**Real-world data-based external controls for assessing long-term benefit-risk of cell and gene therapies: insights and perspectives**

**Background**

Cell and gene therapies (CGTs) are becoming increasingly important to the treatment landscape for rare, chronic, and debilitating genetic diseases. Evaluation of long-term safety and effectiveness of CGTs usually includes extension studies that follow patients for up to 15 years after clinical trial completion, as well as post-authorization safety studies of patients receiving CGT in routine clinical practice. However, long-term follow-up (LTFU) studies are often designed as non-interventional, single cohort studies, and comparative analyses contextualizing findings can be challenging. External controls using real-world data (RWD) are critical to understanding the long-term benefit-risk of CGTs.

**Objectives**

The objective of the symposia will be to highlight the opportunities and challenges in the design and conduct of LTFU studies for CGT, discussing the solution of external controls using RWD for comparative research.

Presentations will highlight perspective and/or experience from several stakeholders on practical, operational and methodological specificities of RWD-based external controls applicable to LTFU studies for CGTs.

**Description**

The proposed structure of the symposium is as follows (all speakers are confirmed):

|  |  |  |
| --- | --- | --- |
| Time allotment | Topic | Potential Speakers |
| 10mn | Opening - Welcome introduction on benefit-risk and potential long-term clinical consequences of CGTs | Moderators:  Katie Miller (Genentech)  Seun Osundolire (Pfizer) |
| 10mn | Patient perspective on long-term monitoring of CGTs: constraints, opportunities for tailored patient-centered approaches | Reid D’Amico (IQVIA, CGT SIG member) |
| 10mn | Regulatory considerations on external controls for comparative LTFU studies for CGTs | L.T. (Lourens) Bloem (Utretch University) |
| 10mn | Design and conduct of external comparators, special challenges in rare disease populations for CGT LTFU studies | Reese Sy (Pfizer) |
| 10mn | Identifying fit-for-purpose RWD for external controls in CGT LTFU studies | Hannah Furby (Roche) |
| 10mn | Feasibility of assessing comparative long-term benefit of CGTs using RWD-based external control arm | CY Vossen (Syneos Health) |
| 10mn | Statistical considerations for the LTFU of gene therapy trial participants: Platform LTFU trials for real-world comparative research of CGTs | Avery McIntosh (Pfizer) |
| 20mn | Open discussion | Q&A |