Dr. Jason Lott

Crystal Balls and Counterfactuals: Evidence Generation for Cell and Gene Therapies

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Course duration:
10:15 – 10:45 am

Jason Lott, MD is currently Vice-President, Integrated Evidence Generation, for Specialty Medicine and Cell-Gene Therapy at Bayer. He read Philosophy, Politics, and Economics at Brasenose College, Oxford University as a Marshall Scholar and received his medical degree at the University of Pennsylvania, followed by residency in dermatology and fellowships in the Robert Wood Johnson Foundation Clinical Scholars Program at Yale University and the NCI-sponsored Cancer Research Network. He is a current fellow of the American Academy of Dermatology.

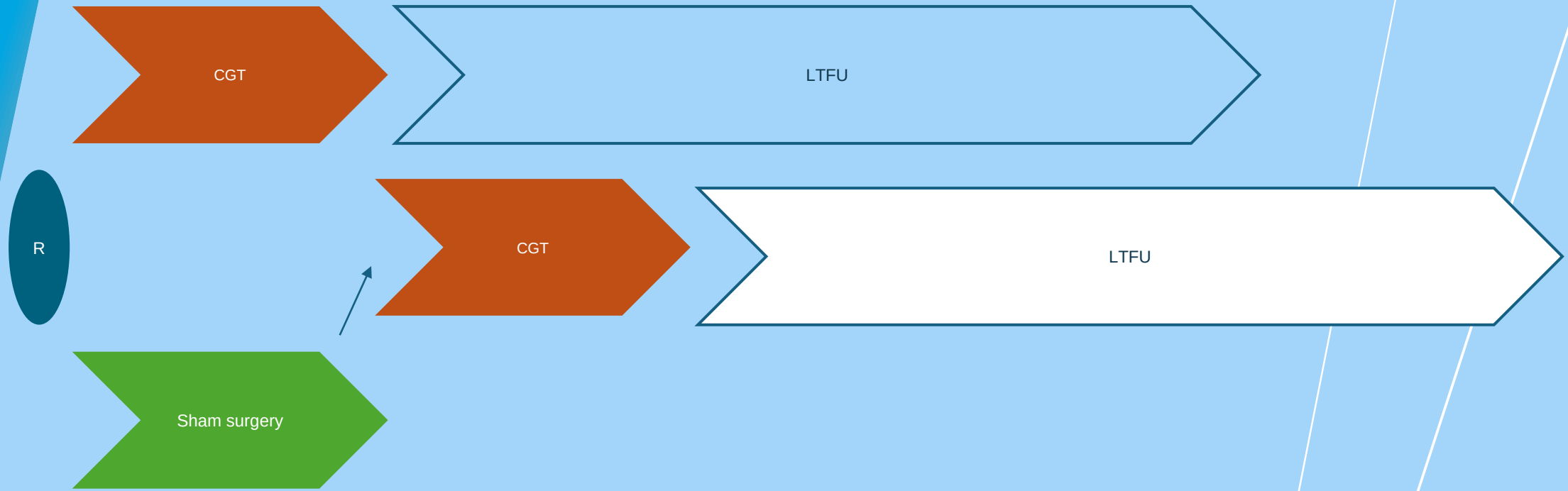
Crystal Balls and Counterfactuals: Evidence Generation for Cell and Gene Therapies

Presentation Abstract:

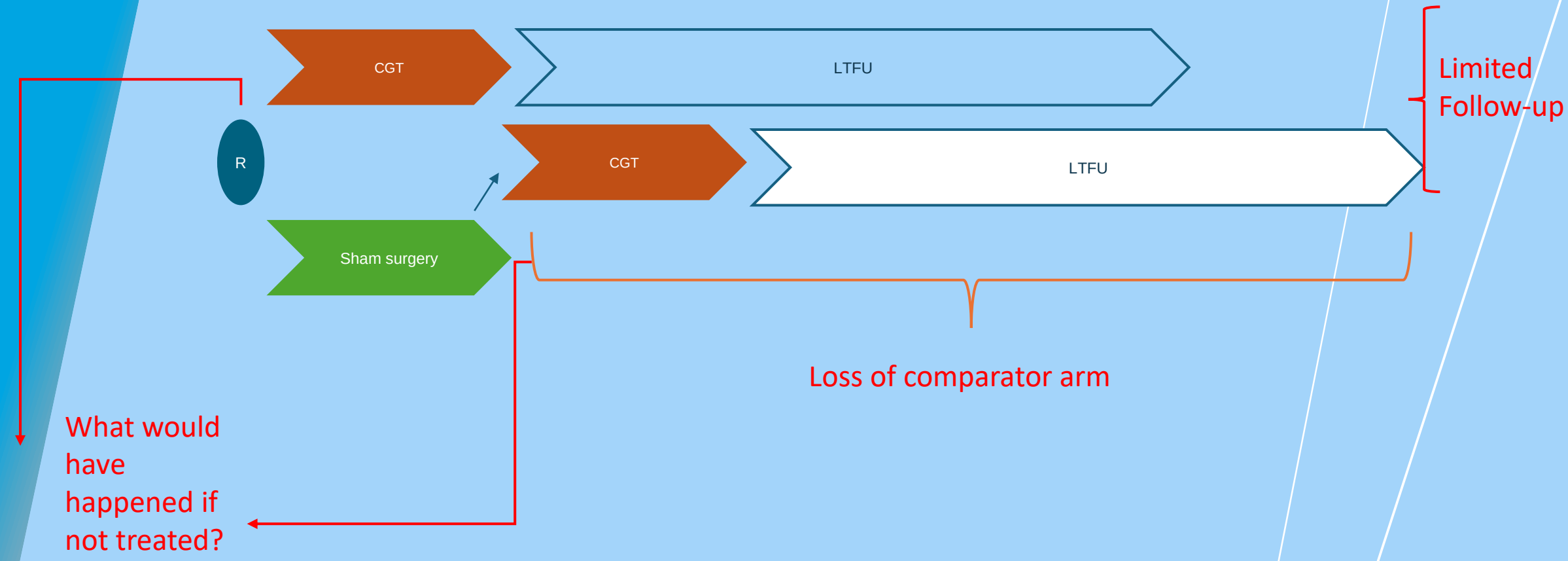
Evidence generation for cell and gene therapies require early and interdisciplinary approaches to enable successful development and commercialization. Specific challenges include refining target populations for non-rare diseases, understanding comparative efficacy in cross-over design trials, planning for long-term follow-up, and predicting durability of response. Robust evidence packages, inclusive of clinical and real-world data, will be needed to meet ever-growing demands of "value" demonstration for these innovative technologies.

Cell and Gene Therapies in Non-Rare Diseases: Challenges

- Loss of comparator arm
- Limited follow-up
  - Empiric measurement
  - Predicting durability of response
- Who should be treated?



R, Randomization; CGT, cell and gene therapy; LTFU, long-term follow-up



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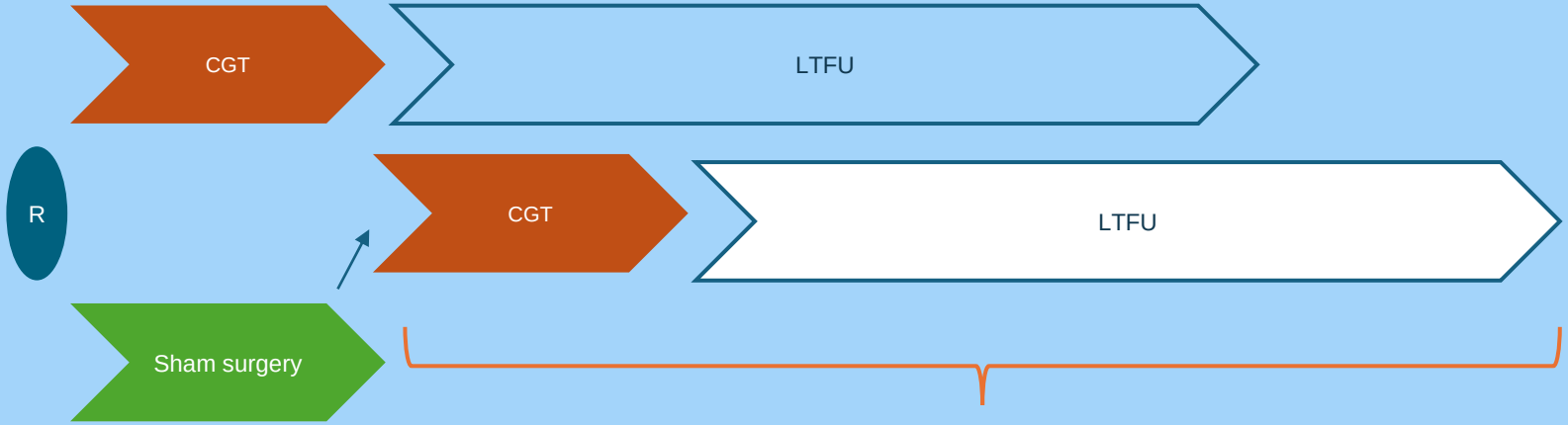


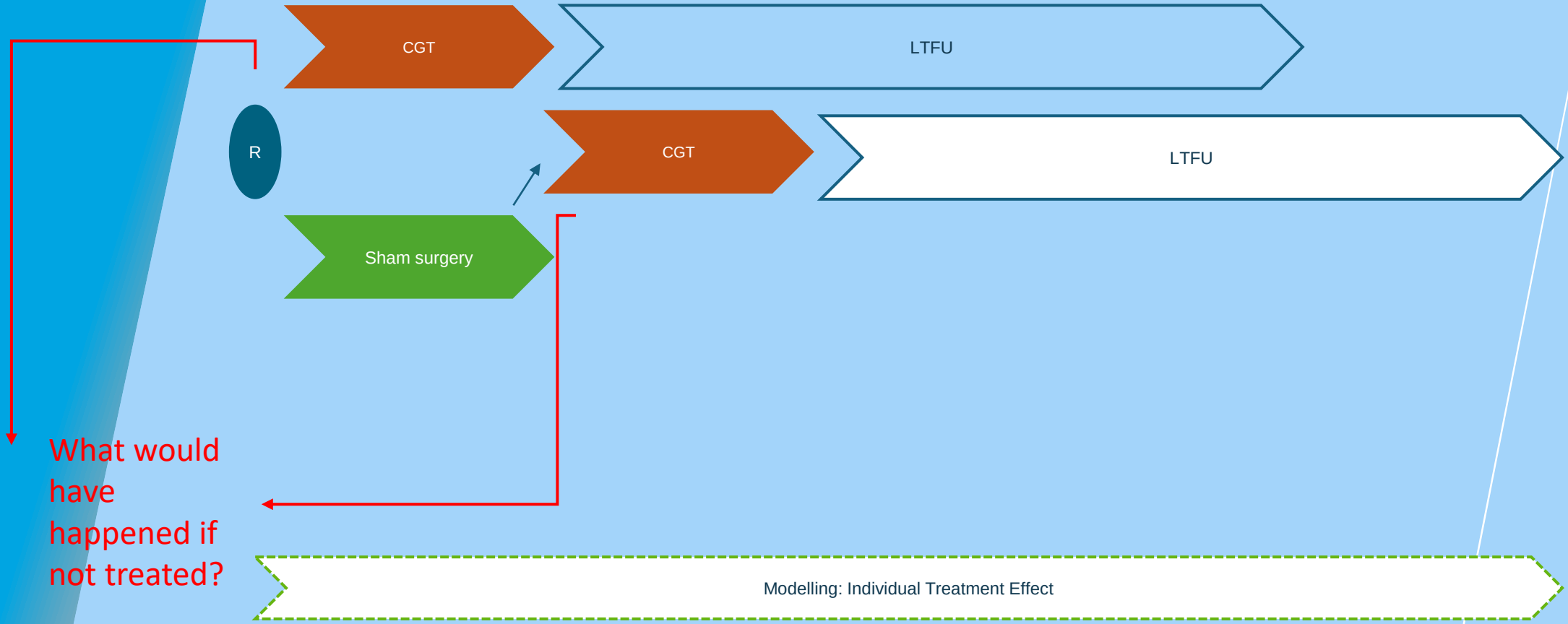
Prospective Natural History Study

Loss of comparator arm

Retrospective Cohort

Source for external control arm
Contextualize sham surgery placebo arm
Source for other external comparators
Source for predictive modeling/extrapolation

R, Randomization; CGT, cell and gene therapy; LTFU, long-term follow-up



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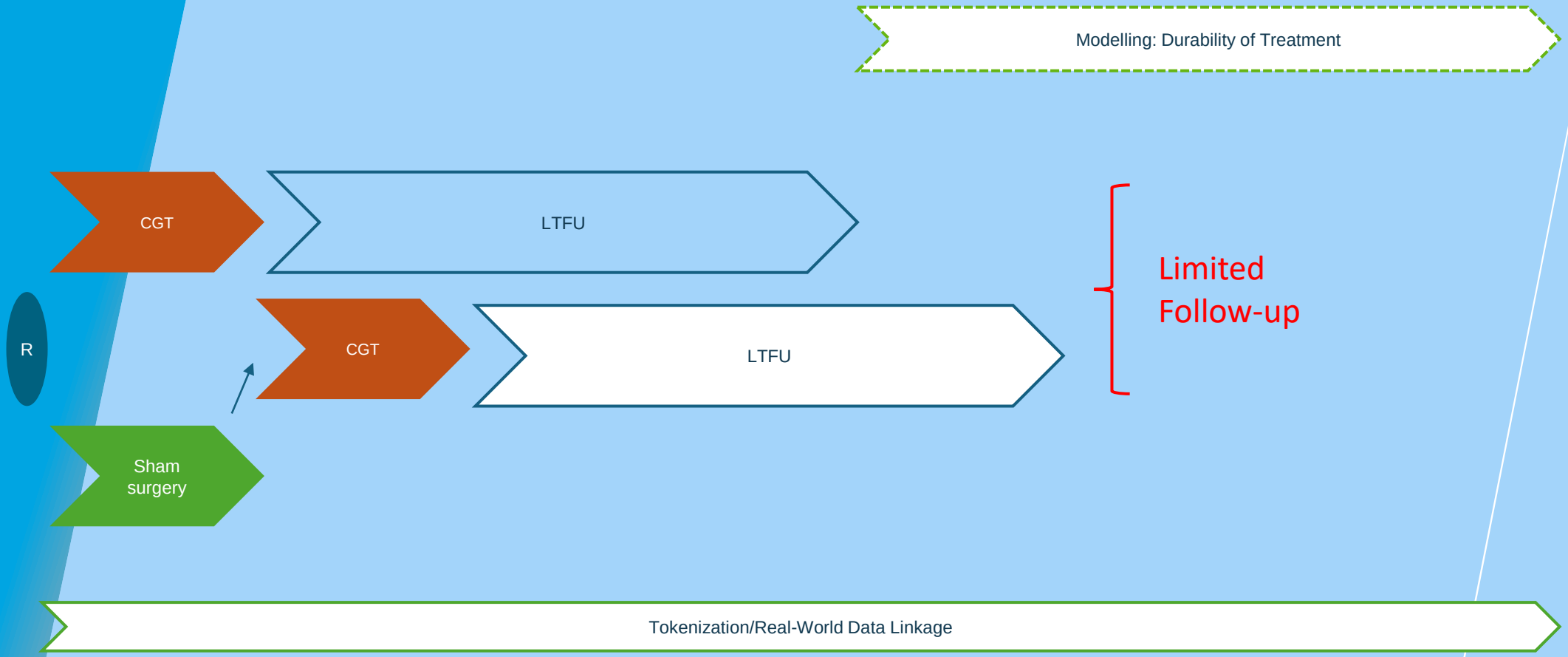

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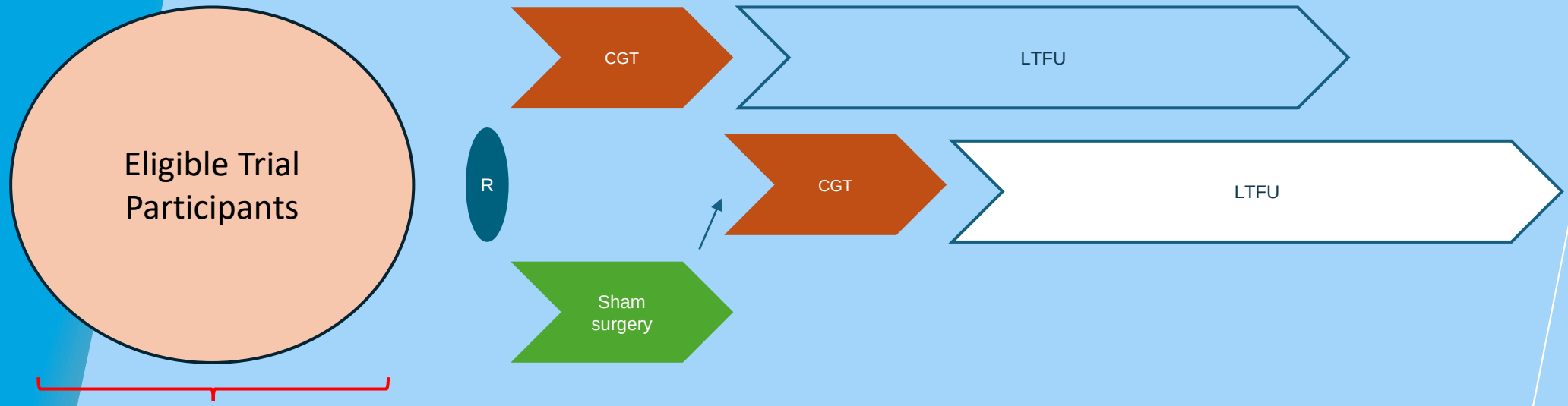
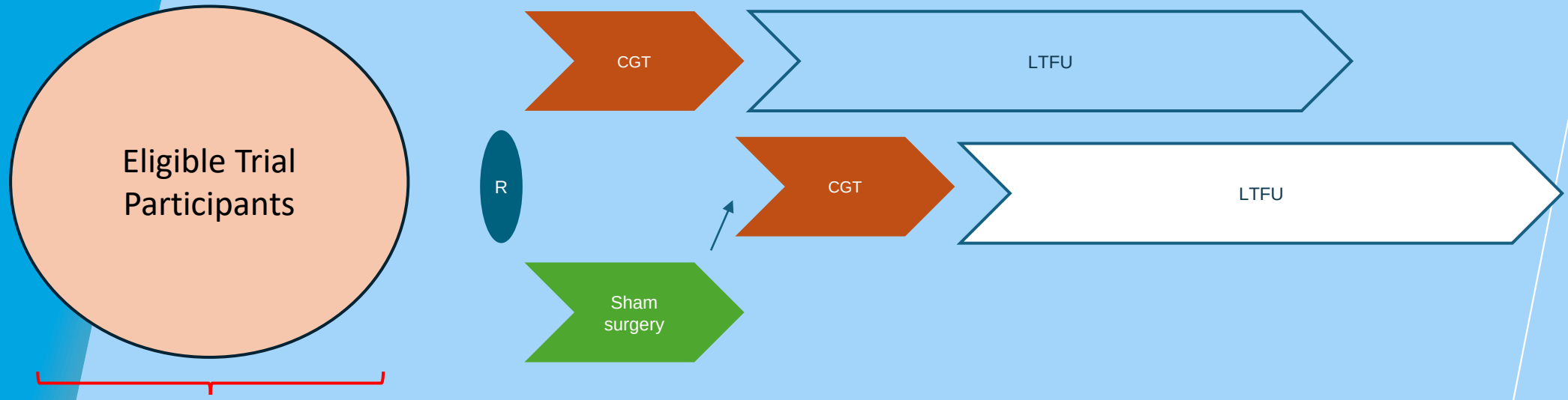


Large population

R, Randomization; CGT, cell and gene therapy; LTFU, long-term follow-up



Large population

Refined

Mapping/calibrating trial to RWD
RWD to identify “high value” patients
Claims-based algorithm development

R, Randomization; CGT, cell and gene therapy; LTFU, long-term follow-up

Cell and Gene Therapies in Non-Rare Diseases: Other Considerations

- Data sourcing
- Risk-sharing
- Adapting to payer needs

R, Randomization; CGT, cell and gene therapy; LTFU, long-term follow-up

Thank you.