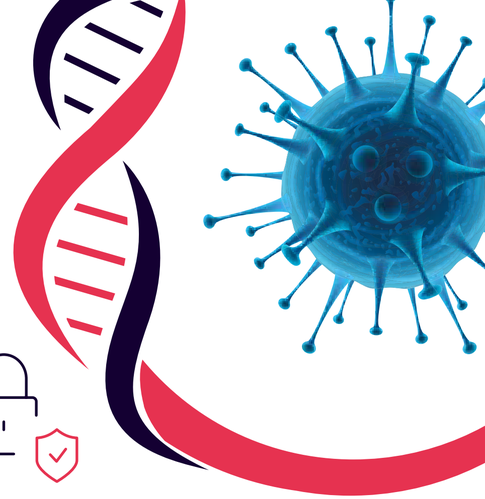
The 5th Annual Gene Therapy for Rare Disorders Congress



Across 4 days in March, the emotive team were excited to join delegates from innovative biotechs, large pharmaceutical companies and key service providers in Boston, MA, to discuss how to overcome the challenges with bringing novel gene therapy into the forefront of clinical practice.

To ensure the full breadth of this field was covered, the sessions were divided into several streams relating to specific challenges, from clinical development to market access. Take a look below for a summary of the key insights gathered throughout the sessions.

Here are our key takeaways:

The meeting began with a recap of the progress made in the gene therapy space to date. There is record-breaking investment in the field, with over 130 gene therapy trials in rare diseases currently in place. Building on strong results in 2021, nine cell and gene therapy BLA/MMA submissions have been placed across Europe and the United States in 2022 so far.

Key challenges for the industry, however, persist:

- Limitations of current manufacturing platforms that restrict gene therapy production
- Leveraging of non-clinical and manufacturing data from one application to another
- Use of complex and innovative clinical trial designs and advanced planning for clinical trials transitioning from Phase 1 to pivotal trials
- Product access and effectively engaging patients with rare diseases

Finally, engagement tips were shared from the patient perspective. It is important to communicate with patients early and regularly, and when looking at the disease landscape, support any PAG or foundation priorities and strive for stories that cover the broad spectrum of disease.

Clinical stream:

It is critical to optimise patient identification and engagement early in the clinical process to ensure meaningful endpoints are developed and to improve access to studies in the rare disease community.

To streamline this process, four key pillars were highlighted:

- Improving patient and healthcare provider education to support informed decision making
- Building comprehensive, multi-pronged, multi-channel targeting strategies
- Emphasising roles and responsibilities to ensure alignment across stakeholders
- Leveraging the first-hand experience of PAGs to raise awareness and increase access to studies in the rare disease community

Regulatory stream:

Presenters shared their experiences and case studies when seeking regulatory approval in the US and EU. Some of the key takeaways included:

- Knowing when to engage with agencies, and with what amount of data
- Ensuring good communication with agencies, including taking advantage of opportunities for collaborative meetings with the FDA and EMA
- Understanding the differences between regulatory designations and their requirements

Market Access stream:

Presenters outlined the barriers faced in the reimbursement of gene therapies in rare diseases, sharing examples of what strategies are working for approved therapies so far. Some of the key discussion points included:

- The significant cost of gene therapies, and the 'one-and-done' concept behind them which poses a new challenge for payers. In some instances, some gene therapies may need to be readministered which may add further strain on healthcare bodies
- How payers can accommodate more gene therapy approvals in the coming years
- Value demonstration challenges for potentially curative therapies in chronic diseases with a history of limited treatment options
- The requirement of investment in real-world evidence collection to elucidate the unmet need and disease burden associated with rare diseases

Collaboration across all stakeholders in the industry is heralded as a key driving force to unlocking the full potential of gene therapy. Throughout the congress, fostering connections and building networks was encouraged among the attendees to act as kindling for the start of new relationships across the industry.

Abbreviations:
BLA, biologics licence application; EMA, European Medicines Agency; FDA, Food and Drug Administration; MAA, marketing authorisation application; PAG, patient advocacy group.

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