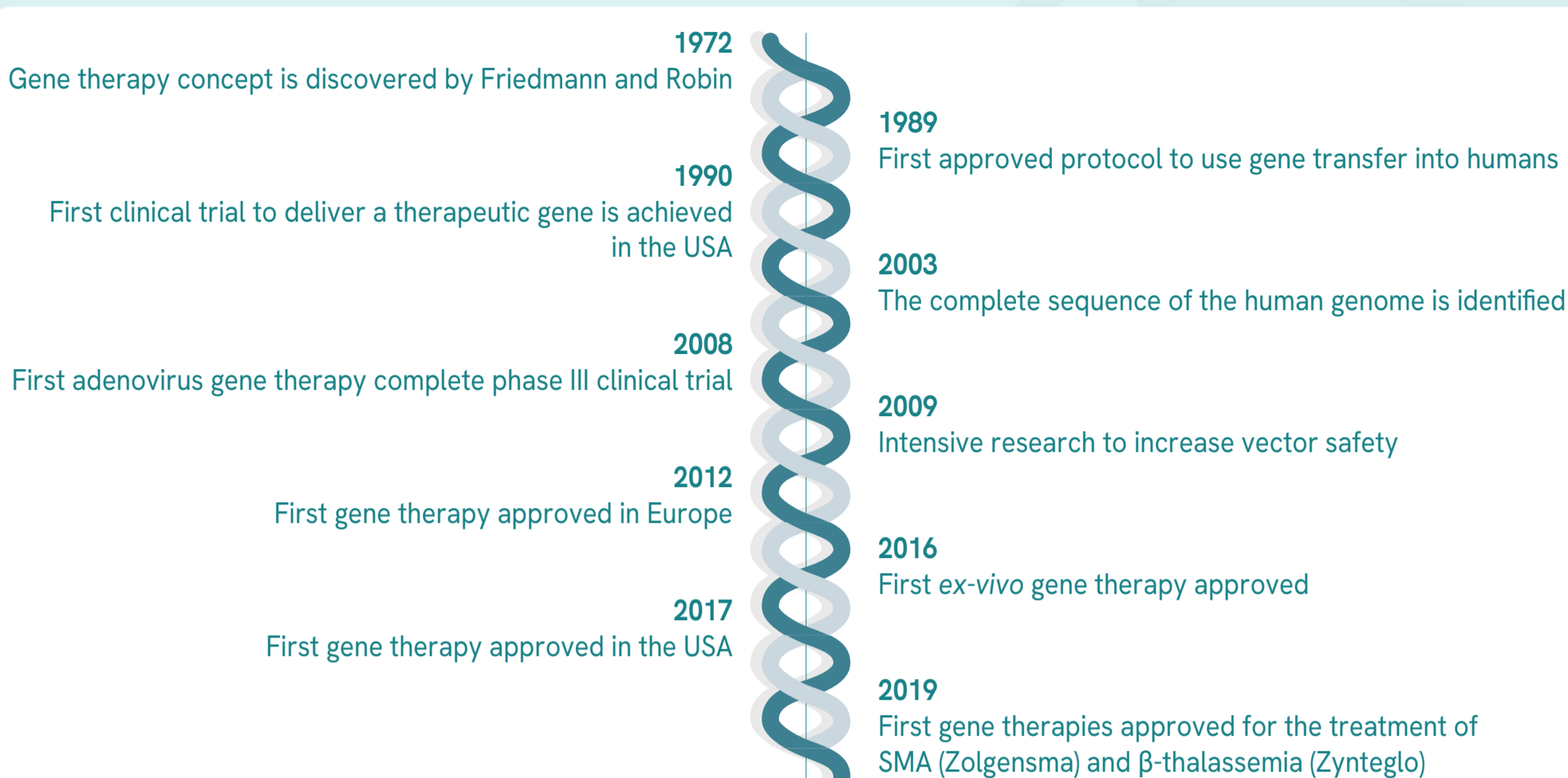


The great story of gene therapy

Scene 1

Significant advancements

over the last decade



Scene 2

Major key facts

in gene therapies

Approaches

- Replacing a mutated gene
- Inactivating a mutated gene
- Introducing a new gene

20 approved gene therapies

>1350 Preclinical

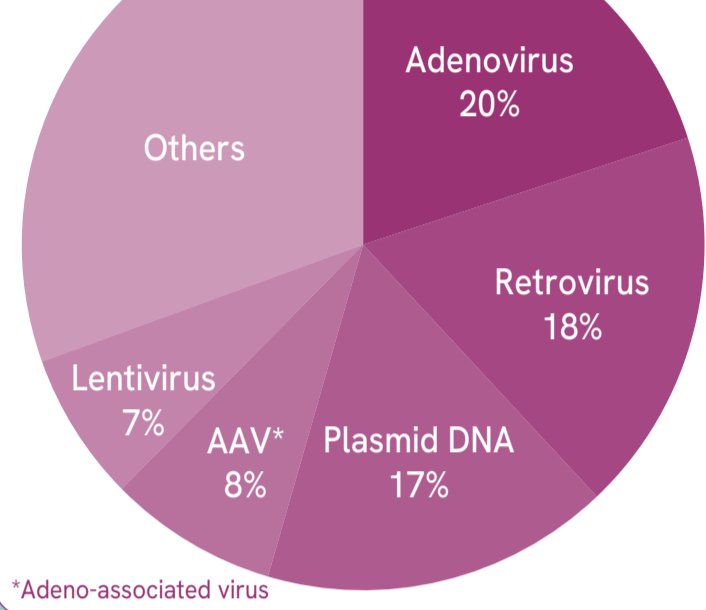
>2400 Product candidates

>300 Phase I

>250 Phase II

>50 Phase III

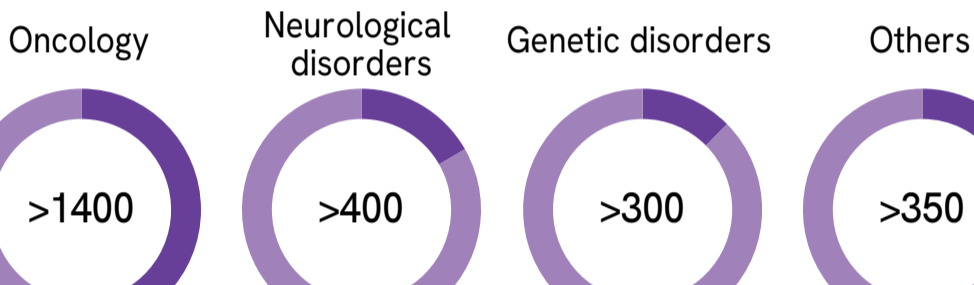
Vectors



Challenges

- Long term safety
- Efficacy over time
- Neutralizing immune responses
- Manufacturing scale up
- Evolving regulatory guidance

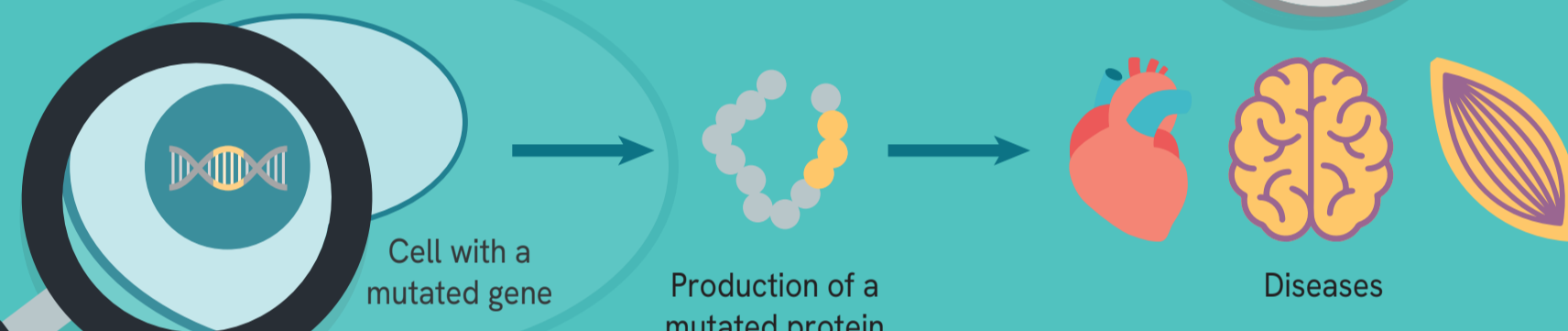
Therapeutic areas



Scene 3

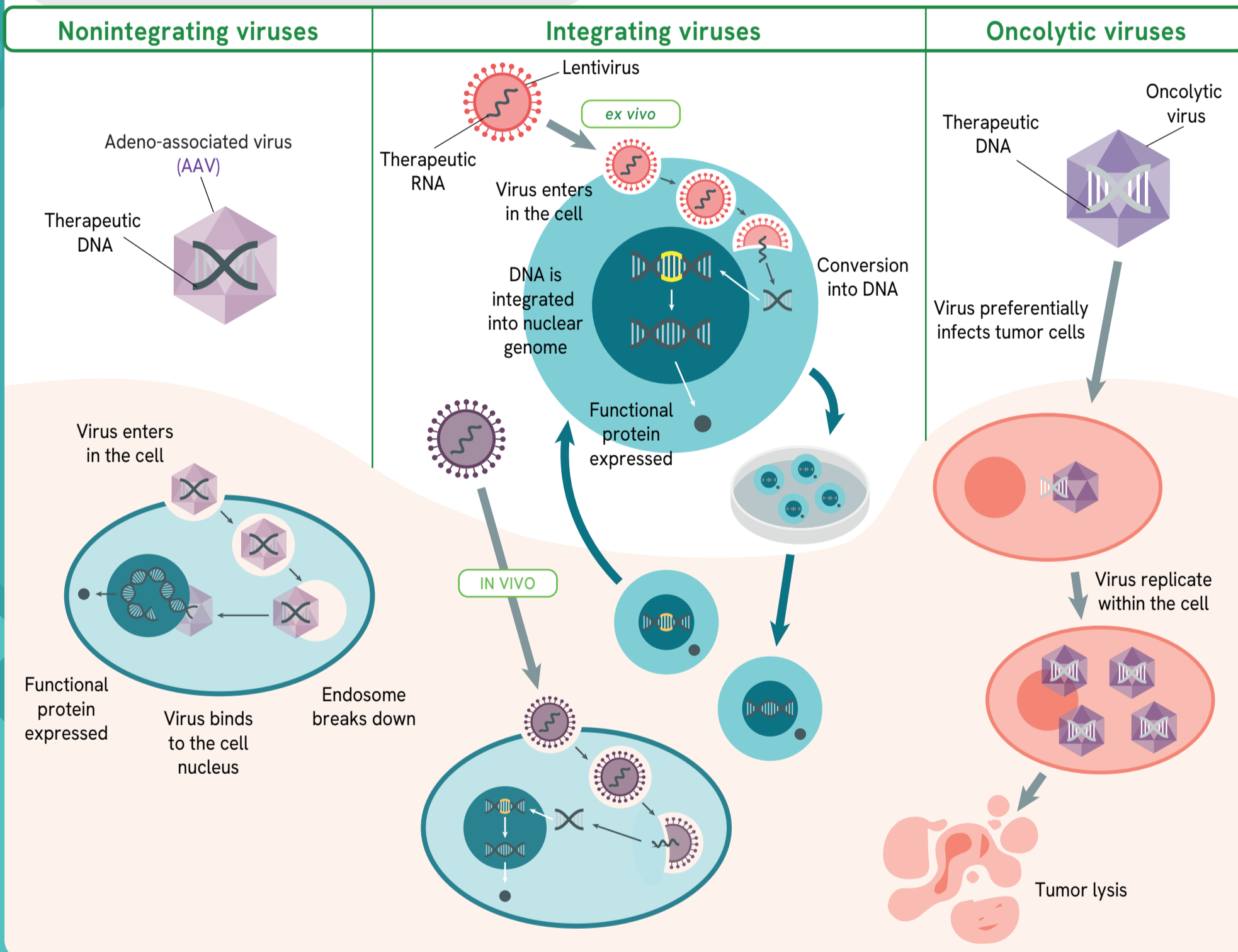
How does gene therapy work?

work?

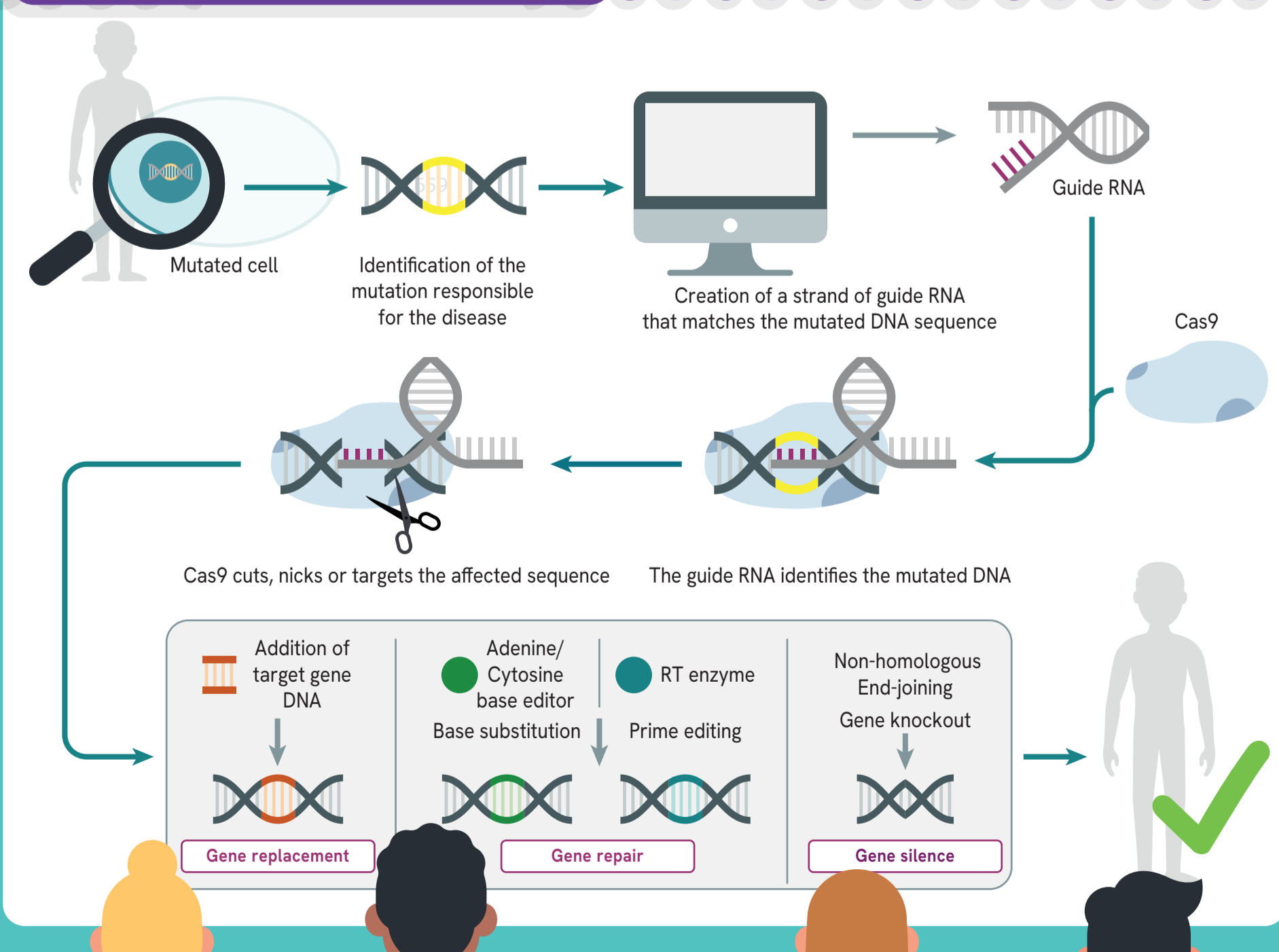


Gene therapy Can correct these mutations

By using viral vectors



By using CRISPR/CAS9



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- Jef Akst. Targeting DNA. The Scientist. | Lucy Reading-Ikkanda. Delivering New Genes. June 2012.
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Are you ready to see more about Gene Therapy? Check the Revvity website <https://www.revvity.com/>

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