GRG Case Study: Effectiveness of tisagenlecleucel versus real-world standard of care in r/r FL with EMA review

Challenge

- To contextualize effectiveness of **tisagenlecleucel**, an autologous anti-CD19 CAR-T cell therapy, against physician's choice standard of care in **relapsed/refractory follicular lymphoma**, to support a submission to the **European Medical Association** (EMA) requesting a Type II variation to the marketing authorization.

Approach

- Genesis Research Group **collaborated with multiple stakeholders** including clinical development, statistics, RWE, and regulatory colleagues to develop a **target trial**.
- From the target trial, Genesis Research Group developed a **fit-for-purpose external control arm (ECA) study** for submission to EMA as part of a briefing book.
- After incorporating EMA feedback, **the ECA study was executed**, showing favorable effectiveness for tisagenlecleucel versus standard of care.

Impact/Solution

- The ECA study was **accepted as supportive evidence** for the submission to the EMA.
- The **EMA ultimately approved** the Type II variation to the marketing authorization allowing tisgenlecleucel to be used in r/r 2+ FL patients.

- **Published Article in *J Comp Eff Res*: link**
- **EPAR Assessment Report: link**

Hao Y, Hsu WC, Parzynski CS, Degtyarev E, Hampson LV, Masood A, Wu WH. Effectiveness of tisagenlecleucel versus real-world standard of care in relapsed/refractory follicular lymphoma. J Comp Eff Res. 2023 Jul;12(7):e220173.