

The great story of Gene Therapy

Significant Advancements

over the last decade

Scene 1

- 1972 Gene Therapy concept is discovered by Friedmann and Robin
- 1990 First clinical trial to deliver a therapeutic gene is achieved in the USA
- 2008 First adenovirus gene therapy complete phase III clinical trial
- 2012 First gene therapy approved in Europe
- 2017 First gene therapy approved in the USA

- 1989 First approved protocol to use gene transfer into humans
- 2003 The complete sequence of the human genome is identified
- 2009 Intensive research to increase vector safety
- 2016 First ex-vivo gene therapy approved
- 2019 First gene therapies approved for the treatment of SMA (Zolgensma) and β -thalassemia (Zynteglo)

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

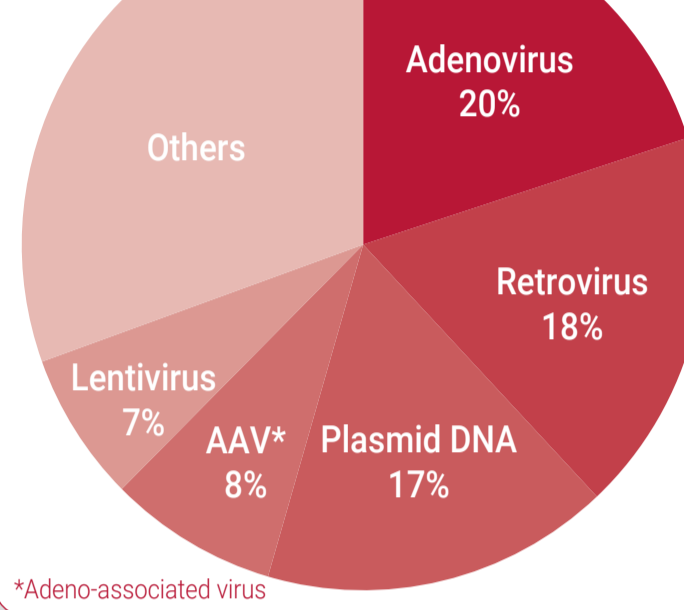
>2400 Product candidates

>300 Phase I

>250 Phase II

>50 Phase III

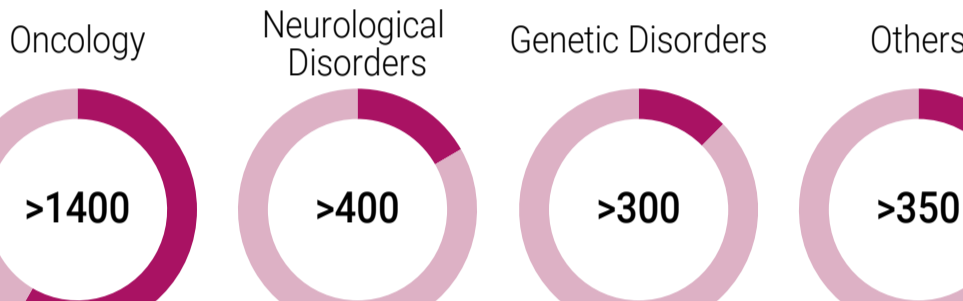
Vectors



Challenges

- Long Term Safety
- Efficacy Over Time
- Neutralizing Immune Responses
- Manufacturing Scale Up
- Evolving Regulatory Guidance

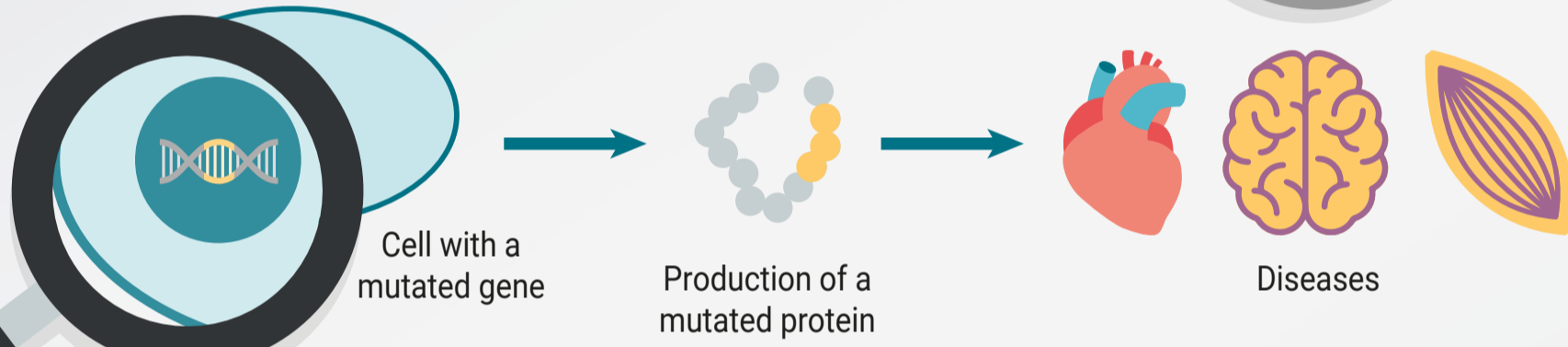
Therapeutic areas



Scene 3

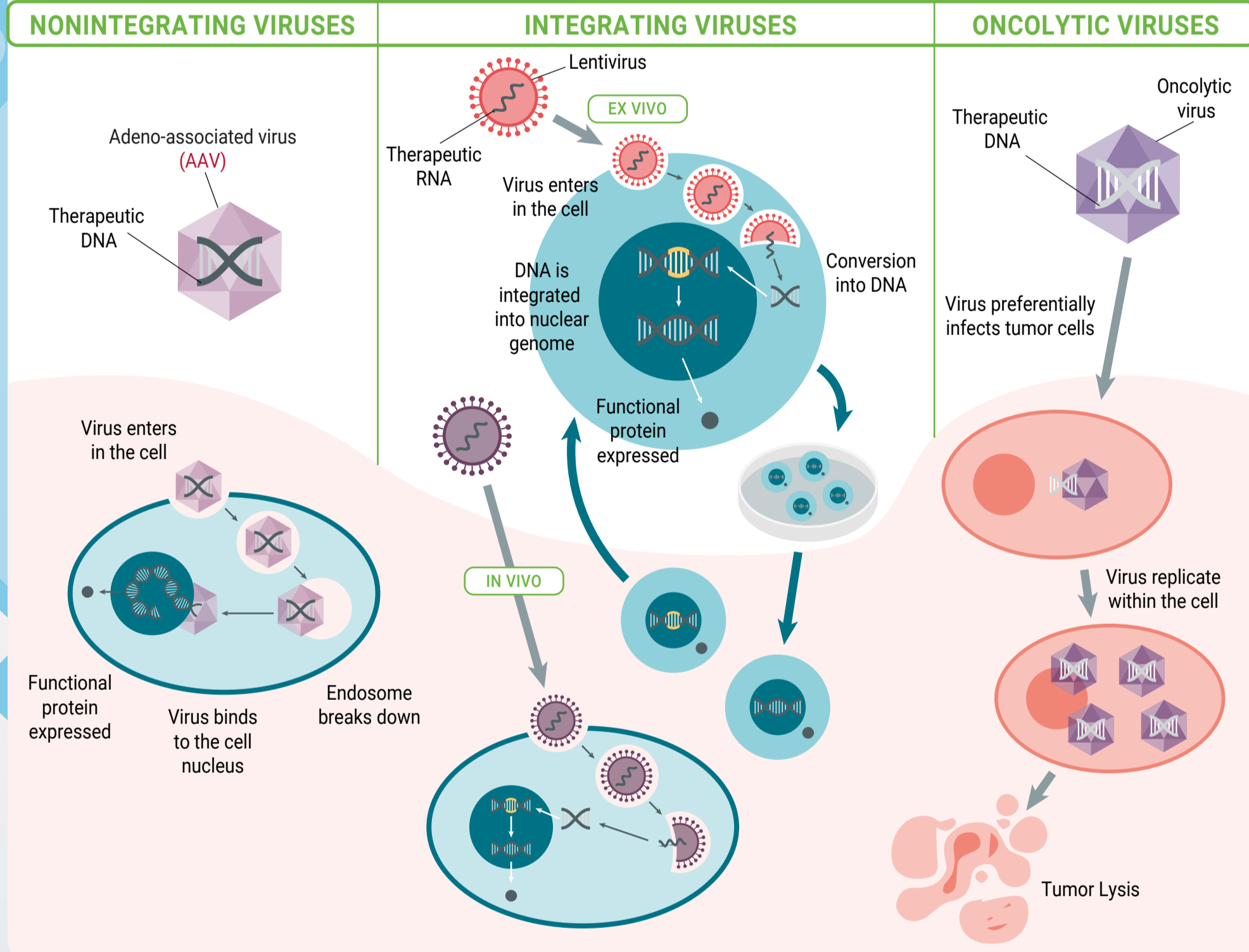
How does gene therapy work?

work?

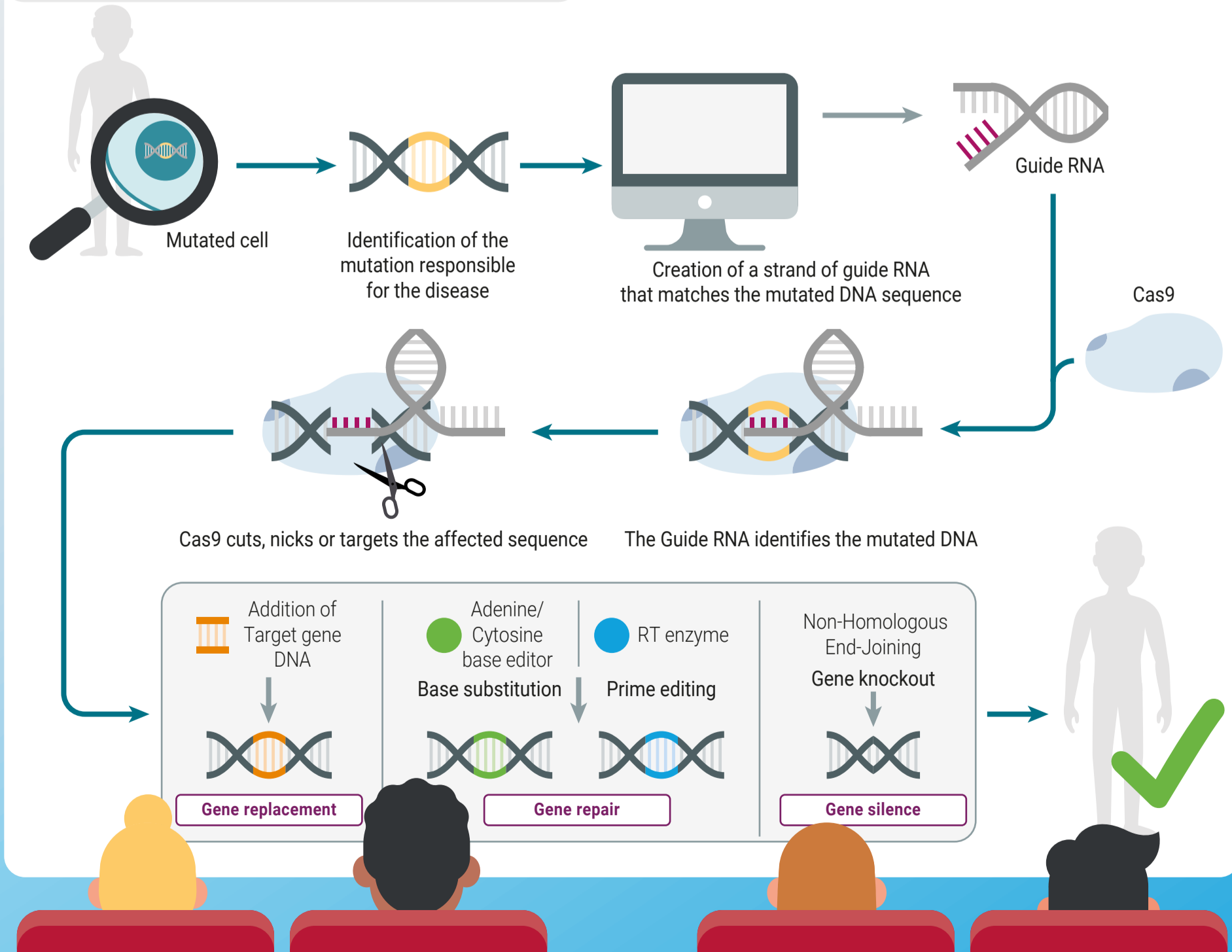


Gene Therapy can correct these mutations

By using Viral Vectors



By using CRISPR/CAS9



Are you ready to see more about **Gene Therapy**?

Check the PerkinElmer website <https://perkinelmer-appliedgenomics.com/home/applications/aaav-characterization/>

and follow us on:



References

- Gene Therapy Industry Report 2021. Labiotech. June 2021.
- Jef Akst. Targeting DNA. The Scientist. | Lucy Reading-Ikkanda. Delivering New Genes. June 2012.
- Ansari Karimian et al. CRISPR/Cas9 technology as a potent molecular tool for gene therapy. Journal of cellular Physiology. January 2019.