

Portfolio Modeling of Single-Use Rare Disease Treatments

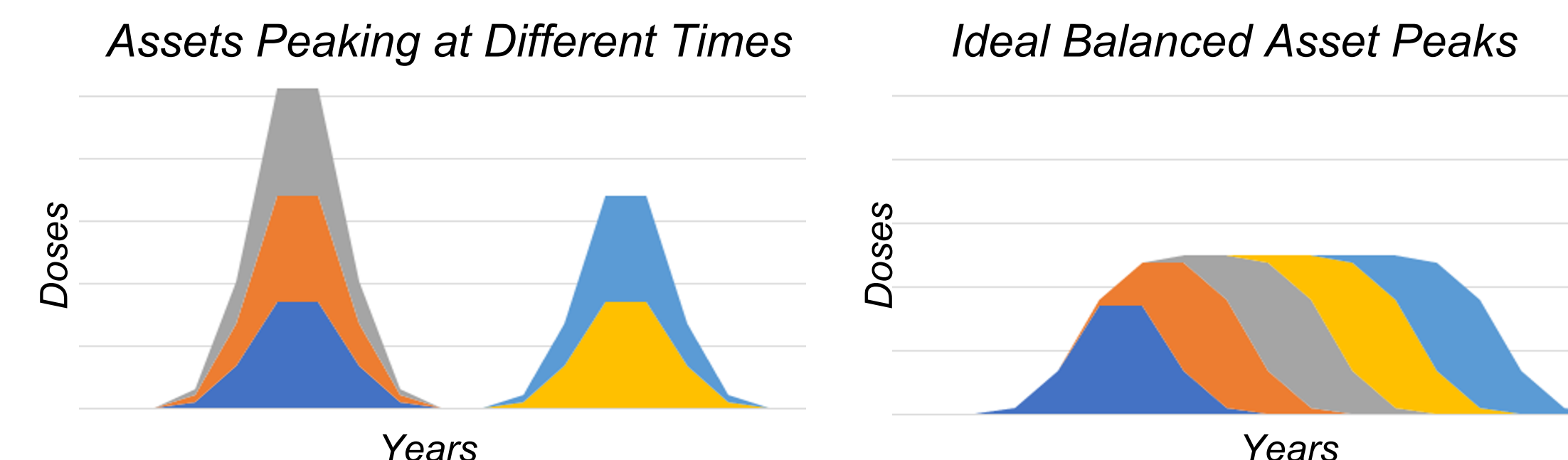
Problem Motivation / Background

- > Growing pipeline to serve small rare disease populations
 - Cellular and gene therapies (CGTs) are cutting-edge
 - Only 3 GTs approved by FDA to date
- > Single-use demand further narrows limited market size
- > Lack of commercial scale production capabilities
 - No current network infrastructure
 - Assets may utilize similar manufacturing



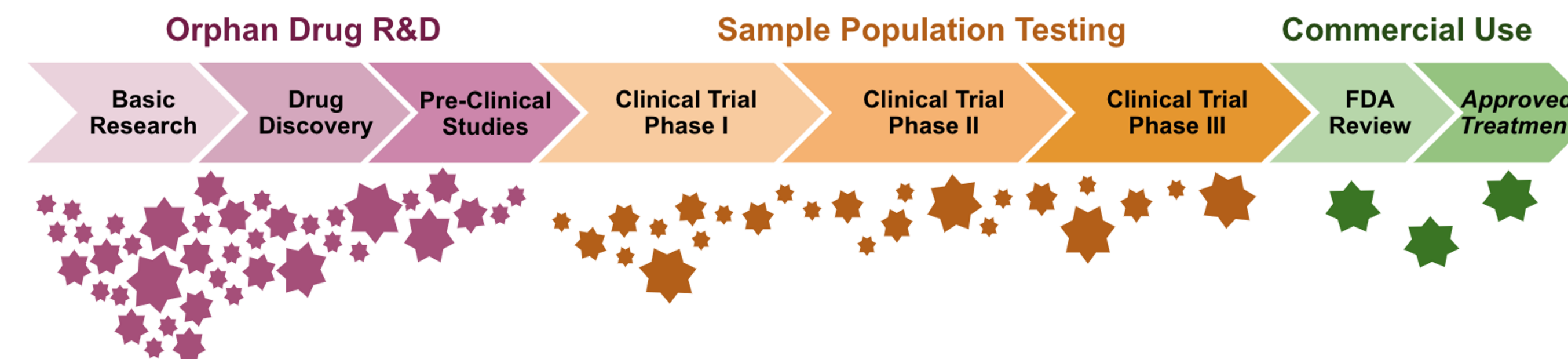
Key Question / Hypothesis

Can a forecasting model support Roche's strategic network development for sharing new supply chain capacity across an evolving portfolio of novel single-use gene therapies?



Relevant Literature

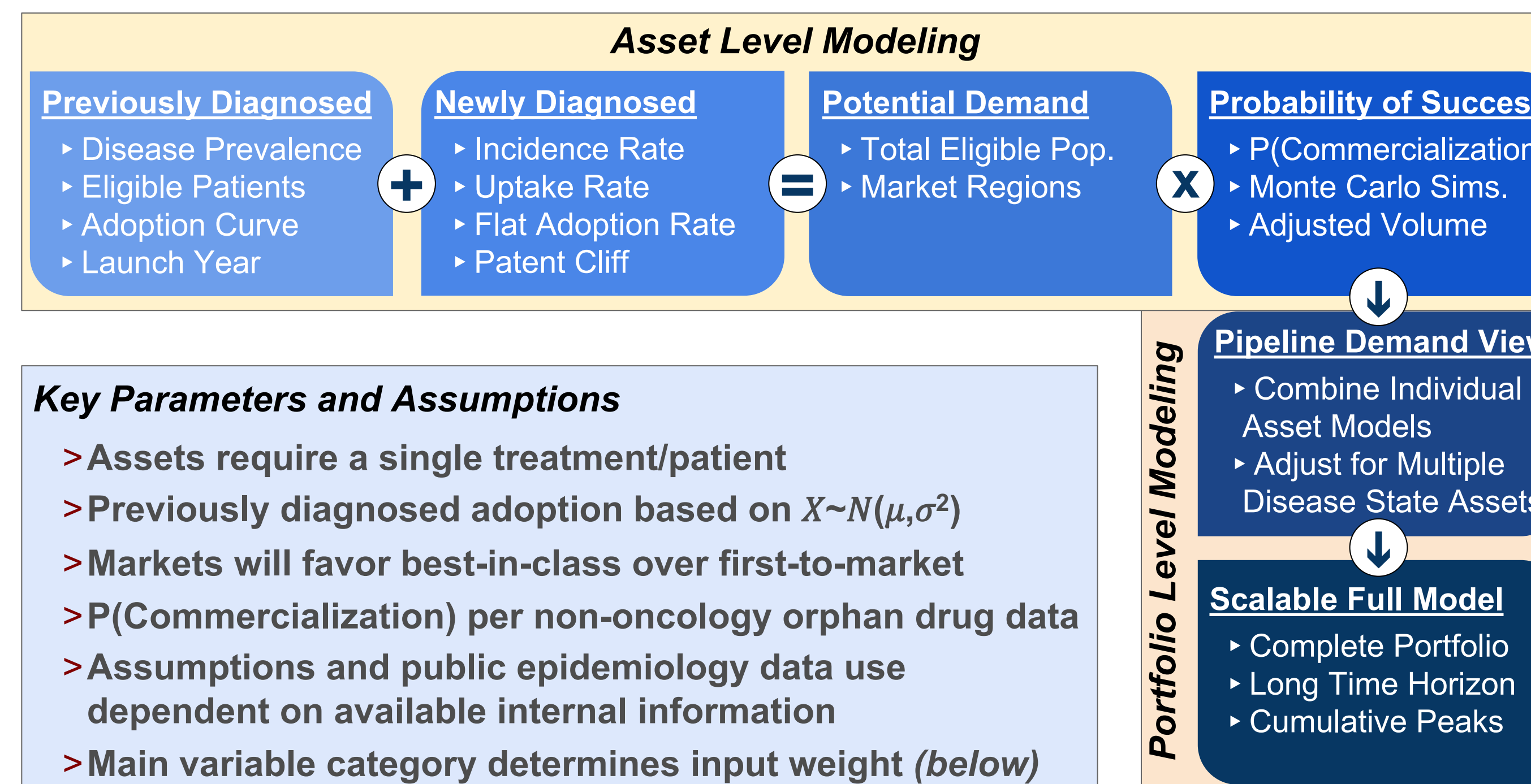
- 1) Cook, A. G. (2006). *Forecasting for the Pharmaceutical Industry: Models for New Product and In-market Forecasting and how to Use Them*. Gower. <https://books.google.com/books?id=5laddXKSNTkC>
- 2) Quinn, C., Young, C., Thomas, J., & Trusheim, M. (2019). Estimating the Clinical Pipeline of Cell and Gene Therapies and Their Potential Economic Impact on the US Healthcare System. *Value in Health*, 22(6), 621–626. <https://doi.org/10.1016/j.jval.2019.03.014>
- 3) Wong, C. H., Siah, K. W., & Lo, A. W. (2019). Estimation of clinical trial success rates and related parameters. *Biostatistics*, 20(2), 273–286. <https://doi.org/10.1093/biostatistics/kxx069>



Commercializing gene therapies impacts end-to-end supply chain functions

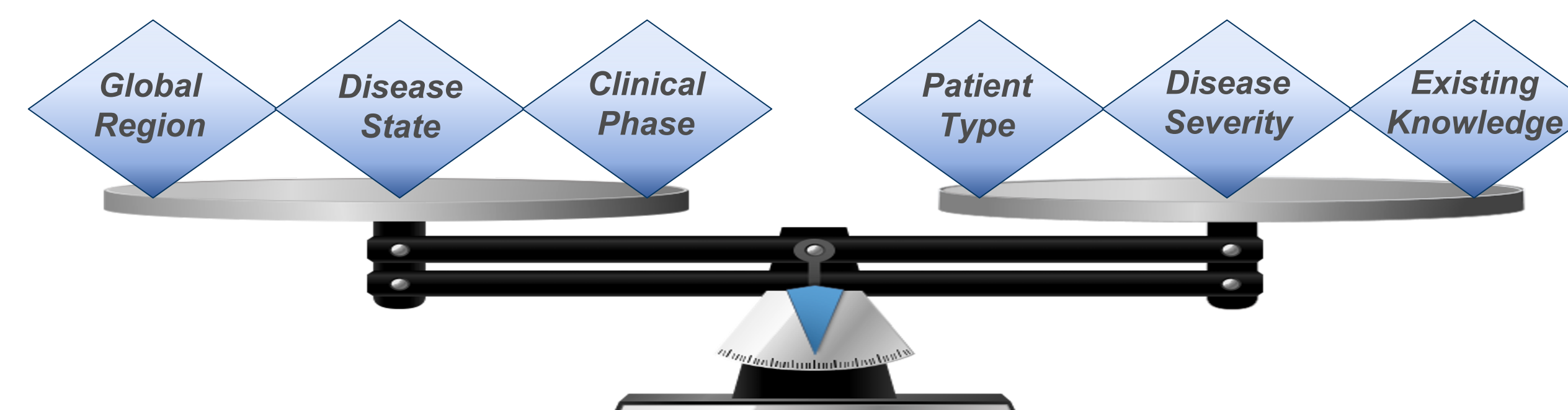
This immature, but radically developing niche market requires substantial investment to build a network that can accommodate the varying additional capacity long-term

Methodology



Key Parameters and Assumptions

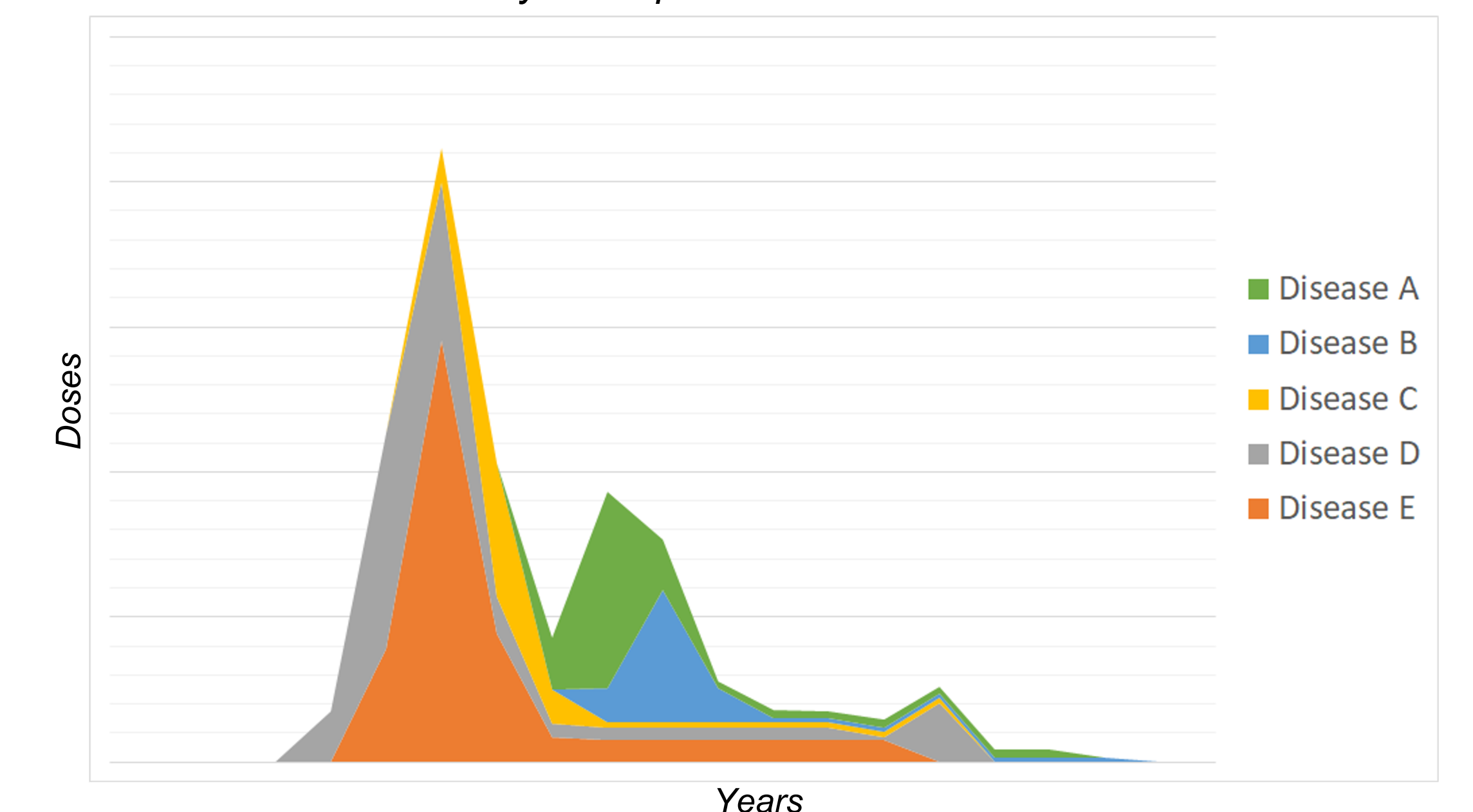
- > Assets require a single treatment/patient
- > Previously diagnosed adoption based on $X \sim N(\mu, \sigma^2)$
- > Markets will favor best-in-class over first-to-market
- > P(Commercialization) per non-oncology orphan drug data
- > Assumptions and public epidemiology data use dependent on available internal information
- > Main variable category determines input weight (below)



Initial Results

- > Toy model is highly sensitive to prevalence inputs
 - DMD: Competitive pressures adjust forecast down by 10x
 - Huntington's: Projected high adoption rates due to onset severity
 - Hemophilia A: Balancing high prevalence with lower disease impact

Preliminary GT Pipeline in Doses over Time



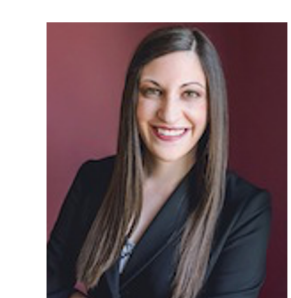
- > Point model helps shape refinement opportunities
 - Further asset level sensitivity points
 - Enhance previously diagnosed adoption curve
 - Address incorporation of future in-licensed/acquired assets

Expected Contribution

- > Demonstrate the portfolio effect and potential application
- > Useable, scalable model to support network design decisions
- > Conduct scenario planning exercise on projected volume



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