



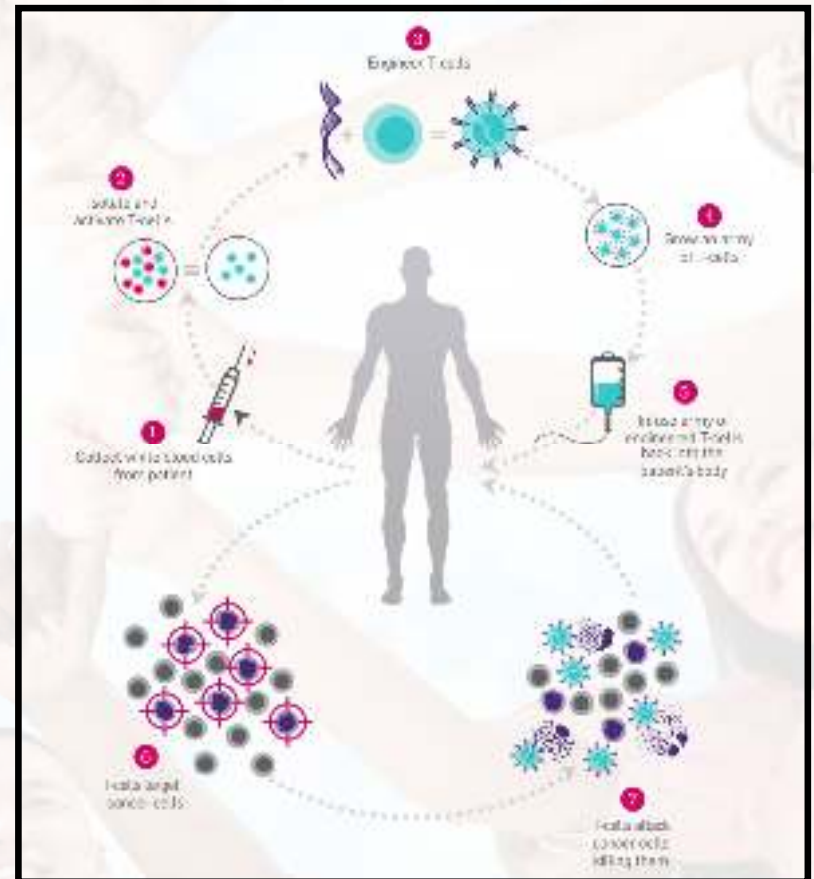
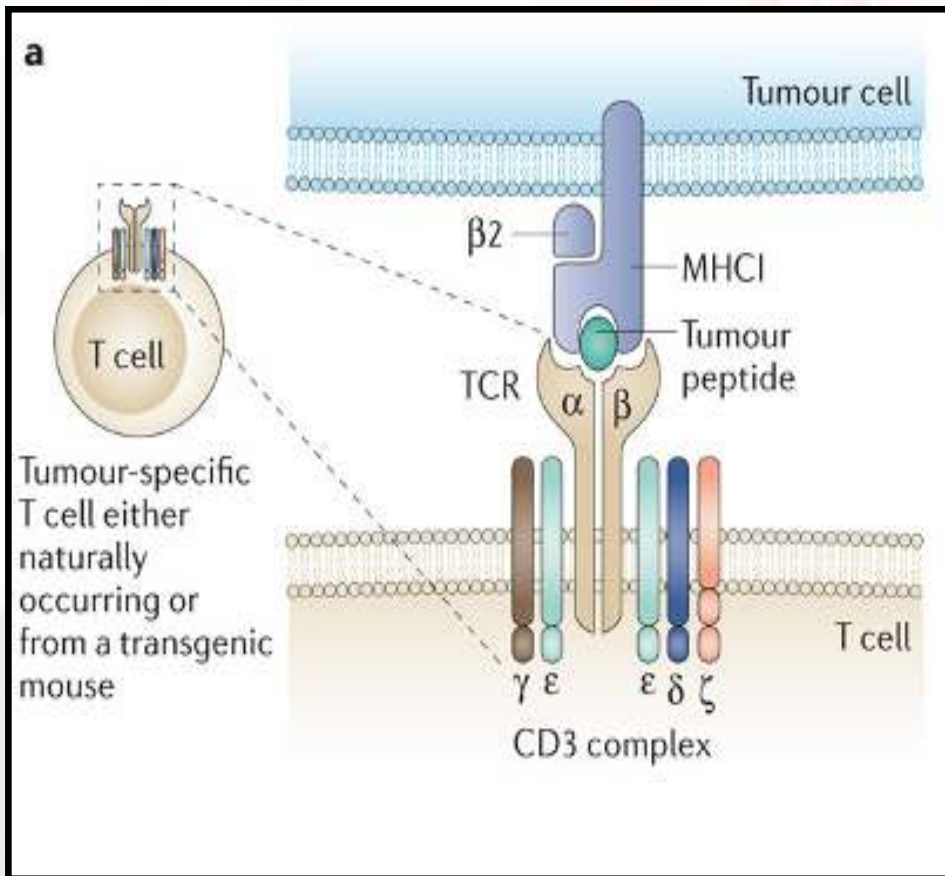
Reflection on CDDF Workshops 2021

# Gene and cell therapies

Jaap Verweij

Cancer Drug Development Forum

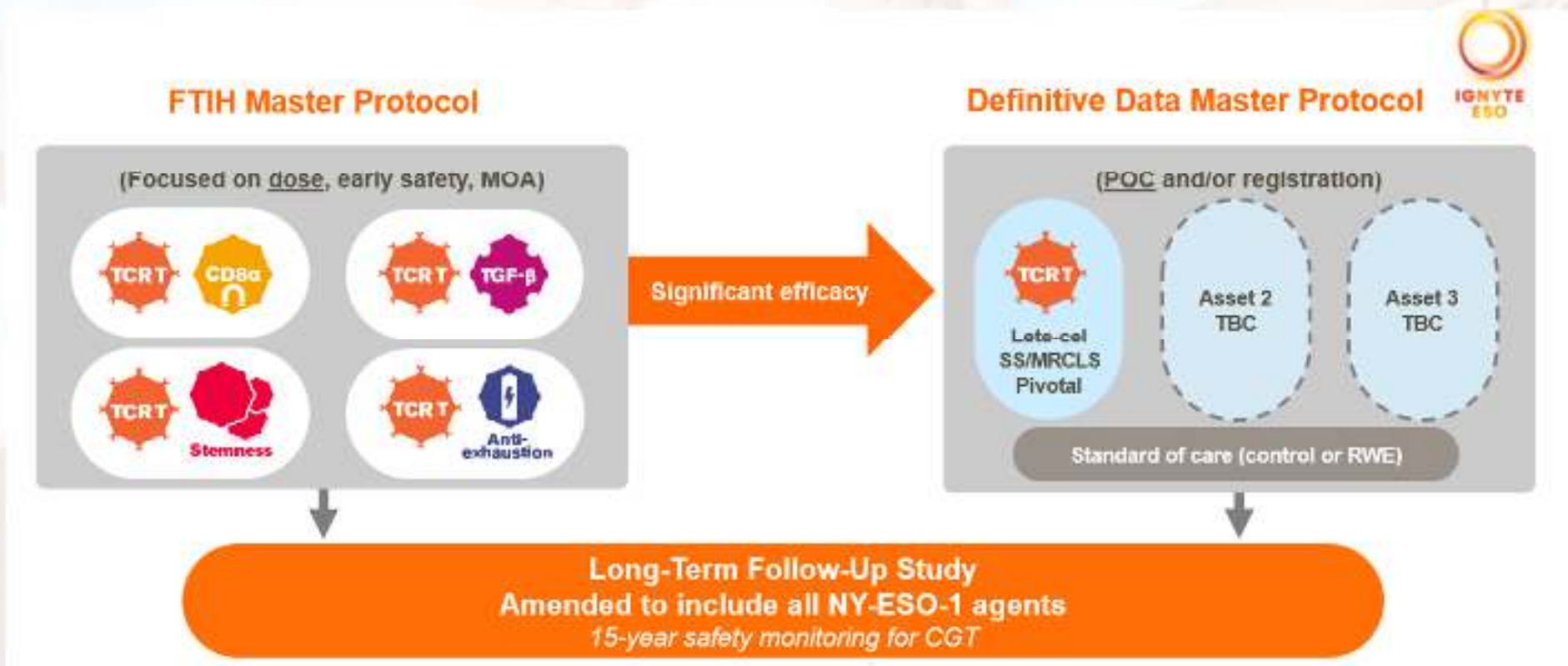
# TCR and CAR modified T cells as immunotherapy



Courtesy: prof. Emma Morris

Cancer Drug Development Forum

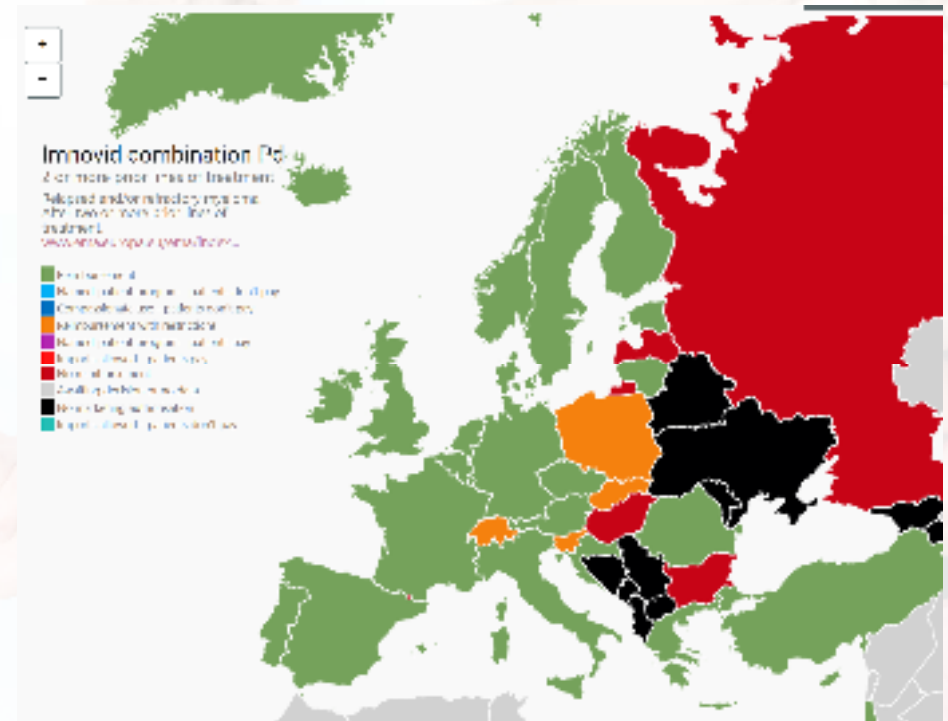
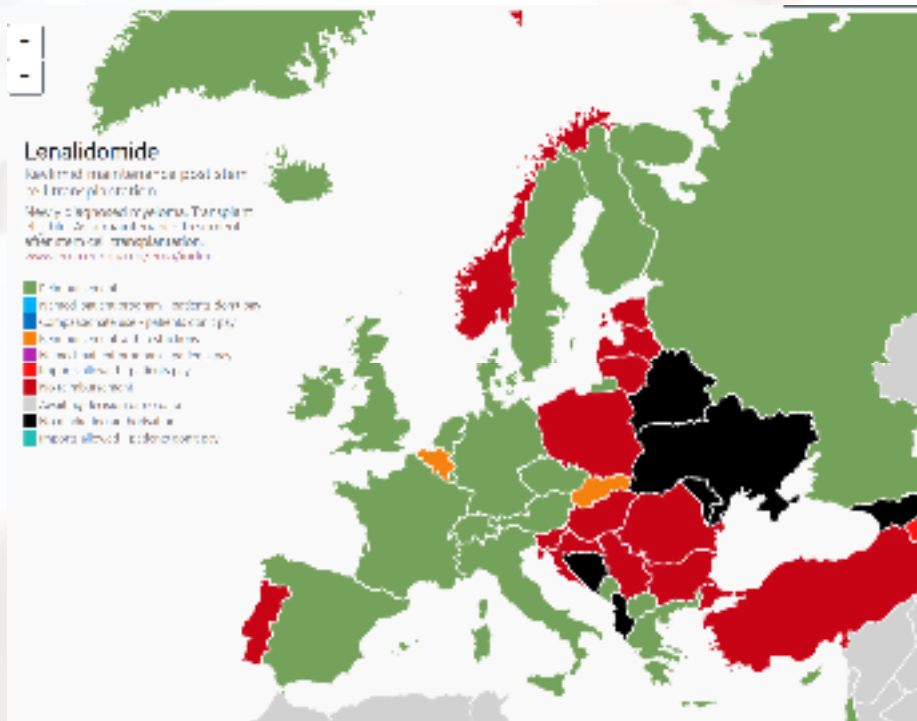
# Gene and Cell therapies: Clinical trial challenges



Courtesy: prof. Laura Pearce

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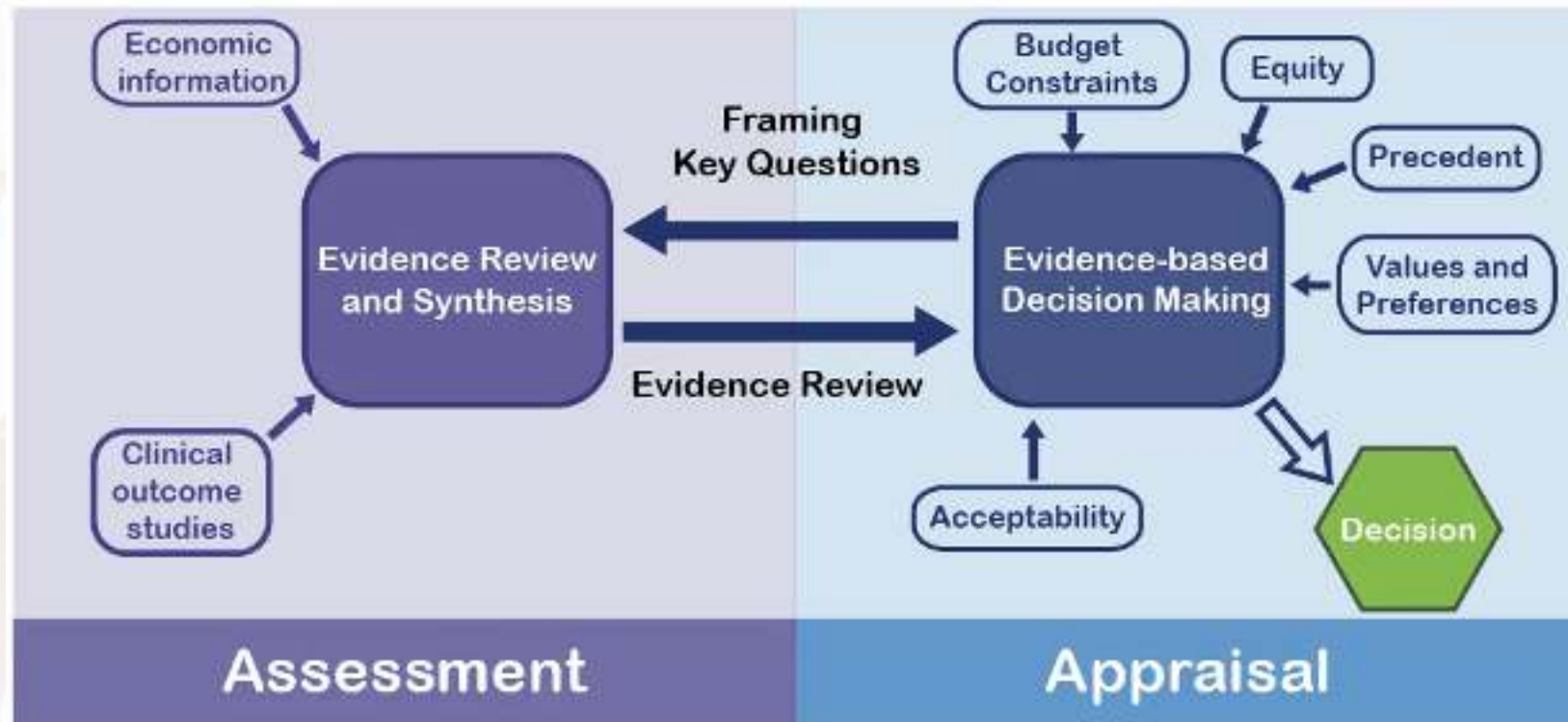
# Gene and cell cancer therapies: How to improve patient access



Courtesy: Kate Morgan

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# Gene and cell cancer therapies: How to improve patient access



Courtesy: prof. Carin Uyl

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# Gene and Cell therapies: Future Perspectives

## Internal Control

Not feasible because:

- Unethical to randomize
- Rare outcomes so impractical to randomize
- Patients unwilling to participate due to risk of not receiving drug

## Treatment Group



## External Comparator



- Patient cohort derived from real-world data (RWD)
- Patients match the inclusion / exclusion criteria for the trial
- Benchmark the efficacy data from the treatment group in a cohort of similar patients



# Gene and Cell Therapies: Key take-home messages

- Complex technology
- Manufacturing time-line challenges
- Active in hematological malignancies, early hints in solid tumors
- Regulatory paths activated
- Several options for improvement:
  - Master protocols for clinical trials
  - Use of RWD/RWE?
  - Use of AI in registries
  - HTA/patient access



Thank you for your attention