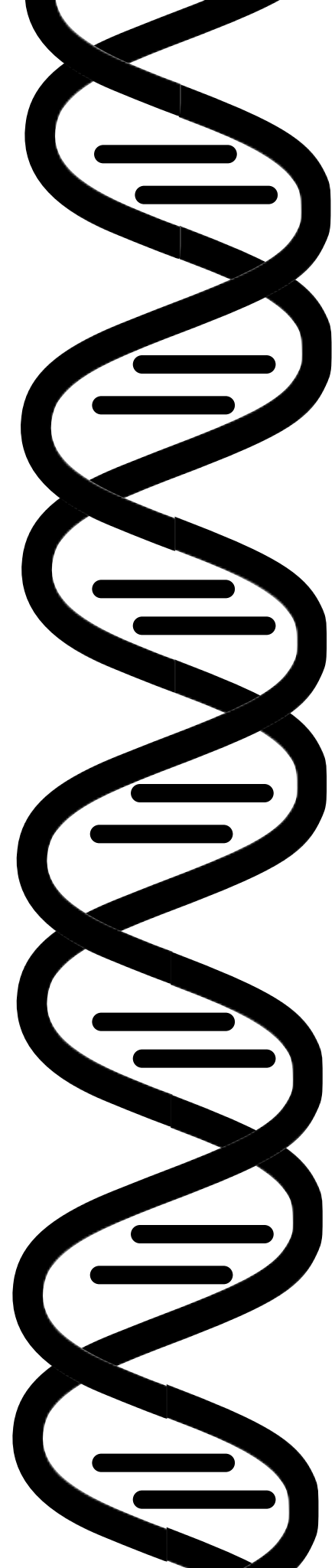


**Matt Might, Ph.D.**  
**Associate Professor**  
**Presidential Scholar**

**University of Utah**

**[matt.might.net](http://matt.might.net)**

**[@mattmight](#)**



# Ultra-rare drug development

Matt Might, Ph.D.

Associate Professor

Presidential Scholar

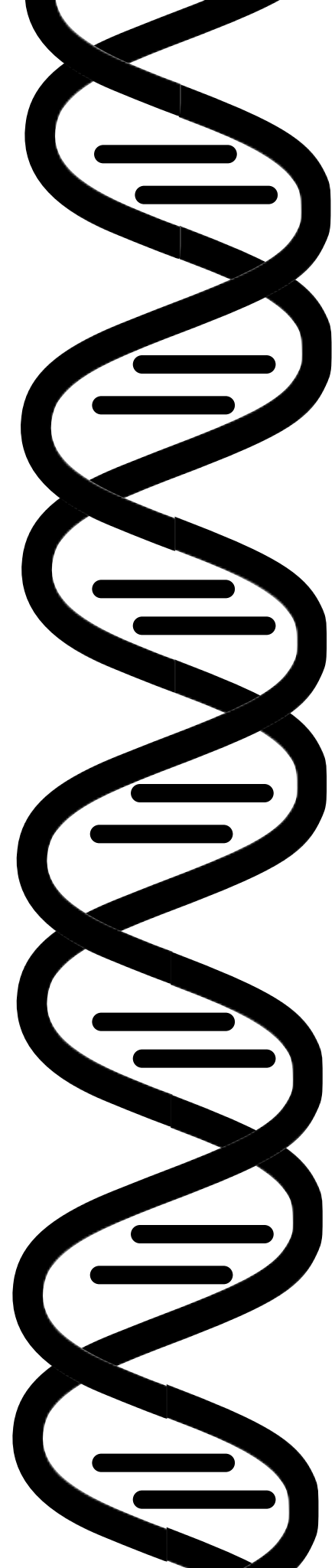
University of Utah

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# Ultra-rare drug development

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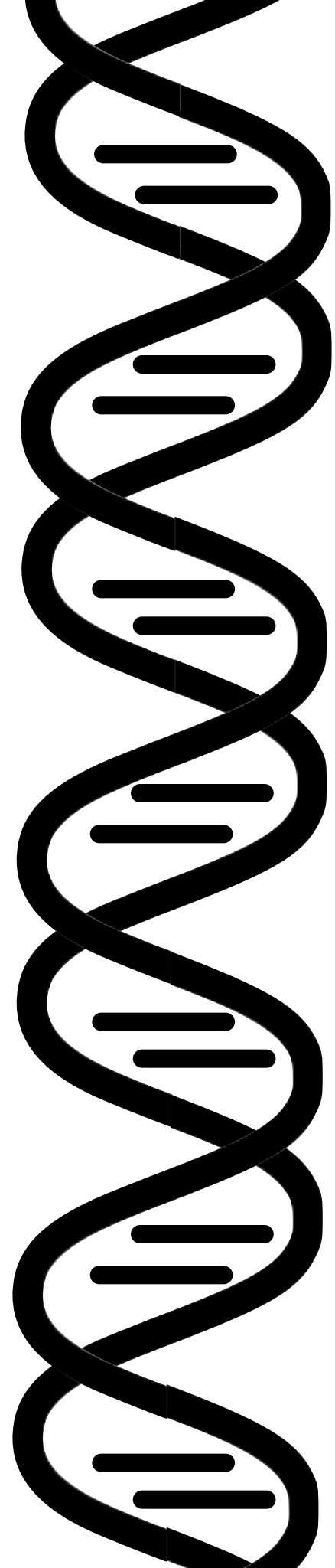
Associate Professor

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University of Utah

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[@mattmight](https://twitter.com/mattmight)



# Ultra-rare drug development

Matt Might , Dad

Finding & organizing patients

Funding for basic science

Tuning regulation for ultras

















# Undiagnosed island





















































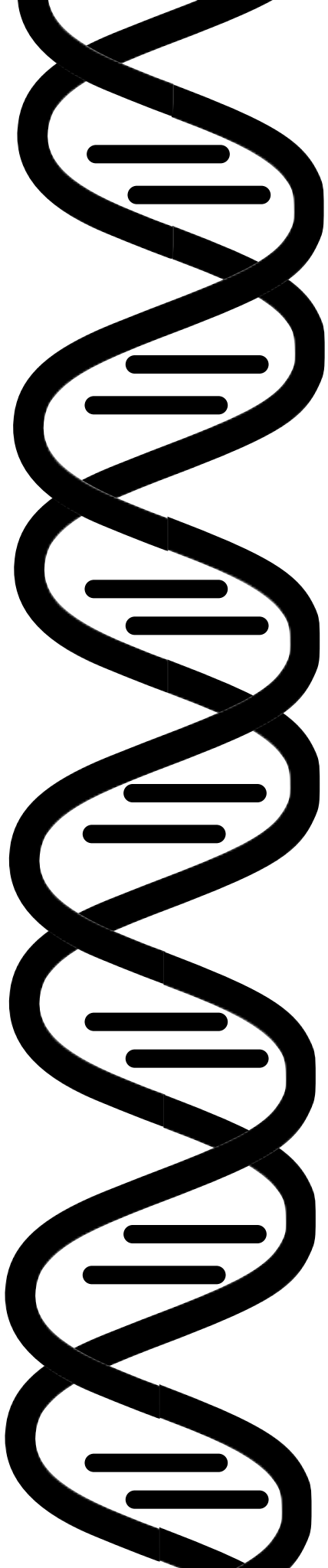


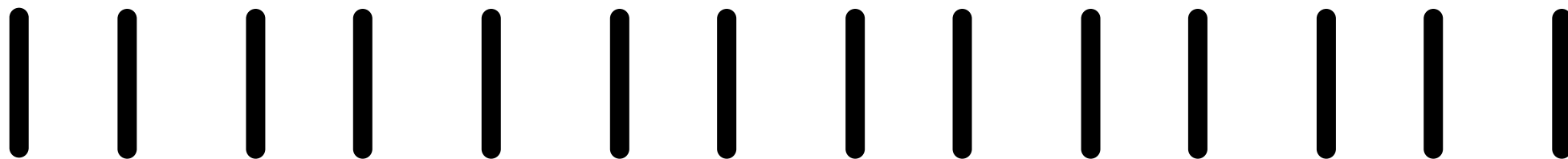
4.5

Duke  
UNIVERSITY

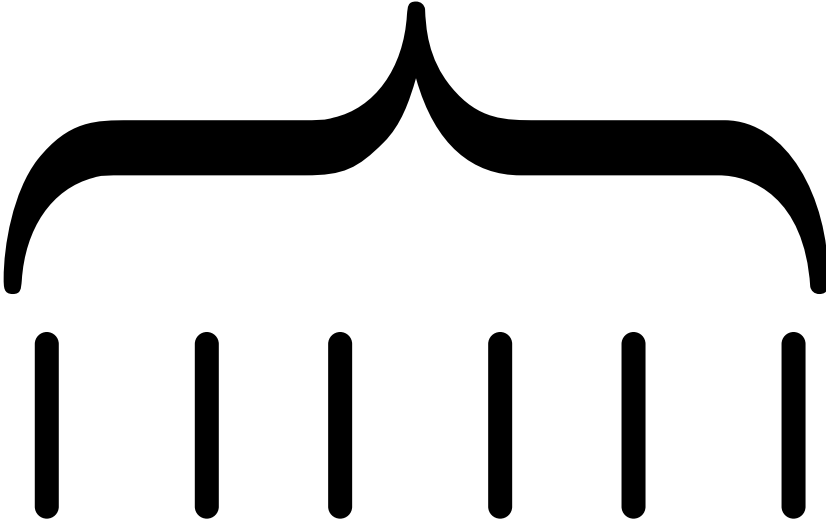




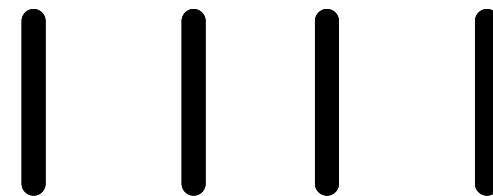
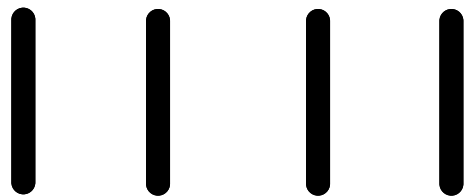
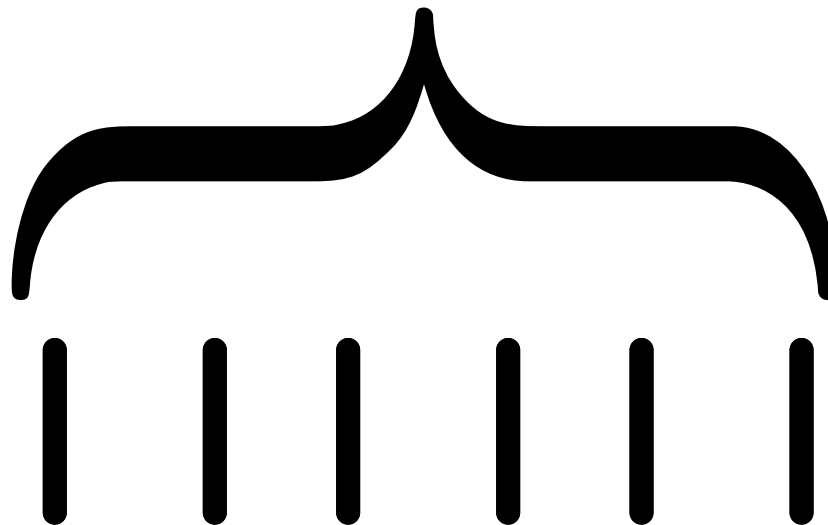




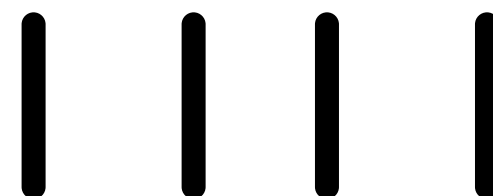
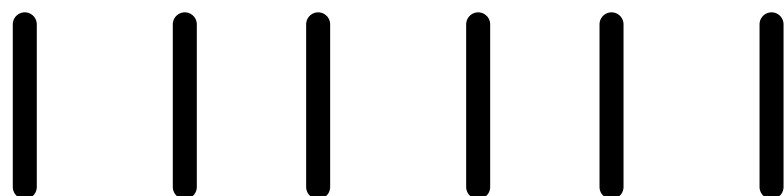
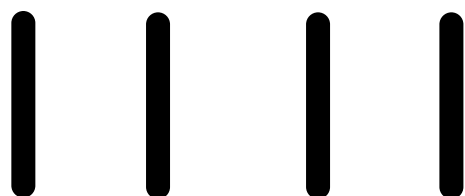
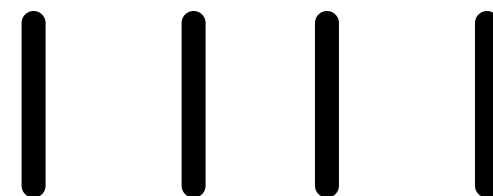
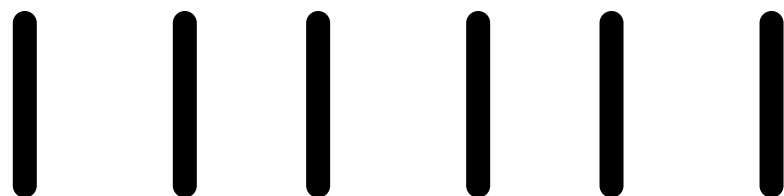
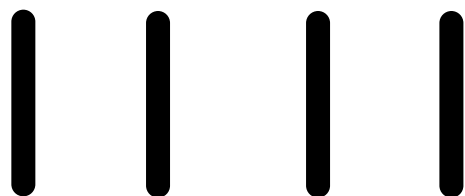
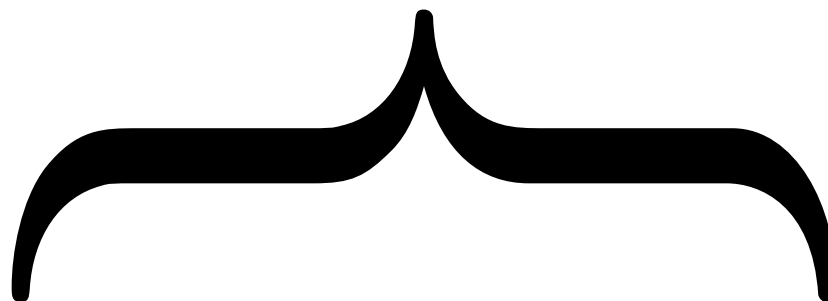
NGLY1



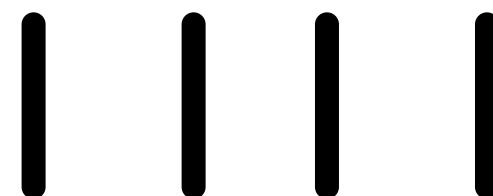
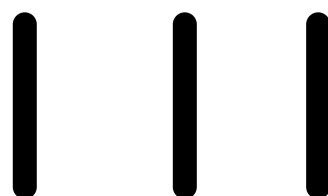
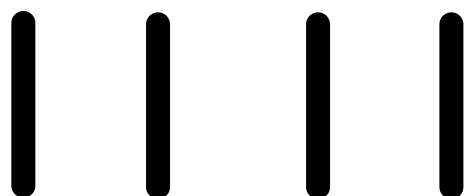
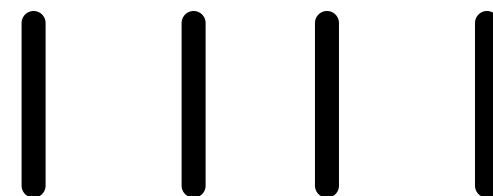
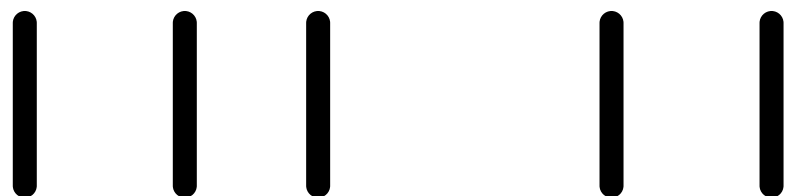
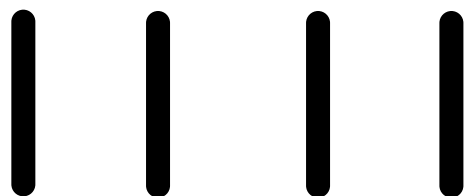
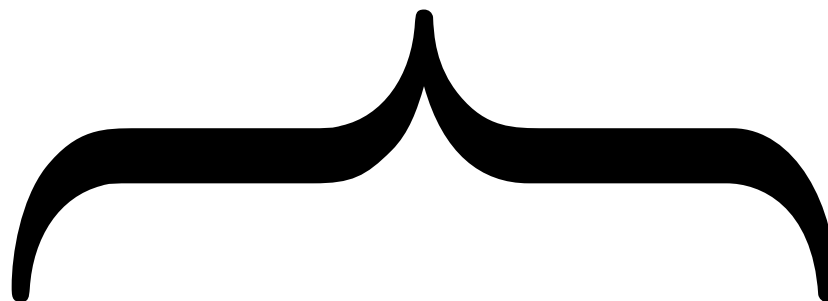
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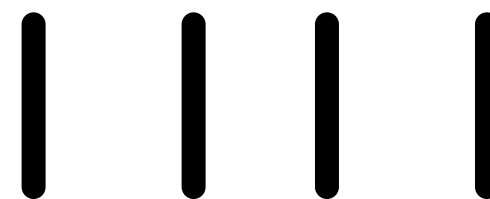
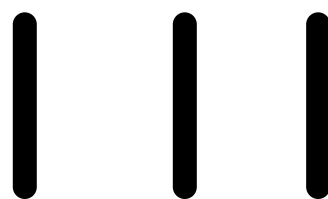
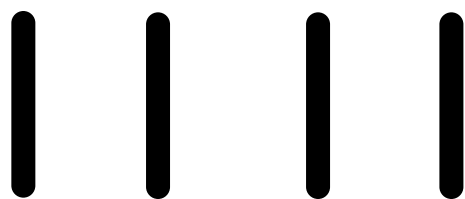
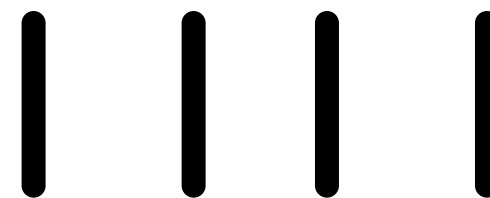
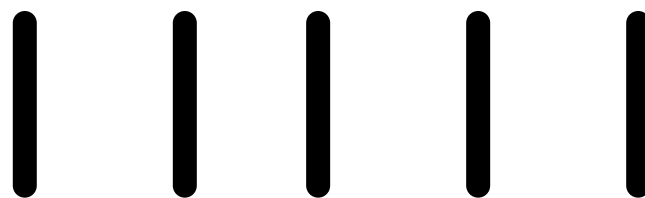
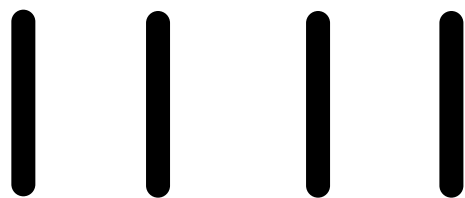
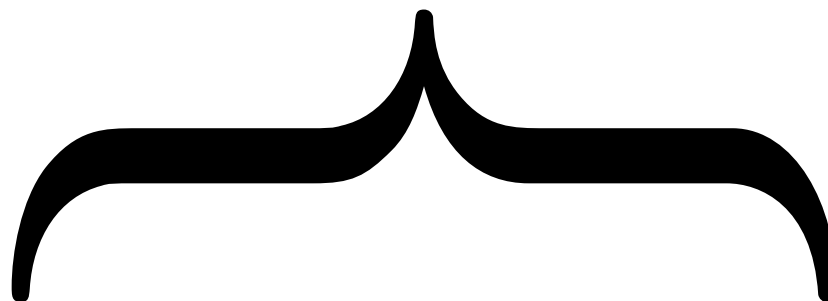
NGLY1



NGLY1

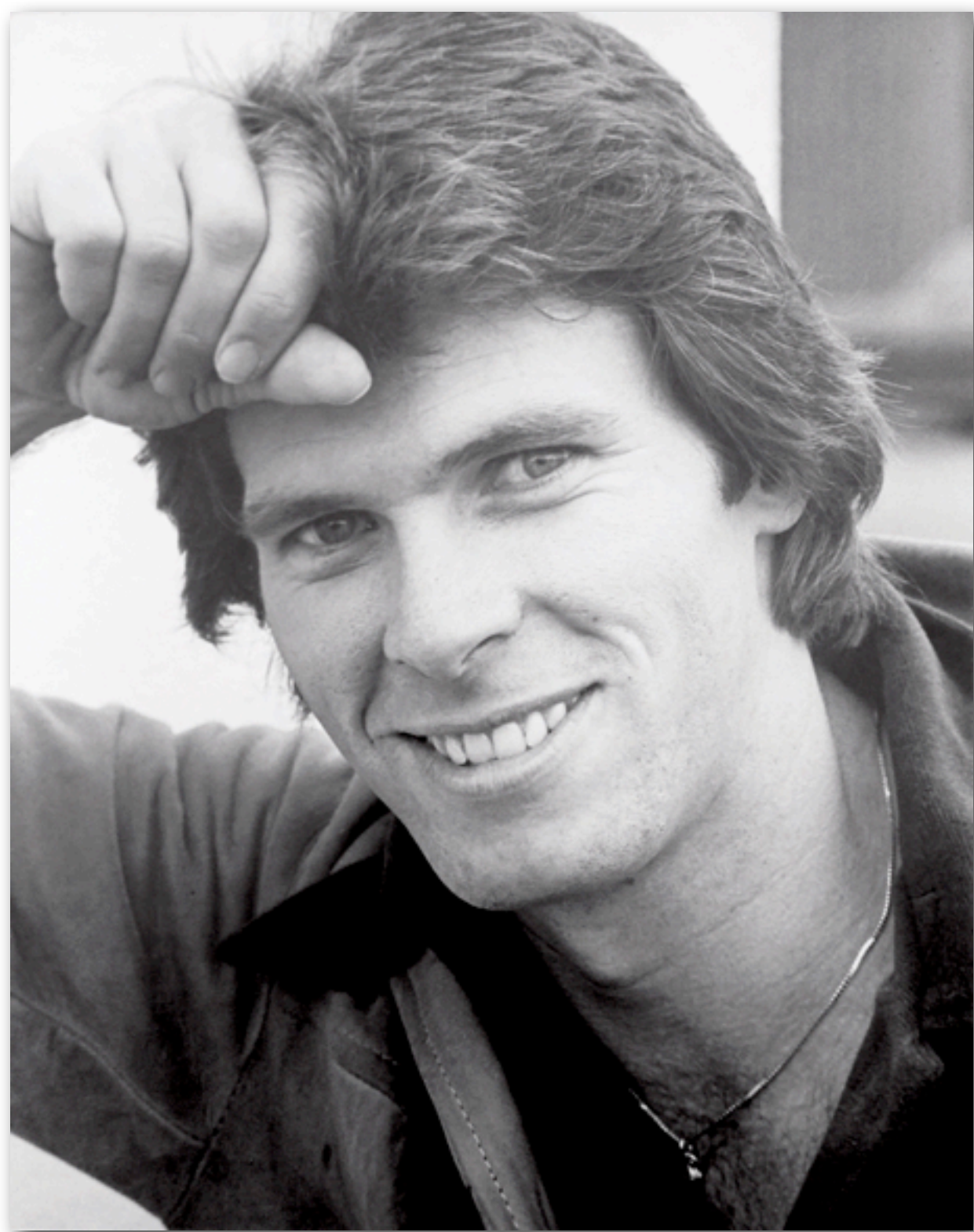


NGLY1



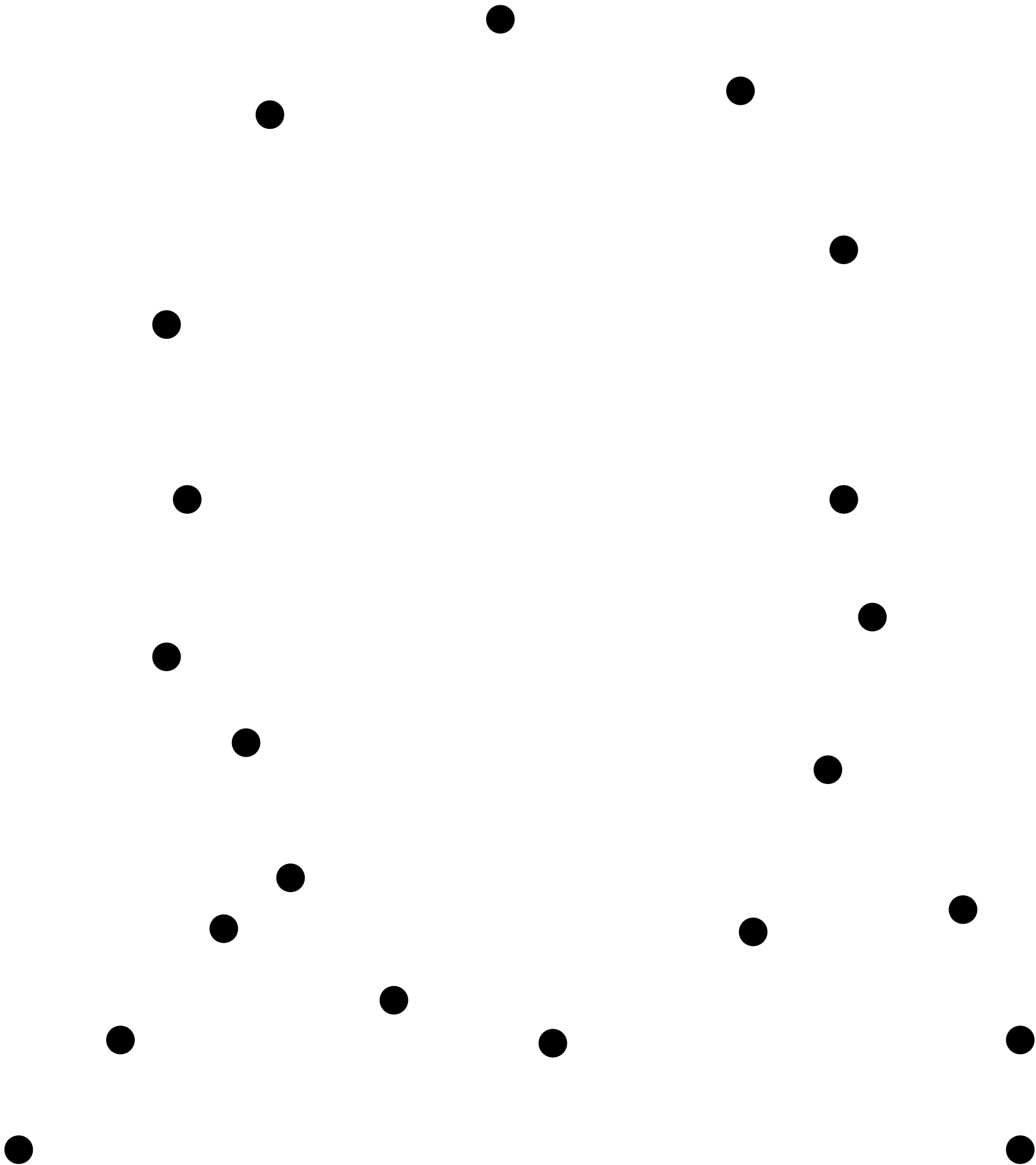


What if only one?



**Dr. Hudson Freeze, Sanford-Burnham**















NGLY1



**“Actionable”**







FASTEN SEAT BELT WHILE SEATED

USE BOTTOM OPTION FOR FLIGHT

SKYY























# The Aftermath



1,700

# Hunting down my son's killer

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I found my son's killer.

It took three years.

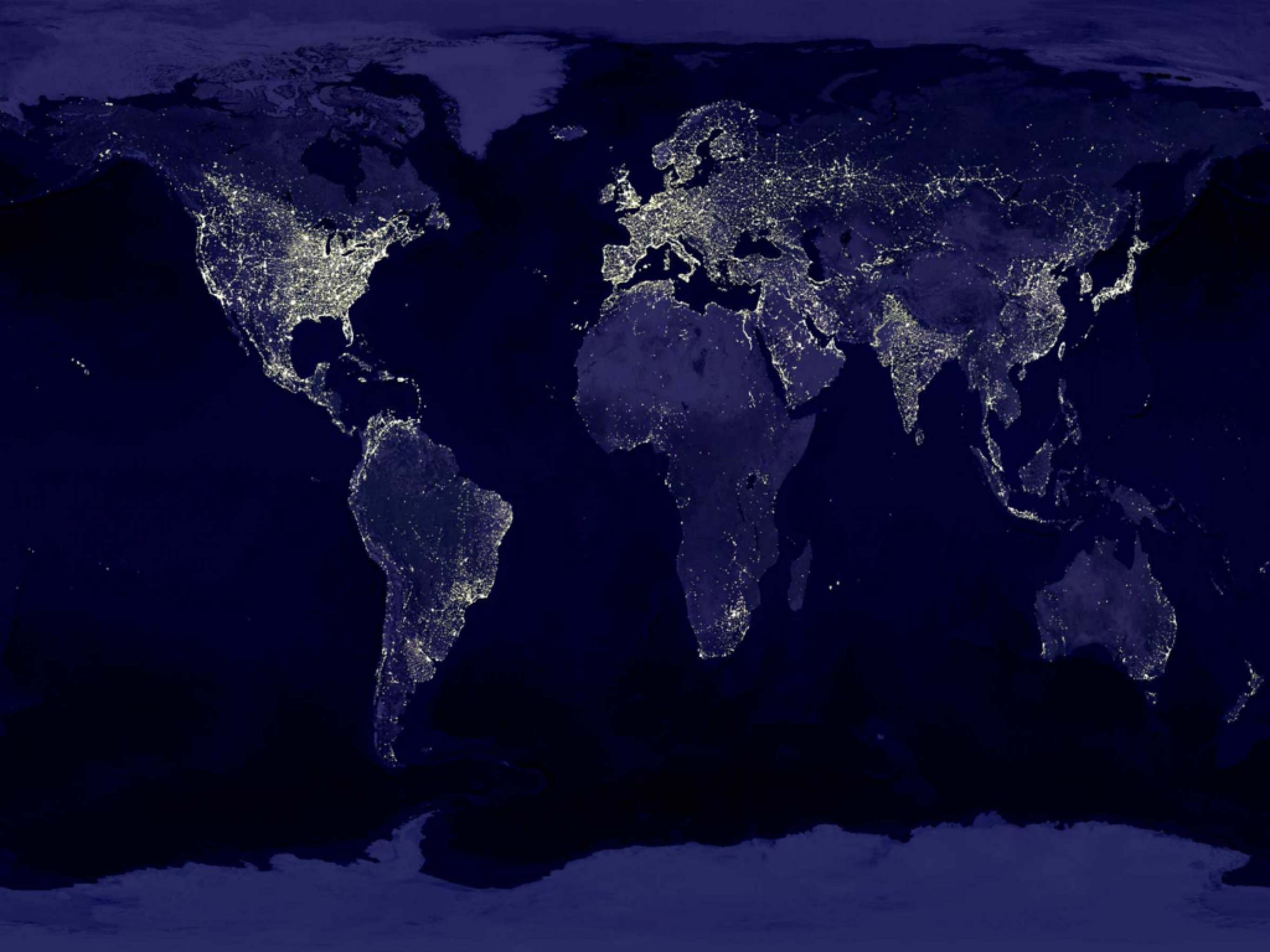
But we did it.



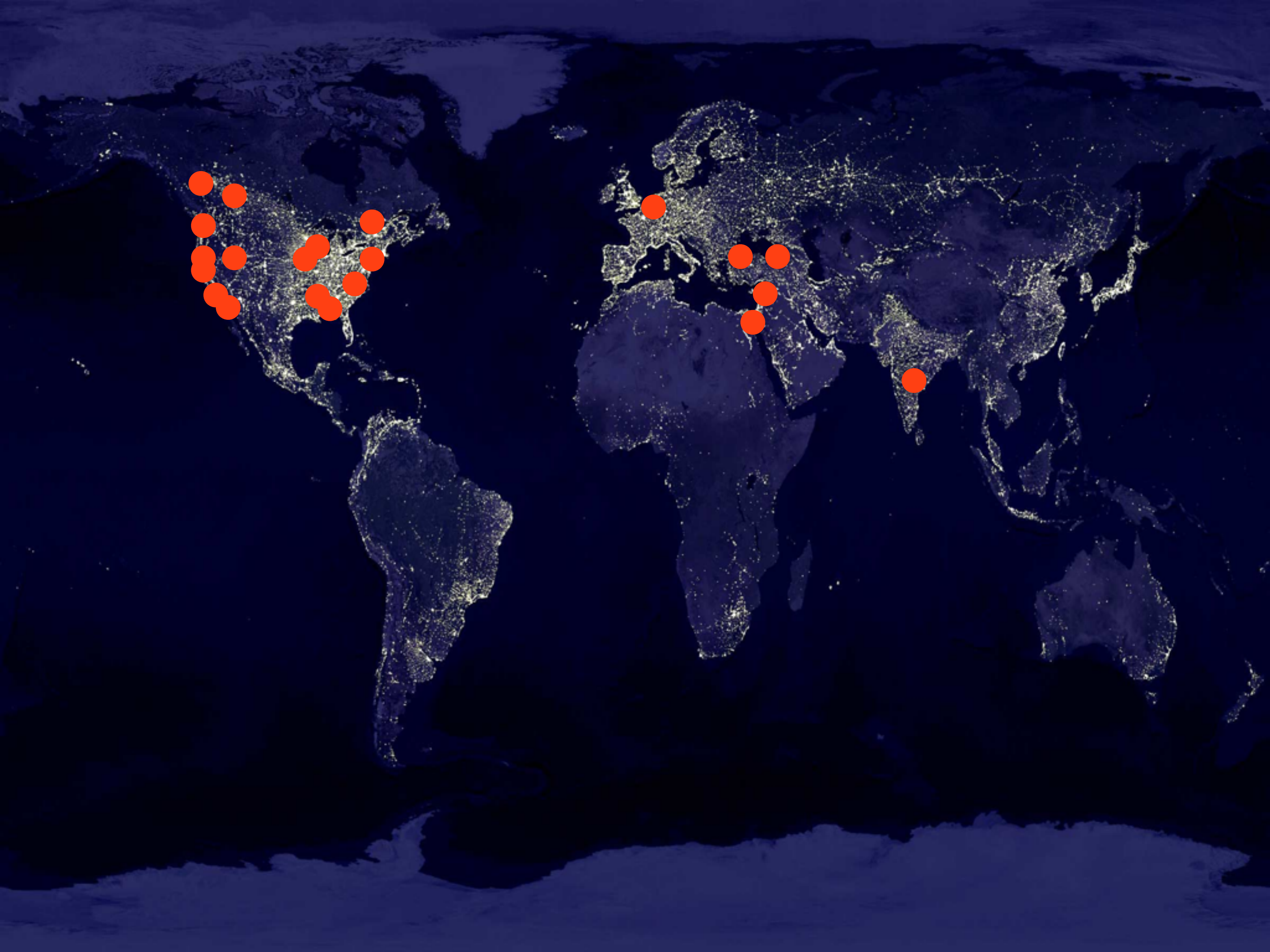
*Not quite like this.*

2,000,000+



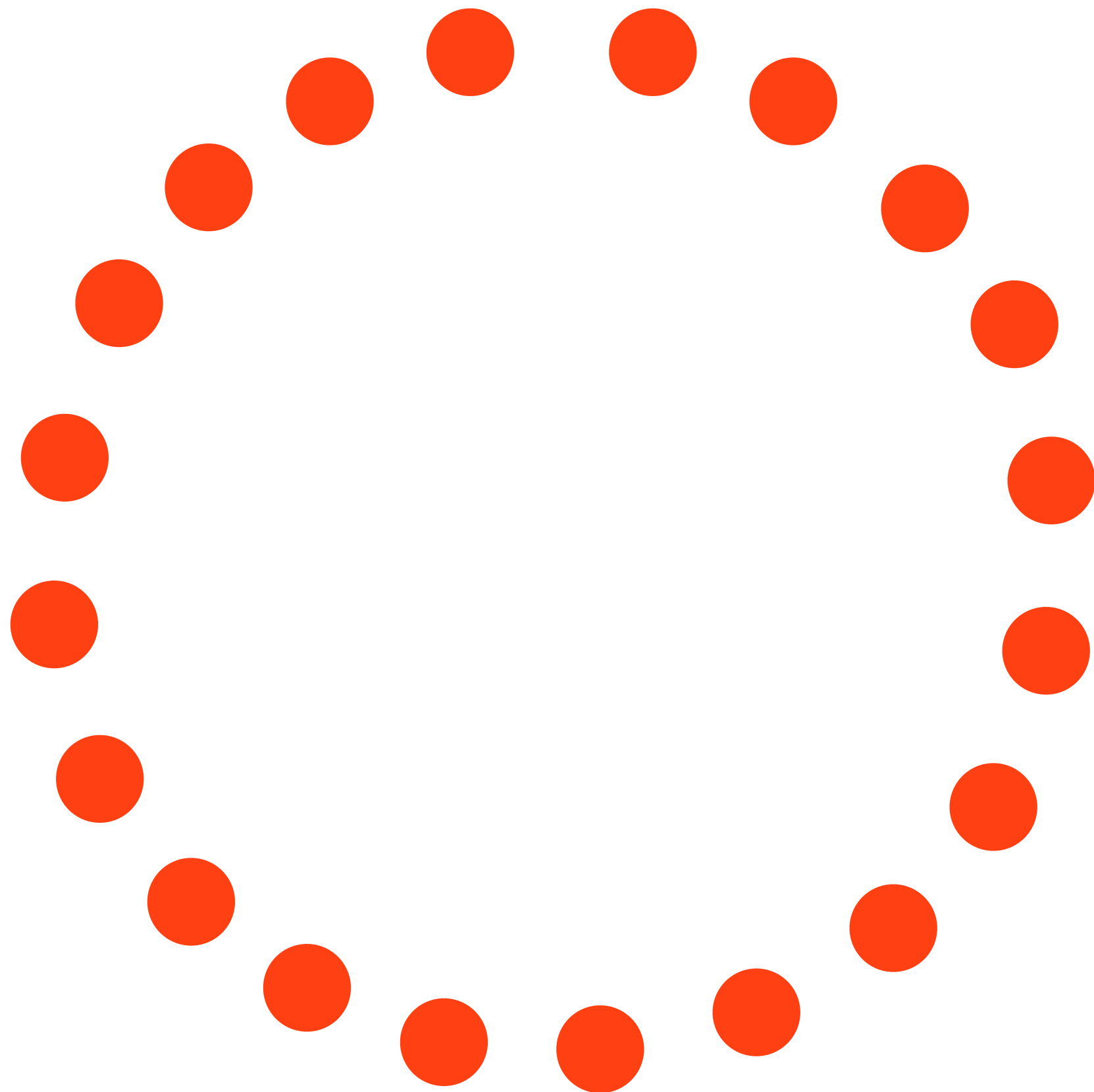














18

20







# Toward a Drug



# Step 1: Understanding



**National Institutes  
of Health**

**Natural History Study**



























Qty: 100  
Reorder No. 8820000  
Test Blue  
Store in a cool, dry place.

WEST PAS UN































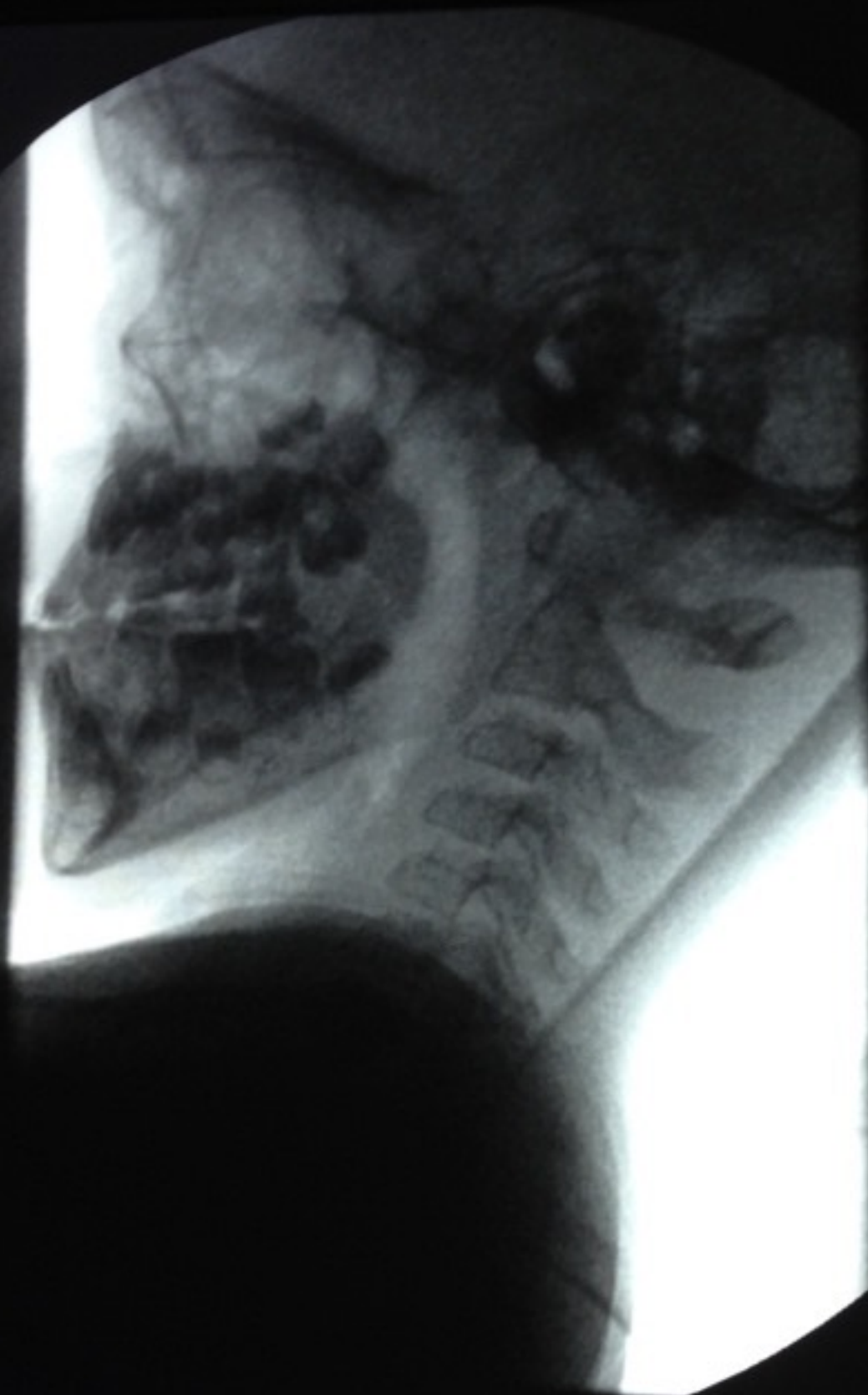












MIGHT  
BERTRAND

Barium Single shot  
Singleshot  
Continuous 25 f/s

0 0 1000



Patient

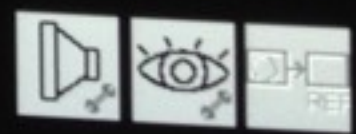
Examination

Postprocessing

Documentation

LIH

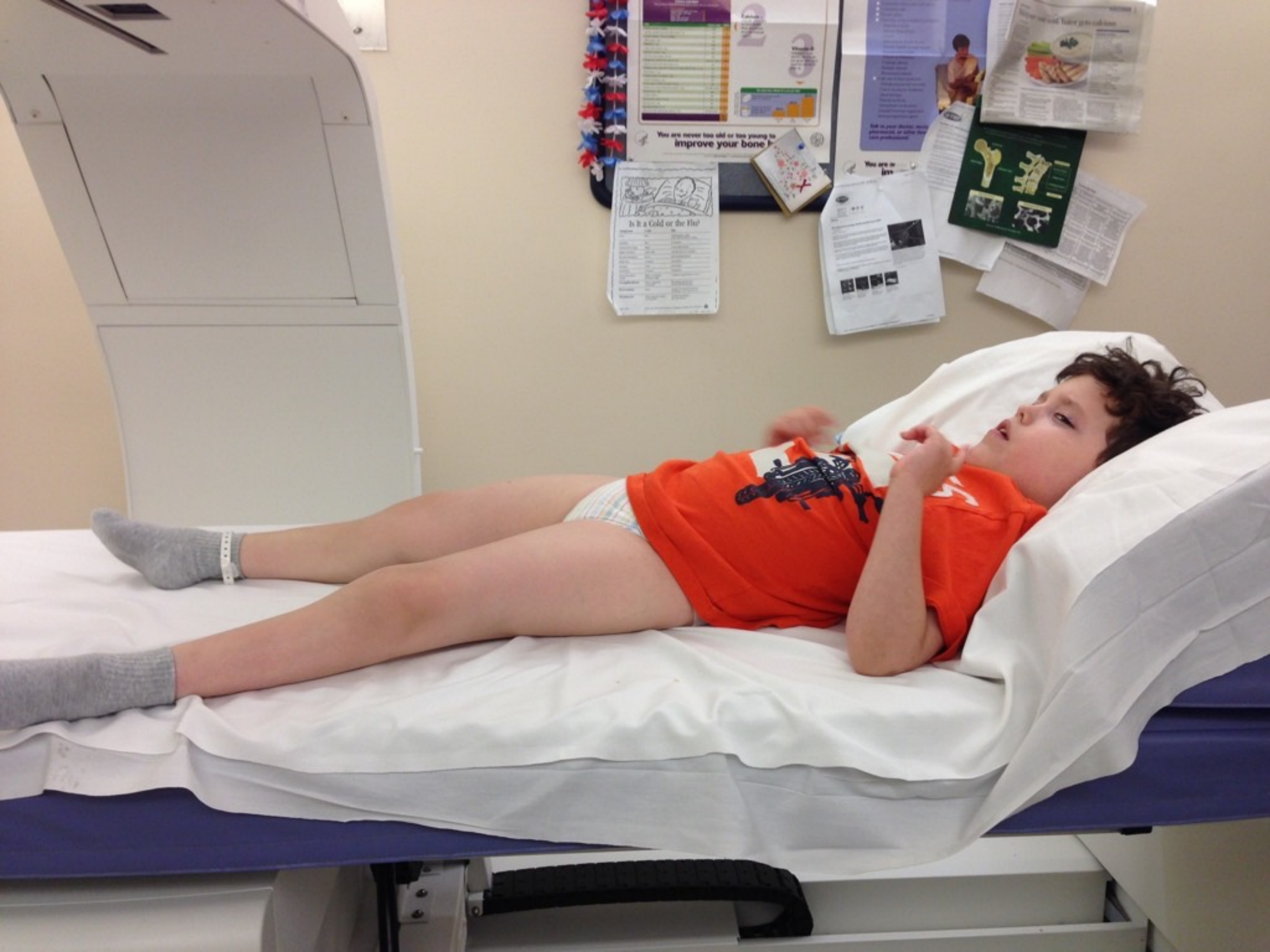
Fluoro: Cu 0.0mm  
Acquis: Cu 0.0mm  
25.4 cGycm2











































































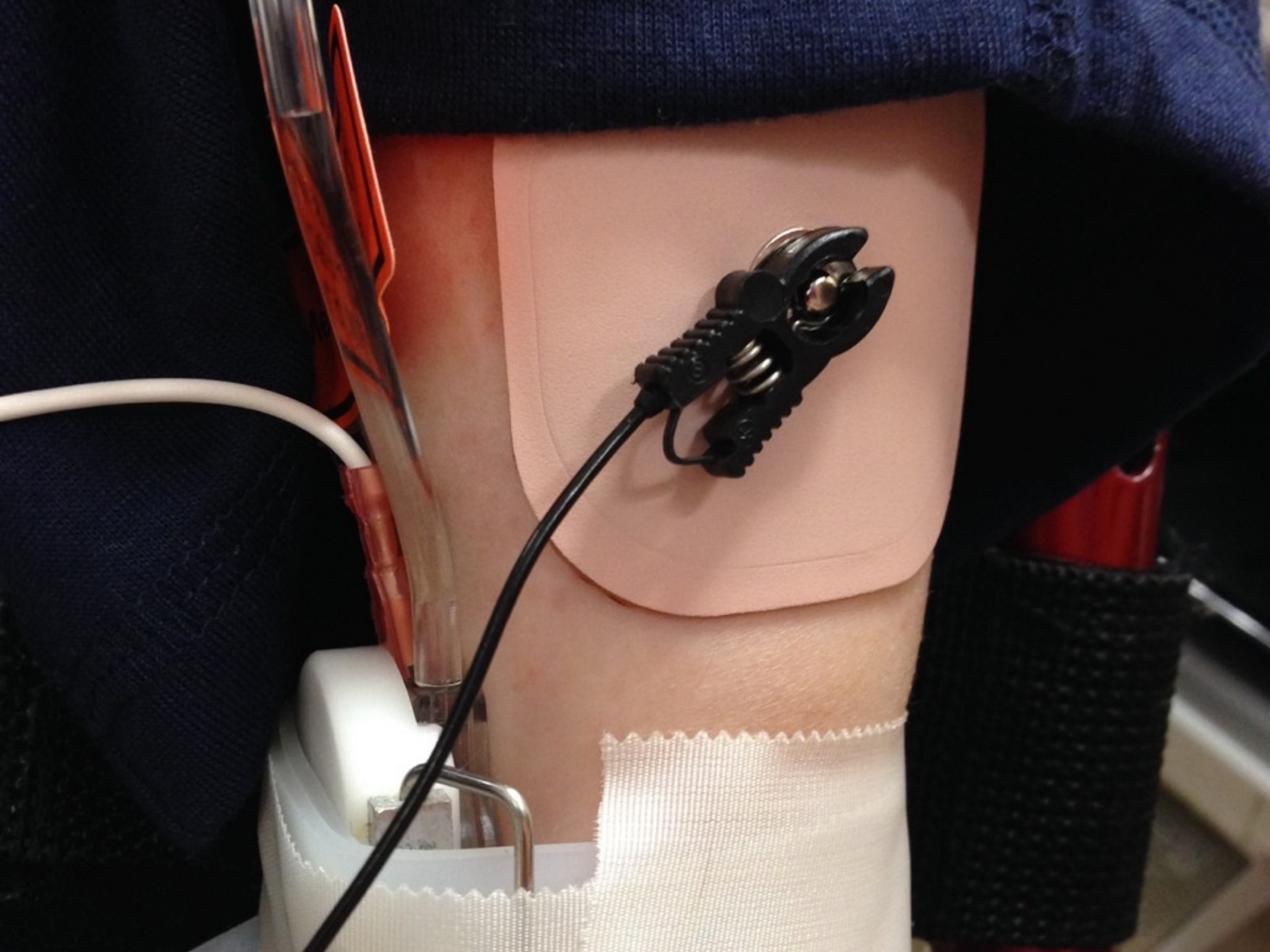




































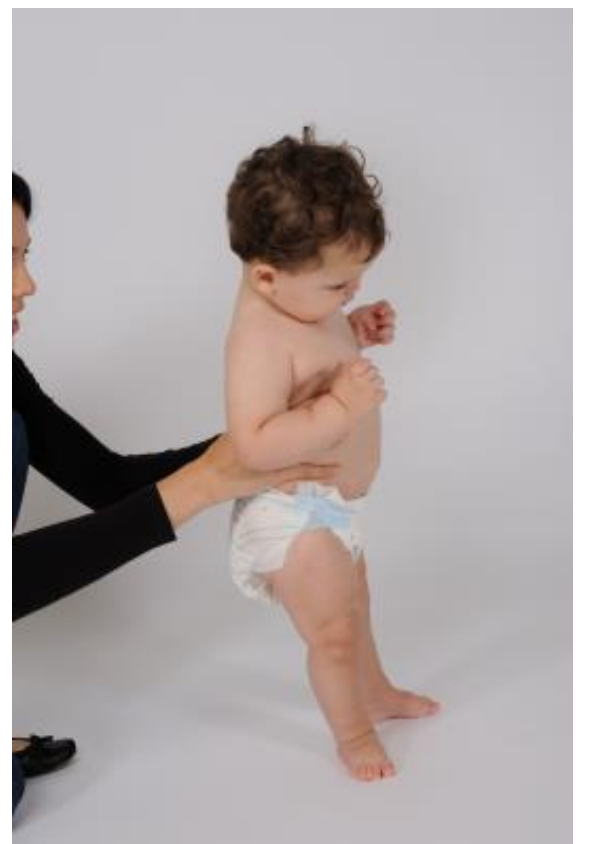


























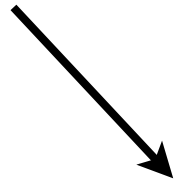








**Biomarkers!**



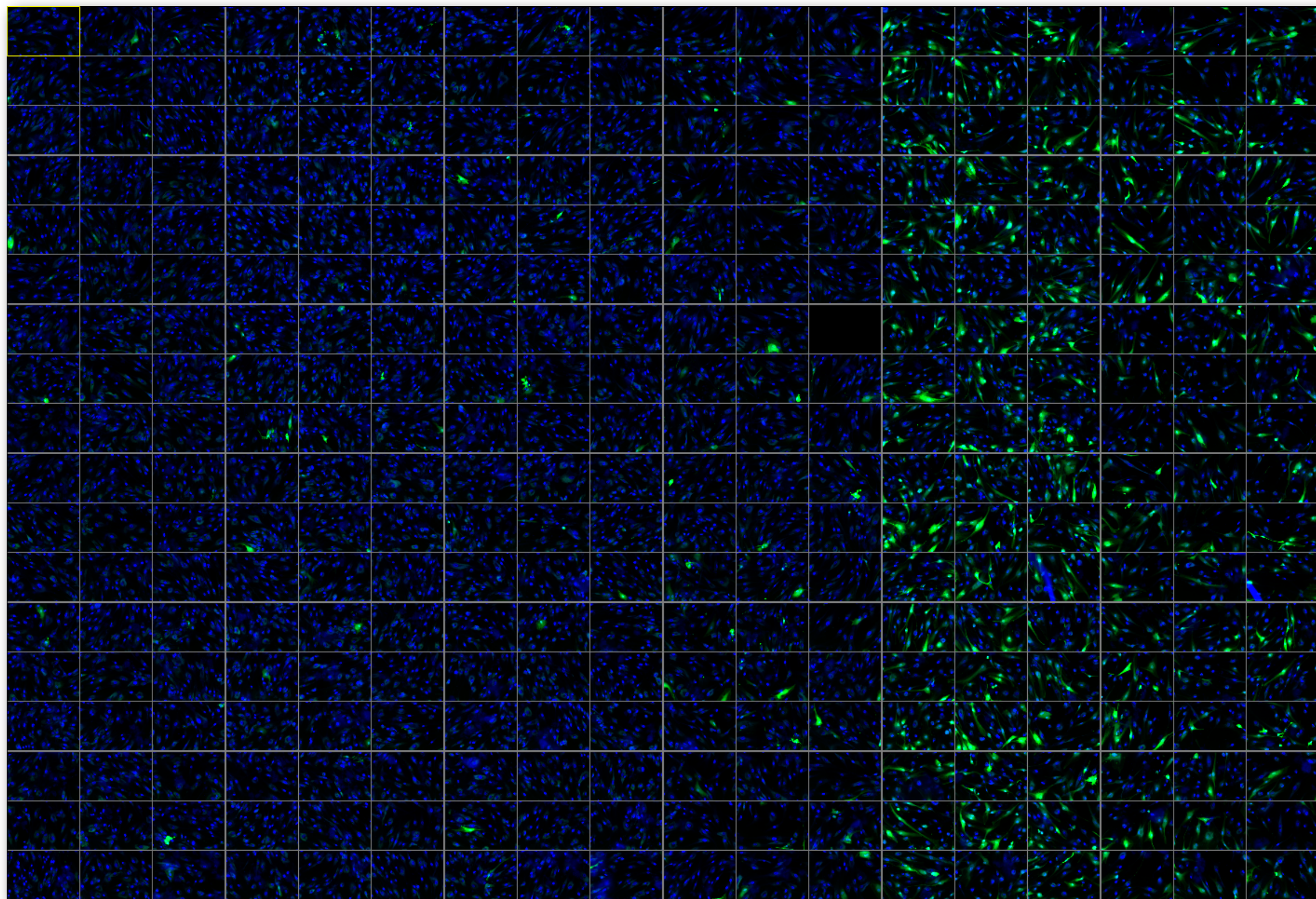


# Step 2: Screening









Source: Hudson Freeze







# Animal models



















NUTRITIONAL



# YEAST

*Flakes*

Vegan  
Friendly

PREMIUM • UNSWEETENED • FORTIFIED

*Gluten Free*

Wonderful Nutty Flavor  
Non-GMO

NET WT. 22 oz. (624 g)

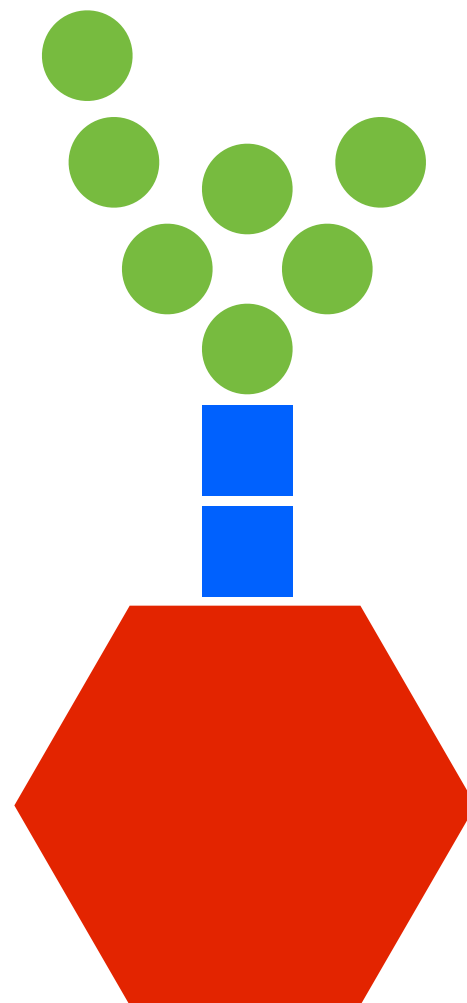
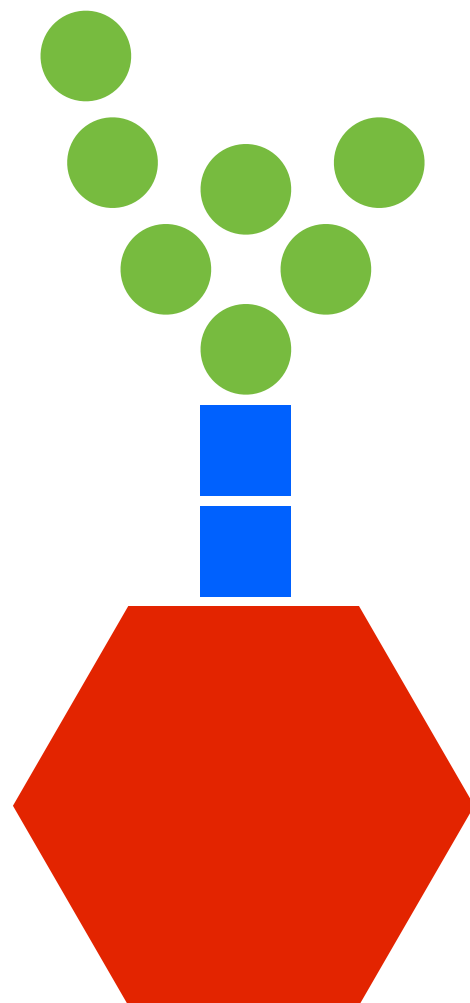
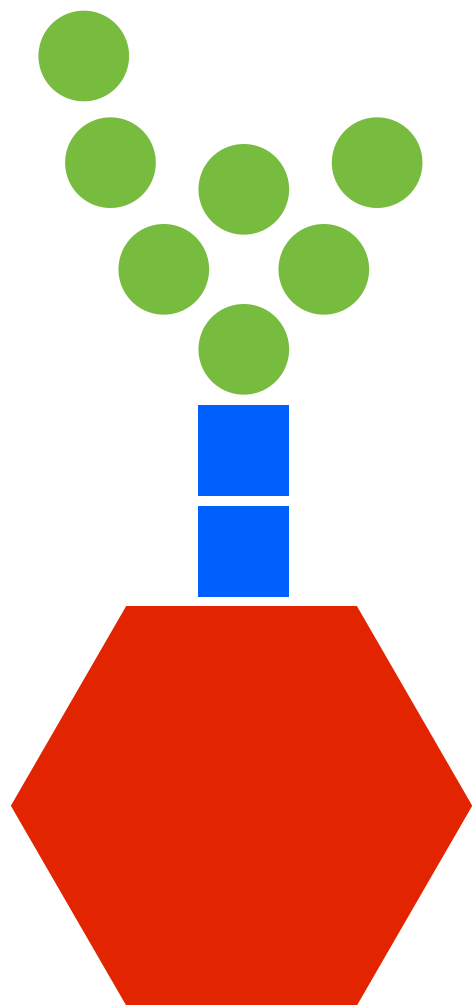
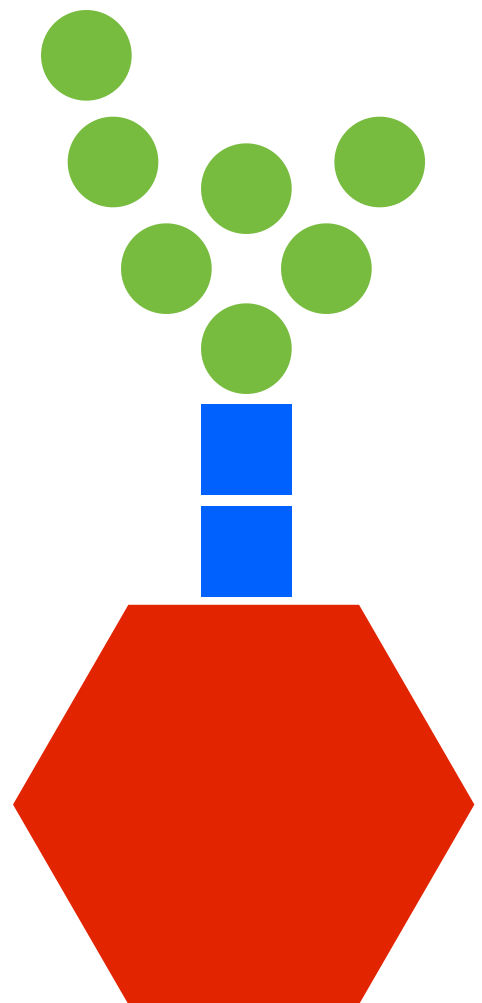


**Early success!**

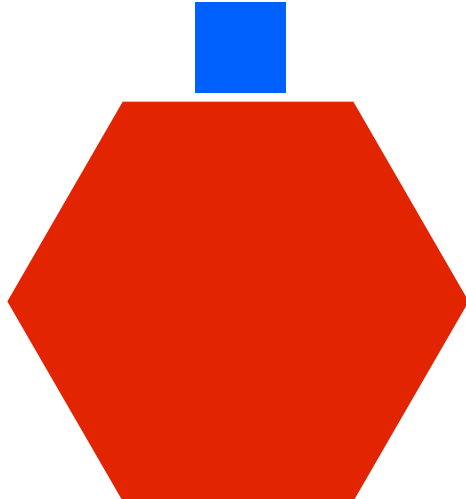
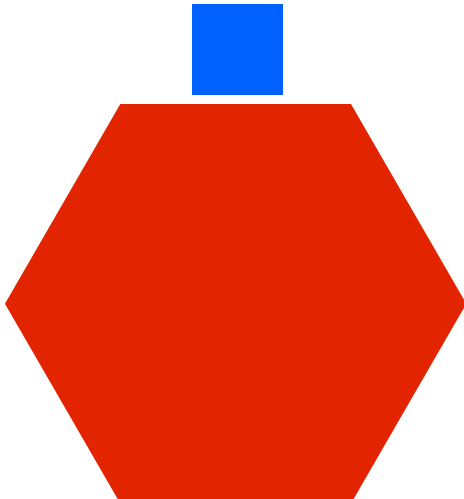
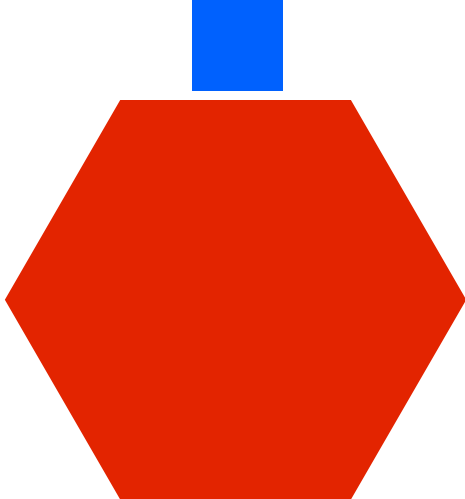
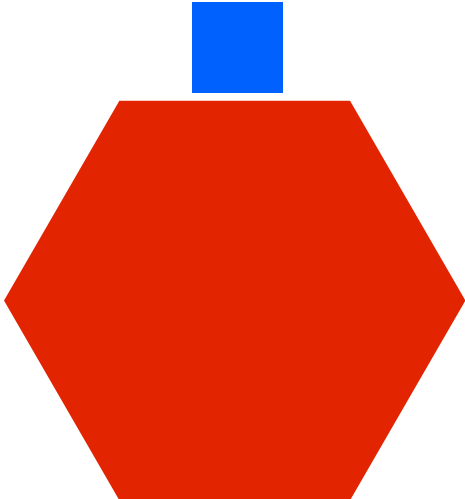




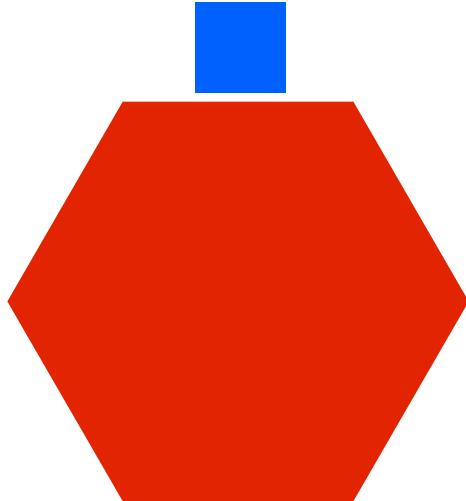
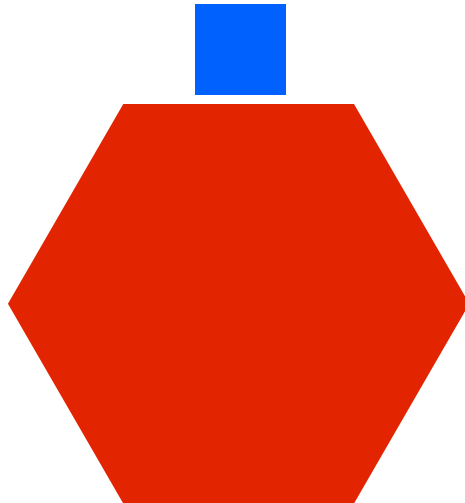
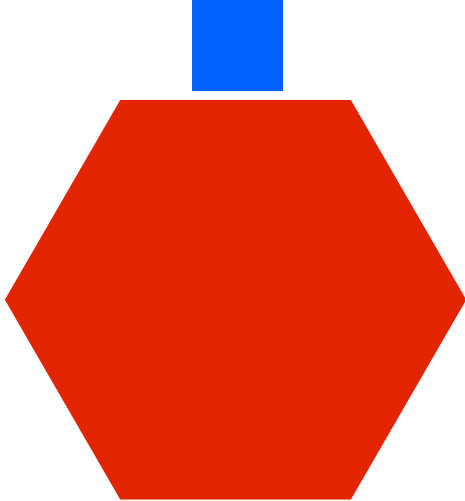
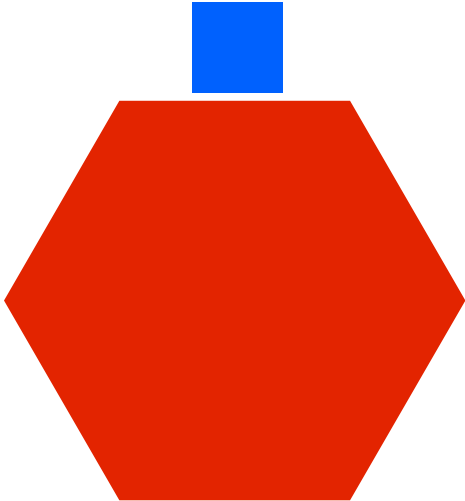


































Diabetes



HIV/  
Ebola



M.S.

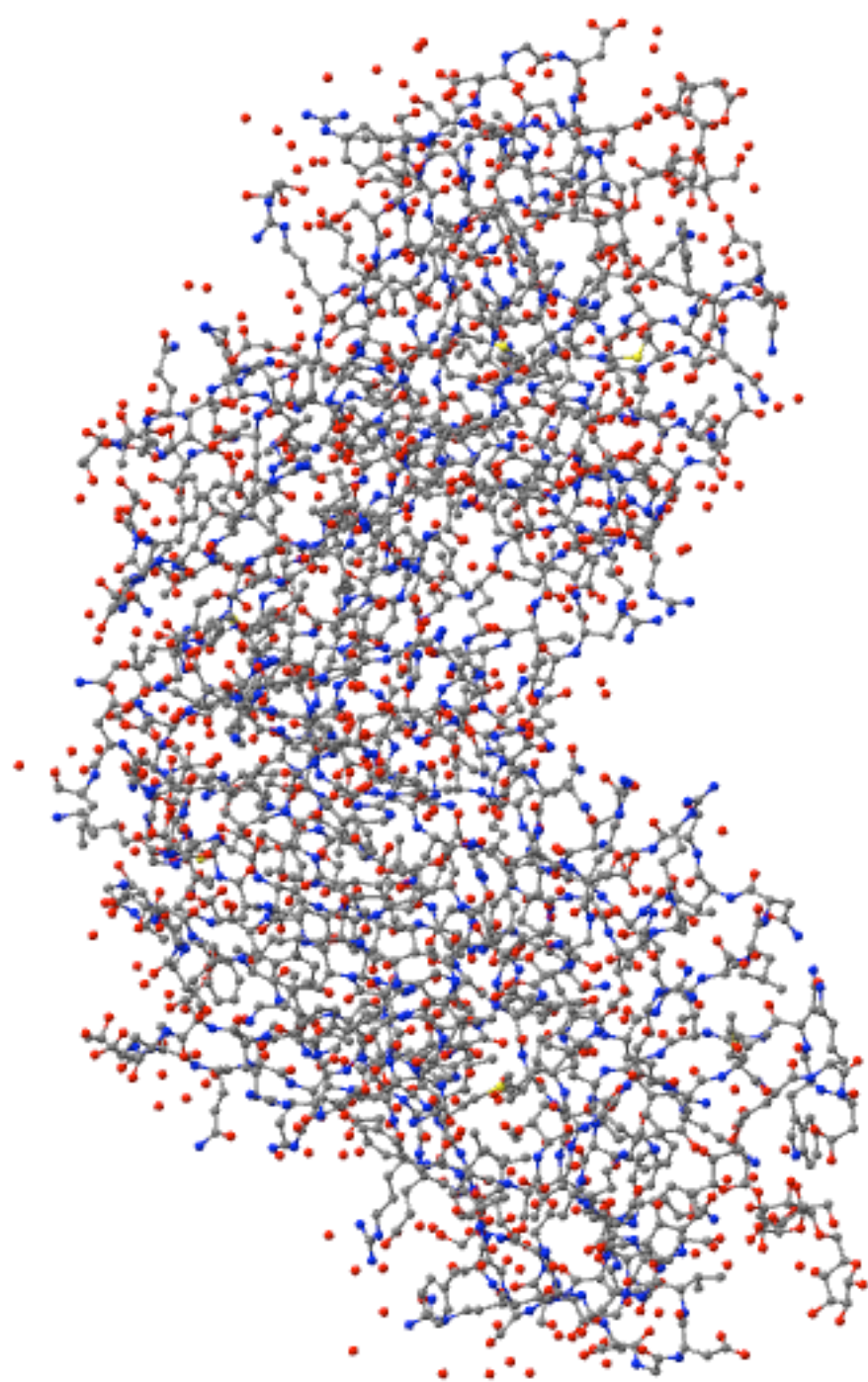


Alzheimer's

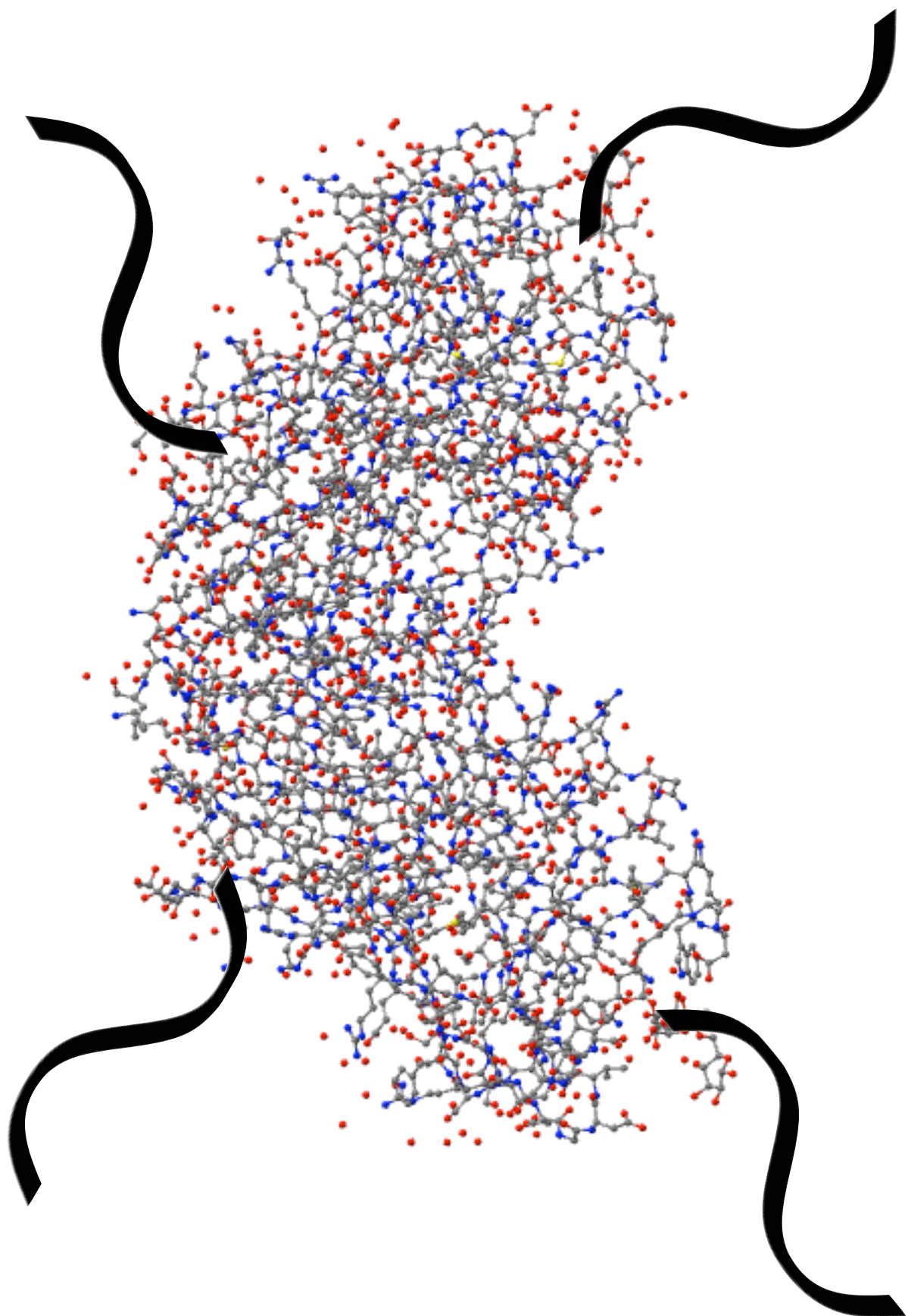


# Enzyme replacement



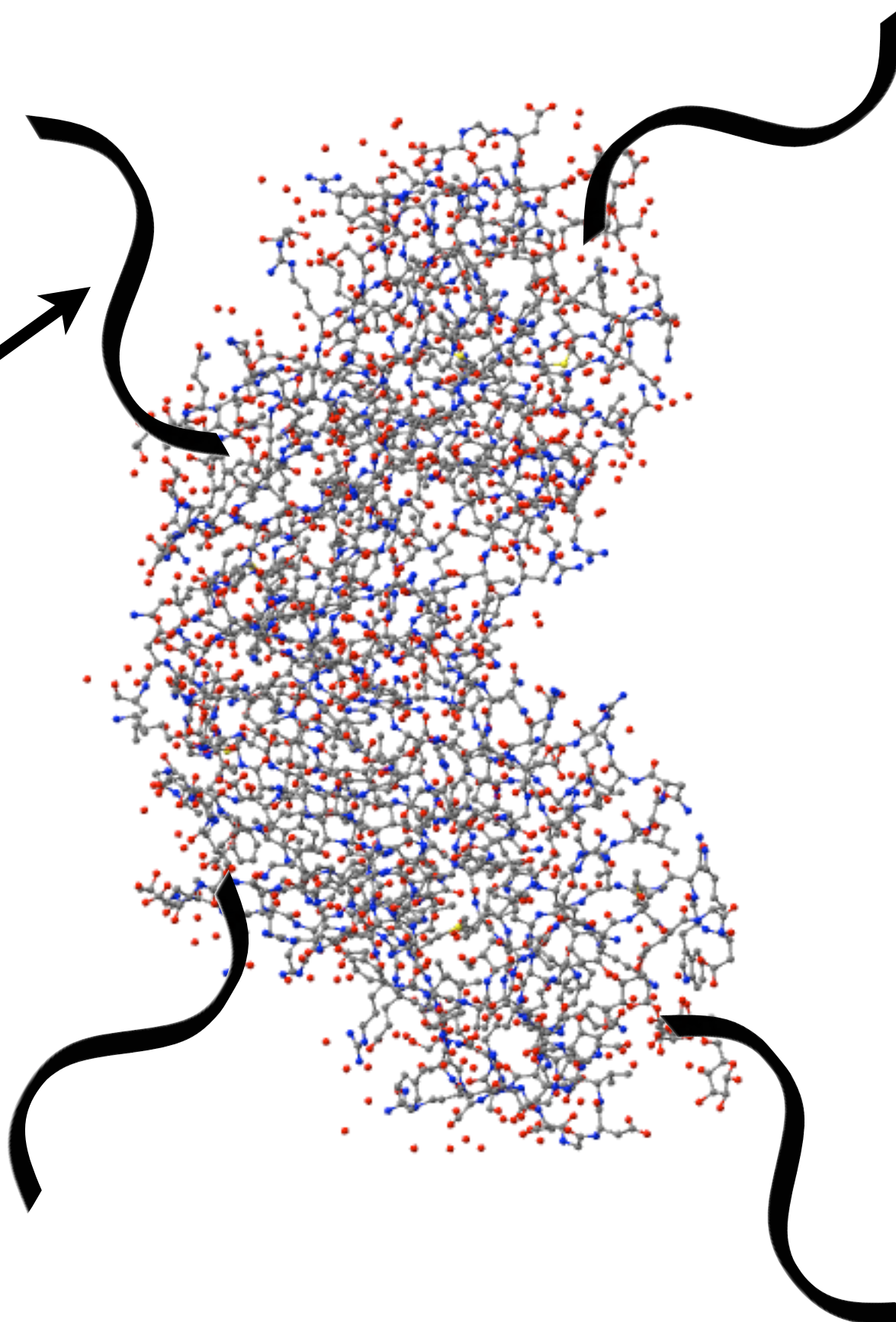








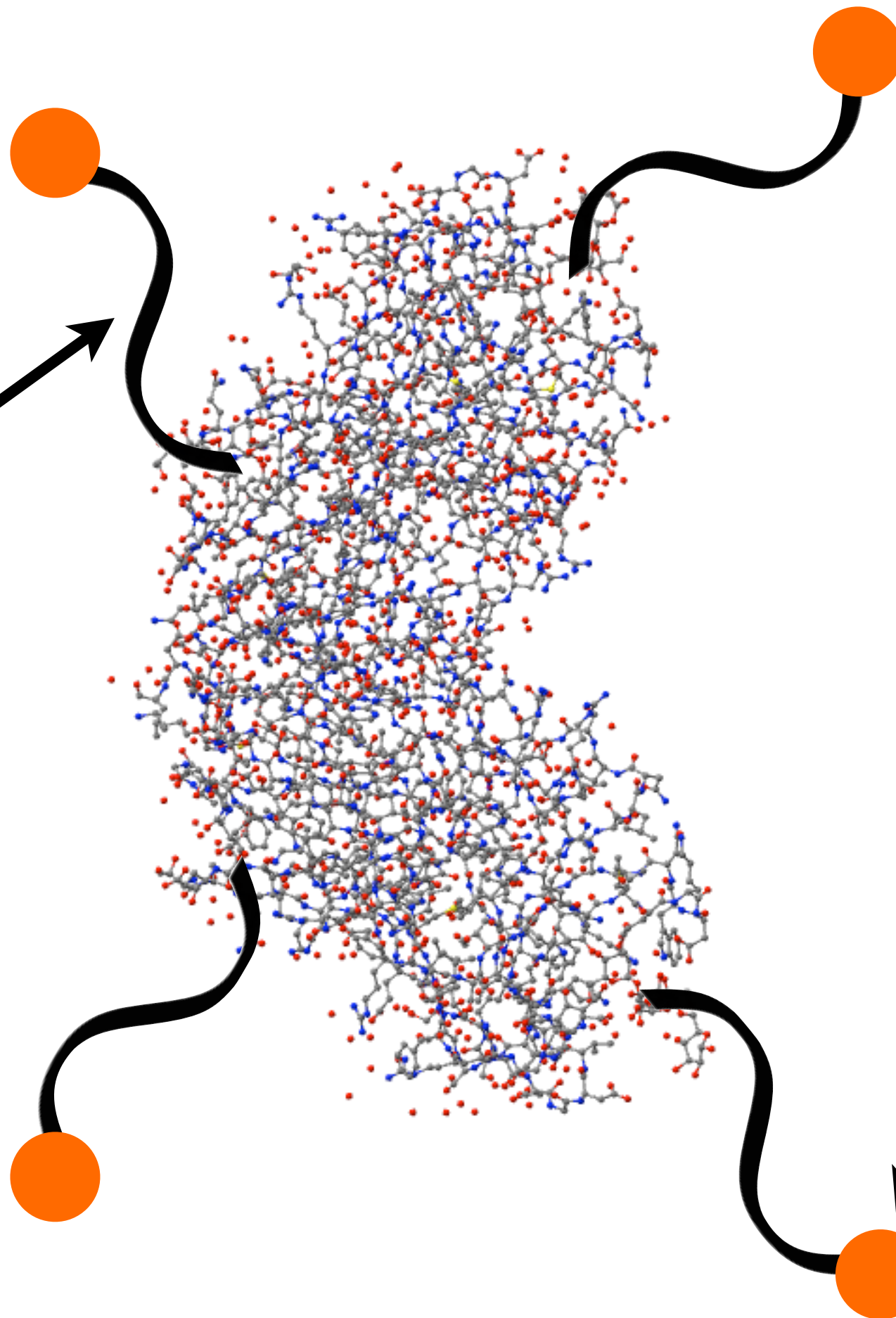
PEG polymer





PEG polymer

TAT peptide





# Repurposing



healx



**Low-hanging fruit**



*De novo* gain of function in Gene X



# Google

Google Search

I'm Feeling Lucky



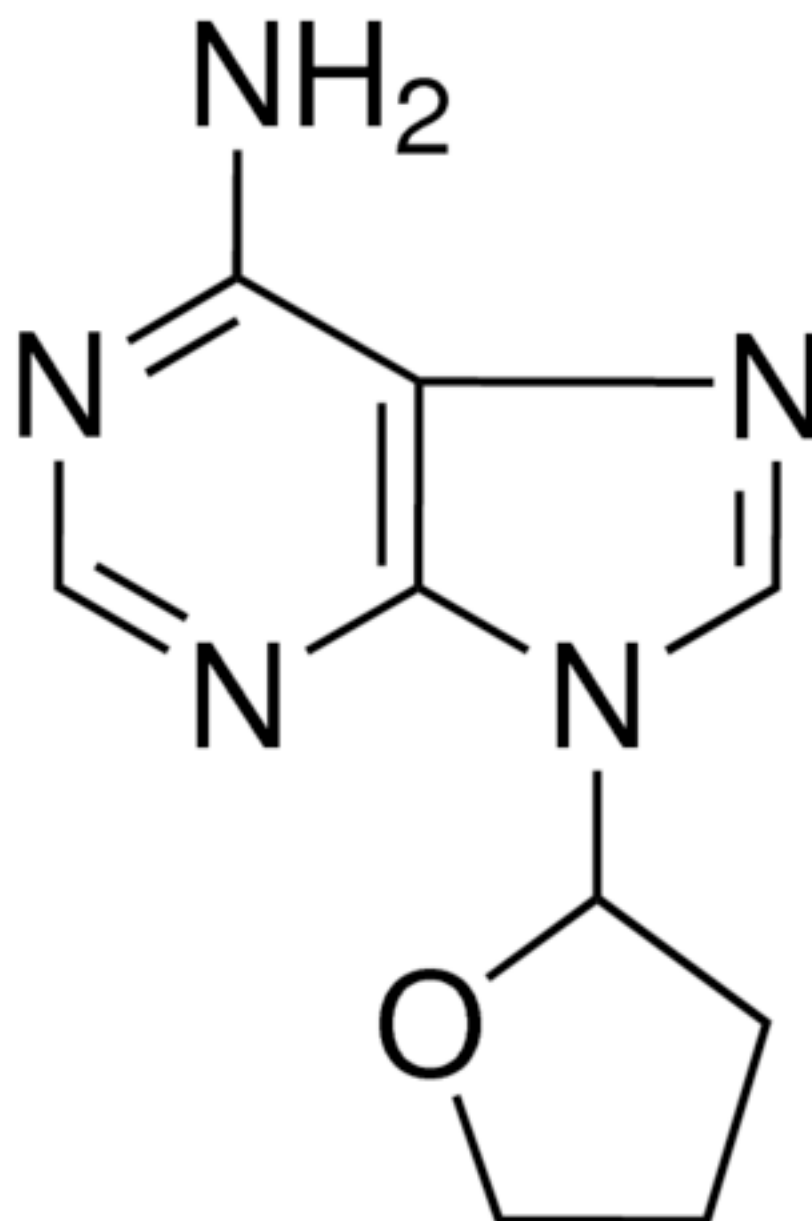


Gene X inhibitor

Google Search

I'm Feeling Lucky





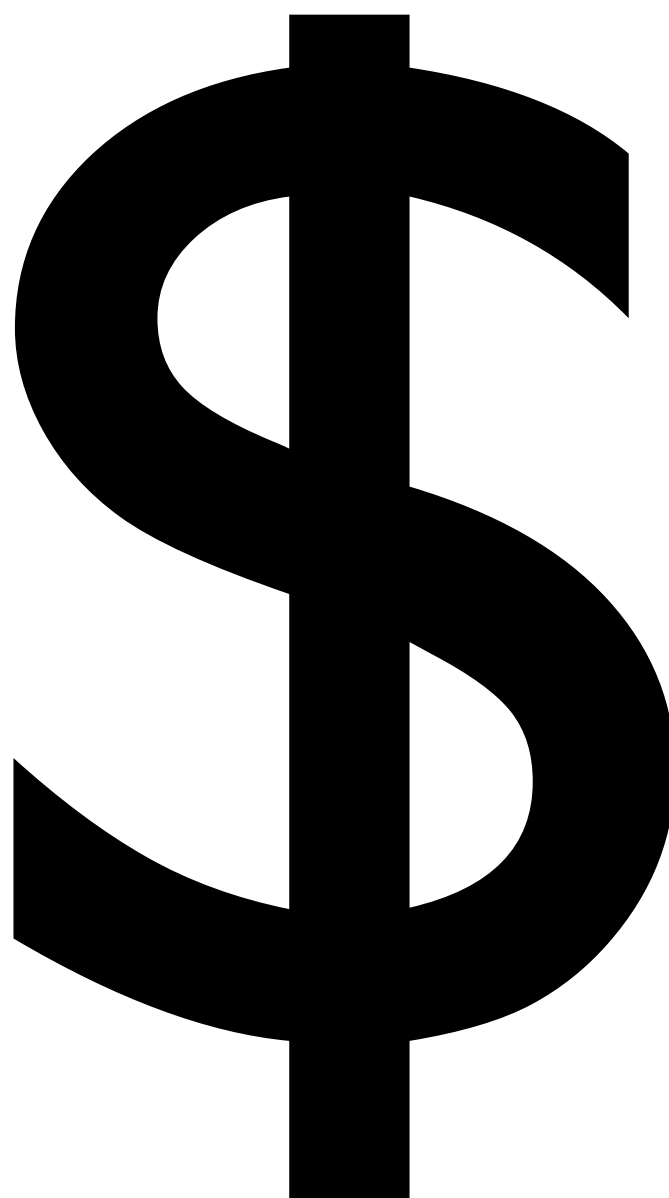
Gene X inhibitor







# Obstacles





**Safety & Efficacy**

# Safety





EVERY ISSUE.  
EVERY STORY  
EVERY DEVICE.

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# Thank you!