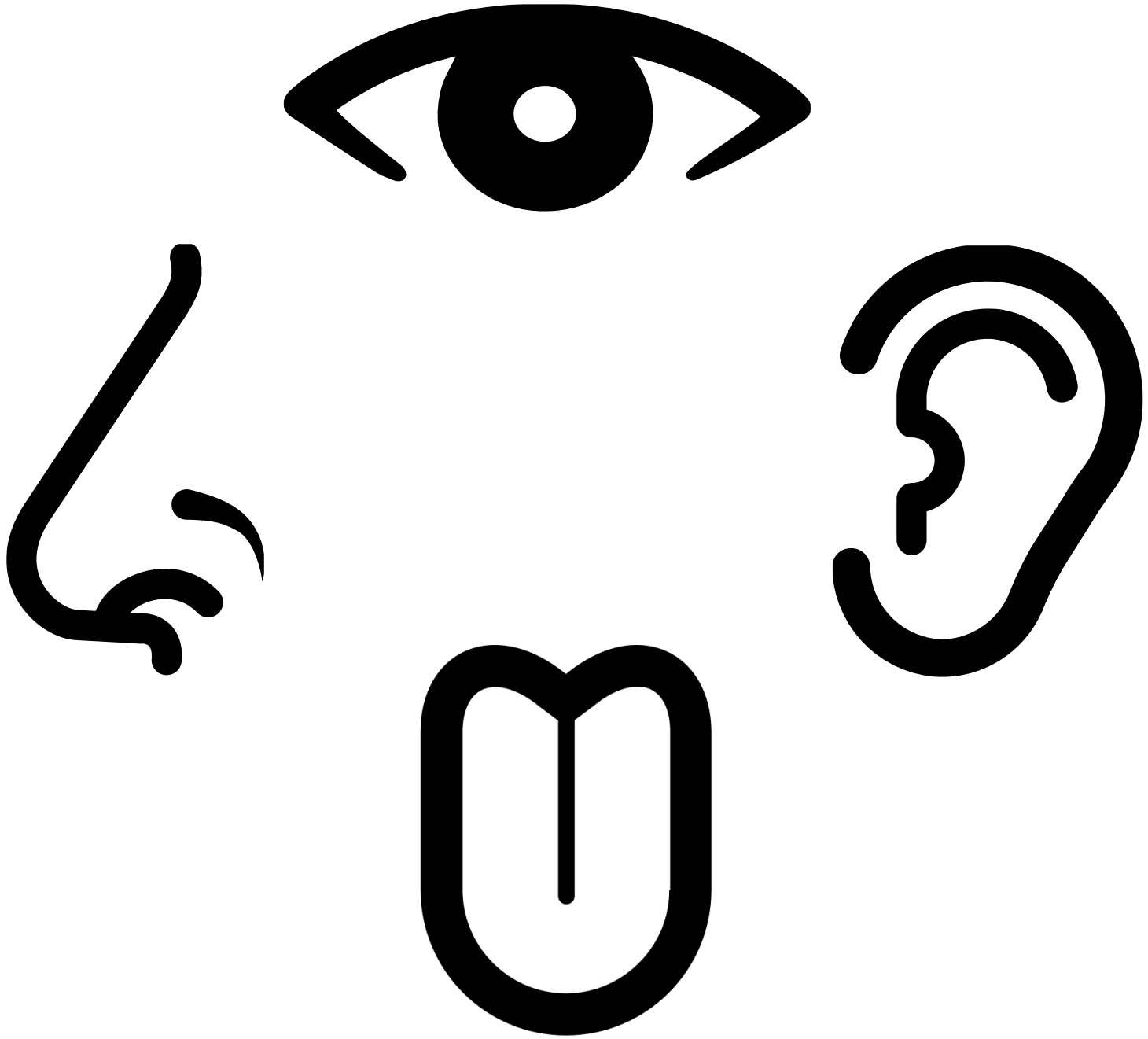
Clinical research
Tests the safety and efficacy of potential treatments in patients



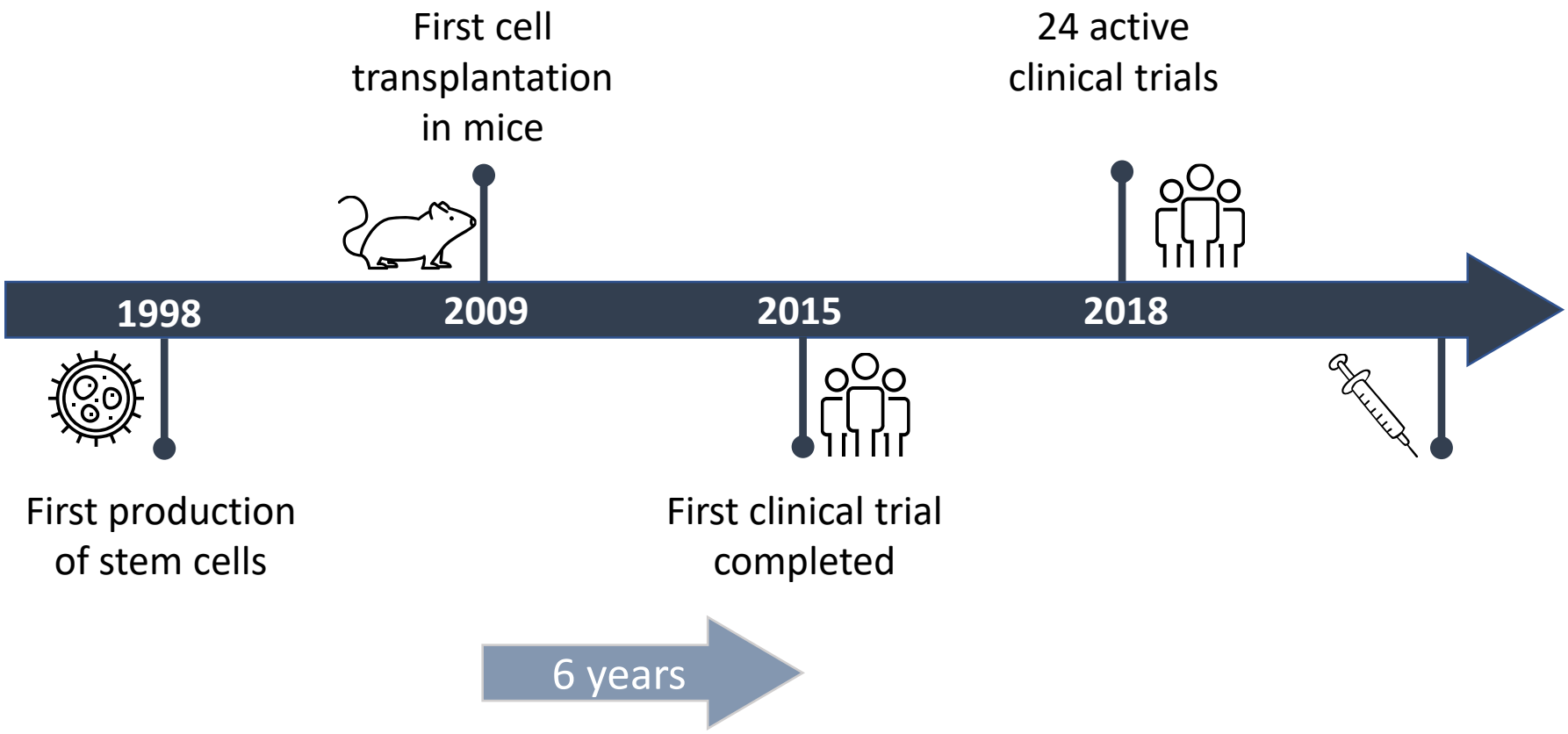


Preclinical research
Translates the findings of basic research into potential treatments tested in animals and human tissues

FDA approval
Available for treatment

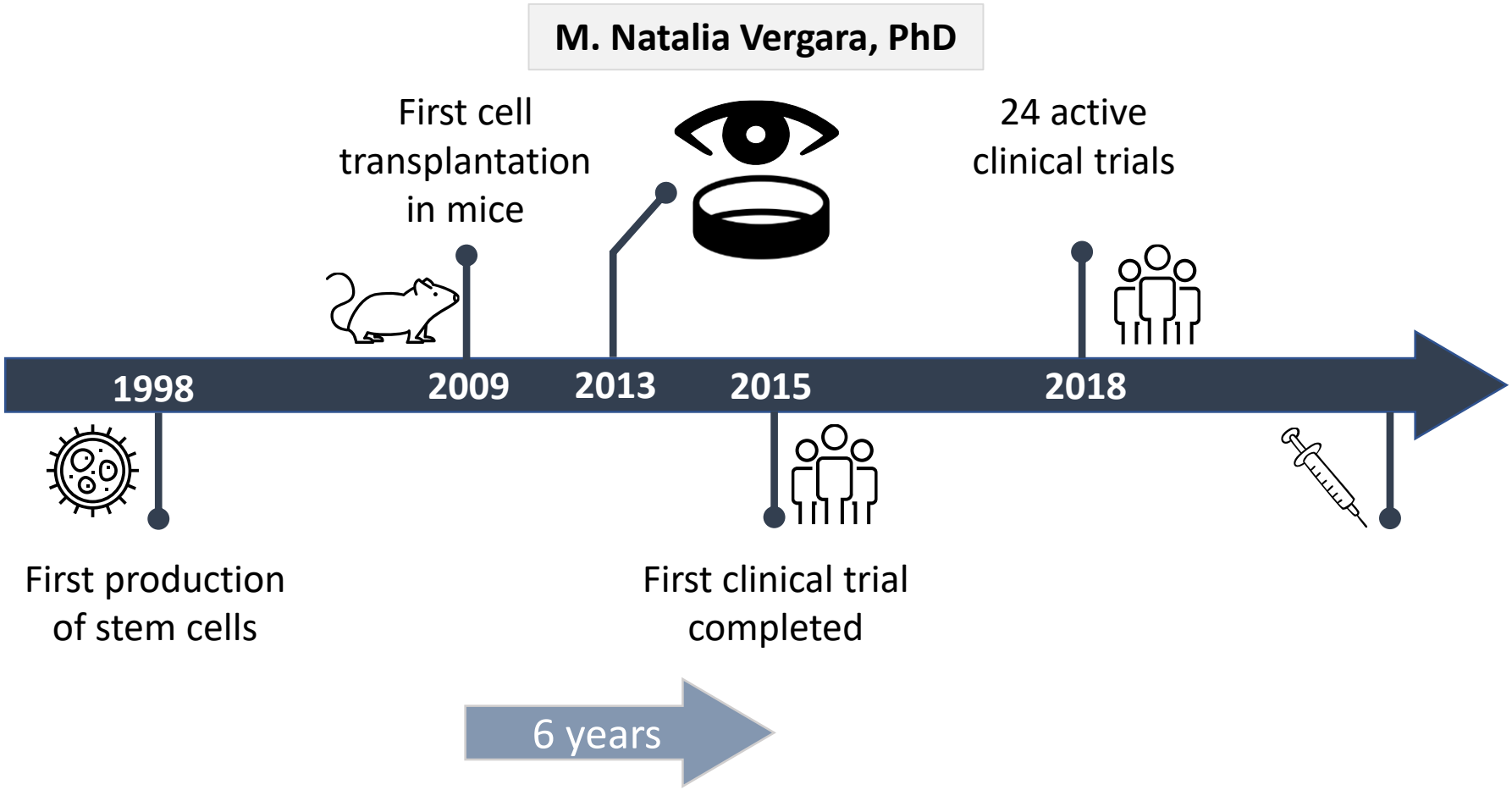


Repairing vision loss with stem cells

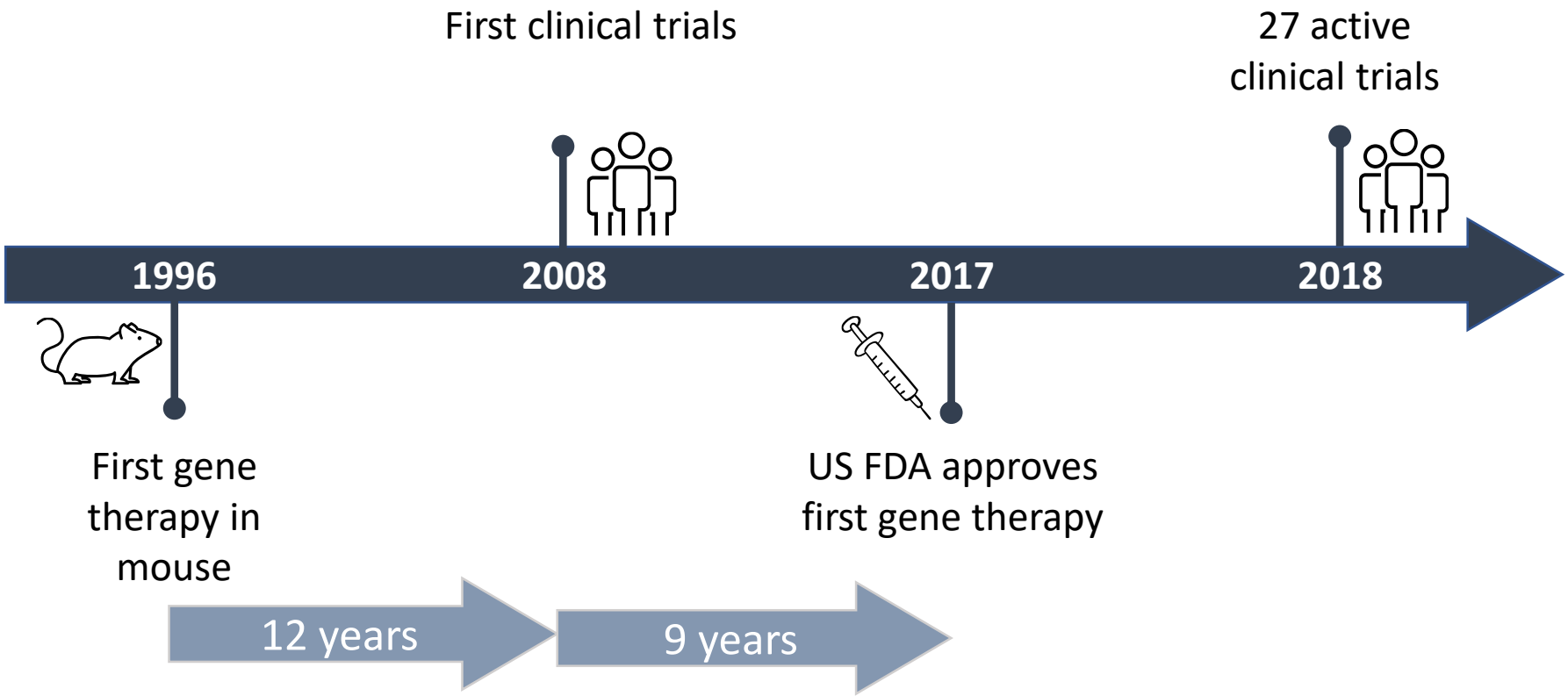


Repairing vision loss with stem cells

M. Natalia Vergara, PhD



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



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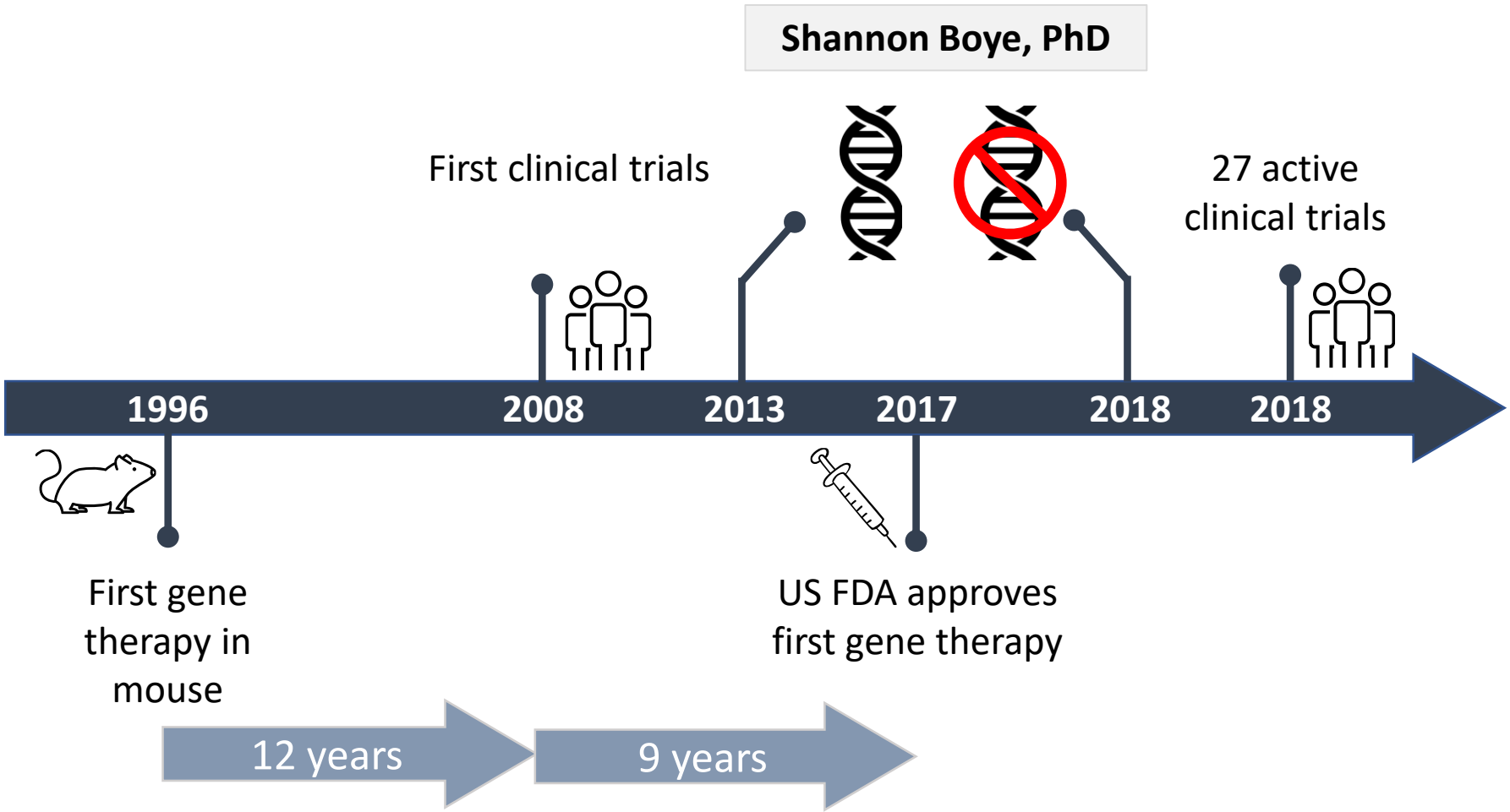
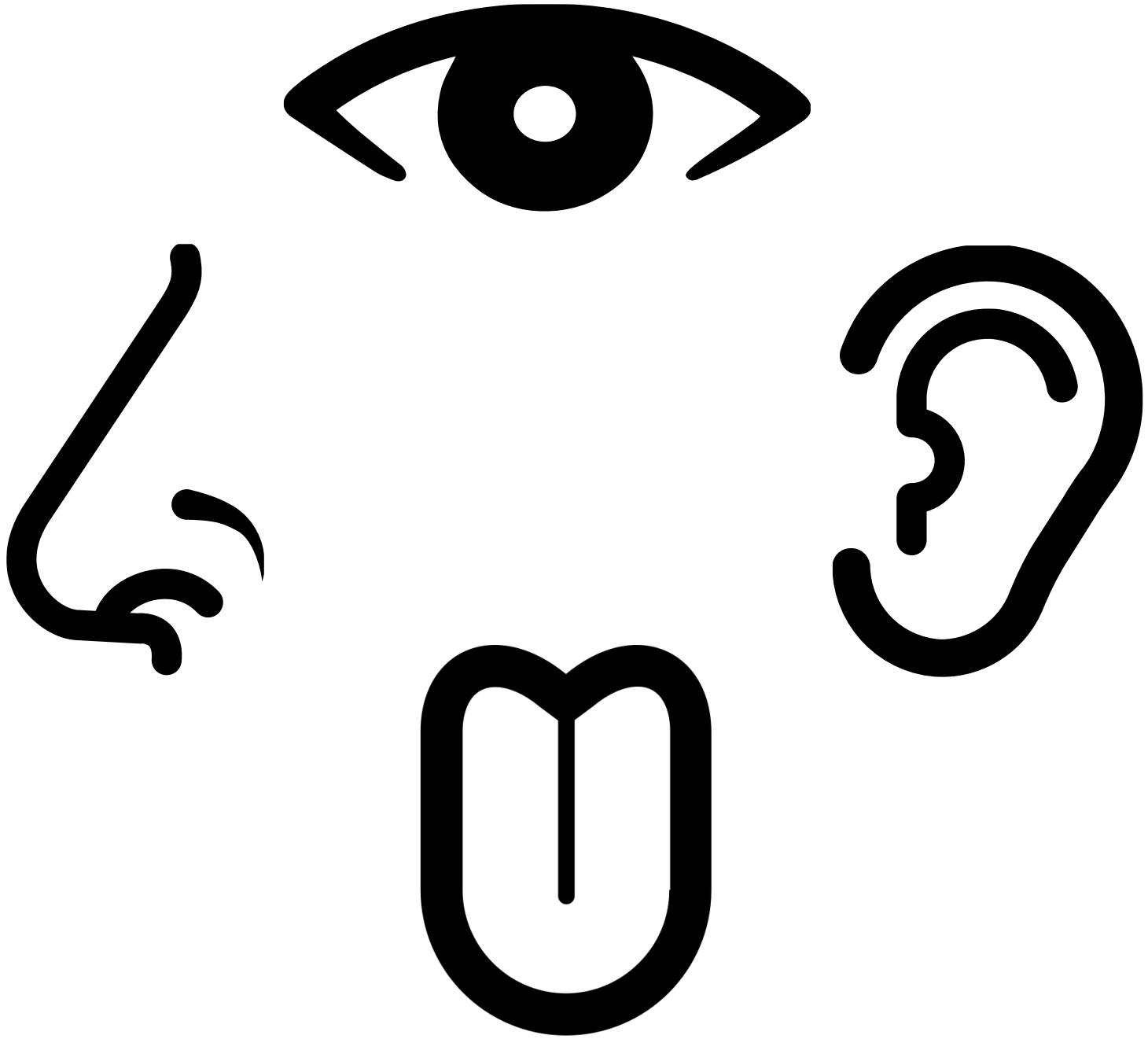
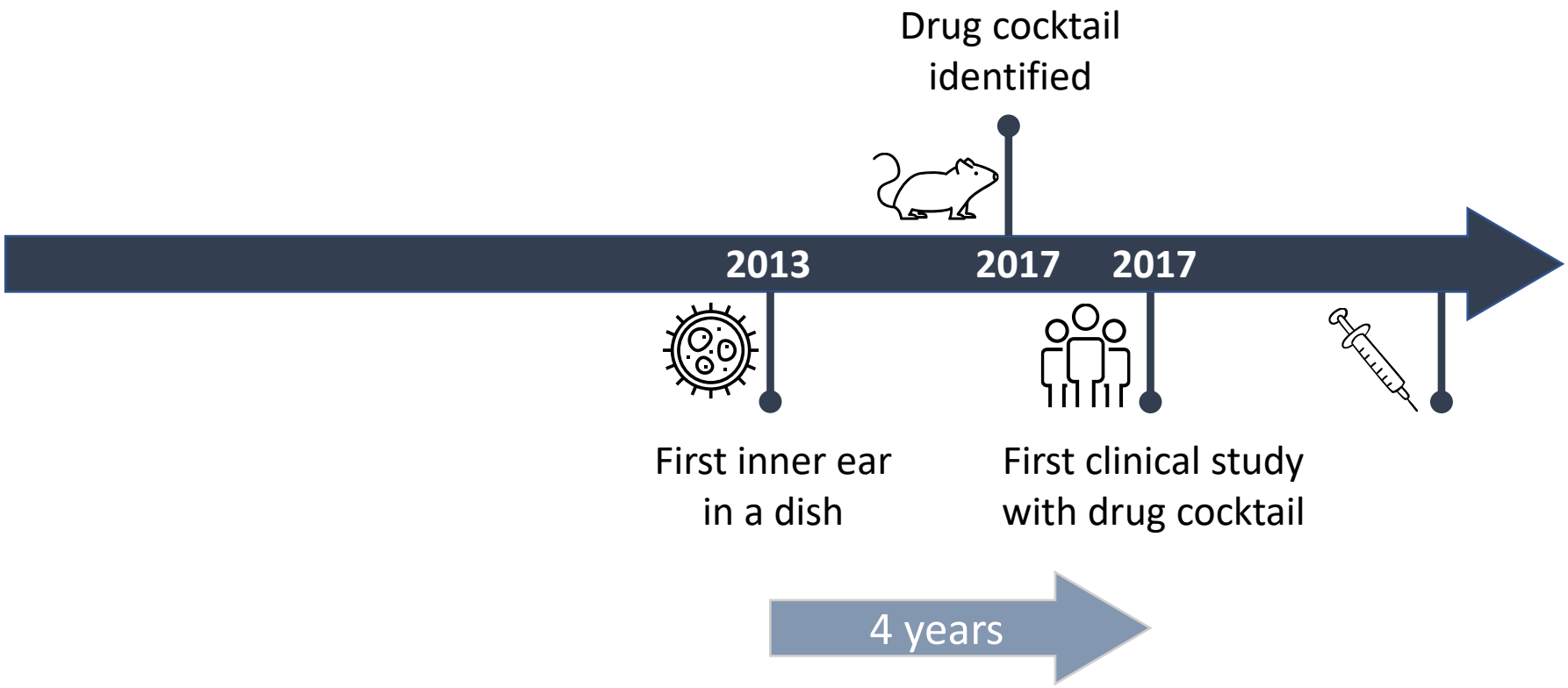




Repairing hearing loss with stem cells



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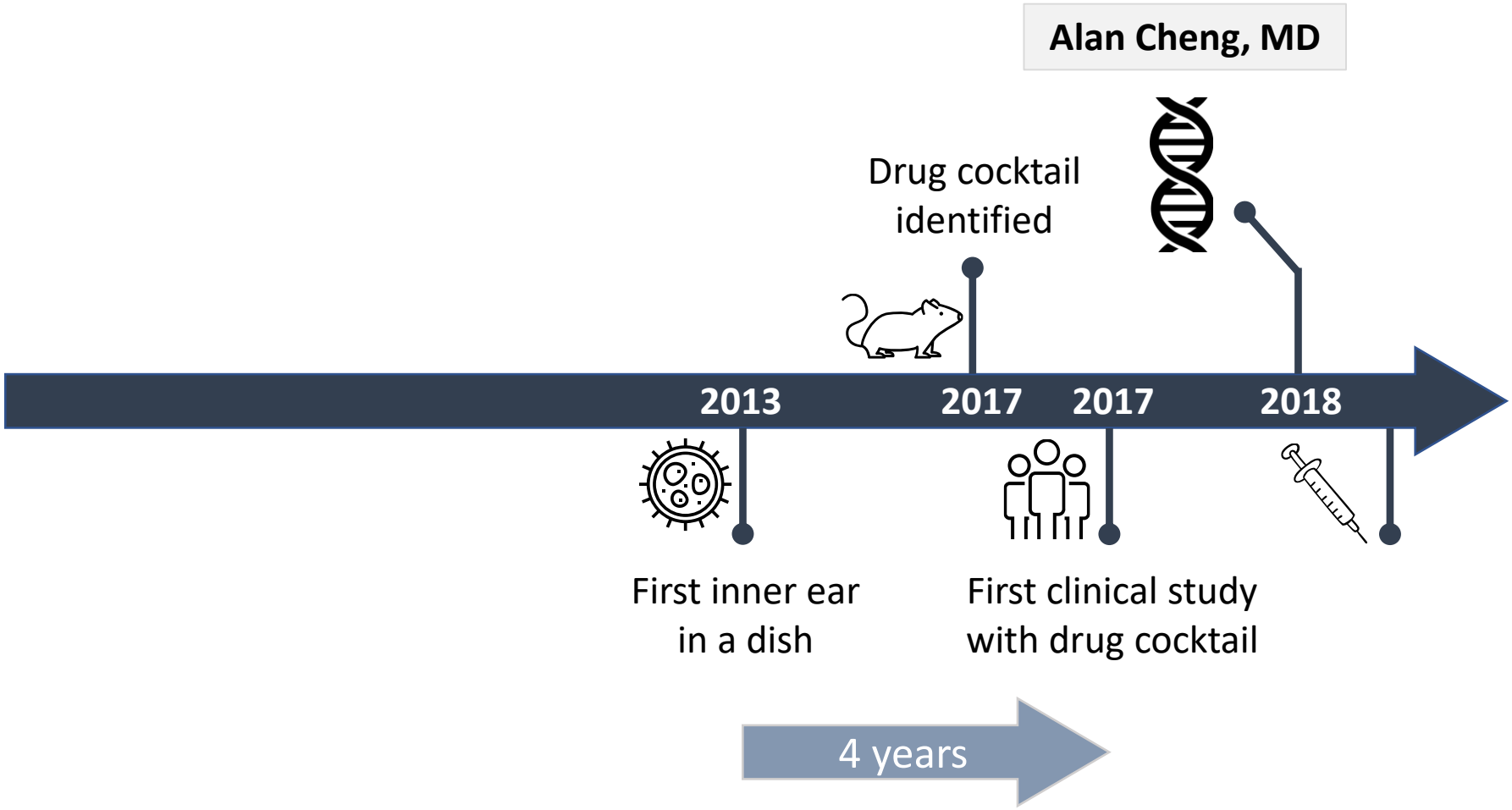

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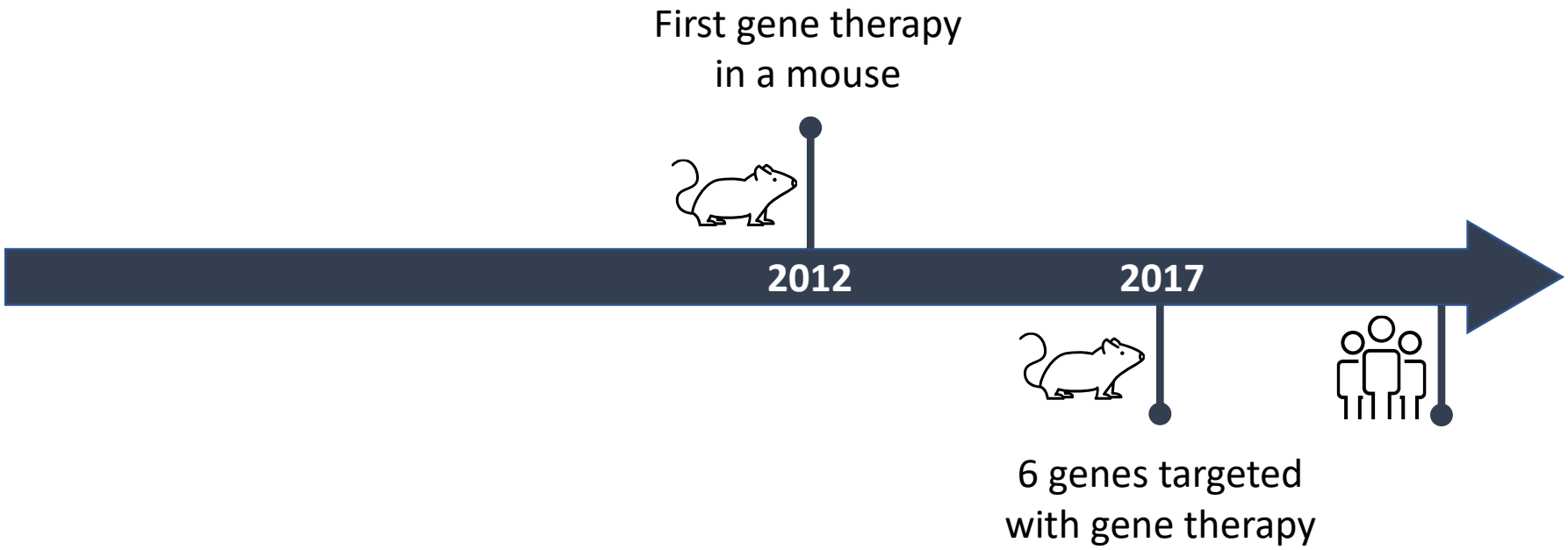

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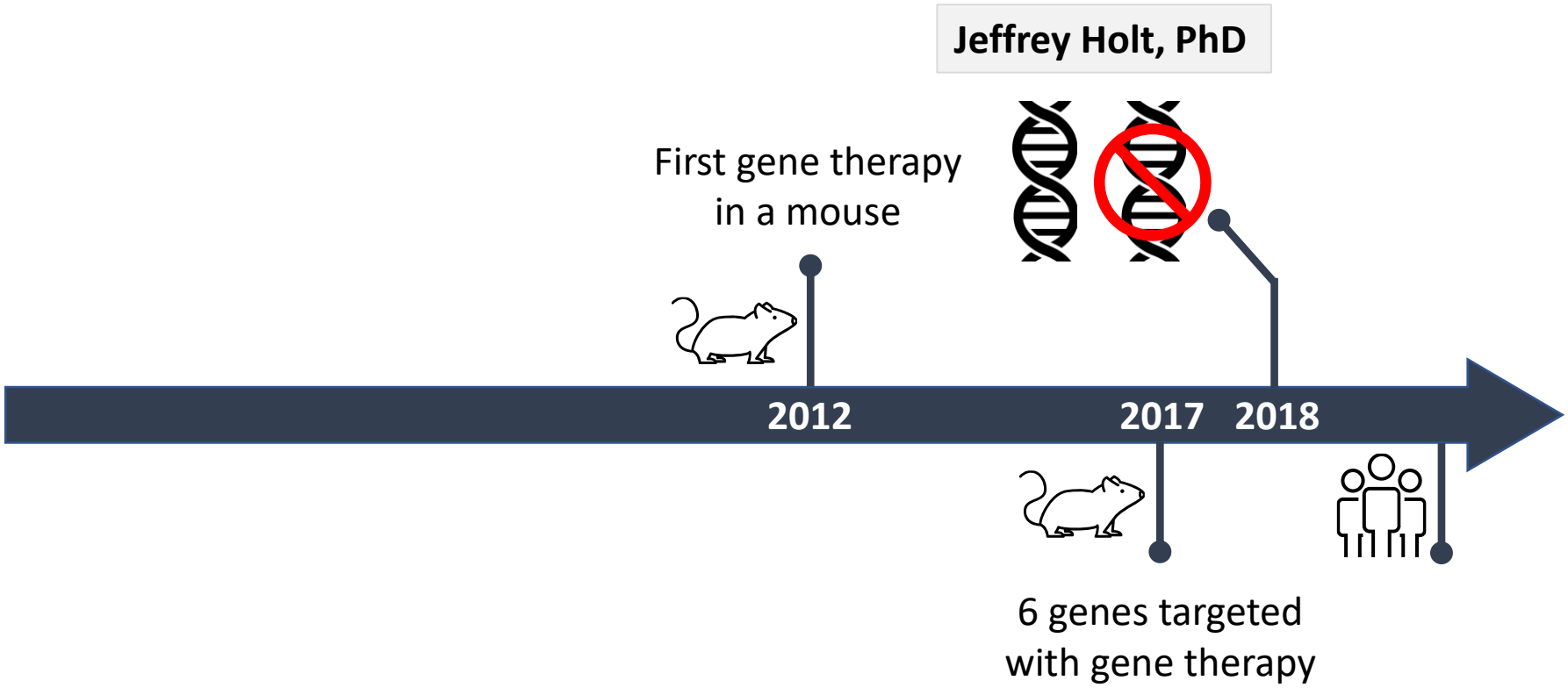
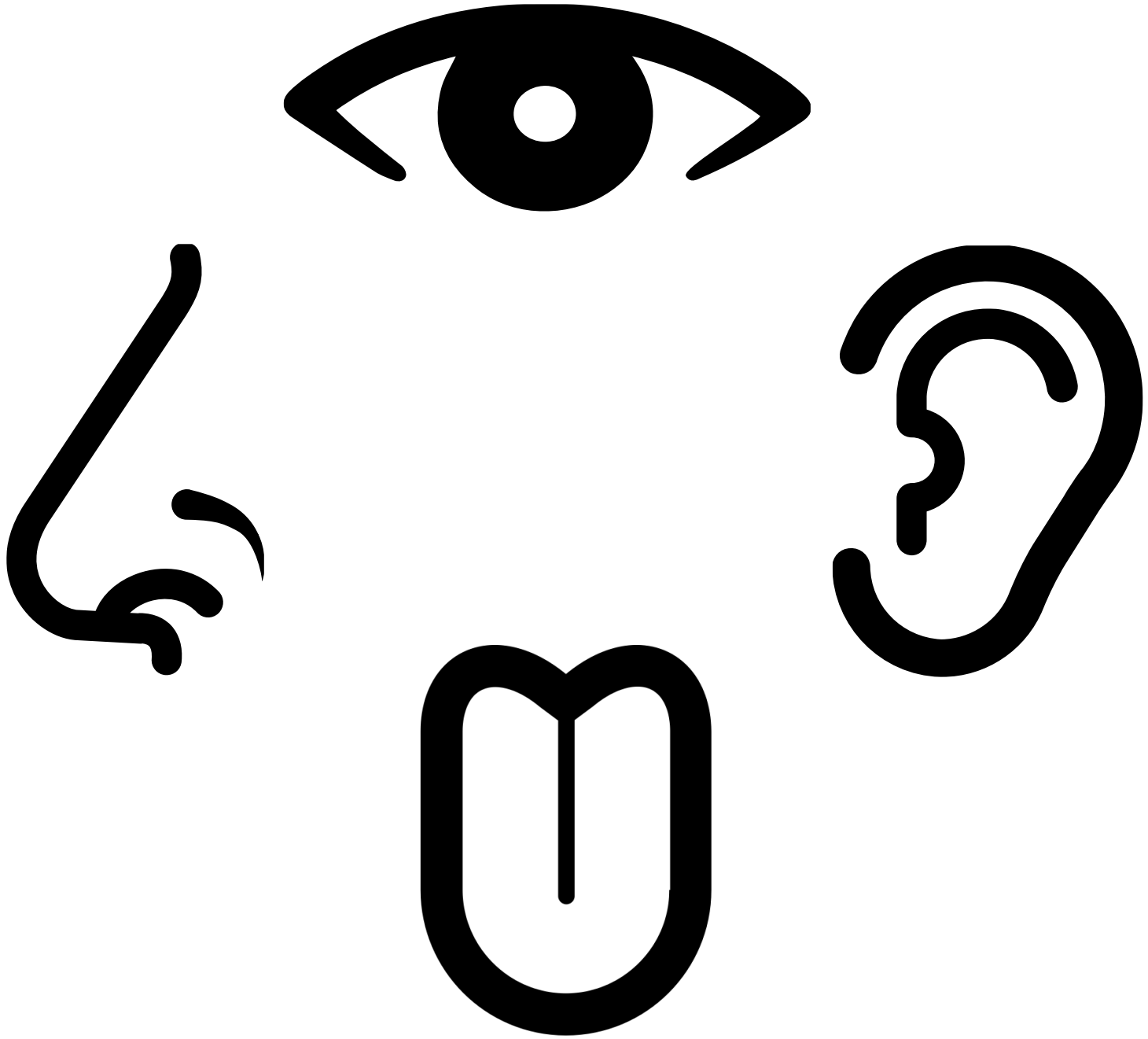
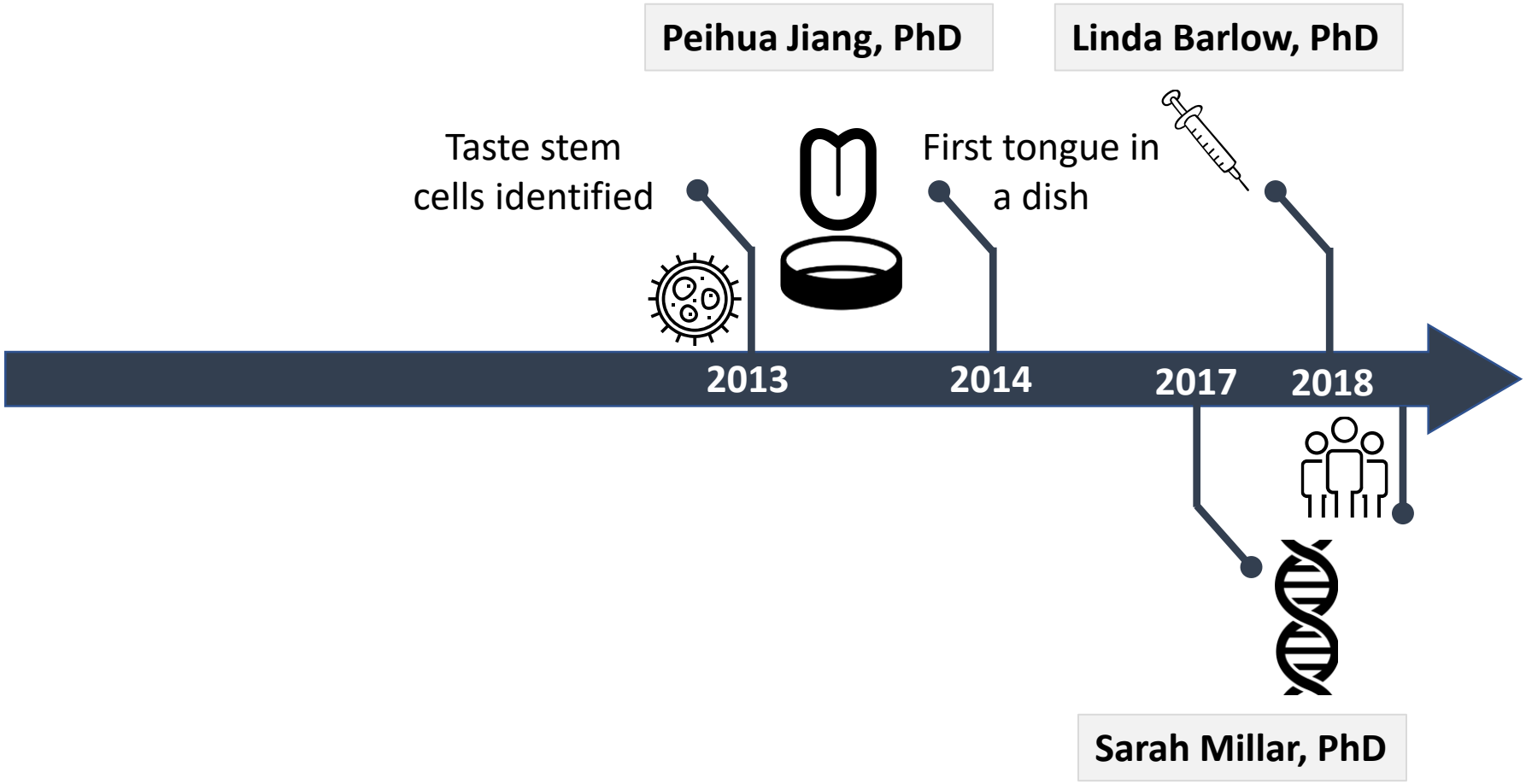
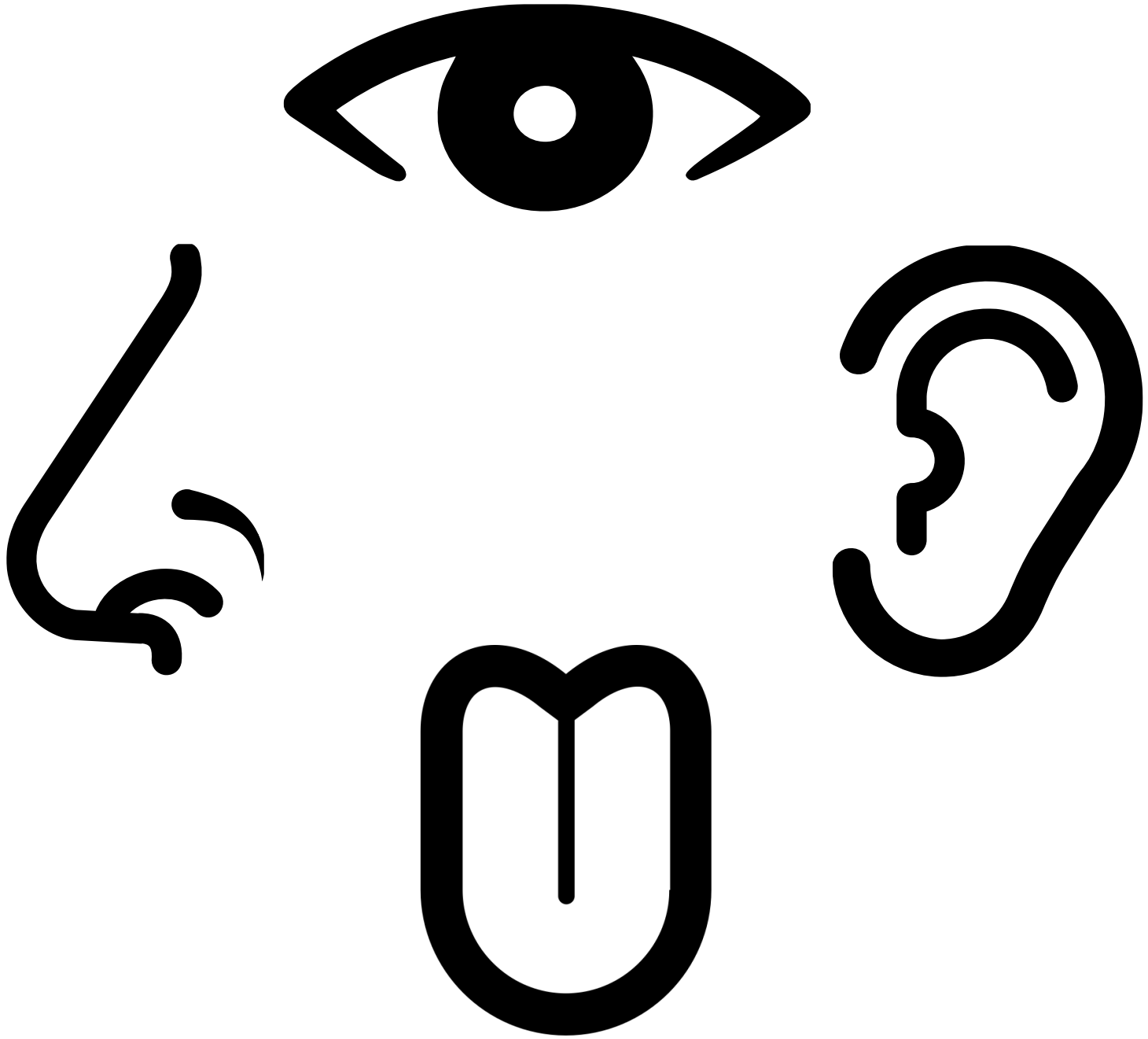


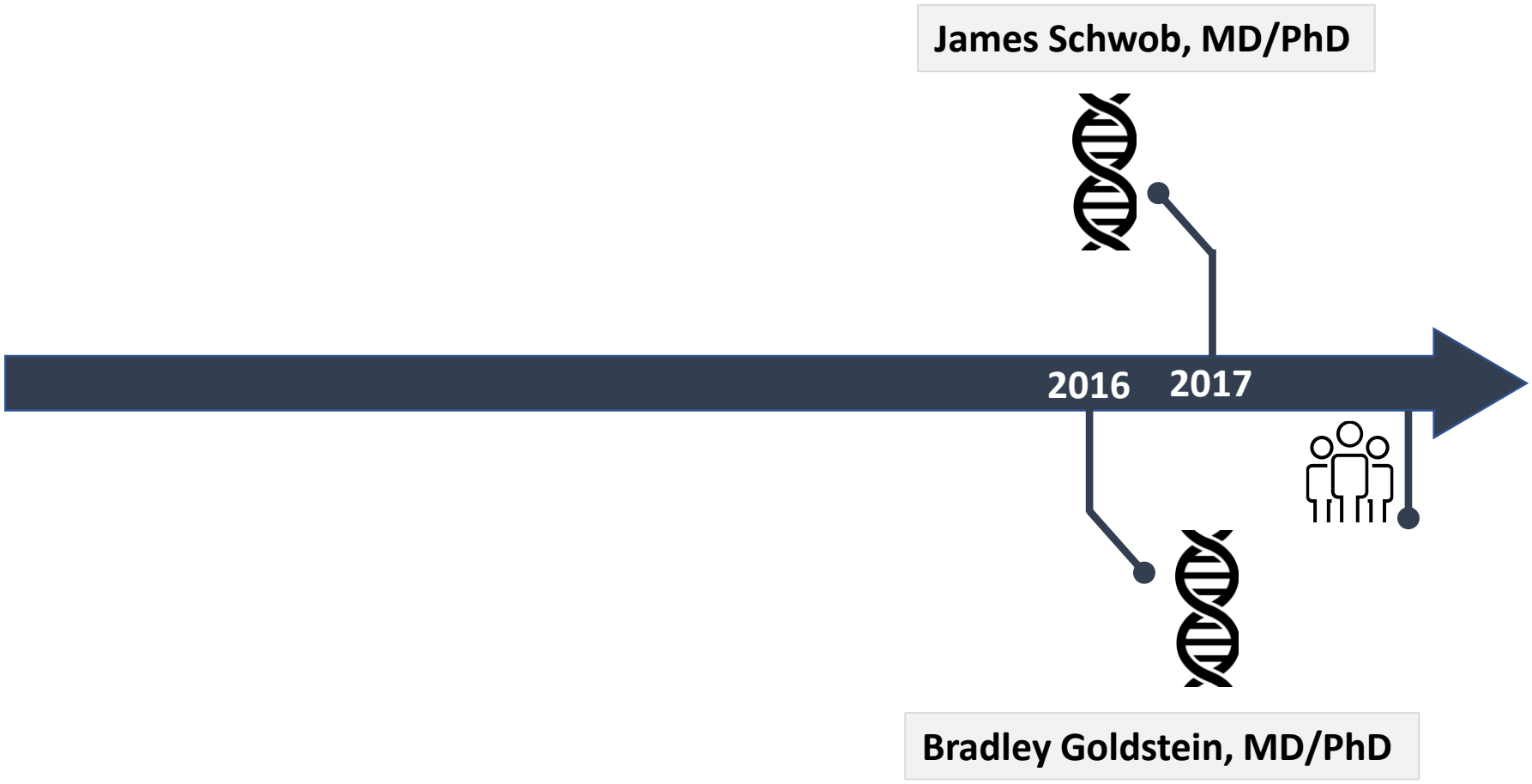


Repairing taste loss with stem cells

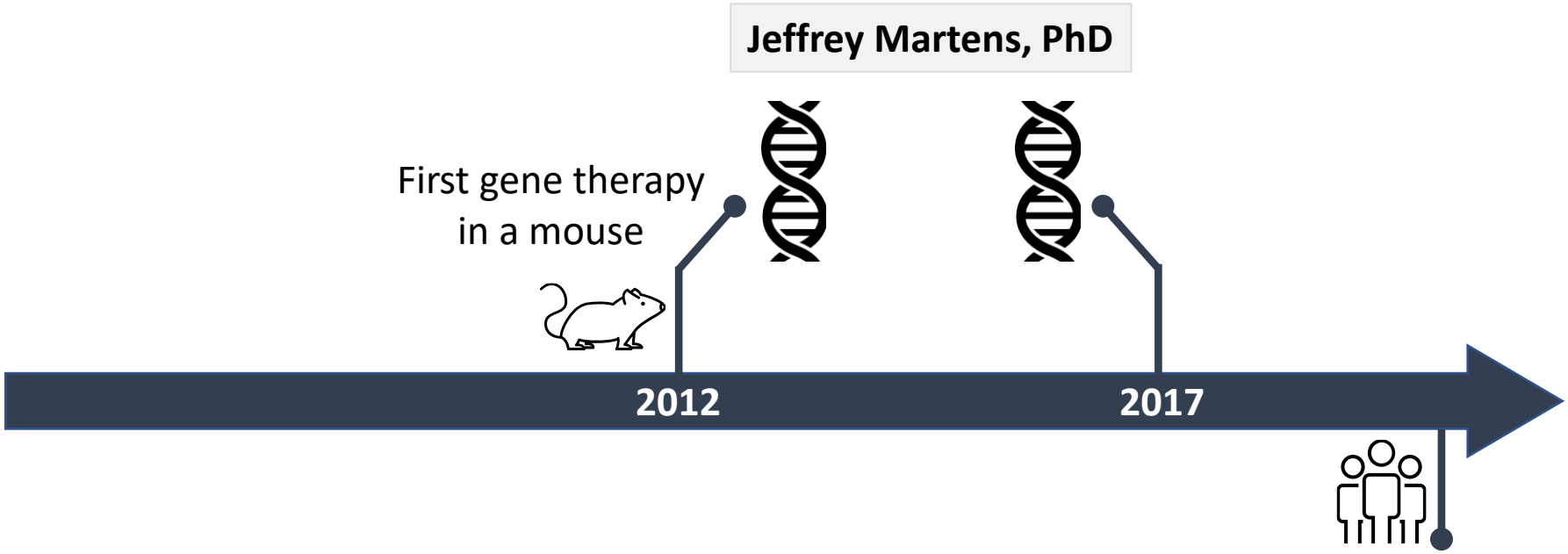


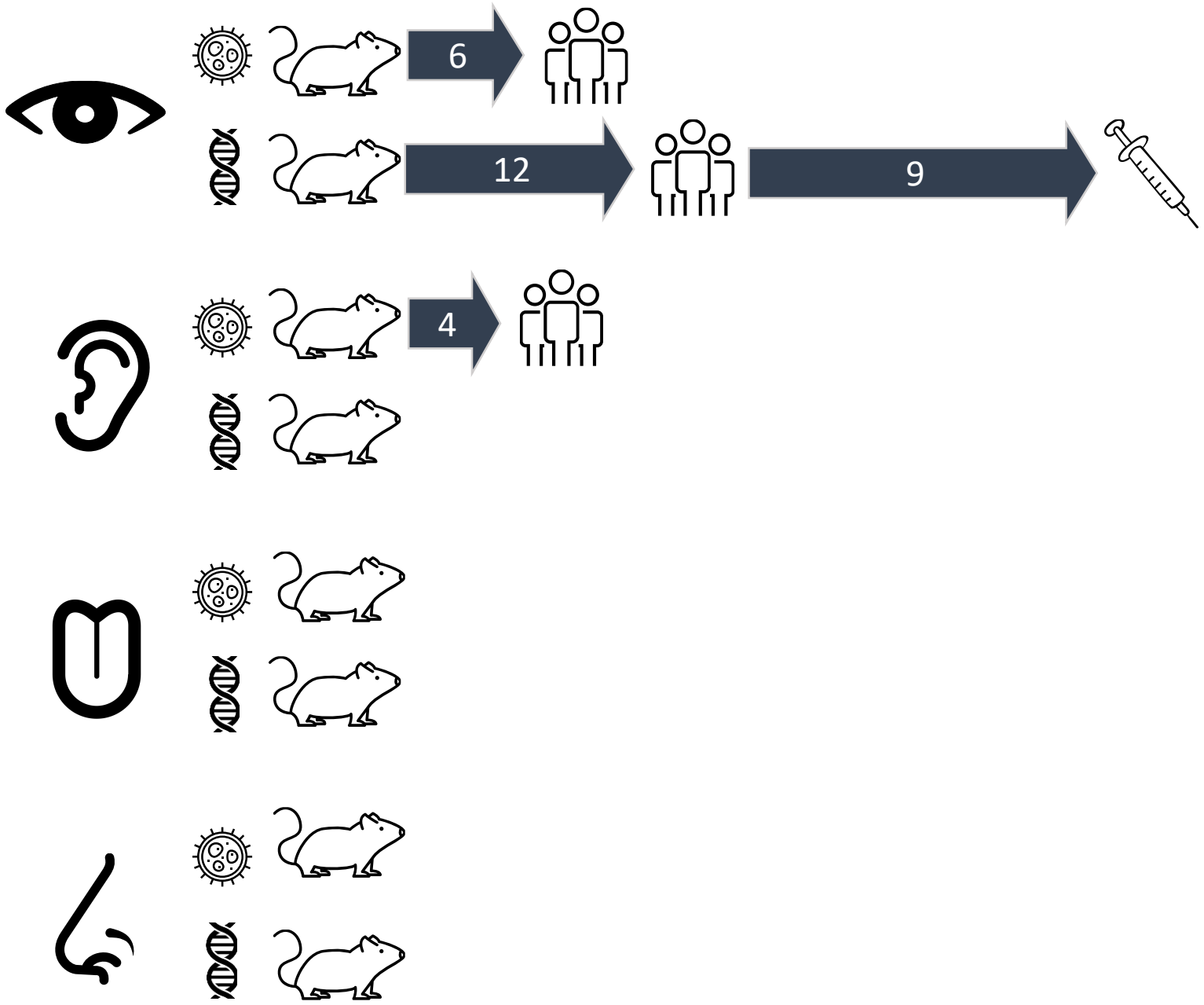


Repairing smell loss with stem cells



Repairing smell loss with gene therapy: An example with ciliopathies

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