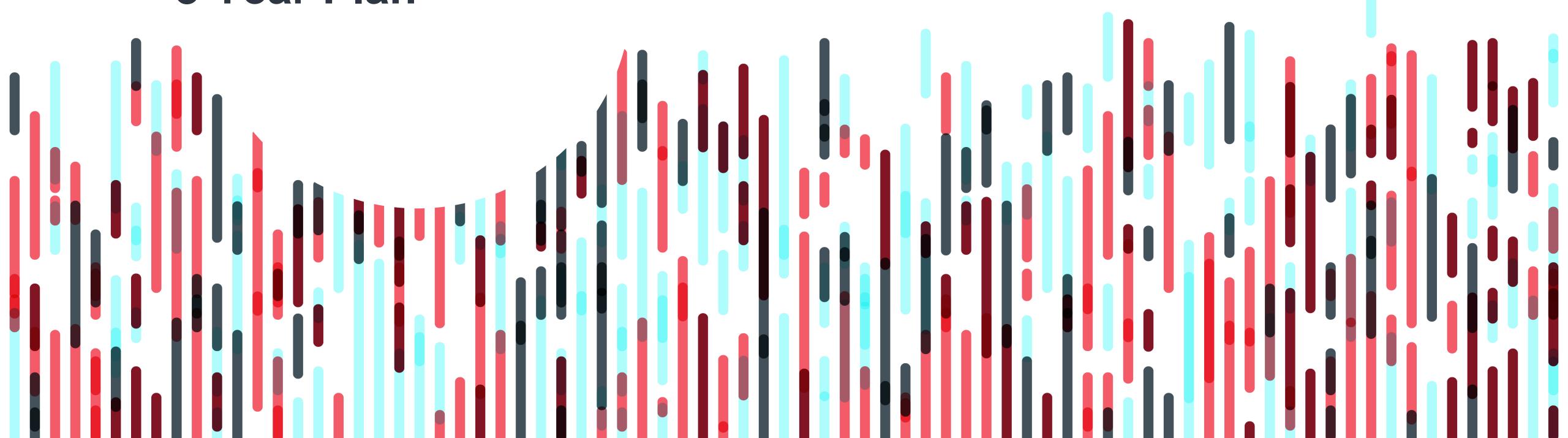
STXBP1 Fast Forward
3 Year Plan



# SIMPLICASU Edevisied

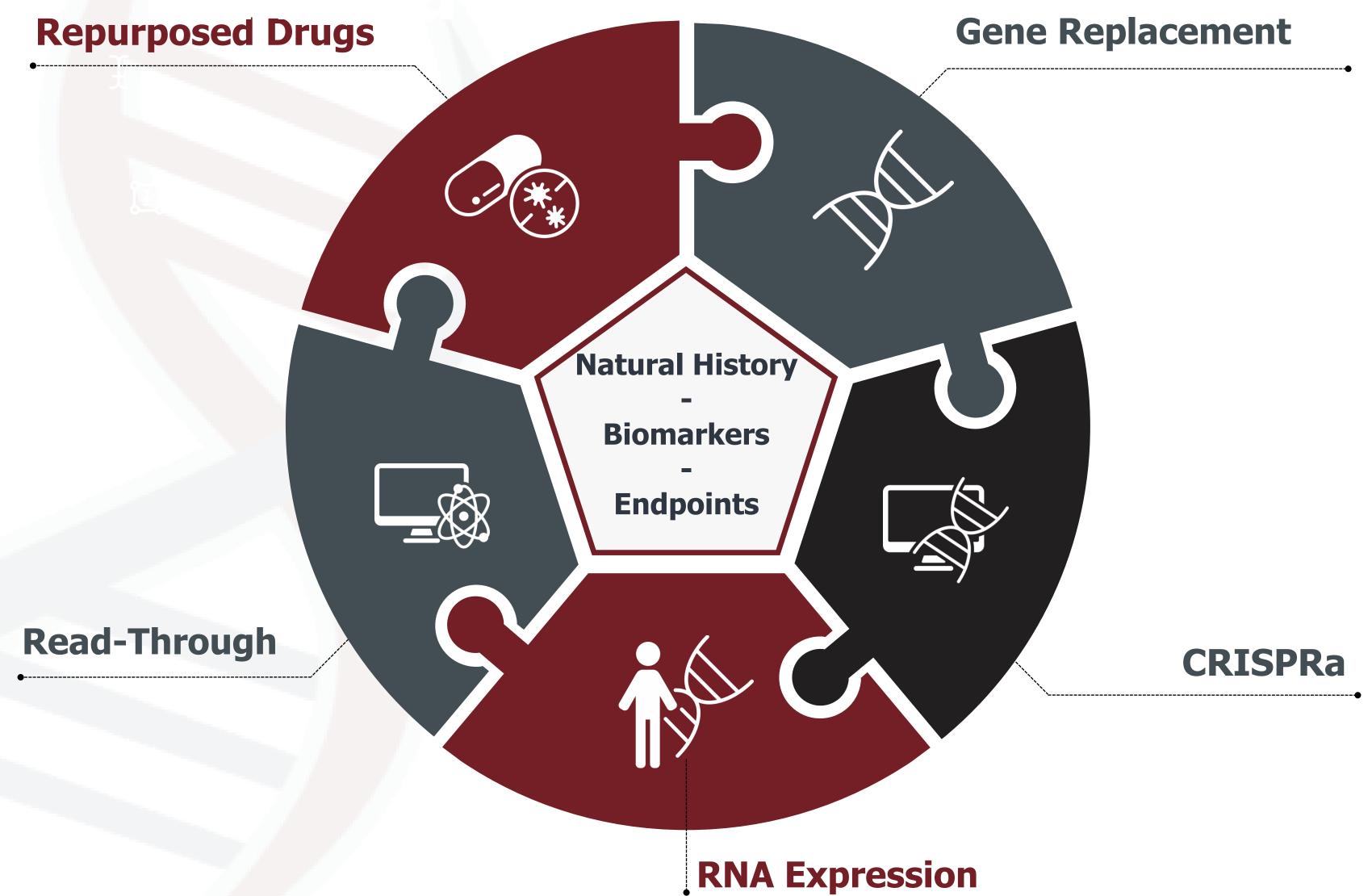
- Propel Precision Therapies for STXBP1
- Prepare for Clinical Trials
- Build Biopharma Company Interest
- Support Patients Today

https://www.stxbp1disorders.org/fast-forward



# SIABPII Fasu

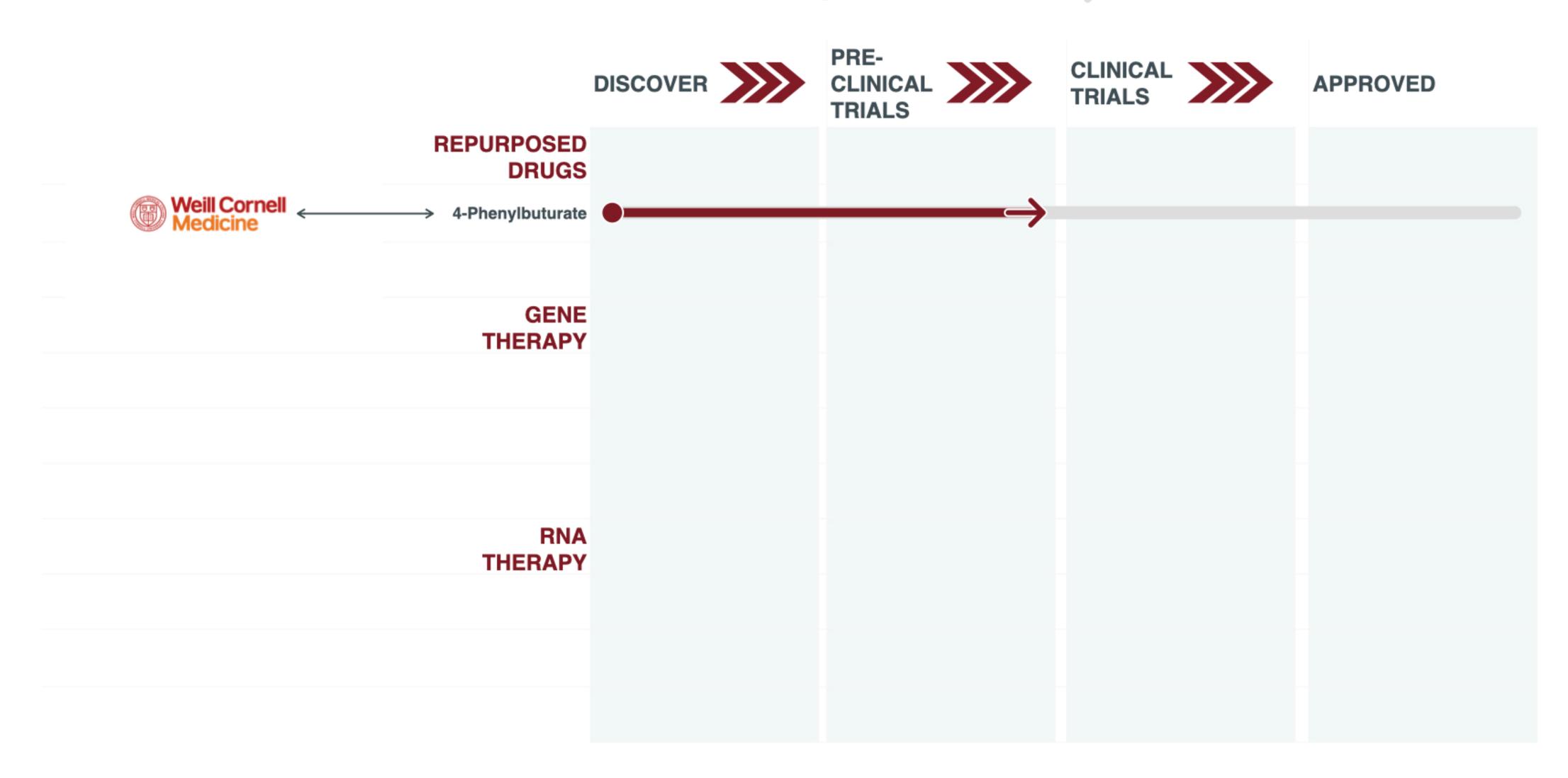
### FORWORD





### Therapeutic Pipeline 2019







#### **Therapeutic Pipeline 2022** where research started where we are in 2022 DISCOVER PRE-CLINICAL TRIALS **APPROVED** REPURPOSED / **SMALL MOLECULE DRUGS** Weill Cornell Medicine FDA-Approved Drug Screen Rarebase -FDA-Approved Drug Screen **GENE THERAPY AAV Gene** COMPANY A ← Replacement **AAV Gene** COMPANY B ← Replacement **AAV Gene** COMPANY C ← Replacement **RNA THERAPY** IONIS ₹ Penn Medicine ← Q-State Biosciences ← ASO VU VRIJE UNIVERSITEIT AMSTERDAM Università ← di Genova **SINEUP** Penn Medicine ← Arginine Read-Through

