



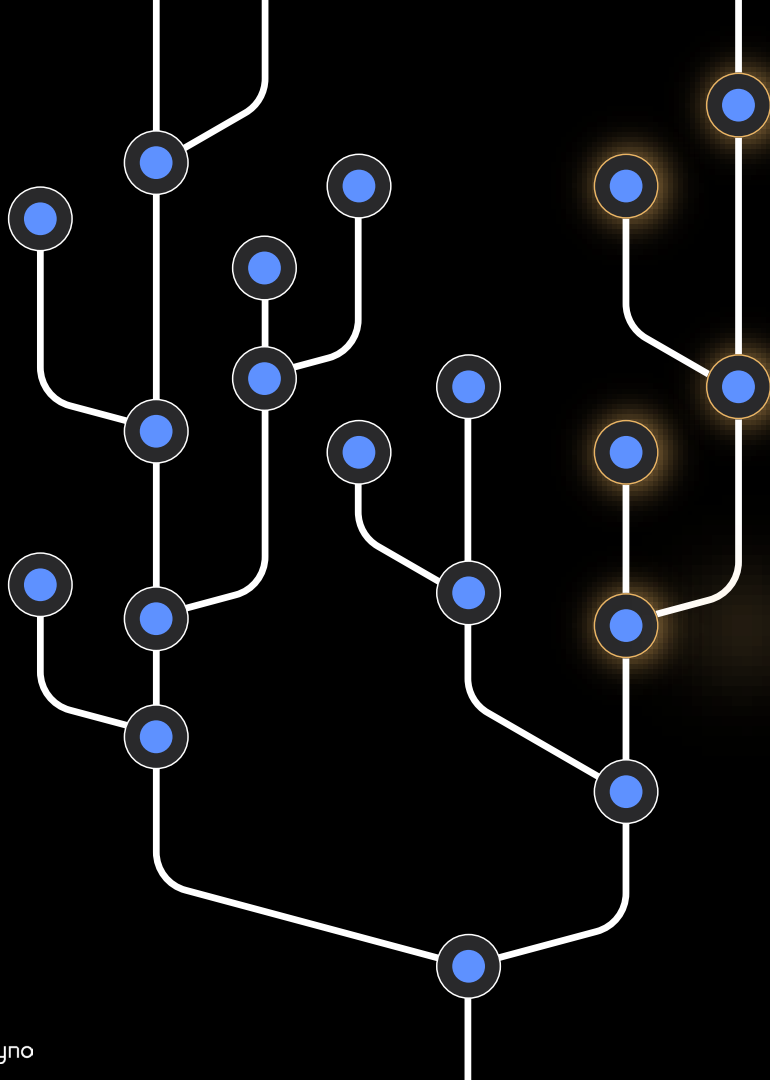
# AI powers the coming age of genetic agency

Sam Sinai  
Co-founder, Head of ML

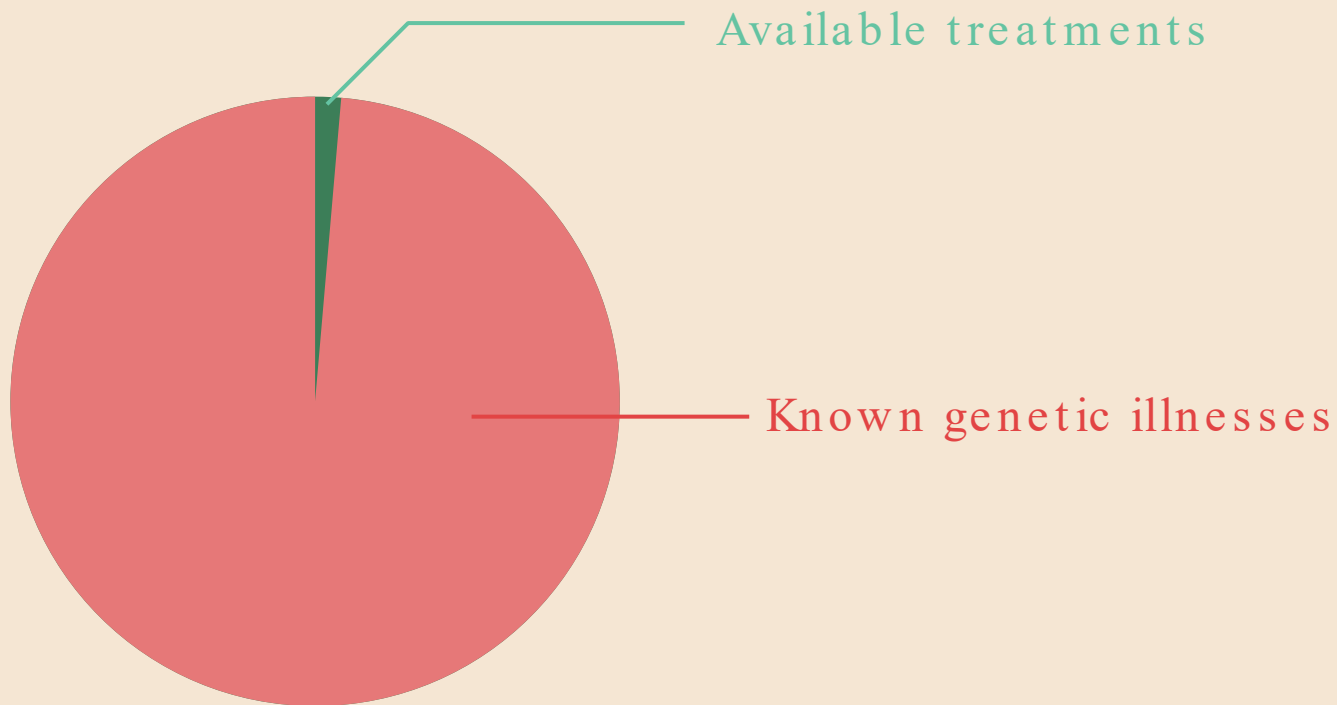
11.18.2025

# Genetic Agency

An individual's ability  
take action at the  
genetic level to live a  
healthier life

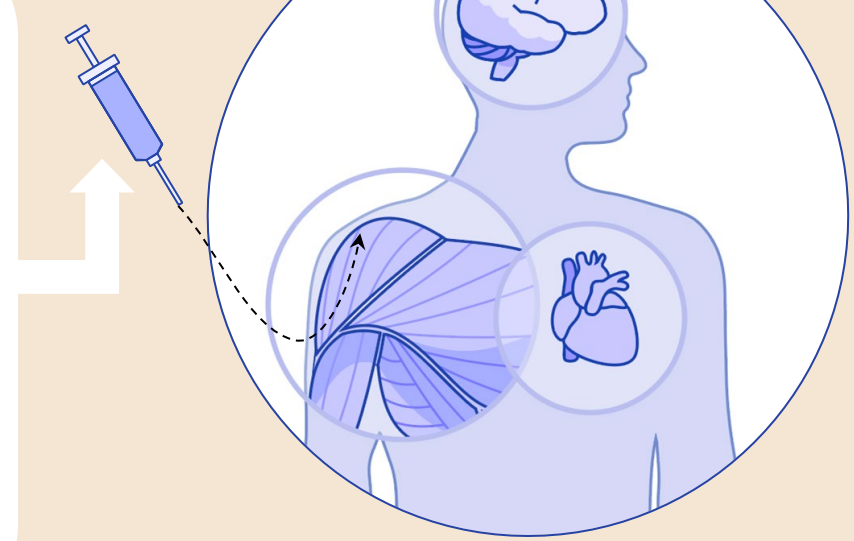
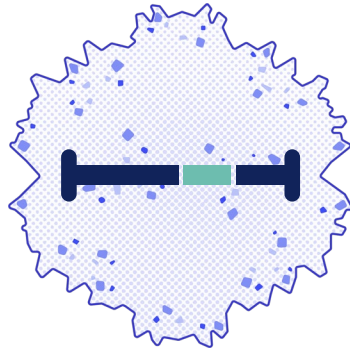


# Why so few treatments for genetic illnesses?



# Gene therapy is a promising but developing technology

Elevidys for DMD

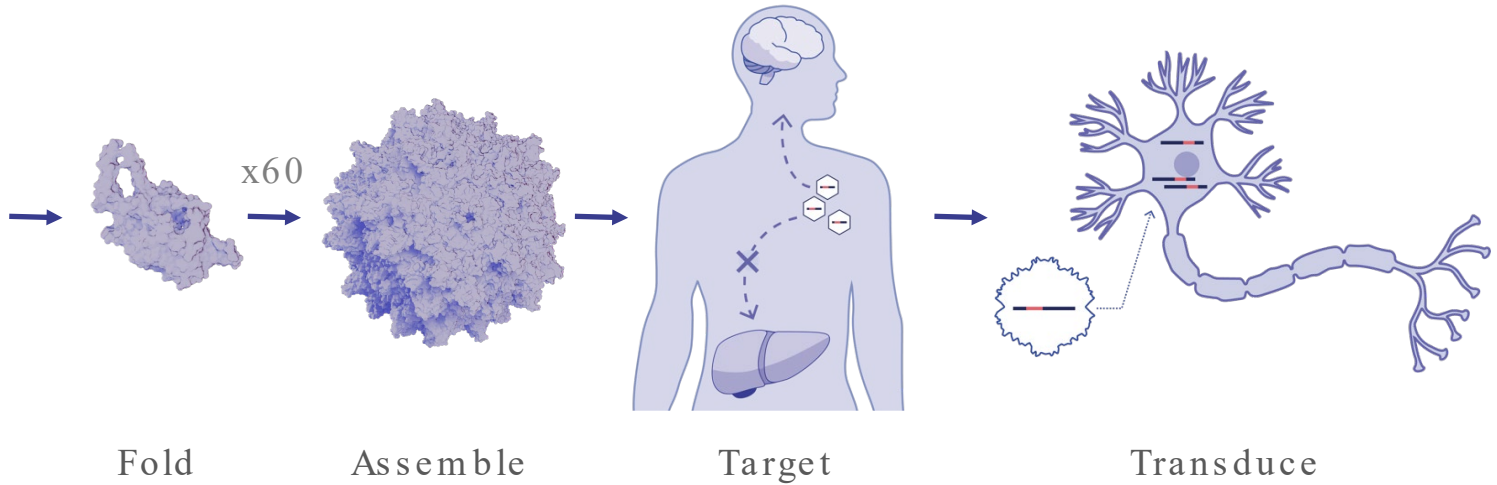


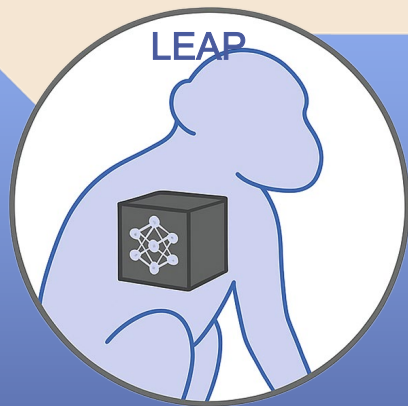
At Dyno, we are  
100% focused on building  
technologies to enable  
genetic agency



# Case study: AAV transduction requires completion of multiple complex steps

~735 AA  
MAADGYLPDWLE  
DNLSEGIREWWA  
LKPGAPQPKANQ  
QHQDNARGLVLP  
GYKYLGPNGLD  
KGEF...





Black-box models  
of billions of in vivo  
capsid  
measurements



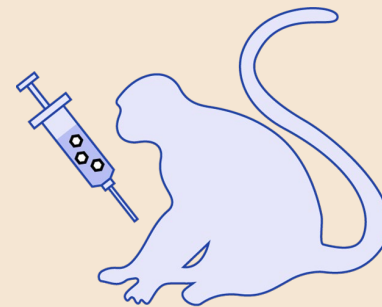
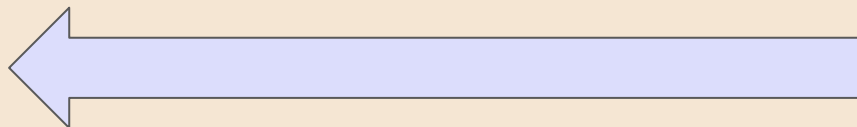
Mechanistic-driven  
design with  
generative models  
for capsids and  
payloads



AI agents as co-  
scientists that can  
make use of  
Dyno's tools

# Learning sequence models from in vivo data

MAADGYLPDWLED  
NLSEGIREWWALK  
PGAPQPKANQQHQ  
DNARGLVLPGYKY  
LGPGNGLDKGEP...



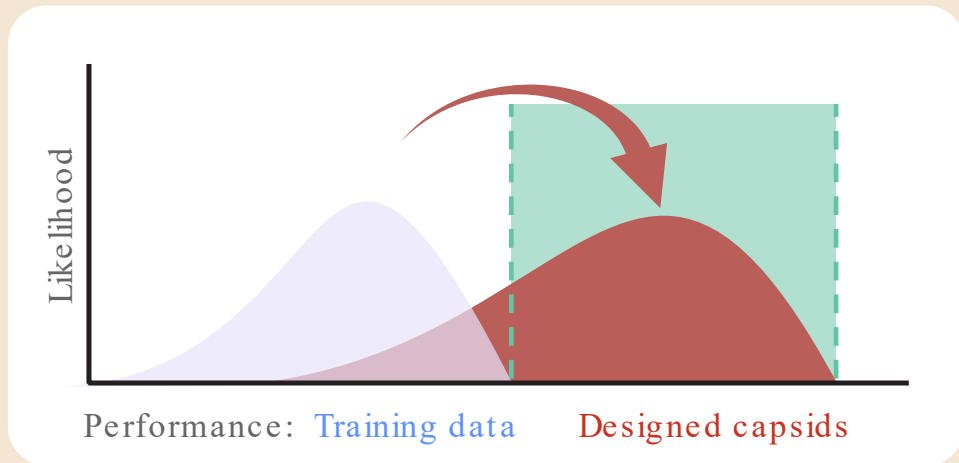




LEAP

# High performance in vivo blackbox design

LEAP's high fidelity enables us to replace an in vivo experiment with compute, saving a year of experiments and >\$1M in expenses.





LEAP

# High performance in vivo blackbox design

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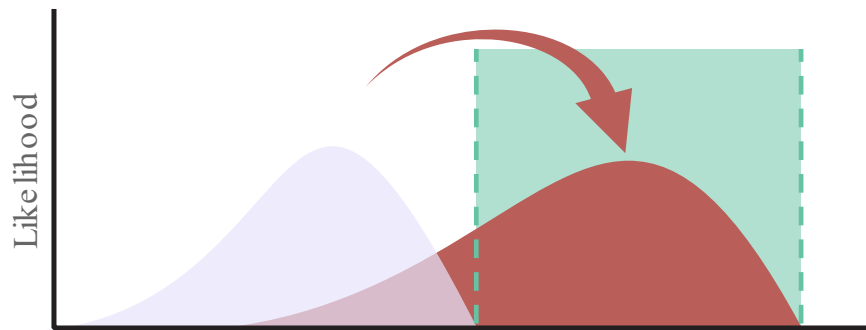
## Head-to-head comparison

- 11 top variants from training data
- 12 new capsids, rationally designed
- 19 new capsids, designed using LEAP



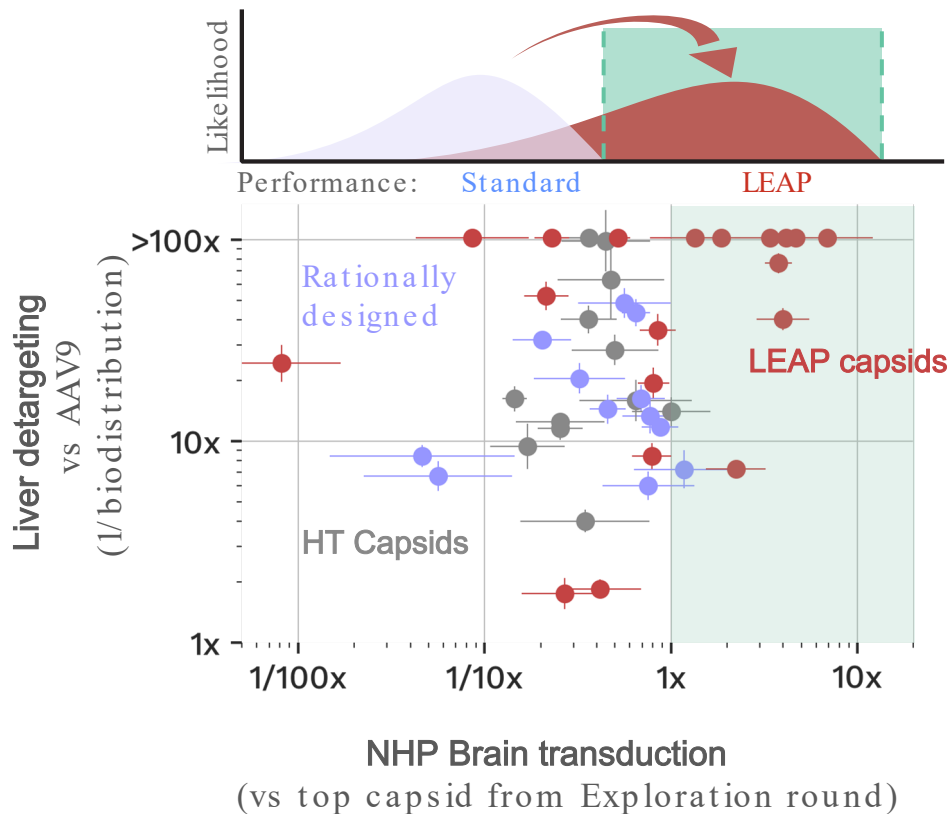
## Pooled validation design

- IV co-injection
- 2 adult Cyno NHPs
- 28 day in-life period
- $1.5 \times 10^{13}$  vg/kg dose for total pool



Performance: Training data    Designed capsids

# With LEAP we replaced in vivo experiments with ML



90%

17 **packaged**  
effectively

47%

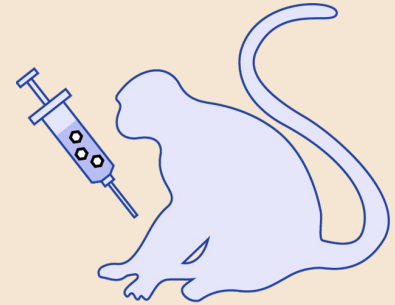
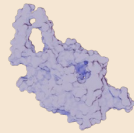
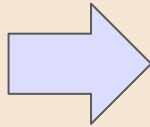
9 performed  
**better than anything we**  
had observed before

6x

Top capsid  
performed up to  
**6x better** than the best  
known capsid before

# Connecting sequence to mechanism

MAADGYLPDWLED  
NLSEGIREWWALK  
PGAPQPKANQQHQ  
DNARGLVLPGYKY  
LGPGNGLDKGEP...



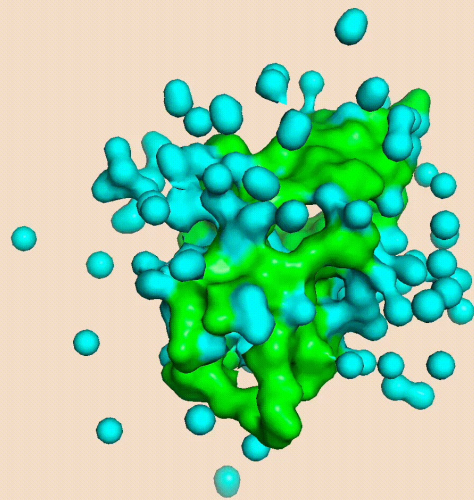


Dyno Psi-1

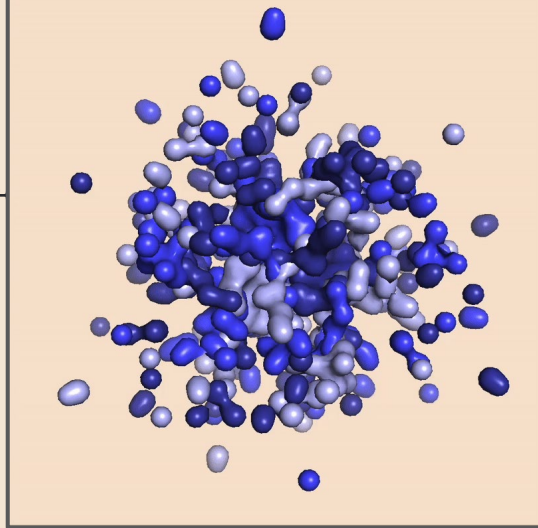
# Controllable design of every component of a gene therapy

Dyno Psi-1 is a structure-based foundation model capable of designing delivery or payload proteins with high efficiency, scale and fidelity.

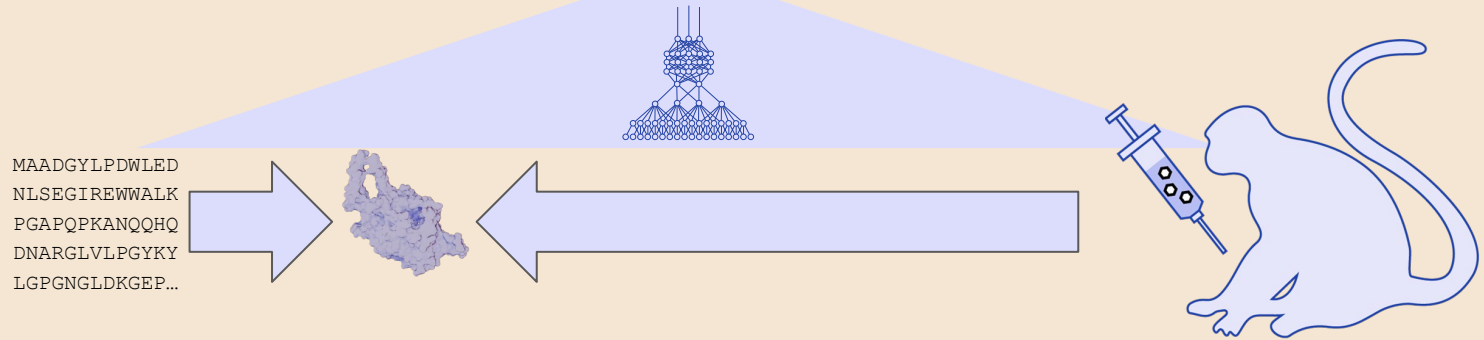
Multi-target binders



Protein complexes



# AI-assisted hypothesis exploration





Link to view >

p0

# Reducing cost and time from idea to therapeutic design

Agents integrate information and tooling for informed and efficient decision making, reducing the expertise, risk, and time needed to generate effective therapies.

User

Help me build a mechanistically informed gene therapy for sickle cell disease

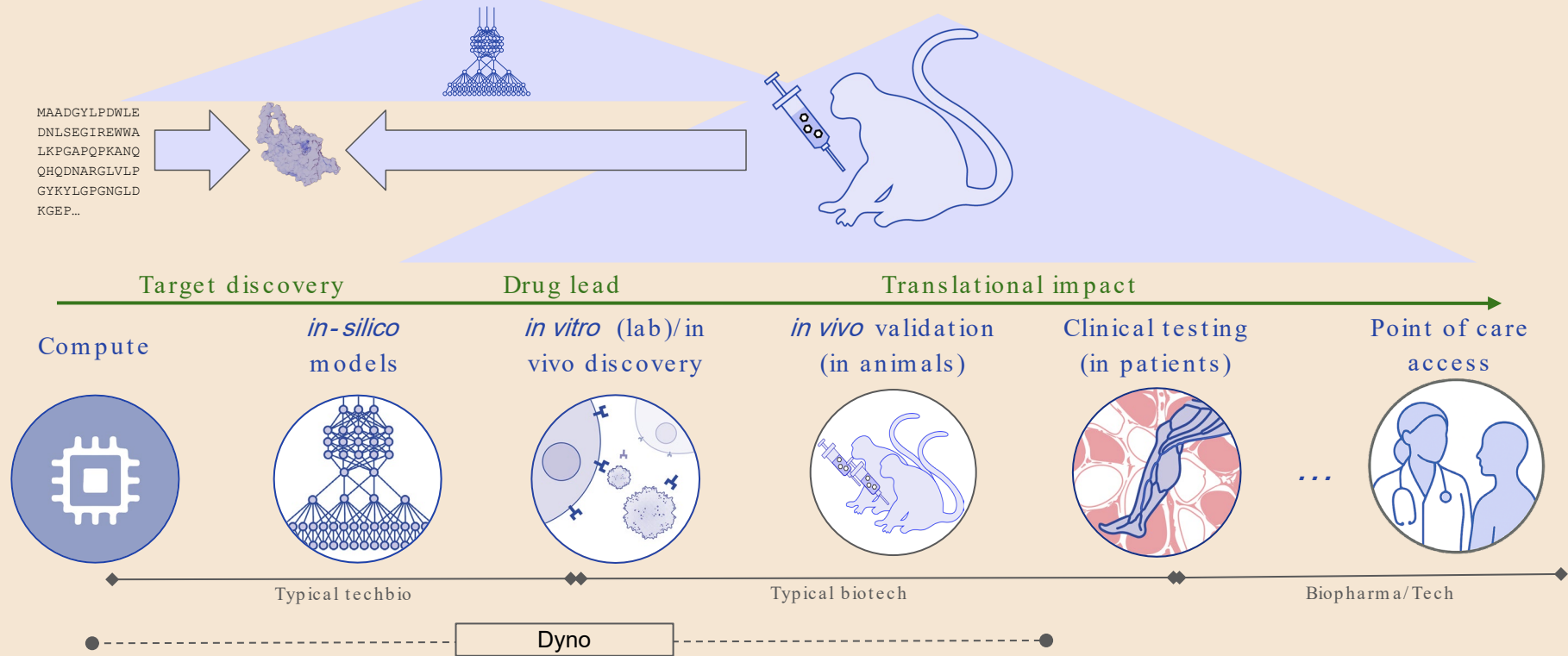
Agent

Thinking...

9EGR 6

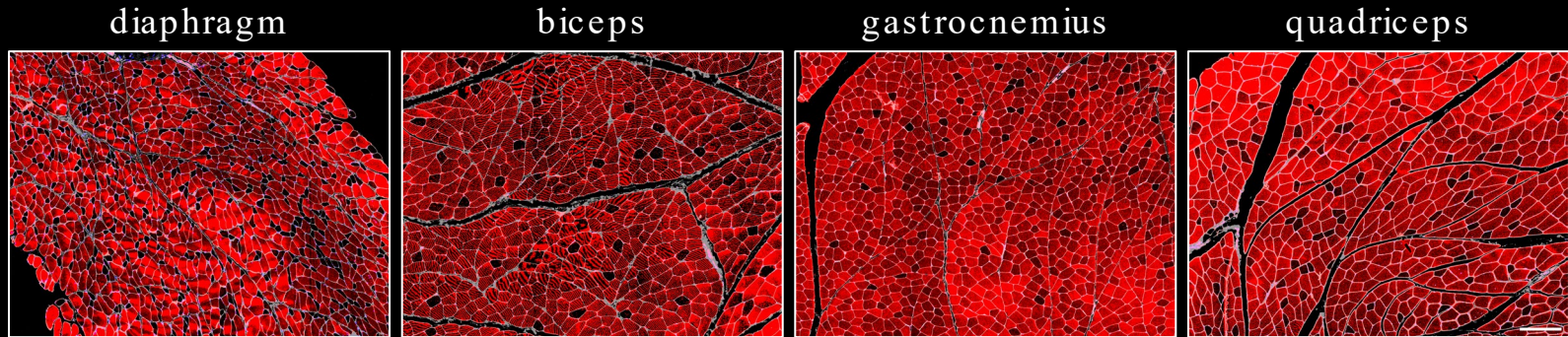
File Zoom Rotate Link Grid

# Connecting technology to patient impact requires deploying AI beyond model organisms



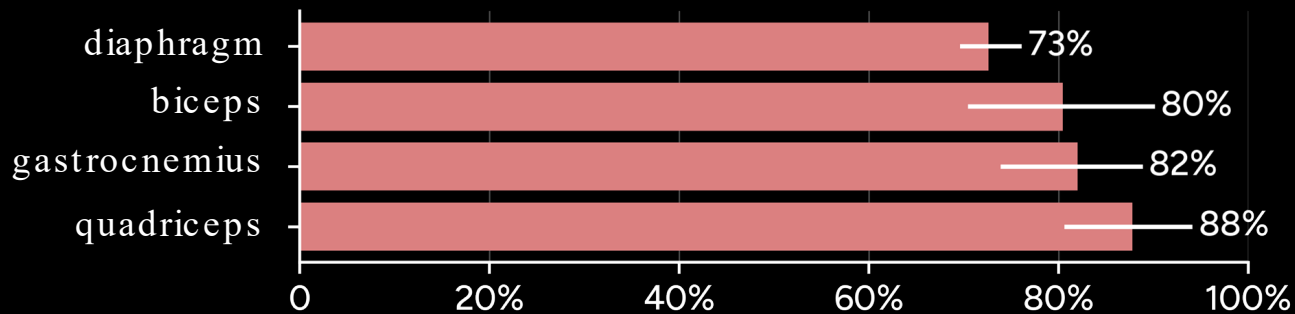


At  $5.2 \times 10^{12}$  vg/kg, **Dyno-bn8** evenly transduces the vast majority of skeletal myofibers in all tested tissues



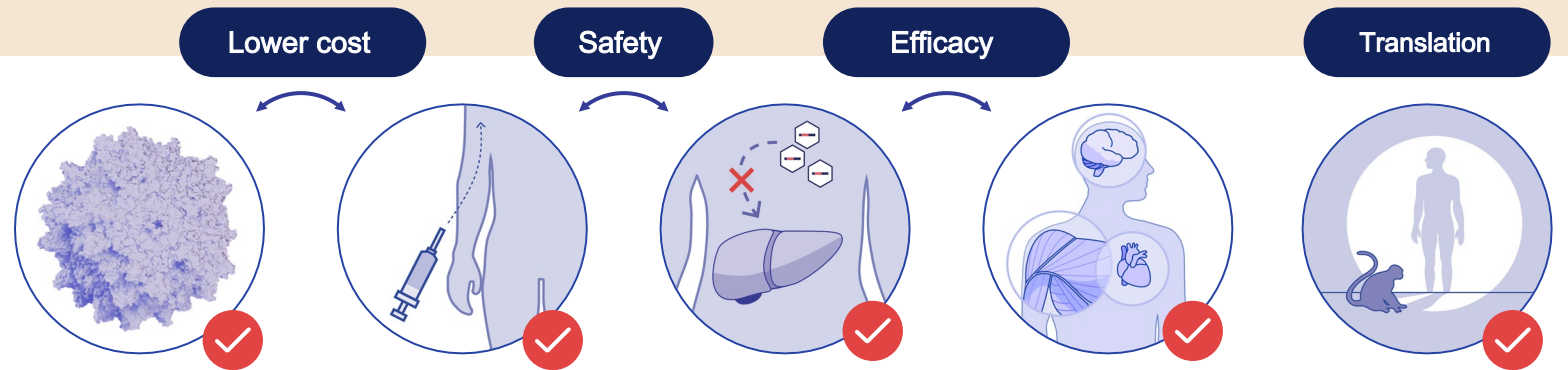
WGA, mCherry (Dyno-bn8), DAPI

Scale = 200  $\mu$ m



error bars  
show 95% CI

# Dyno-bn8 achieves therapeutic delivery to muscle at significantly lower doses\* and with improved liver detargeting



Efficient production

similar production vs AAV9

Efficient at low doses

$1.6e13$  vg/kg  
 $5.2e12$  vg/kg

Detargeted from liver

34 vg/dg liver at  $5.2e12$  vg/kg

Efficient delivery to target organs

81% avg muscle at  $5.2e12$  vg/kg  
40% avg heart at  $1.6e13$  vg/kg

Predict performance in humans

Conserved receptor in NHP and humans

# Final thoughts

- I've shown you today that we have rapidly accelerated how well we can model therapeutics for non-human primates with AI.
- Translation to patients **requires grounding in human data** .
- We are motivated to treat real patients, **many of whom have no options today** .
- U.S. leading position in biotech is impacted by how much we invest and accelerate it right now (through a forward looking policy).