

Repurposing Approved Drugs

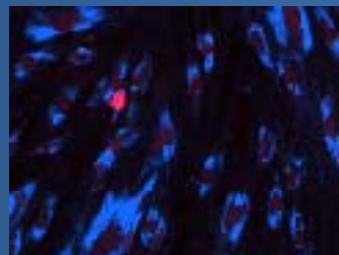
Niemann-Pick Type C (NPC) Disease

- Autosomal recessive neurodegenerative disorder characterized by neurovisceral lipid storage
- Incidence: 1/120,000
- No FDA-approved therapy
- TRND pilot project: repurposing an approved drug that can clear cholesterol from NPC patient cells



Courtesy of National Niemann-Pick Disease Foundation

NPC patient cells



Control



+ Compound

Cholesterol (Blue)
Cell stain (Red)

Repurposing Approved Drugs

Niemann-Pick Type C (NPC) Disease

- Compound: Cyclodextrin
- Collaborators
 - NIH (Genetics, Clinical)
 - Washington University (Biochemistry)
 - Albert Einstein (Animal models)
- NPC disease foundations involved and facilitating
- TRND project milestones
 - ✓ Proof-of-concept in animals
 - ✓ Biomarker development
 - Bio-analytical method development
 - Pharmacokinetics/ pharmacodynamics, toxicology studies
 - IND filing
 - Proof-of-concept clinical trial

