Methods in Drug Development

CRISPR/Cas9

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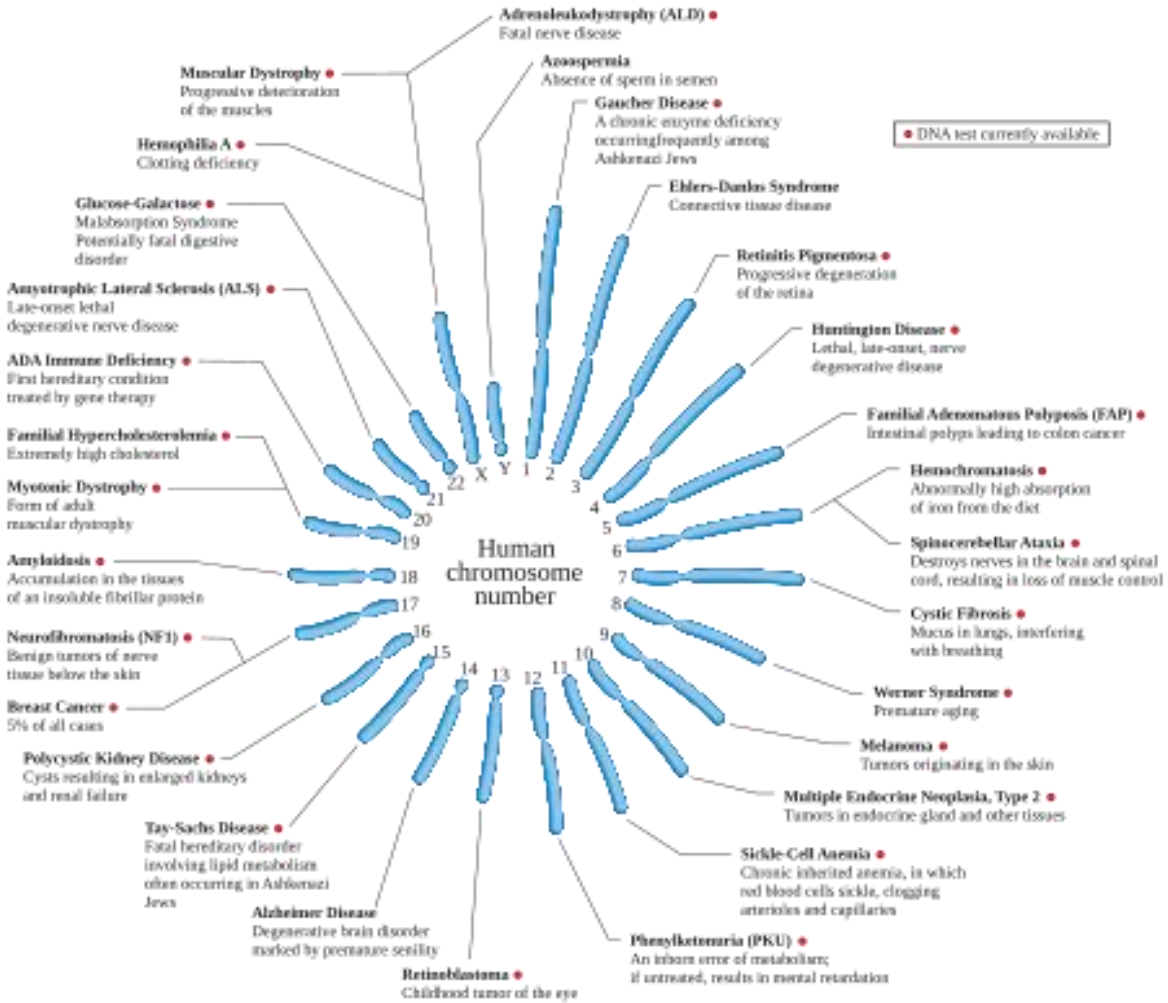
Genetic diseases & gene therapy

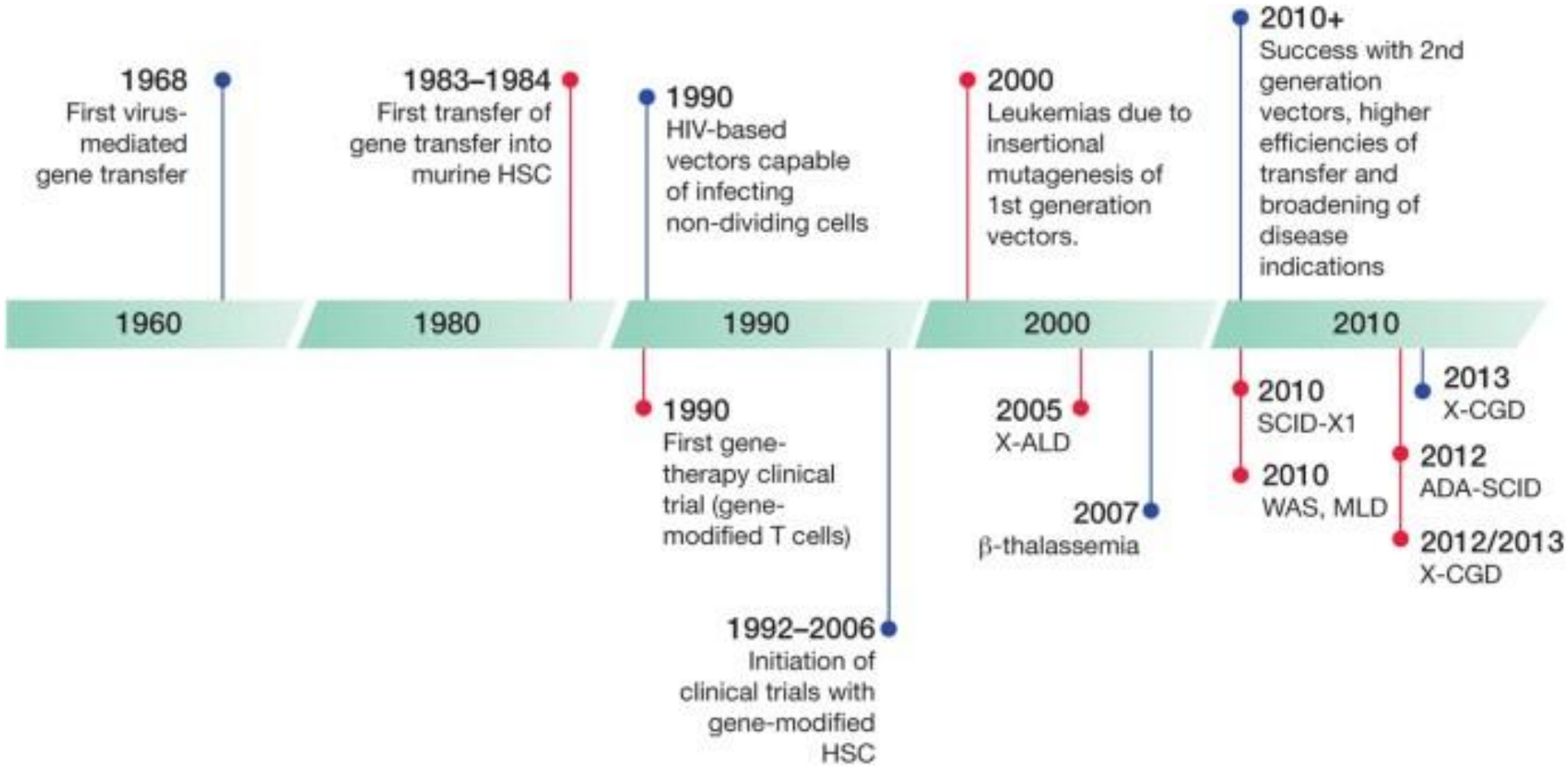


DELETE
a mutated or “misspelled” gene to halt the production of a disease-causing protein

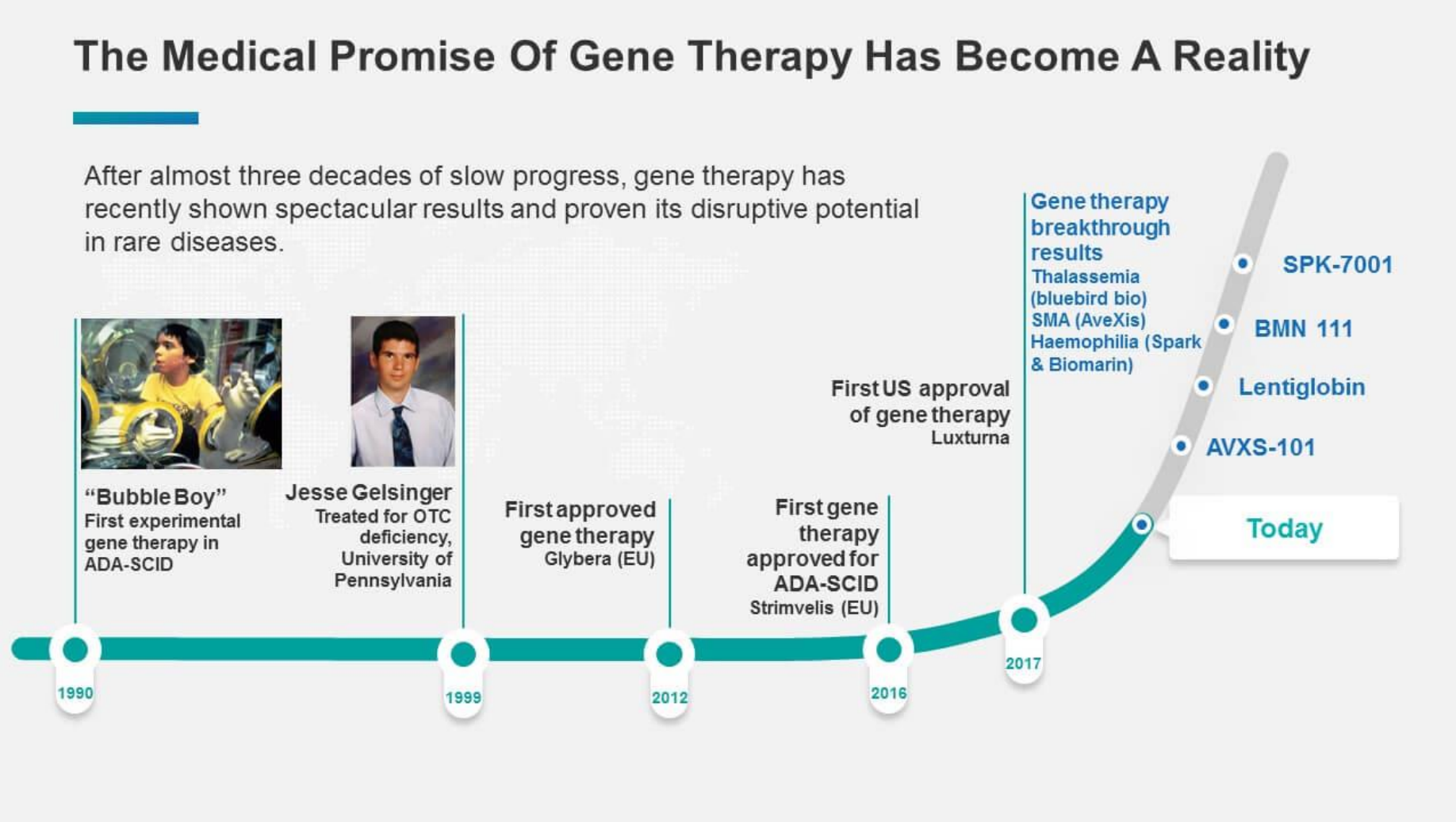
INSERT
a missing gene to produce a needed protein

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

Long history of gene therapy



Gene therapy setbacks and new hope



Note: steep curve may show a too optimistic path

New opportunities with CRISPR/Cas9