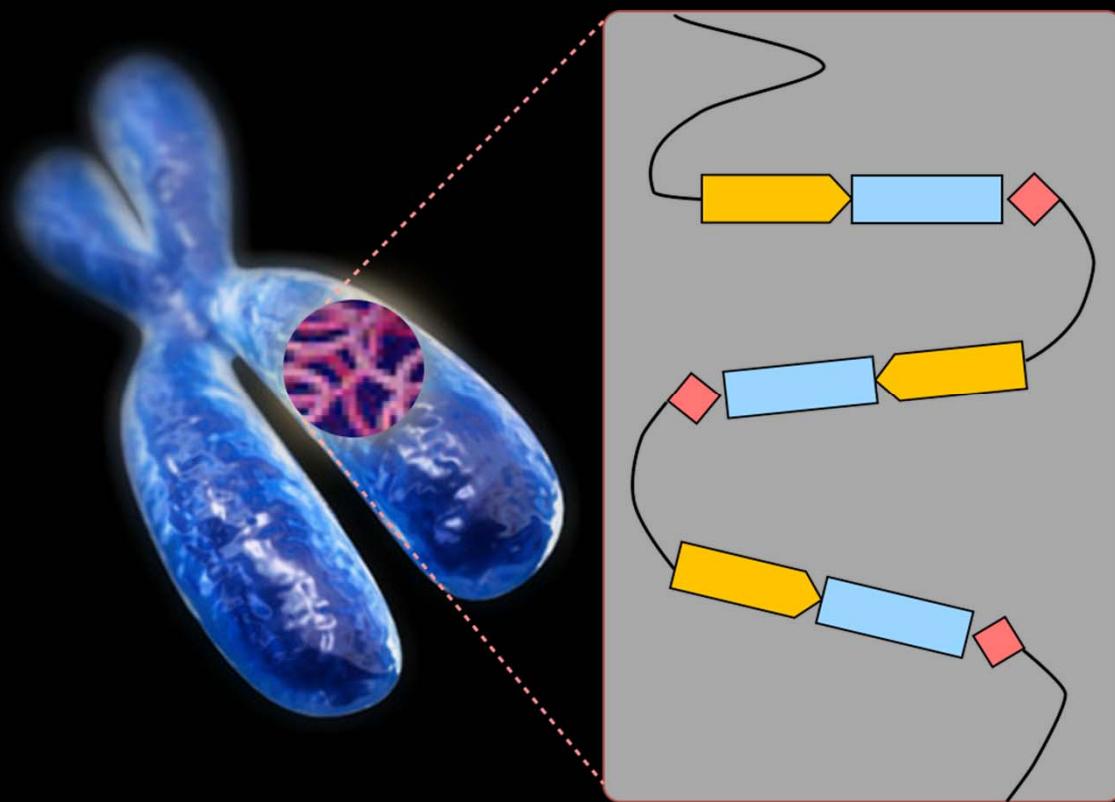


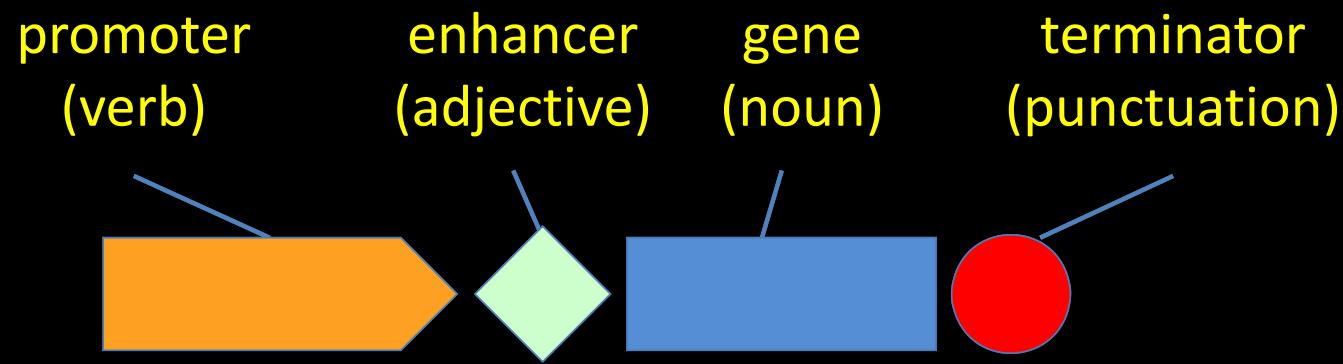


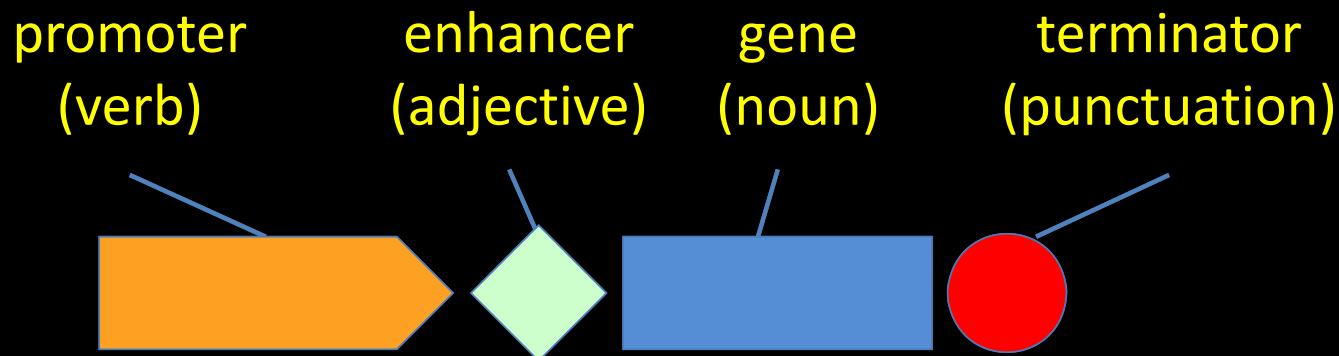




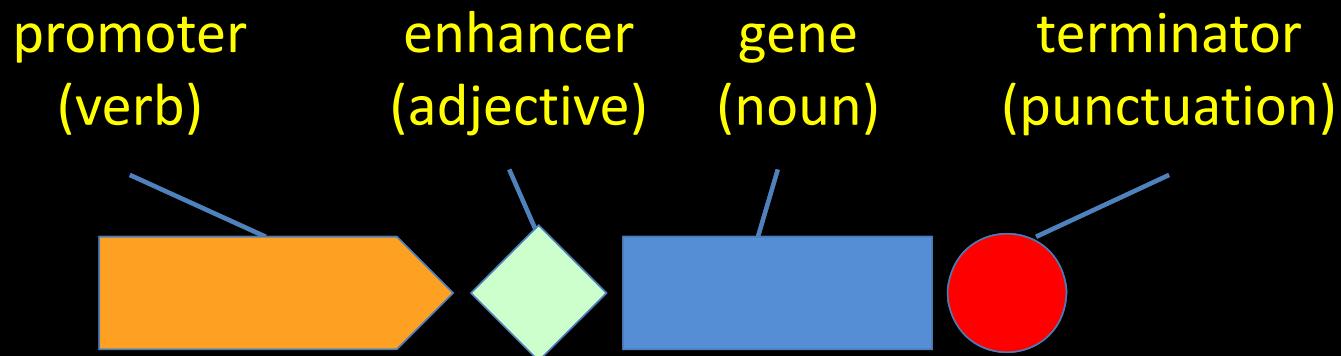
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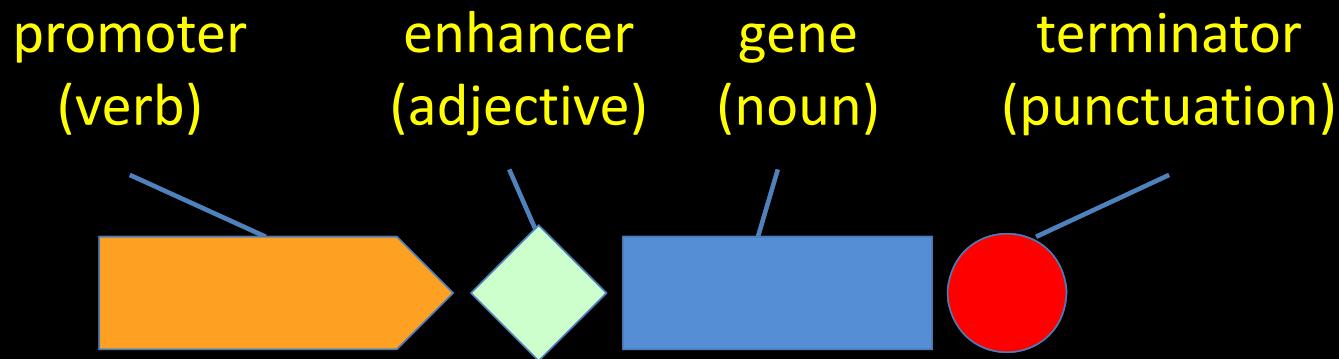




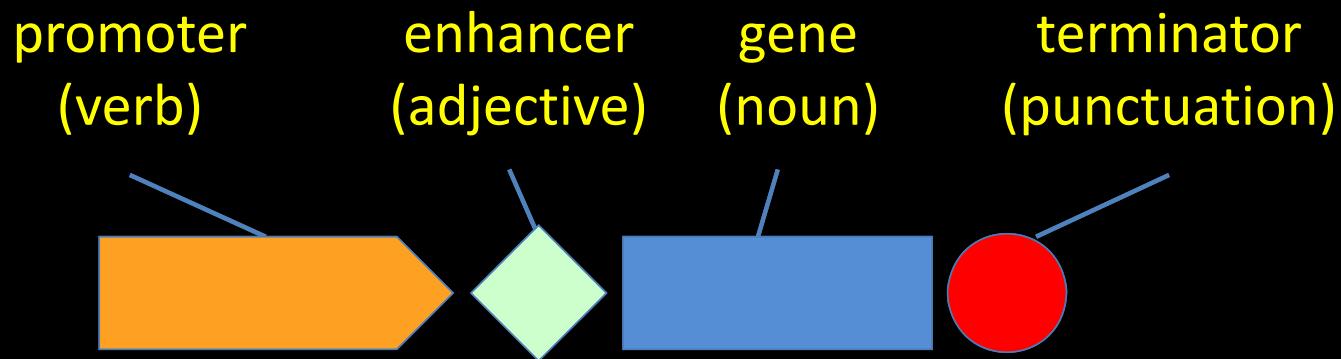
Twinkle twinkle BIG star.



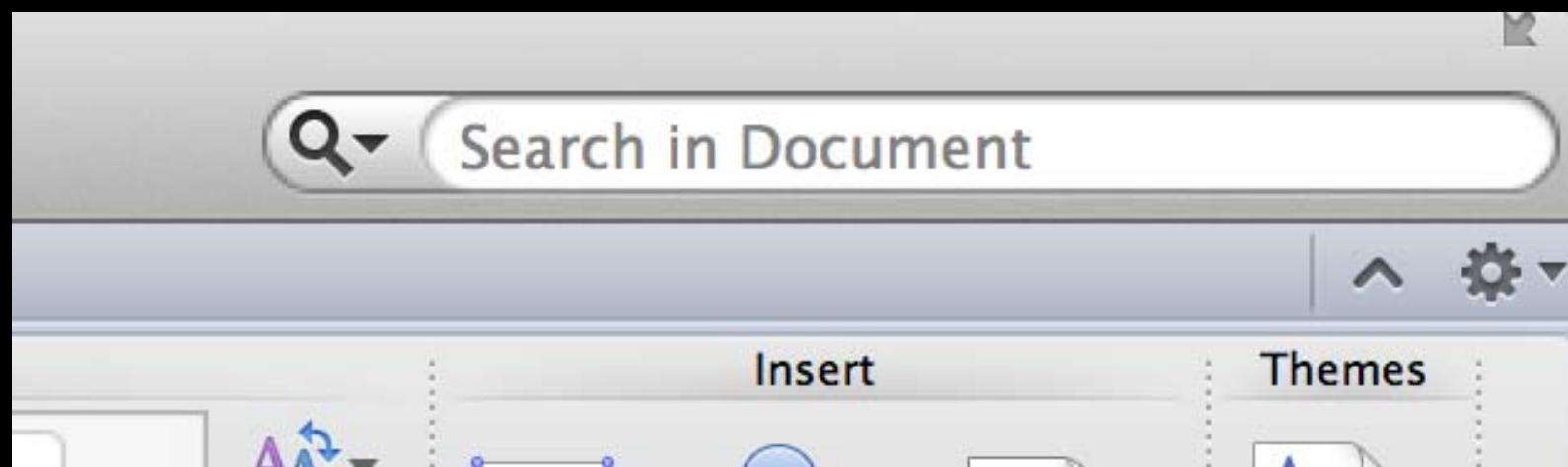
Twinkle twinkle little BIG star.



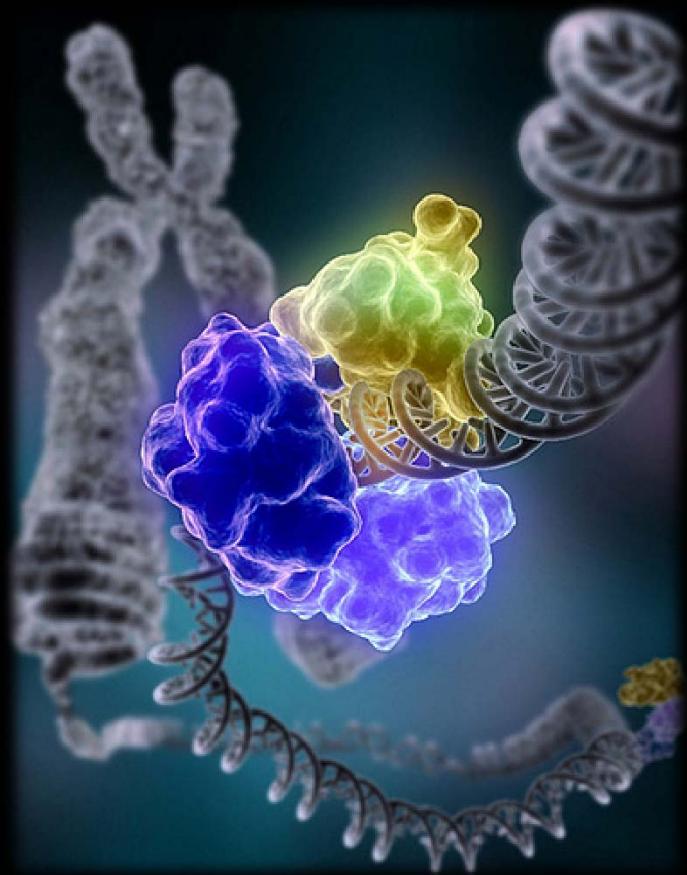
Twinkle twinkle star.



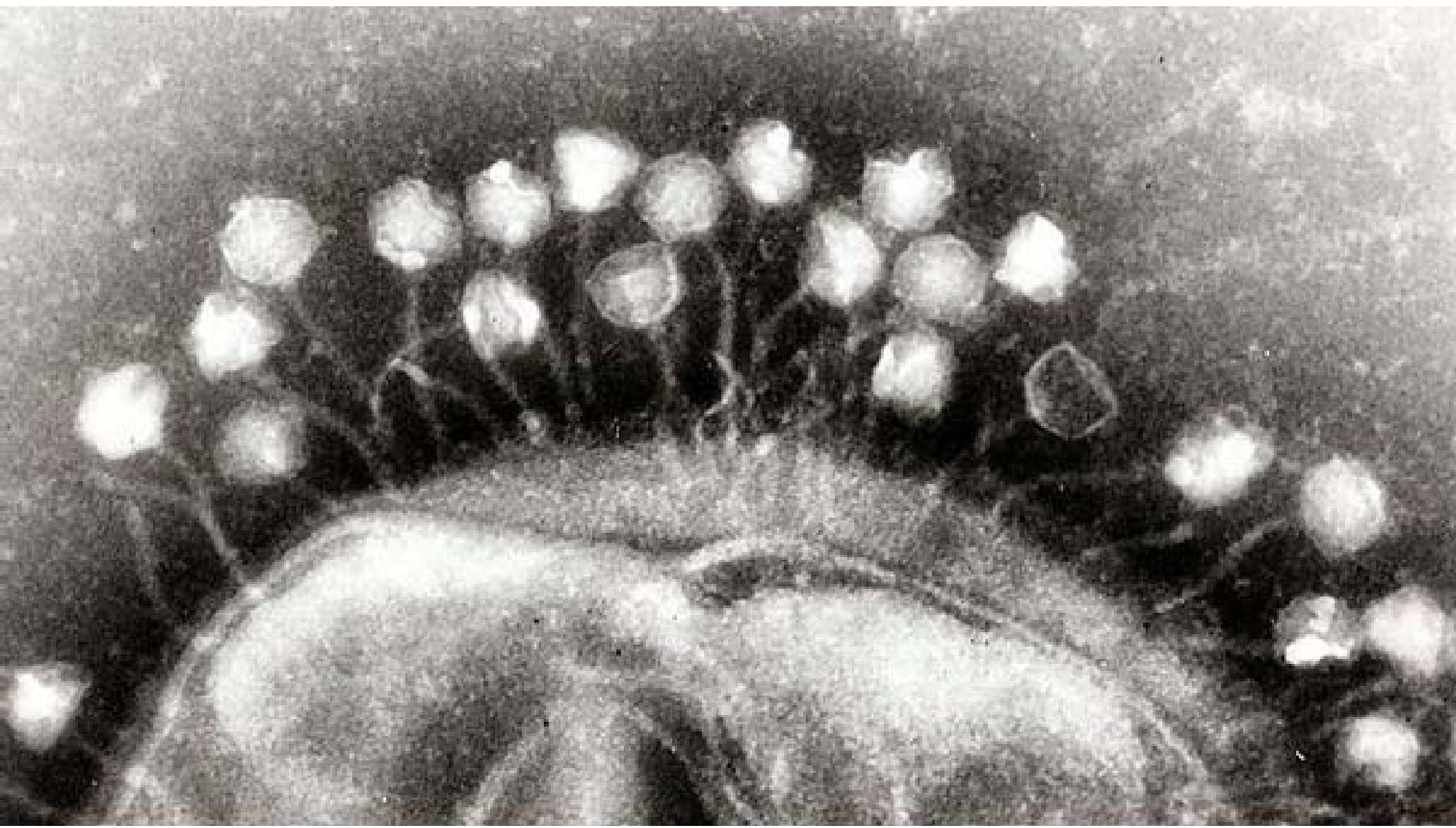
Twinkle twinkle little star.

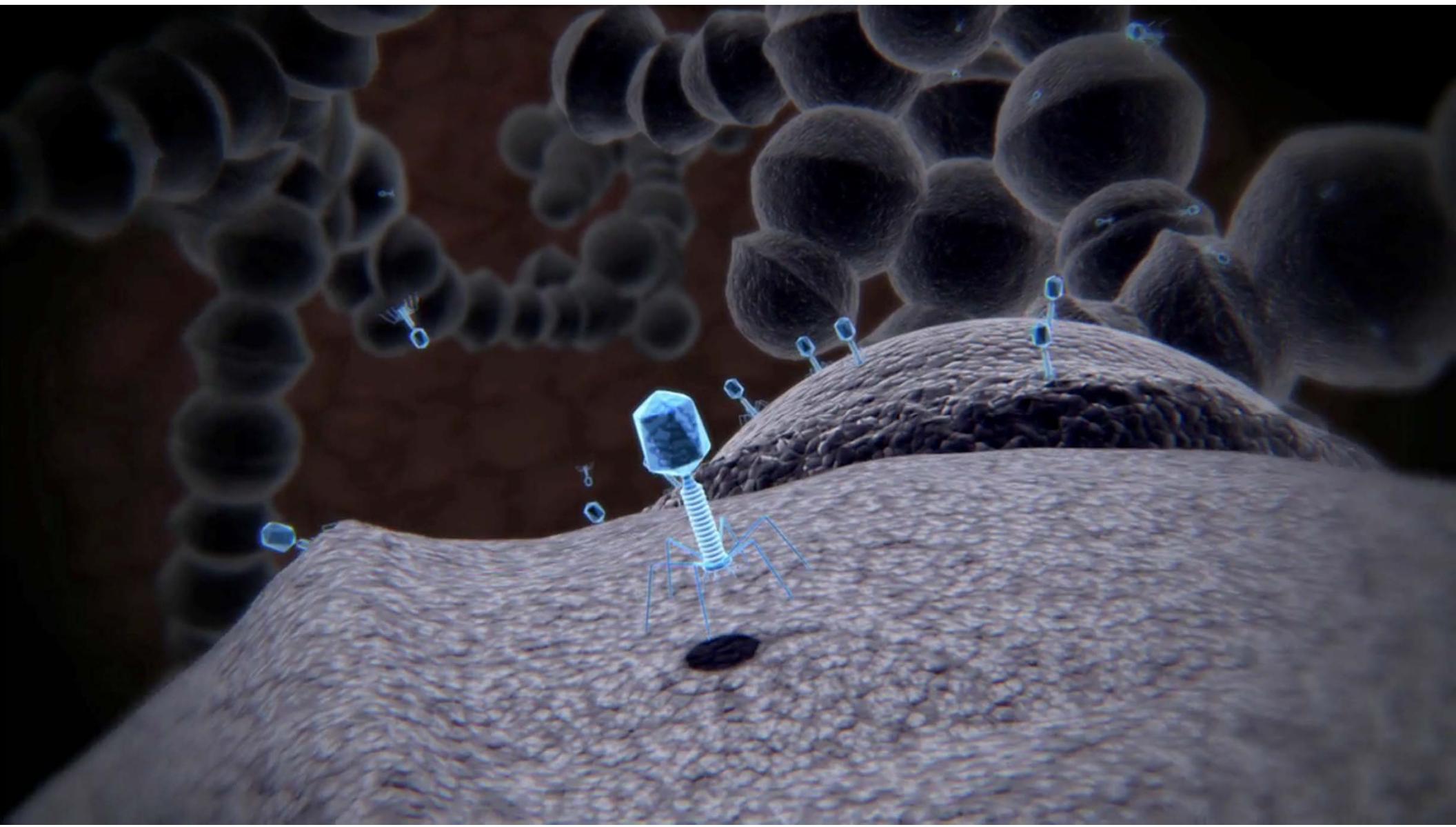


Need to be able to
find a specific spot
on the genome.

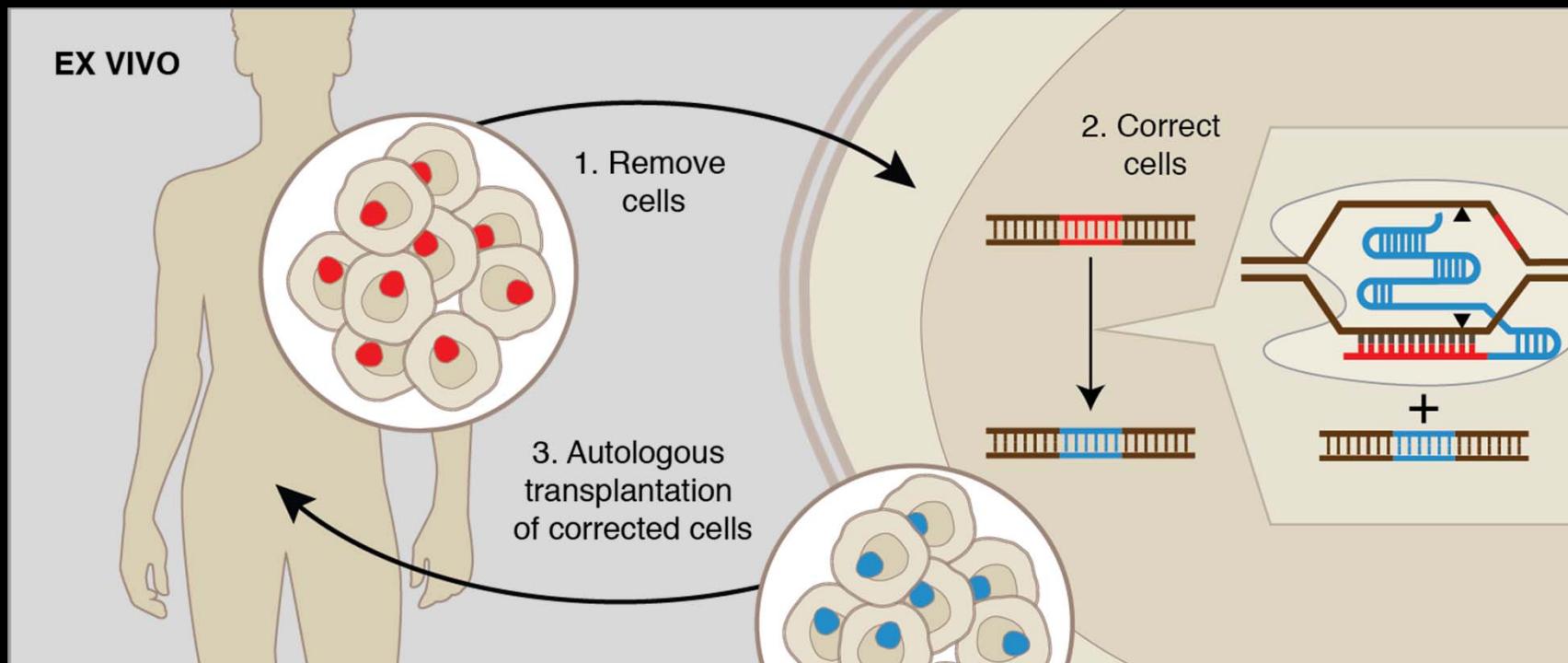


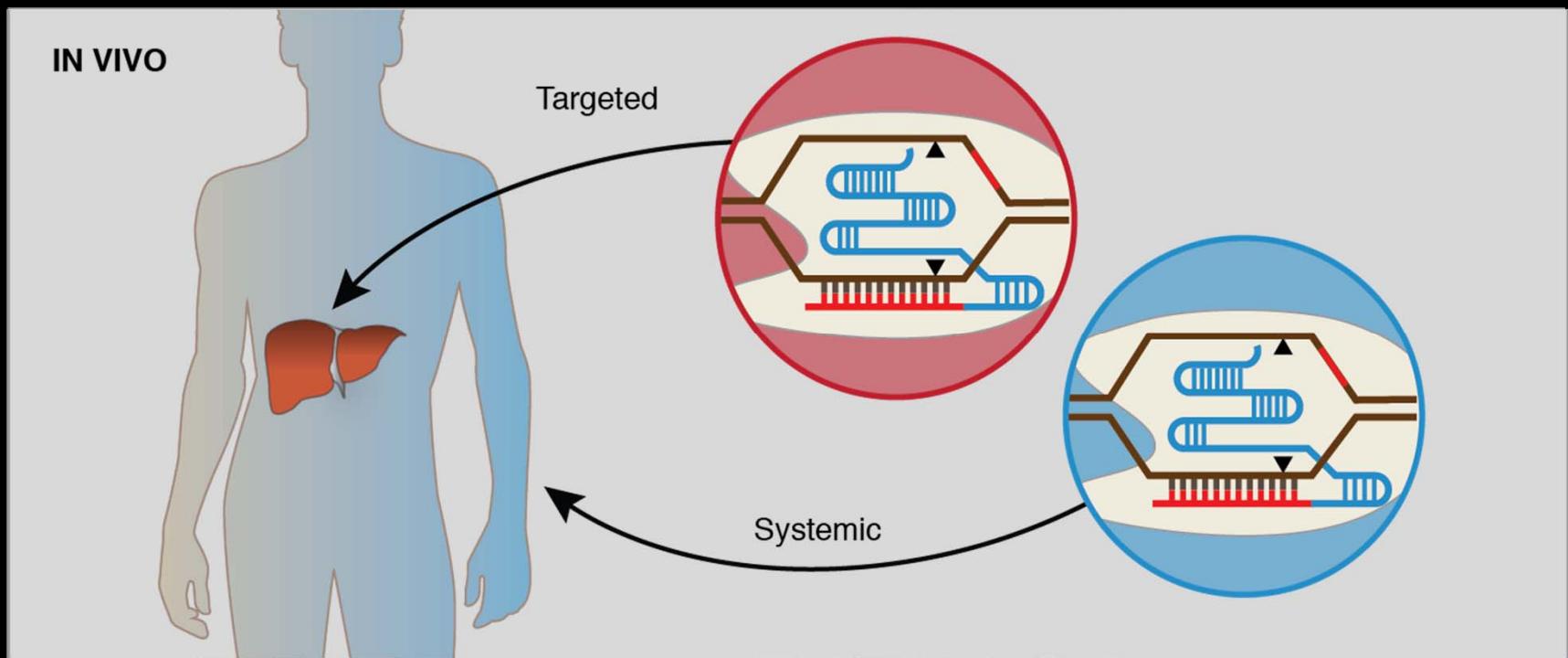


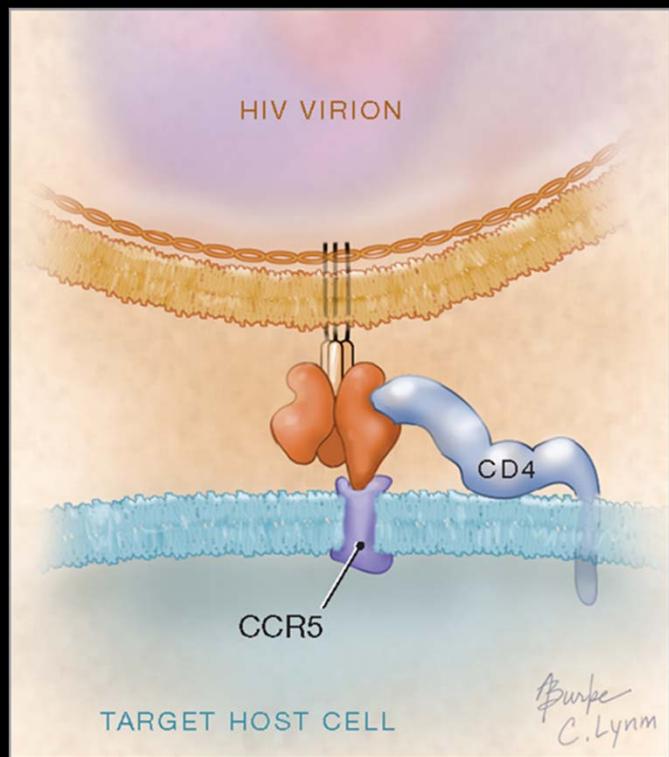












Mucopolysaccharidoses

Lysosomal acid lipase deficiency

myotonic dystrophy

Cystic Fibrosis
Hemophilia

muscular dystrophy Galactosemia

Phenylketonuria

Retinitis Pigmentosa
Usher Syndrome

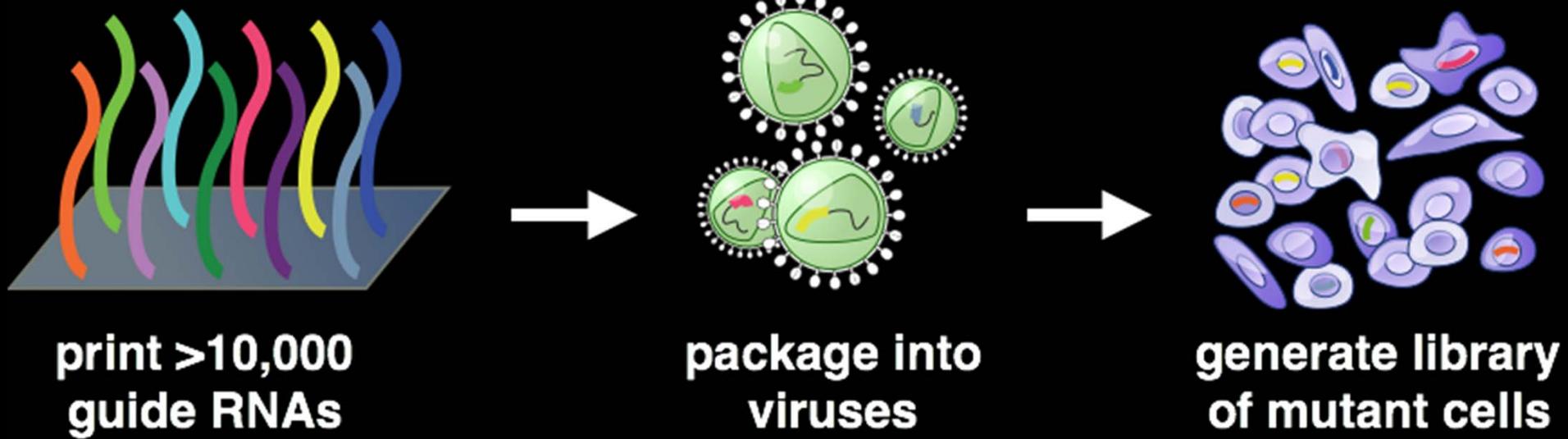
Familial Hypercholesterolemia

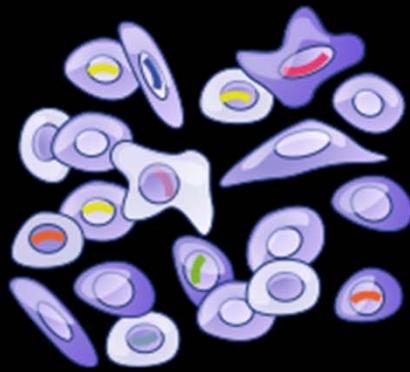
Polycystic kidney disease

Rett's Syndrome Huntington's Disease

ALS Sickle Cell Disease

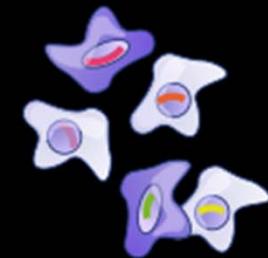
Tay-Sachs disease Lafora's Disease
Neurofibromatosis type I





**start with a
comprehensive
library of cells with
distinct
perturbations**

**add virus of interest
(e.g. HBV, ebola, etc)**



**analyze surviving
cells to identify
gene targets**

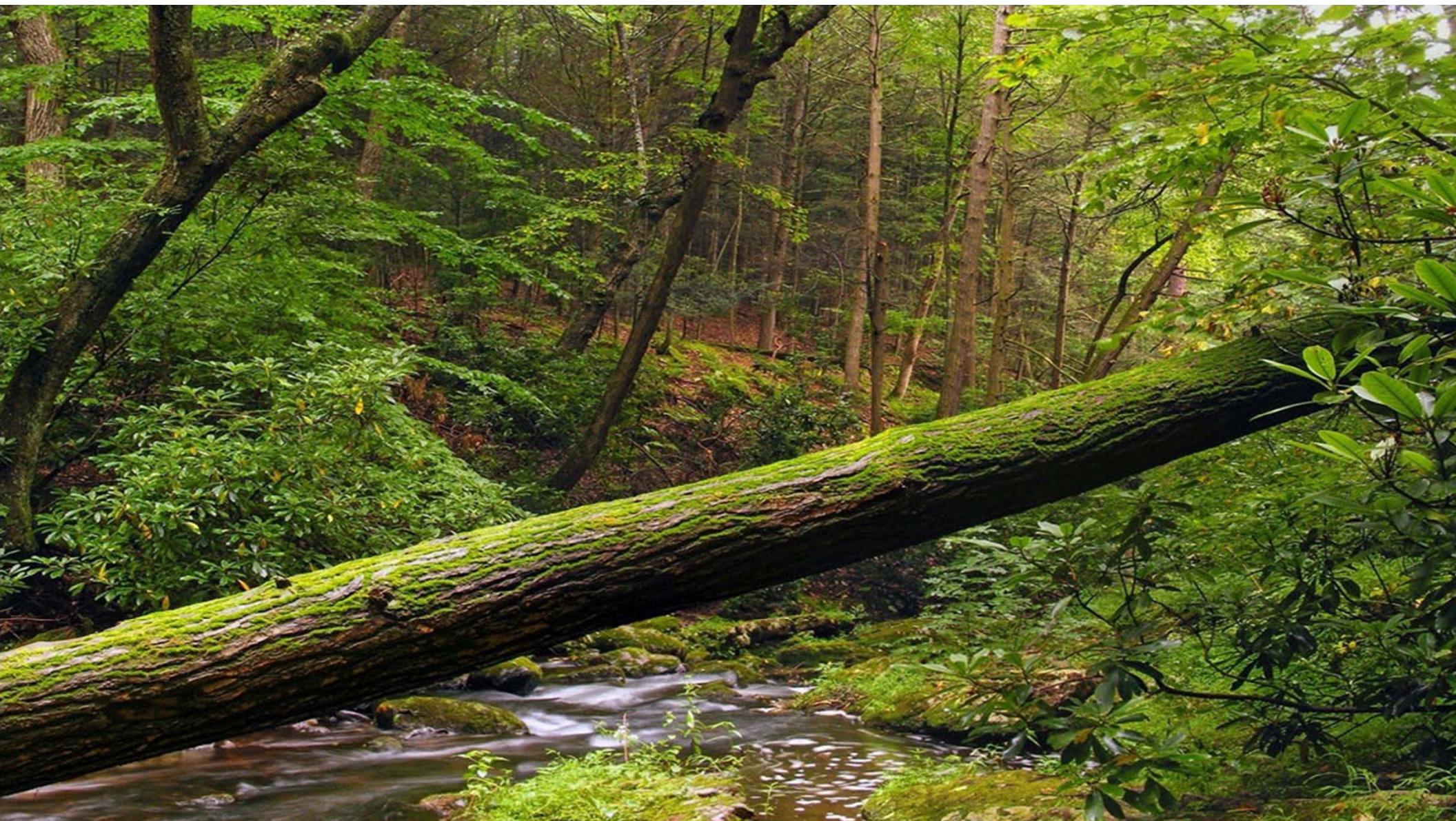


**start with a
comprehensive
library of seeds
with distinct
perturbations**

**plant under desired
condition**



**analyze surviving
species to identify
gene targets**

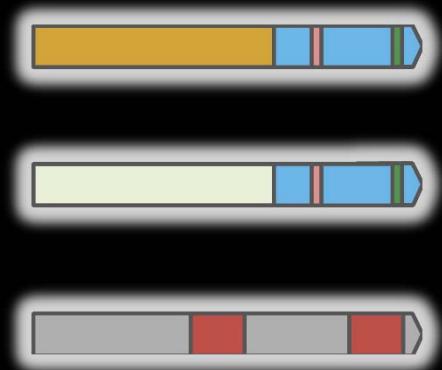




Computational Pipeline
Machine Learning



Novel Enzymes



Collaborators

Charlie Gersbach

Rudolf Jaenisch
Haoyi Wang

Eugene Koonin
Kira Makarova

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Hiroshi Nishimasu

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Support: NIMH, NINDS, NSF, Keck, Allen, McKnight,
Klingenstein, Merkin, Vallee, and Damon Runyon
Foundations; Bob Metcalfe, and Jane Pauley

