Case study: trial design for an ECA in multiple myeloma

Challenge

A new drug is currently being investigated in a single arm study to evaluate its efficacy and safety in MM participants who are refractory to at least one PI, one IMiD, and one anti-CD38 mAb (i.e., triple class refractory, TCR).

- There is a need to generate evidence on the standard of care for patients with TCR MM, which could be done by creating an external control arm using Real World Data (RWD).

Solution

Utilizing Rapid Payer Response* (RPR) to obtain key KOL and payer insights on the appropriate study design elements required to create a robust external control arm, including addressing the following questions:

- What are the must have inclusion and exclusion criteria?
- What are the must have confounders (to ensure the appropriate adjustments are in place during the analytical phase)?
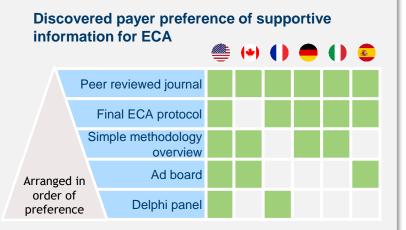
Key findings

Identified top-three best and worst practices for designing an ECA

👍
- Comparable cohort selection
- Similar data collection
- Benchmark to current SoC

👎
- Sparse data and dissimilarity of data analysis
- Patient population incomparable
- Relevance of treatments

Discovered payer preference of supportive information for ECA

Arranged in order of preference

	🇺🇸	🇨🇦	🇫🇷	🇩🇪	🇮🇹	🇪🇸
Peer reviewed journal	🟩	🟩	🟩	🟩	🟩	🟩
Final ECA protocol	🟩		🟩	🟩	🟩	🟩
Simple methodology overview		🟩		🟩	🟩	
Ad board	🟩					🟩
Delphi panel	🟩		🟩			

Identified and ranked top 10 inclusion criteria, exclusion criteria and confounding factors among KOLs and payers

Impact

Within three weeks, RPR was able to gather robust insights from Payers and KOLs, which is not available through a traditional research method, that allowed our clients to:

- Identify best and worst practices for designing an ECA
- Understand that payers and KOLs in different countries value the publication of final results in a peer-reviewed journal as the most crucial evidence. This shows that the ECA design and applicability are reliable and scientifically valid.

GENESIS RESEARCH GROUP

ECA: external control arms, MM: multiple myeloma, RPR: Rapid Payer Response - a Genesis Research Group survey tool to obtain dynamic payer feedback within a short time horizon; KOL, key opinion leader.