Enhancing CMC Regulatory Efficiency in Gene Therapy

The Perspective of Viralgen's Regulatory Team

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Abstract

This study explores the evolving landscape of regulatory affairs in gene therapy from Viralgen's perspective, a leading Contract Development and Manufacturing Organization (CDMO) specializing in viral vector production. Viralgen addresses the complex and dynamic regulatory environment, particularly the challenges in Chemistry, Manufacturing, and Controls (CMC) support for gene therapy products. The study highlights the issues of regulatory compliance, inconsistent guidelines, and the lack of specific regulatory guidance for gene therapy. Viralgen's solutions include a streamlined production platform process, comprehensive CMC support, and the implementation of digital solutions such as Regulatory Information Management (RIM) systems and Health Authority feedback databases. The effectiveness of these solutions is demonstrated by Viralgen's successful regulatory interactions and the approval of numerous clinical trials. The study concludes by discussing future trends in gene therapy regulation, emphasizing faster development pathways, global regulatory alignment, and the necessity for adaptable regulatory approaches to keep pace with scientific advancements.

Current Challenges

Lack of specific guidances[1,2]

The existing requirements are often general and not tailored to this innovative field

High regulatory expectations

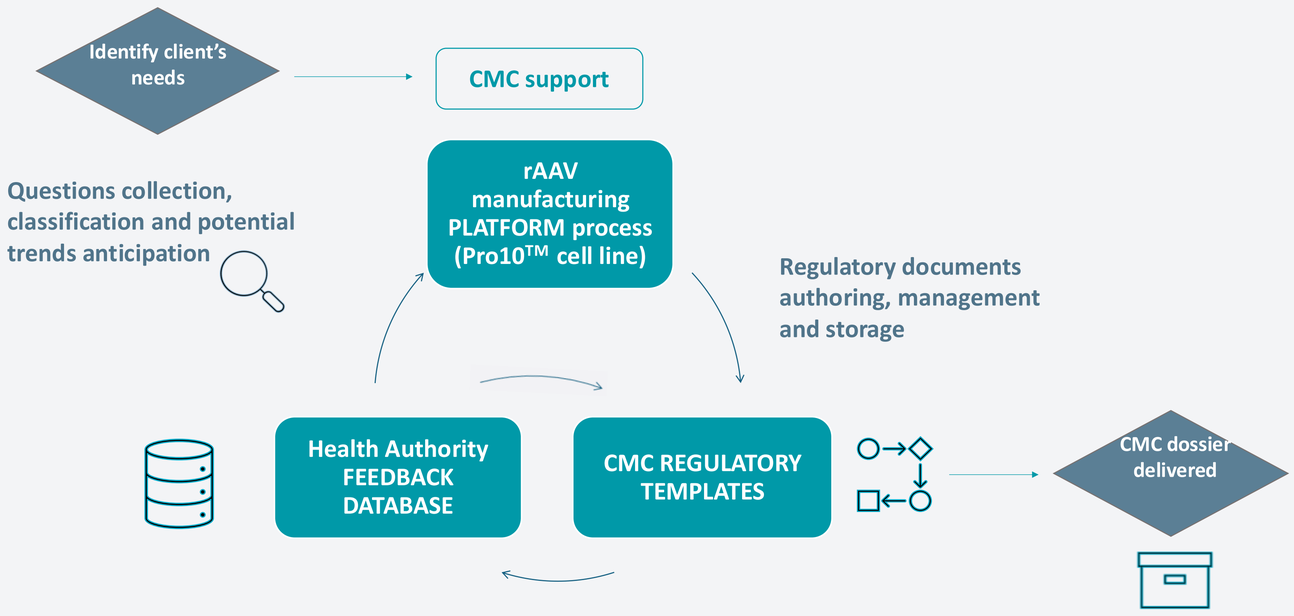
Increased complexity of the submission and approval processes, while HA expectations develop faster than associated guidelines

Inconsistencies and lack of alignment between Global Agencies

Lack of harmonization in regulations across different regions

Viralgen's Solutions

Streamlined process: Viralgen manufacturing platform and regulatory interactions



Viralgen's platform has enabled the creation of **CMC templates compliant with regulatory standards** in the EU, US, UK, and CA, expediting the documentation process and reducing turnaround times. By incorporating feedback from regulatory agencies, Viralgen **continually enhances** and updates its CMC templates to meet evolving requirements. The interaction has been enhanced through digitalization and Regulatory Information Management (RIM) systems implementation.

Regulatory CMC services over drug development

- pIND/Briefing package writing.
- Summary of DS/DP Manufacture and control.
- Support for pIND meeting/Scientific Advice with Health Authorities.

- CMC package writing for initial IND/CTA
- Response to Questions from Health Authorities (related to CMC).
- Commitment to meet Health Authority deadlines.
- CMC writing for amendments.
- Support for Health Authority meetings/Scientific Advices.

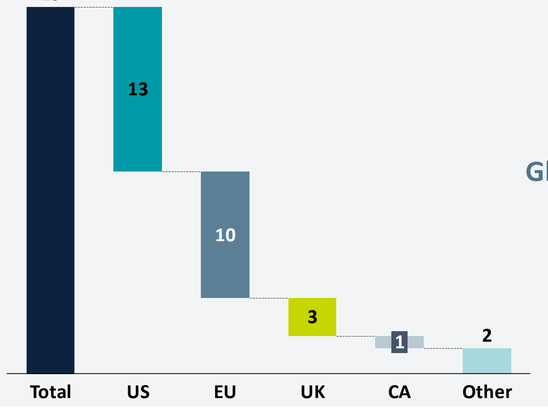
- CMC package writing for BLA/MAA
- Response to Questions from Health Authorities (related to CMC).
- Commitment to meet Health Authority deadlines.
- CMC writing for post approval variations.

Pre-Investigational New Drug application (pIND); Drug Substance (DS); Drug Product (DP); Chemistry, Manufacturing and Control (CMC); Investigational New Drug application (IND); Clinical Trial Application (CTA); European Medicines Agency (EMA); U.S. Food and Drug Administration (FDA); Health Canada (HC); Medicines and Healthcare products Regulatory Agency (MHRA); Biologics License Applications (BLA); Marketing Authorization Applications (MAA).

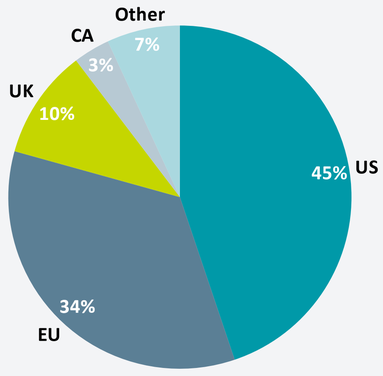
Results

100% CMC submissions accepted by Health Authorities

29 on-going Clinical Trials with material produced at Viralgen, in multiple jurisdictions



Global IND/IMPD submissions authorized



Future Outlook

Accelerating Approval Pathways

Reducing time from Discovery to patient access

Bridging the Science-Regulation Gap

Adapting regulations to keep pace with scientific advances

Utilizing a Platform Approach[4,5]

Adopting standardized development platforms and selective process characterization will expedite approvals

Tailored Regulatory Approaches

Flexible regulations tailored to gene therapy challenges

Preparing for Success from the Beginning

Early planning and robust data collection essential to navigating the regulatory process smoothly

Enhancing Manufacturing Efficiency

Faster and scalable production processes

Global Regulatory Harmonization

Facilitating global market access through unified standards

Data Generated can be Leveraged[3]

Utilizing data effectively can provide valuable insights and streamline the regulatory process

Increasing Accesibility and Affordability

Lowering development costs to make treatments more accesible

Conclusion

As the gene therapy field continues to evolve, the importance of regulatory expertise cannot be overstated. The intricate and fast-paced nature of regulatory frameworks, combined with the specific challenges of Chemistry, Manufacturing, and Controls (CMC) in gene therapy, demands a strategic and proactive approach. Viralgen's Regulatory Affairs team offers comprehensive support that spans the entire product lifecycle, from early planning to market entry, ensuring compliance while **accelerating timelines**.

Our platform-based manufacturing process, combined with deep regulatory knowledge and experience, provides a **streamlined solution** for clients navigating complex regulatory requirements. This approach not only reduces risks and development costs but also enhances product quality and safety, ultimately benefiting both developers and patients.

Looking to the future, the key to regulatory success will lie in flexibility, early planning, and global alignment. By continuing to foster strong collaborations with Health Authorities, leveraging digital solutions, and staying at the forefront of regulatory developments, **Viralgen is well-positioned to help its clients bring life-changing therapies to patients more efficiently and effectively.**

References

[1]FDA Guidance for Industry. Chemistry, Manufacturing, and Control (CMC) Information for Human Gene Therapy Investigational New Drug Applications (INDs) (2020, January).

[2]European Medicines Agency. Draft Guideline on quality, non-clinical and clinical requirements for investigational advanced therapy medicinal products in clinical trials (EMA/CAT/123573/2024) (2024, March).

[3]BioPhorum Operations Group. Leveraging platform and process characterization data to accelerate CGT validation and commercialization (2024, April).

[4]FDA Guidance for Industry. Draft guidance. Advanced Manufacturing Technologies Designation Program (2023, December).

[5]FDA Guidance for Industry. Draft guidance. Platform Technology Designation Program for Drug Development (2024, May).

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