

Strategic Support for Gene Therapy Clinical Development Planning



The Situation

A global clinical research organisation engaged DLRC to provide strategic regulatory support for one of their biotech clients developing a gene therapy programme targeting a rare paediatric neurological condition.

The client sought expert regulatory guidance to accelerate the clinical development of a gene therapy programme targeting a rare paediatric indication. With multiple regulatory pathways and country-specific requirements to navigate, they needed strategic input to optimise timelines and also ensure compliance across the EU, UK, and US.

DLRC's Solution

DLRC facilitated a strategic workshop to explore analogues in gene therapy development as well as identify regulatory acceleration opportunities. The session brought together cross-functional experts to evaluate:

- GMO classification and timelines across key EU Member States
- Expedited pathways including RMAT, RDEA, START, and PRIME
- Paediatric regulatory integration, including PIP planning and Article 32 CTR considerations
- Orphan Drug Designation strategy and maintenance planning
- EU CTR and CTIS submission risks and mitigation strategies

DLRC also provided tailored insights on regulatory expectations for ATMPs, ethics committee interactions, and dossier readiness.

The Outcome

Successful delivery of the workshop and strategic roadmap, with positive feedback from both the clinical research organisation and their biotech client.

- A comprehensive roadmap outlining regulatory touchpoints as well as timelines for Phase 2 and Phase 3 development
- Clarified strategy for orphan designation and paediatric compliance
- Identification of country-specific GMO submission requirements and timelines
- Recommendations for leveraging expedited pathways across regions
- Enhanced client readiness for regulatory engagement and CTA submissions

DLRC's strategic input enabled the client to align internal planning with regulatory expectations, reduce risk of delays, and also position the programme for successful progression through early-phase trials. DLRC's expertise in ATMPs and rare paediatric indications continues to be leveraged in other client engagements.

DLRC also remains a preferred regulatory partner for the client organisation, with active and upcoming projects involving other biotech sponsors developing advanced therapies in rare disease indications.

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