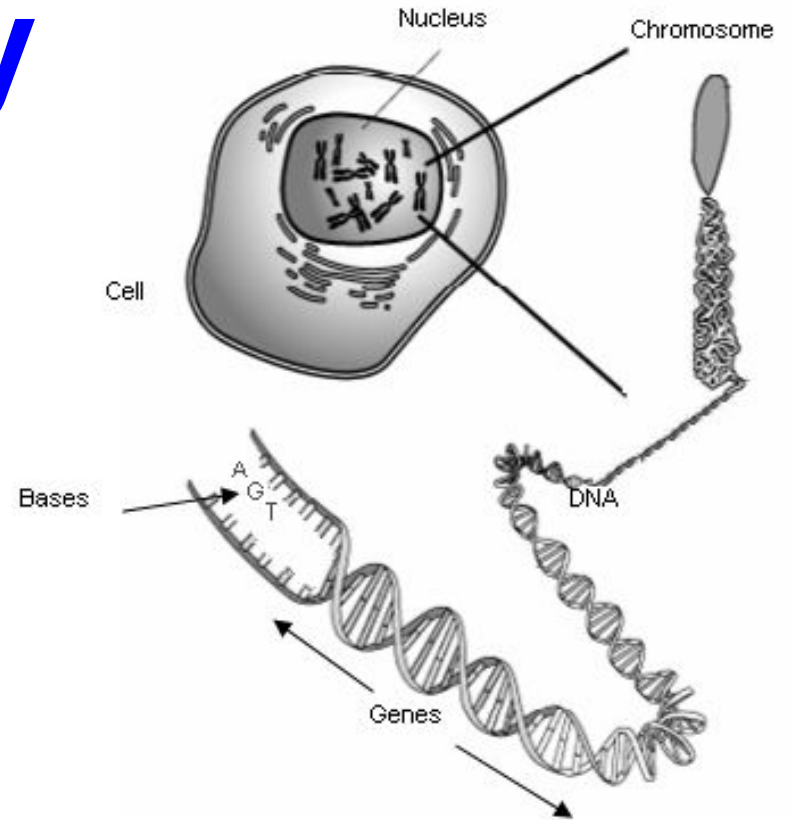
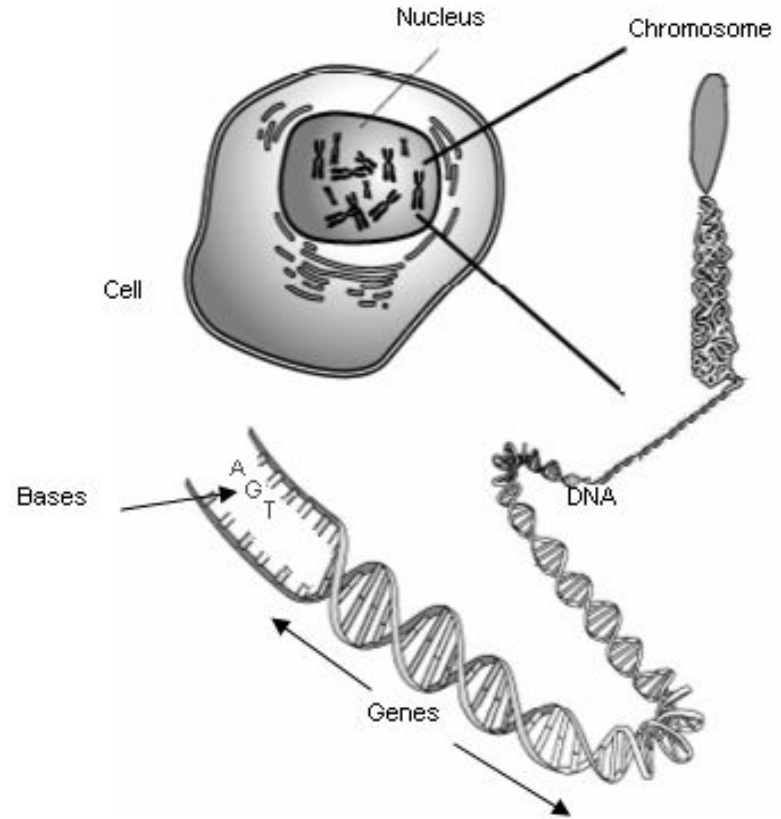
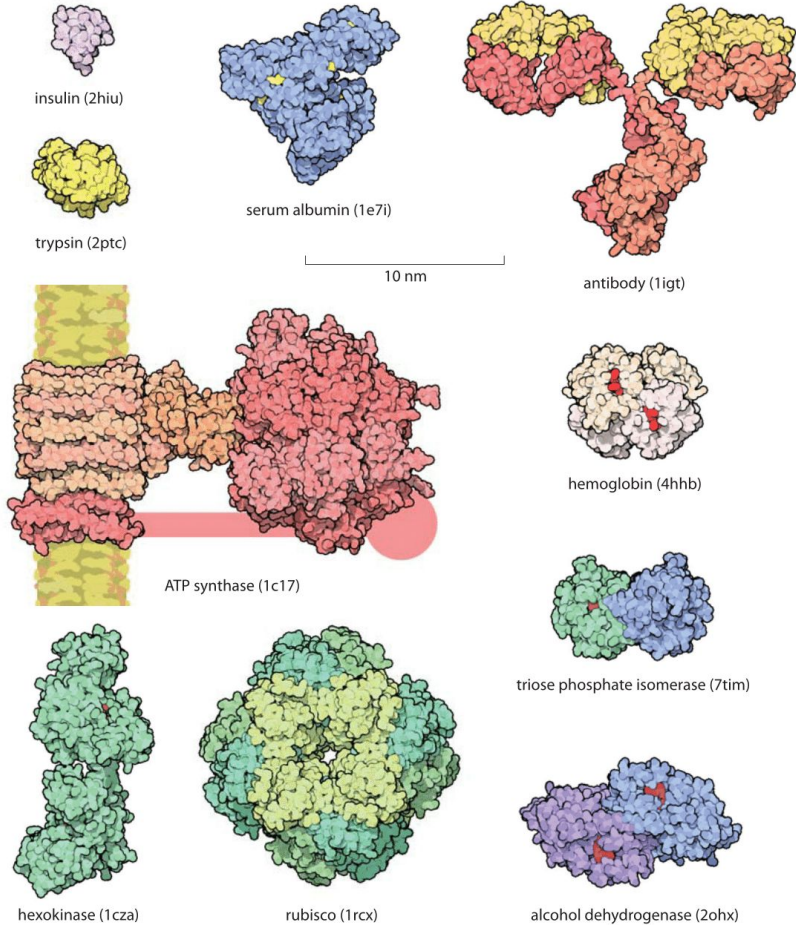


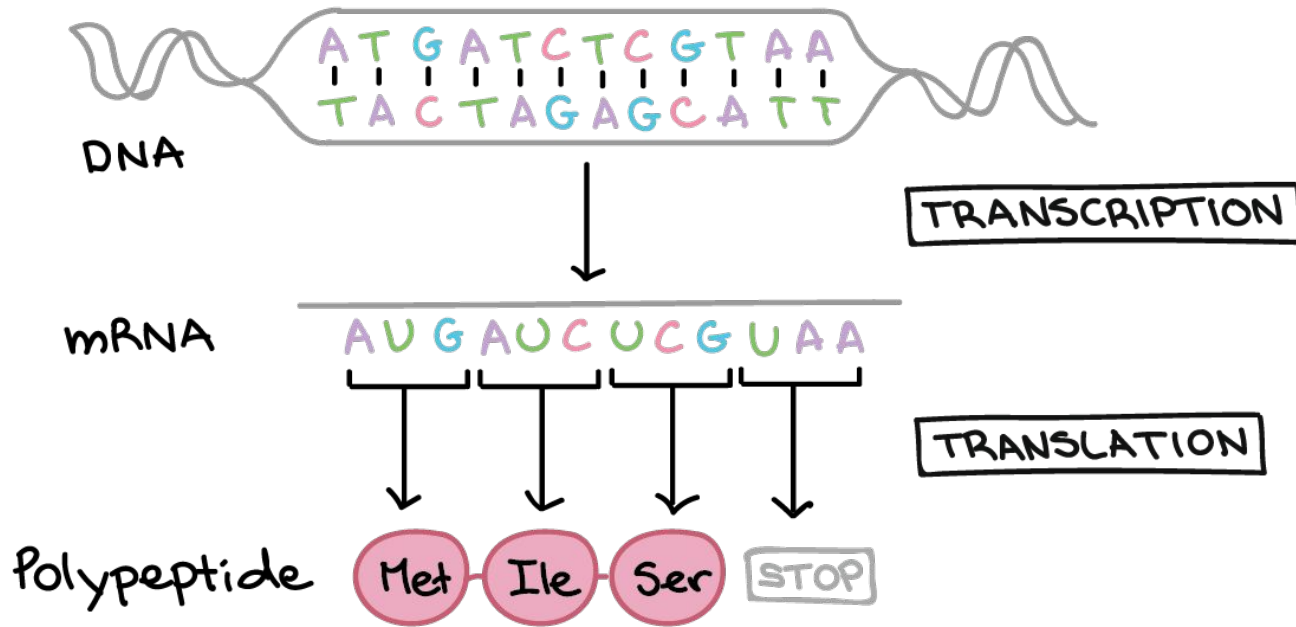
Gene Therapy



How are the body's proteins related to its genes?

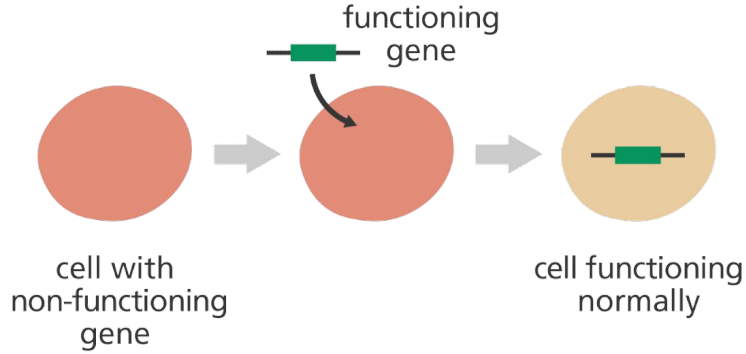


How are the body's proteins related to its genes?

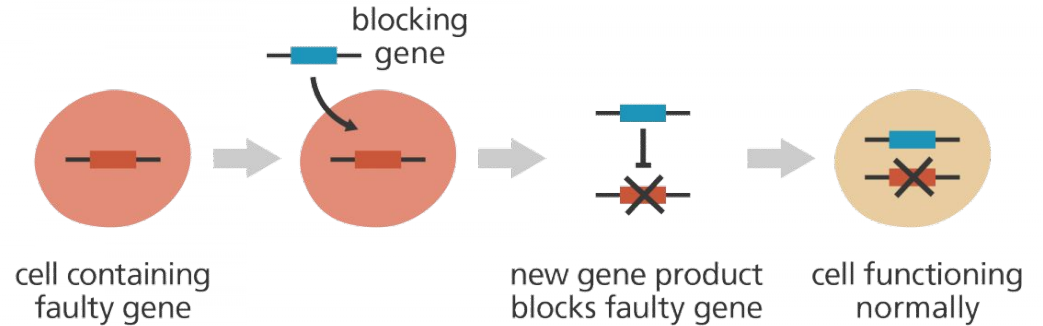


How does gene therapy work?

Gene augmentation therapy

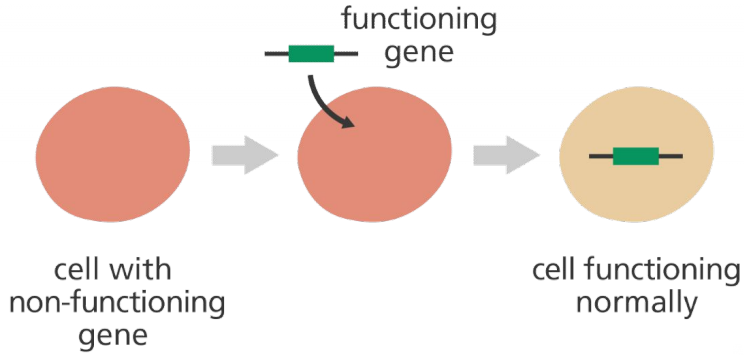


Gene inhibition therapy

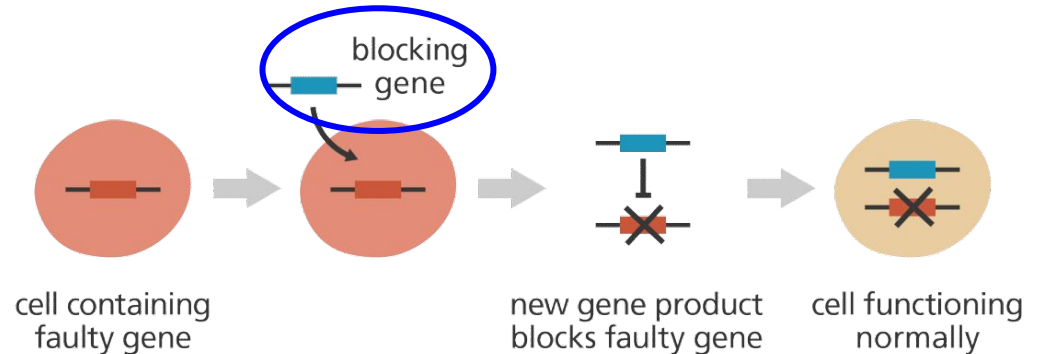


How does gene therapy work?

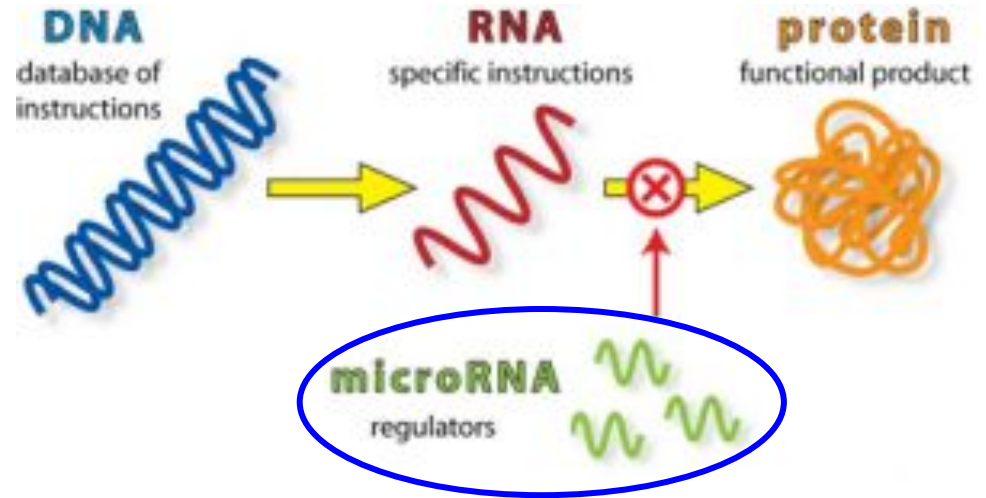
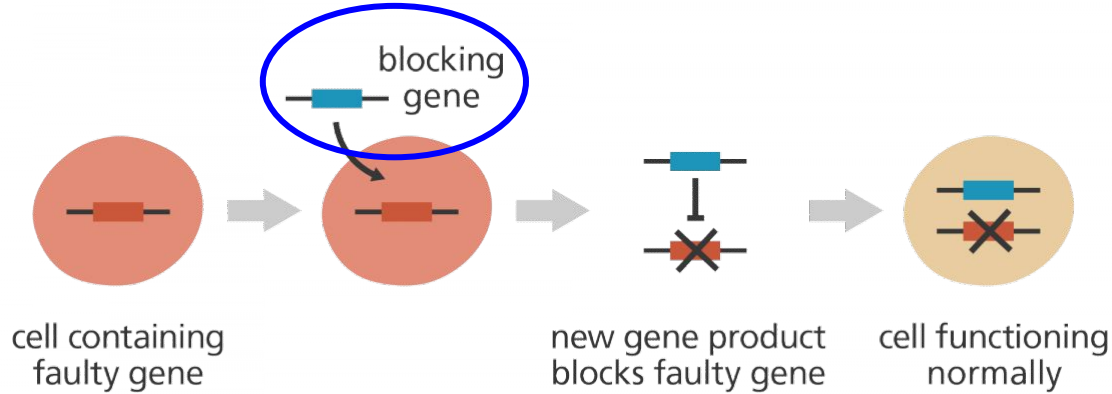
Gene augmentation therapy



Gene inhibition therapy

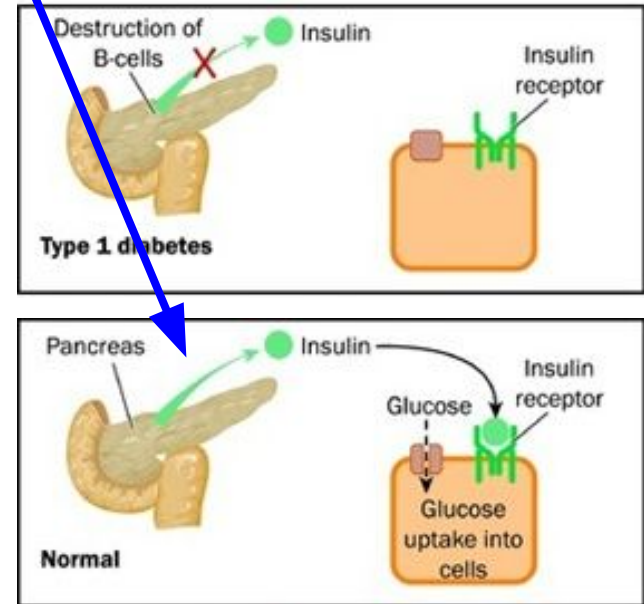
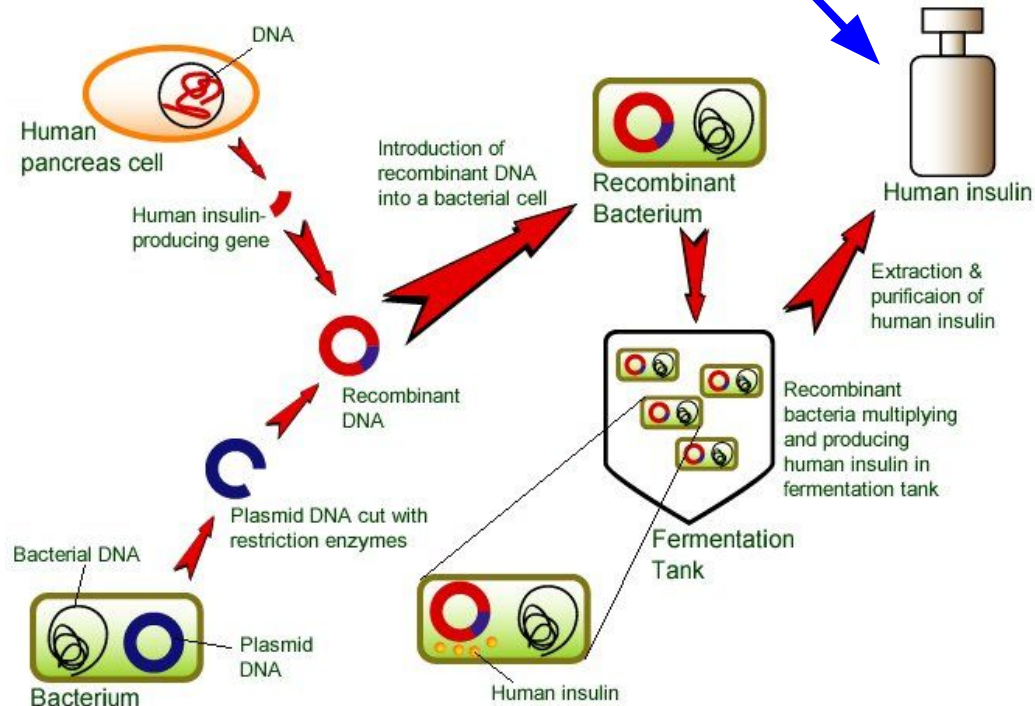


Gene inhibition therapy

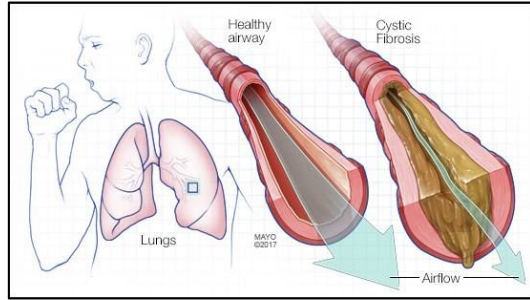


Why might gene therapy be preferred to treatment with proteins like insulin?

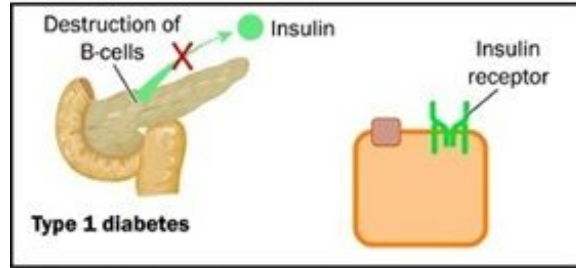
Human Insulin Production



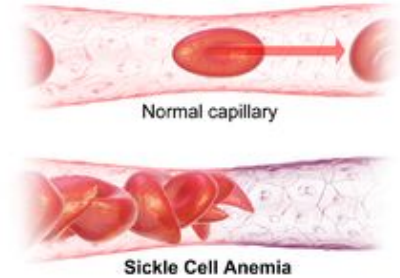
Is this genetic disease a good candidate for **gene therapy**?



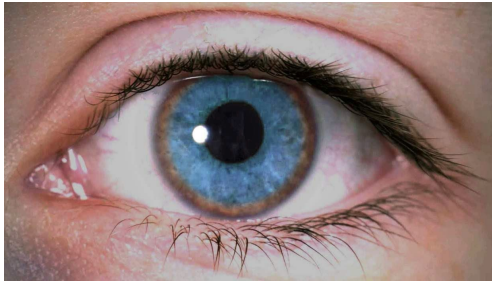
cystic fibrosis



type I diabetes



sickle cell anemia



Wilson's disease

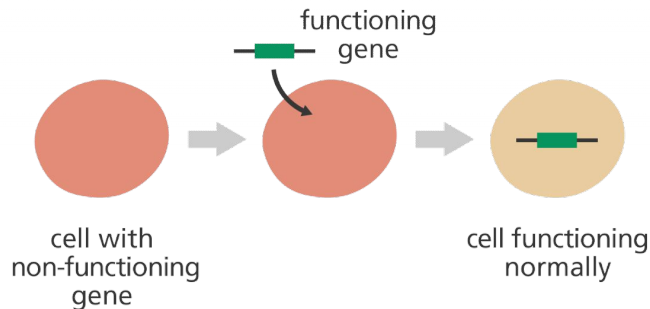


Down syndrome

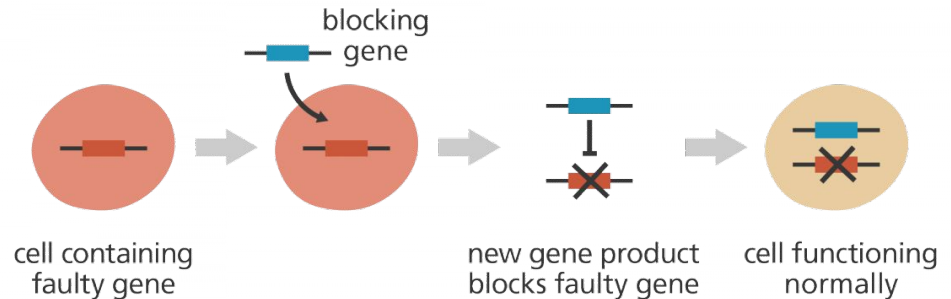
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- 4) Can we deliver the gene to affected cells?

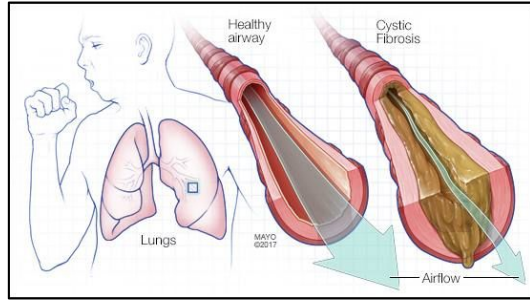
Gene augmentation therapy



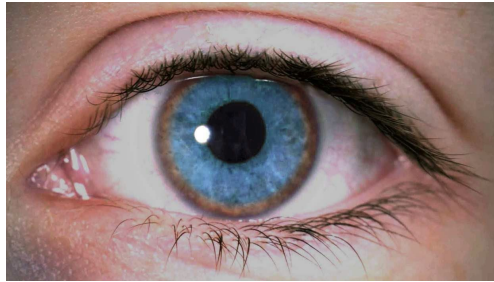
Gene inhibition therapy



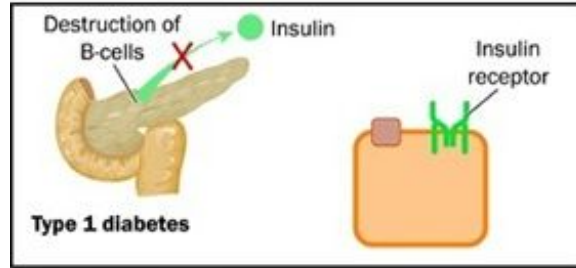
Is this genetic disease a good candidate for gene therapy?



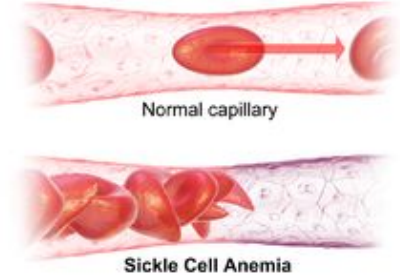
cystic fibrosis



Wilson's disease



type I diabetes



sickle cell anemia

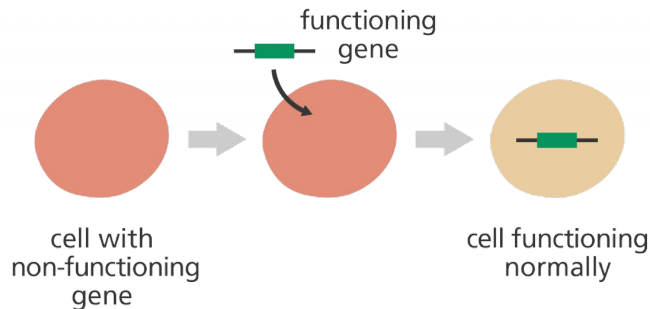


Down syndrome

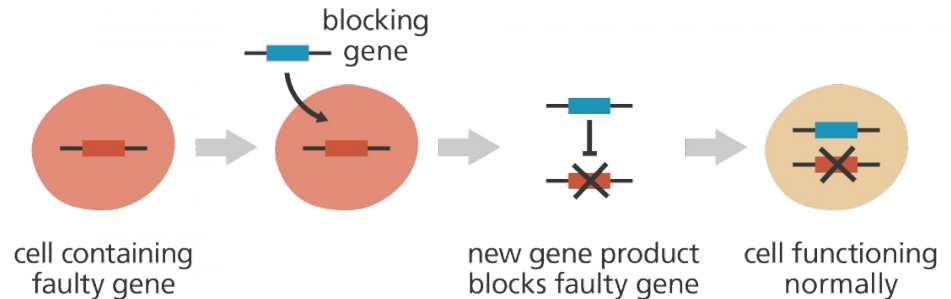
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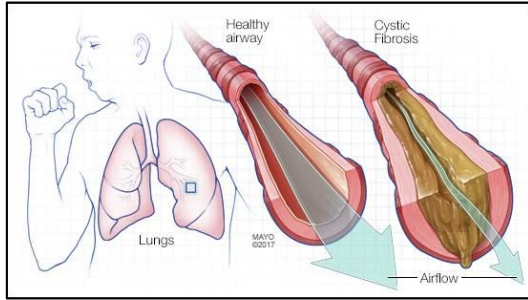
Gene augmentation therapy



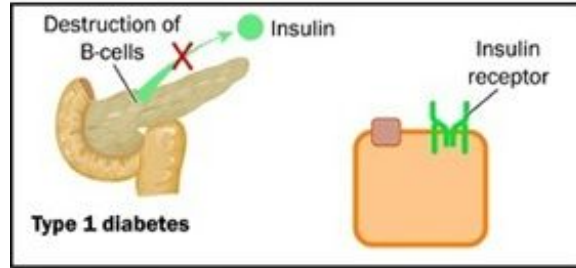
Gene inhibition therapy



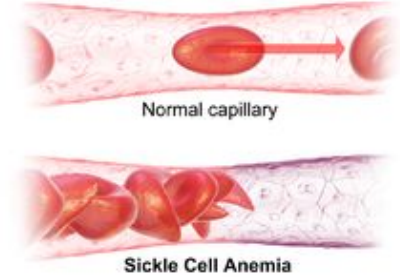
Is this genetic disease a good candidate for gene therapy?



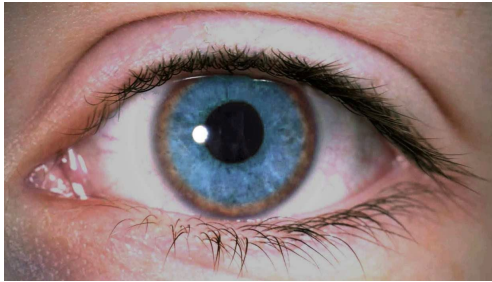
cystic fibrosis



type I diabetes



sickle cell anemia



Wilson's disease

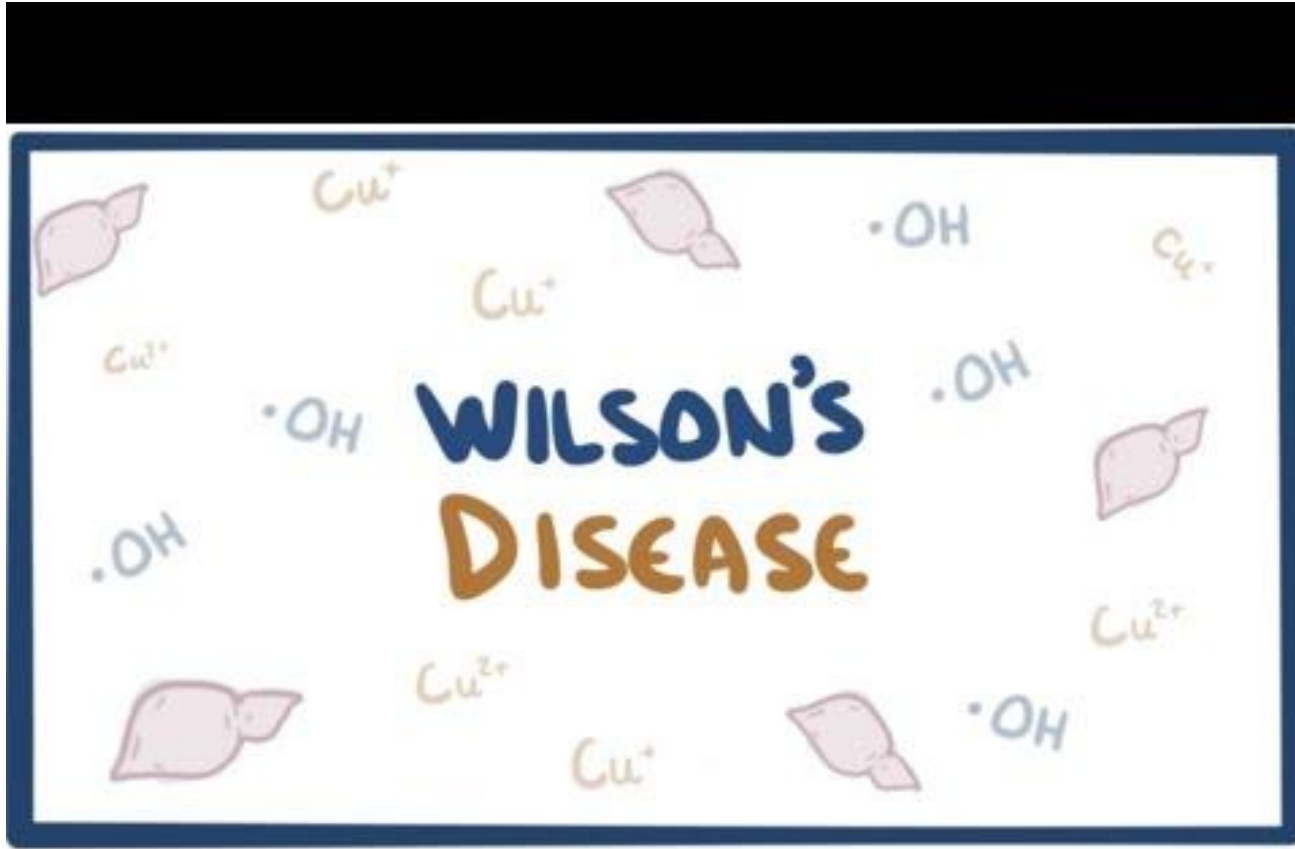


Down syndrome

Is **Wilson's disease** a good candidate for gene therapy?



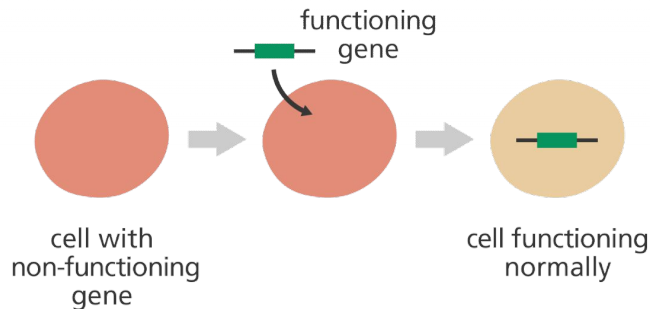
Is Wilson's disease a good candidate for gene therapy?



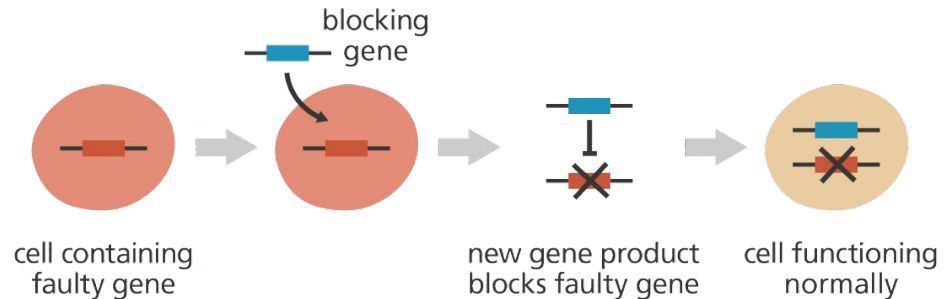
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Gene augmentation therapy

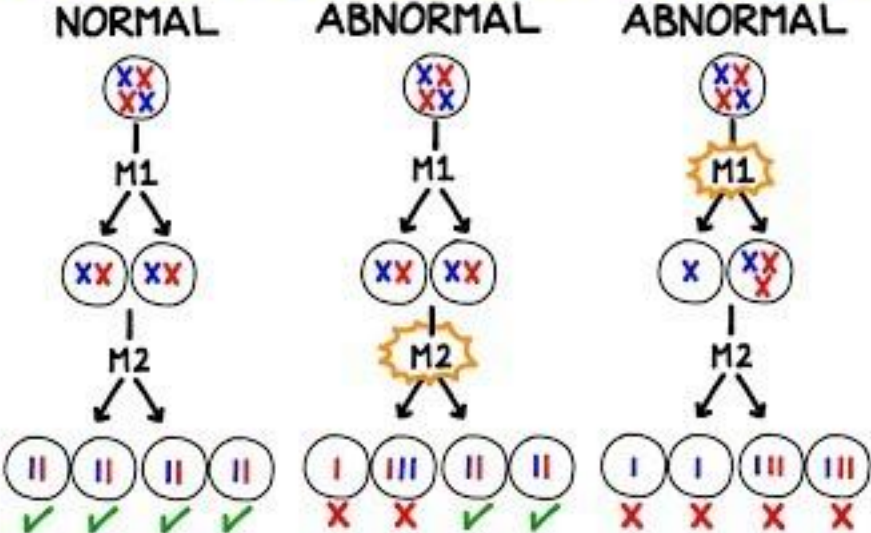


Gene inhibition therapy



Is Down syndrome a good candidate for gene therapy?

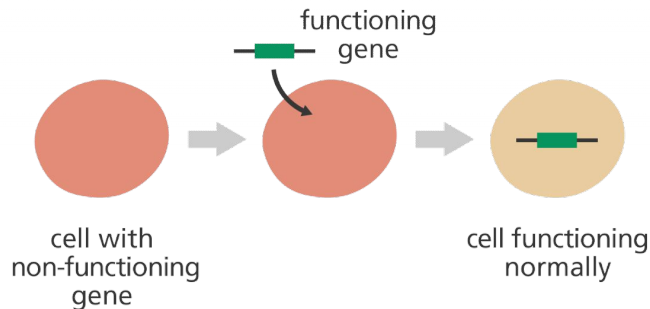
NON-DISJUNCTION



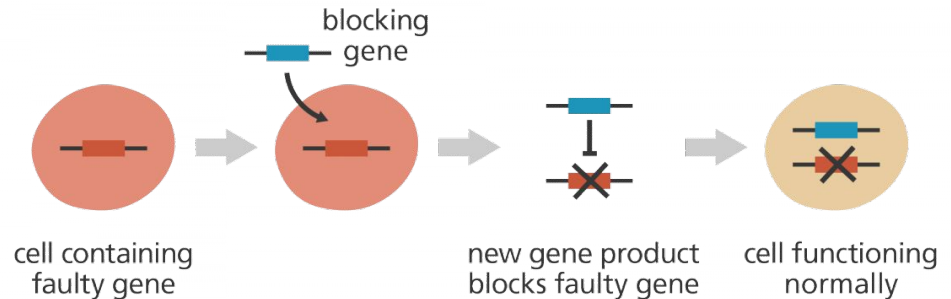
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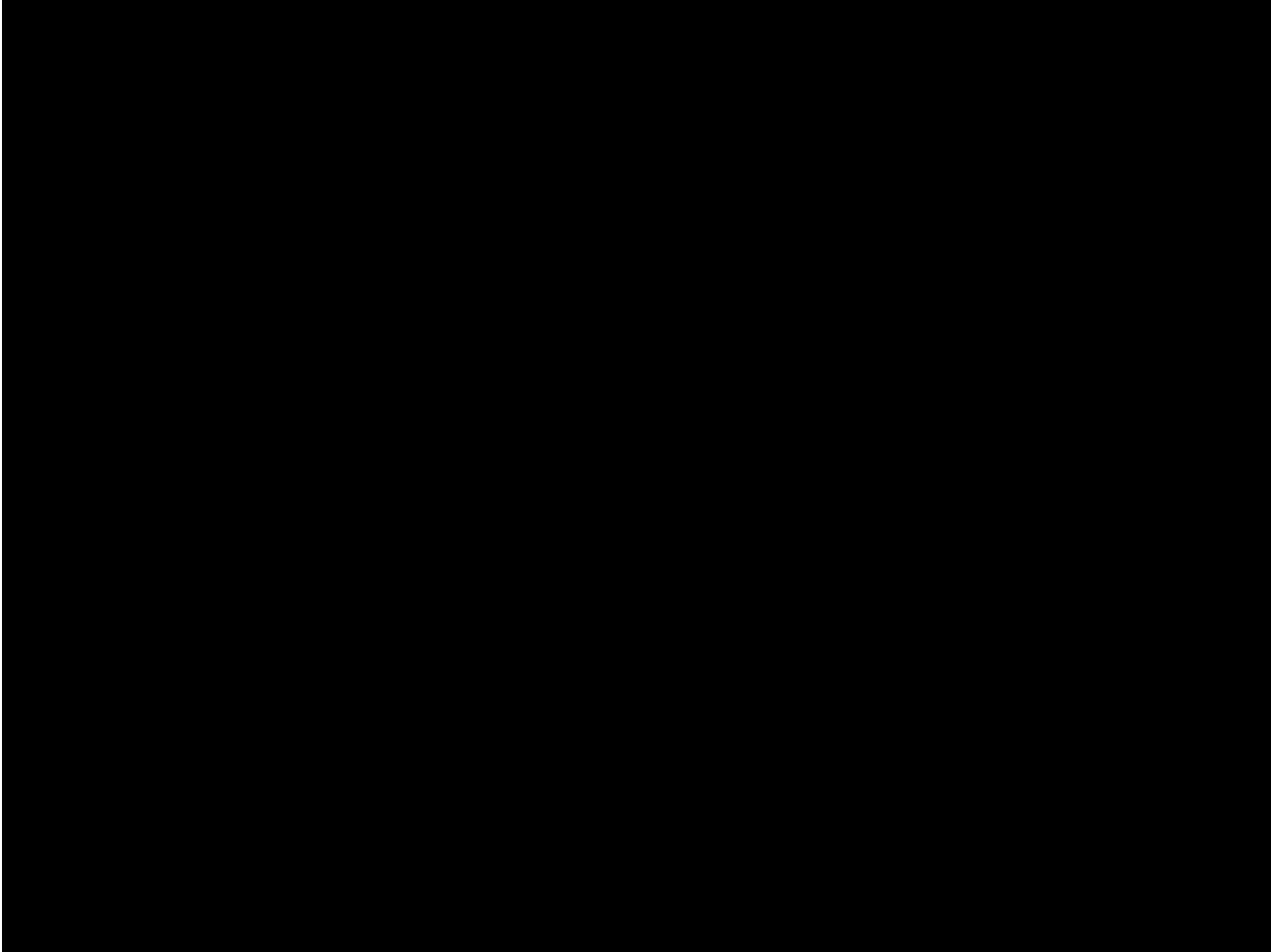
Gene augmentation therapy



Gene inhibition therapy



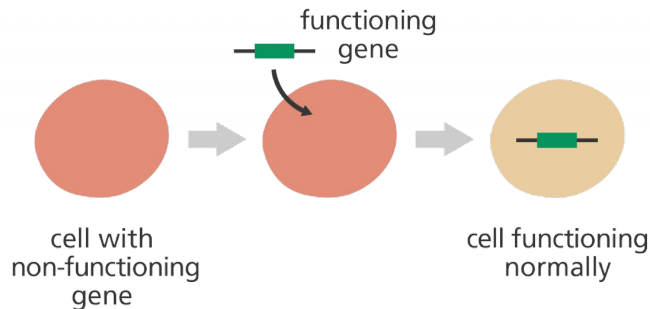
Is sickle cell anemia a good candidate for gene therapy?



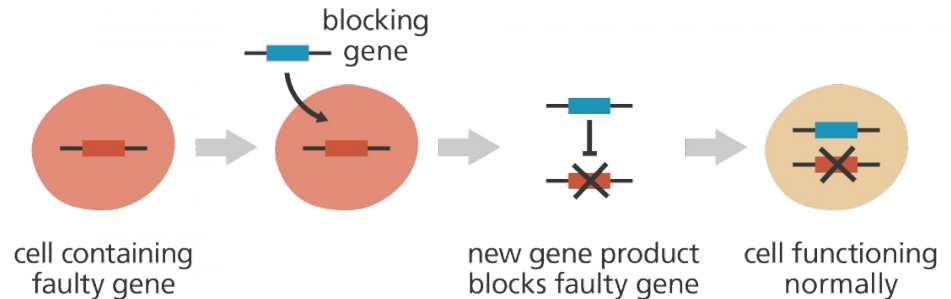
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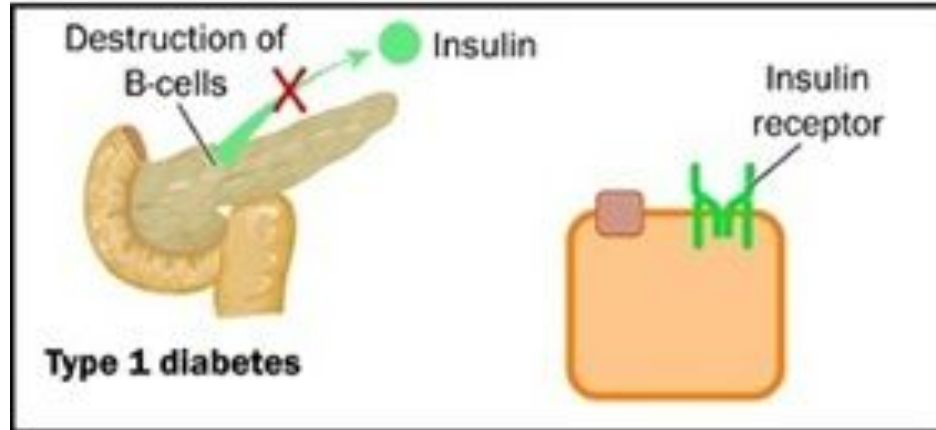
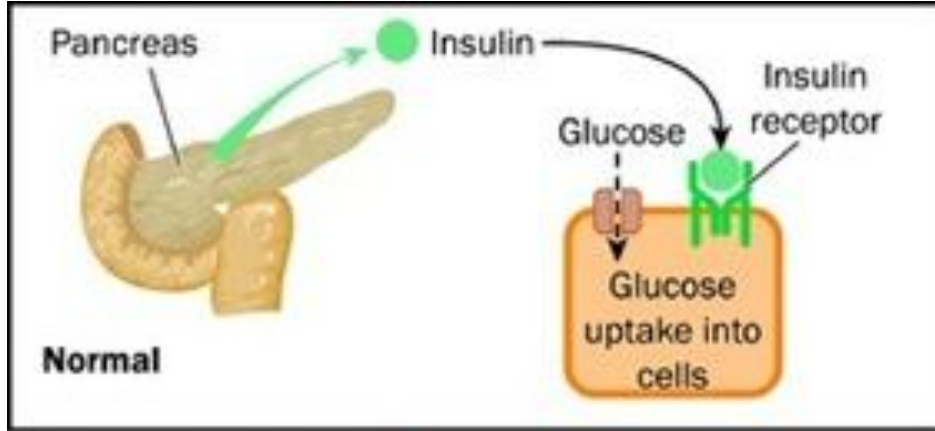
Gene augmentation therapy



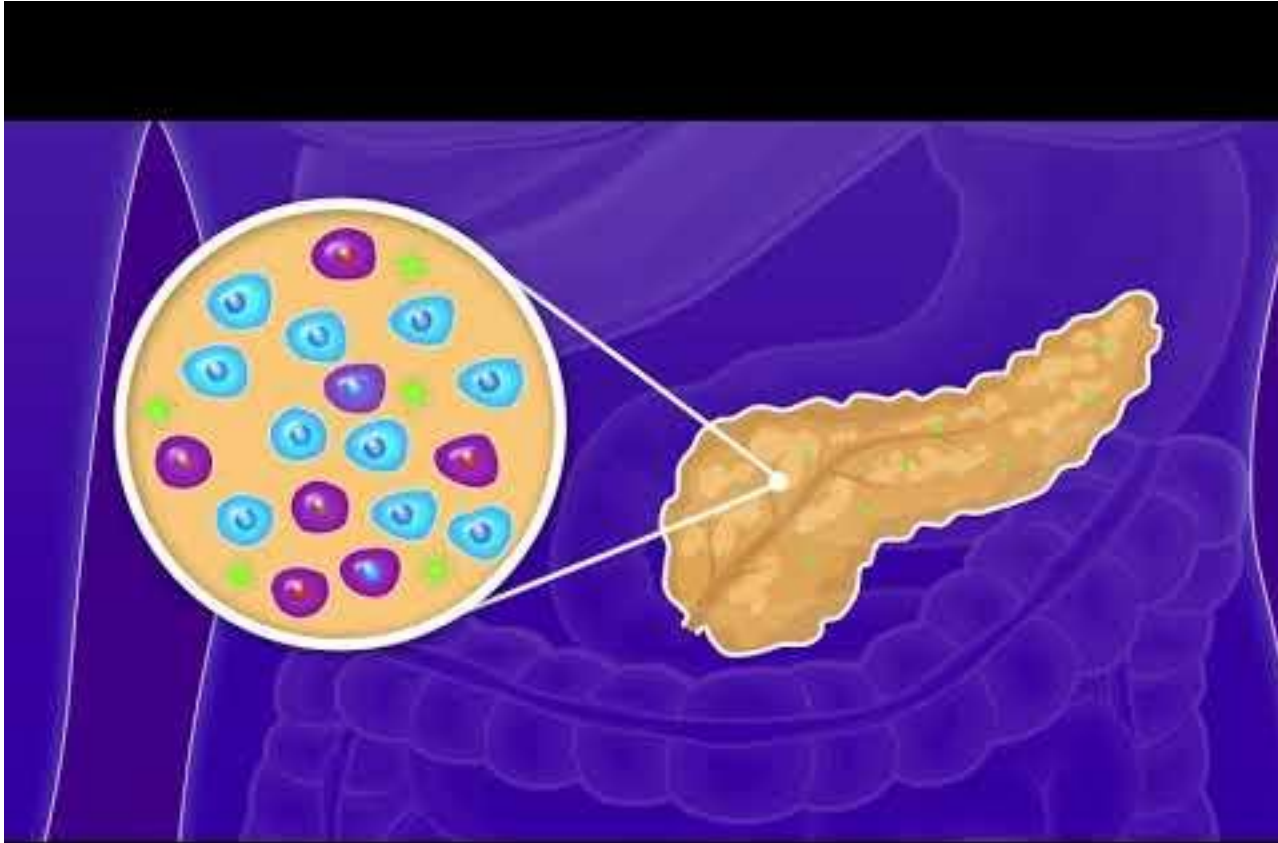
Gene inhibition therapy



Is type 1 diabetes a good candidate for gene therapy?



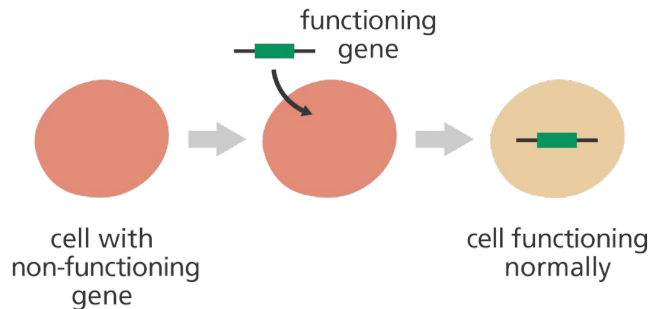
Is type I diabetes a good candidate for gene therapy?



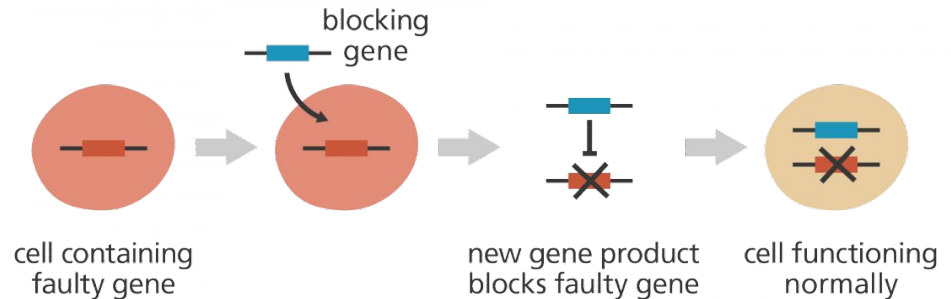
Is this genetic disease a good candidate for gene therapy?

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Gene augmentation therapy



Gene inhibition therapy



How are genes delivered?

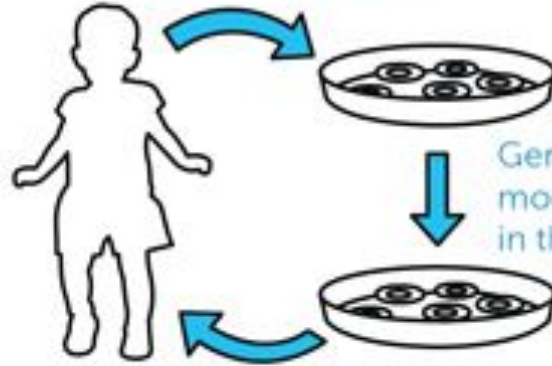
In Vivo

Genes are transferred into cells while still in the patient



Ex Vivo

Cells are taken from the patient



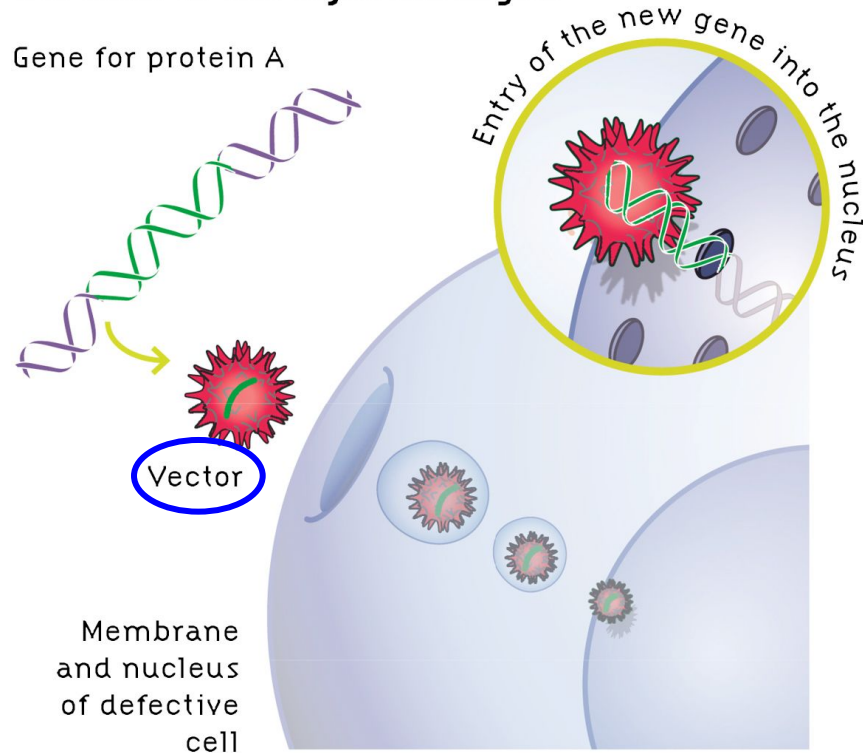
Gene is modified in the lab

Cells are transferred back into the patient

How are genes delivered?

Action of vectors at the level of
the cells of the injected organ

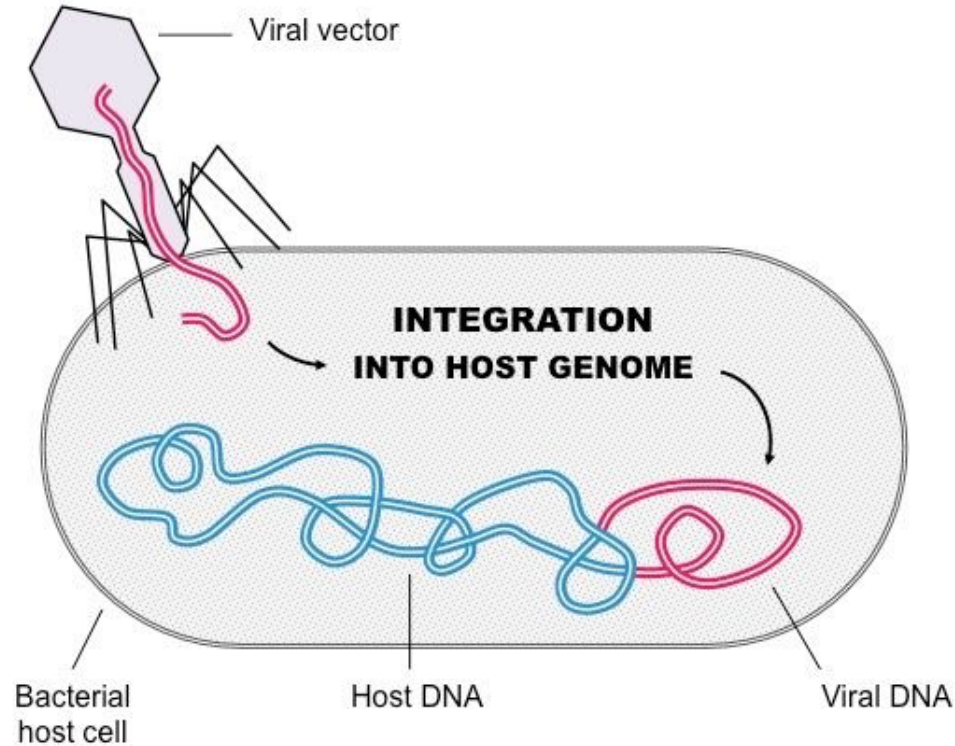
Gene for protein A



Vector

Membrane
and nucleus
of defective
cell

How are genes delivered?

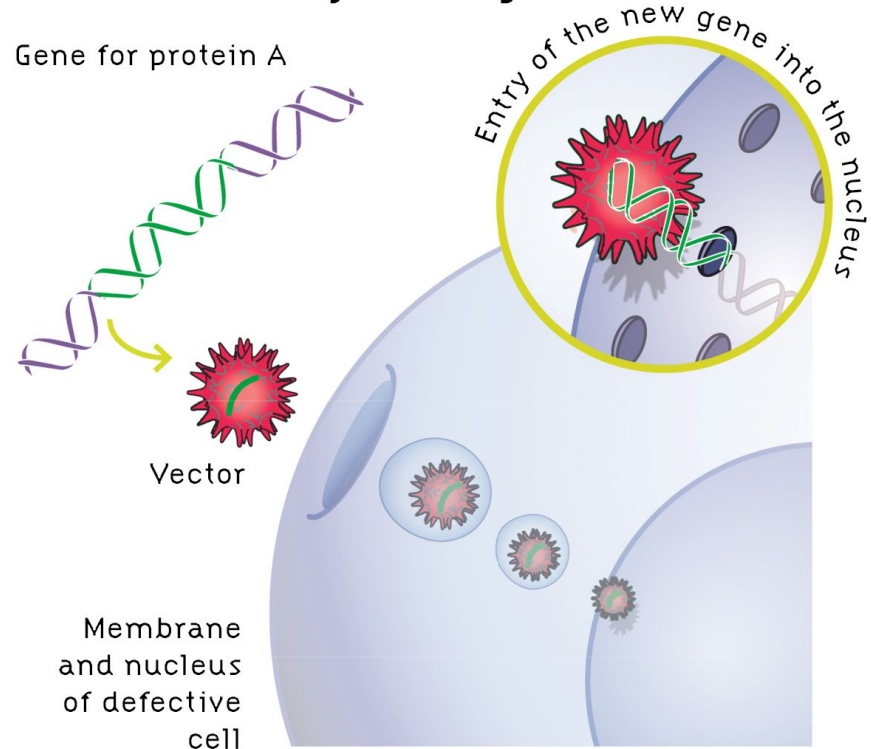


What **vector** should be used to deliver the gene?

- 1) Does it enter the right cells?
- 2) Does it integrate the new gene(s) into the patient's genome?
- 3) Does it switch the new gene(s) "on"?
- 4) Does it avoid harmful side effects?

Action of vectors at the level of the cells of the injected organ

Gene for protein A



MODEL: Gene Therapy* **

non-functioning gene functioning gene blocking gene vector *ex vivo*
in vivo pipette petri dish target cell cell membrane receptor
nuclear membrane nucleus patient's genome protein mitosis

*Model gene therapy for one specific disease of your choice.

**Use labels, arrows, and numbered steps!