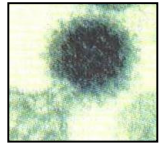
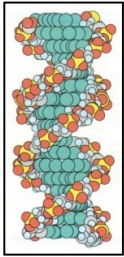
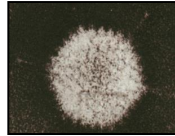


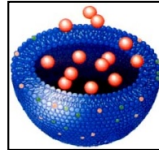
# GENE THERAPY



Lentiviral vectors



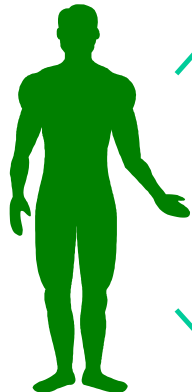
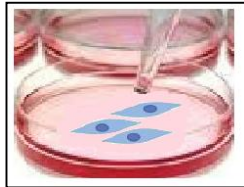
Adeno-associated viral vectors



Non-viral vectors

Adult stem cells  
Fetal stem cells  
Embryonic stem cells  
Induced pluripotent stem cells

# CELL THERAPY

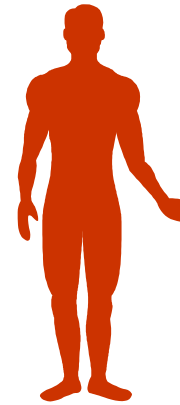


Healthy

Transfected or differentiated therapeutic cells



Transplantation  
EFFICACY  
SECURITY



Patient

## HEMOPHILIA

- Rare disease
- Monogenic disease
- Mild-moderate phenotype [biological activity of coagulation factors: 1-40%]
- Non-regulated gene expression
- Wide variety of experimental animal models

Factor VIII / IX expression  
Bleeding correction