Matt Might, Ph.D.
Associate Professor
Presidential Scholar
University of Utah
matt.might.net
@mattmight
Ultra-rare drug development

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Matt Might, Dad
Finding & organizing patients

Funding for basic science

Tuning regulation for ultras
Undiagnosed island
4.5
NGLY1
What if only one?
“Actionable”
The Aftermath
1,700
Hunting down my son's killer

I found my son's killer.

It took three years.

But we did it.

Not quite like this.
2,000,000+
Toward a Drug
Step 1: Understanding
Natural History Study
Biomarkers!
Step 2: Screening
Animal models
NUTRITIONAL

YEAST Flakes

PREMIUM • UNSWEETENED • FORTIFIED

Gluten Free

Wonderful Nutty Flavor
Non-GMO

NET WT. 22 oz. (624 g)
Early success!
NGLY1

Diabetes

HIV/Ebola

M.S.

Alzheimer’s
Enzyme replacement
Repurposing
Low-hanging fruit
De novo gain of function in Gene X
Gene X inhibitor
Gene X inhibitor
Obstacles
Safety & Efficacy
Safety
Thank you!