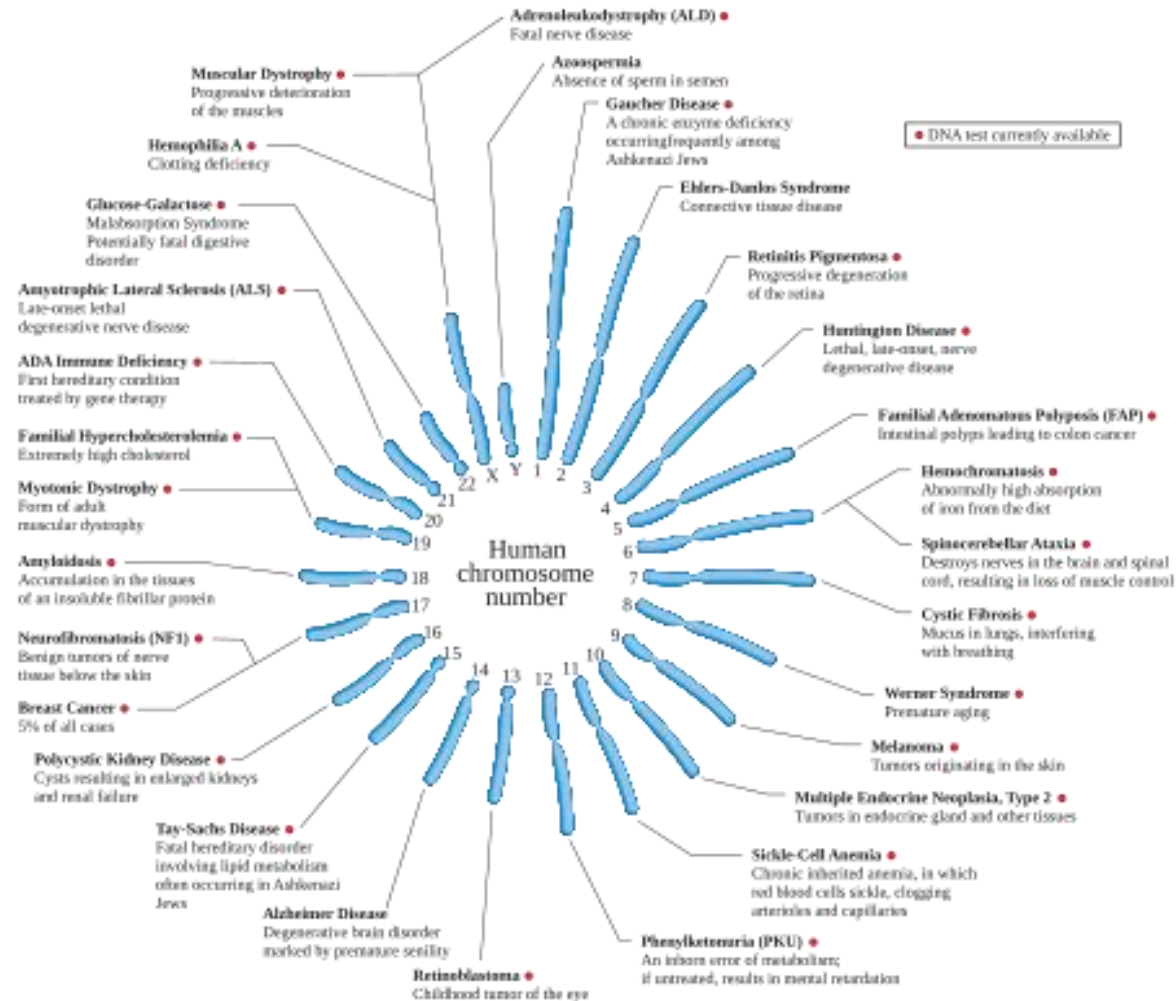




Methods in Drug Development

CRISPR/Cas9

Genetic diseases & gene therapy



DELETE

a mutated or “misspelled” gene to halt the production of a disease-causing protein



INSERT

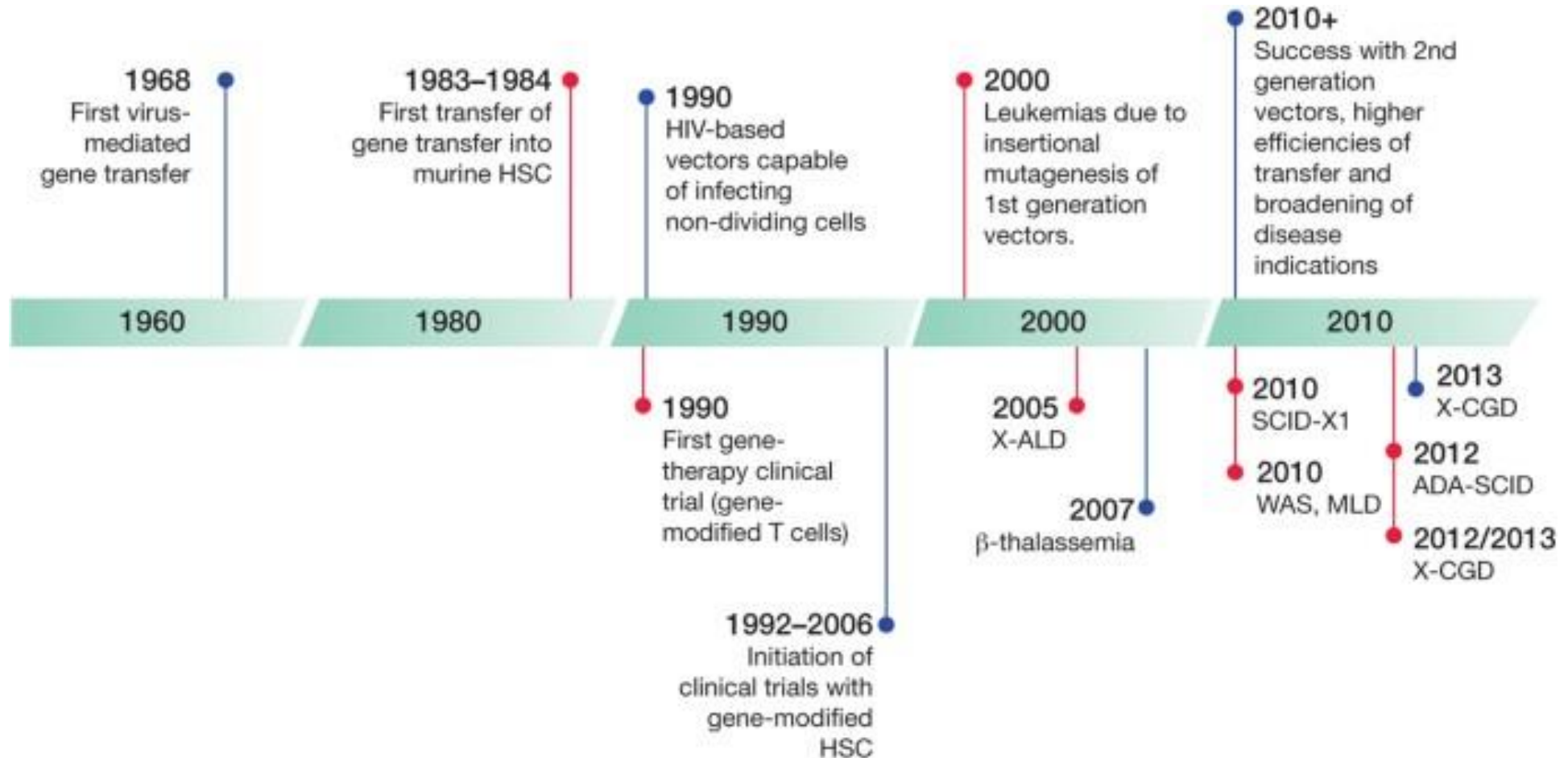
a missing gene to produce a needed protein



CORRECT

a mutated or “misspelled” gene to repair a dysfunctional protein

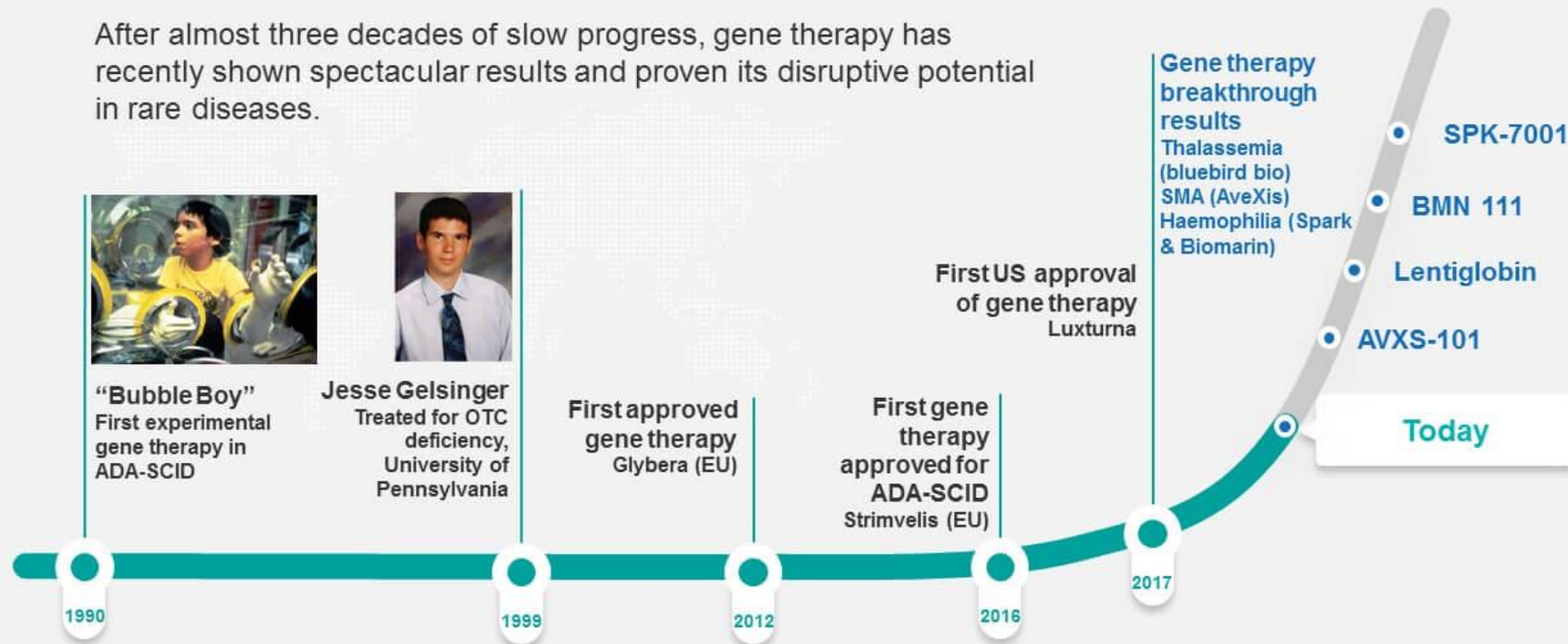
Long history of gene therapy



Gene therapy setbacks and new hope

The Medical Promise Of Gene Therapy Has Become A Reality

After almost three decades of slow progress, gene therapy has recently shown spectacular results and proven its disruptive potential in rare diseases.



Note: steep curve may show a too optimistic path

New opportunities with CRISPR/Cas9

