

PPD Biotech Brings Deep Experience to Advancing Cell and Gene Therapy Drug Development

Emerging technologies have driven cell and gene therapies to the forefront of healthcare, addressing a growing number of disease conditions, including rare diseases with unmet medical needs. Advancing development in these areas, however, requires special expertise and the ability to tailor services across all stages of drug development.

PPD Biotech has deep experience partnering with clients to advance development of their cell and gene therapy trials across numerous indications and all development phases.



Early development, regulatory and market access strategy



Comprehensive laboratory services



End-to-end product development and clinical execution



Patient and sample concierge

Leading Clinical Trial Experience

Over the past five years, **we have conducted 59 cell and gene therapy studies** across multiple regions, and we have broad therapeutic area expertise with multiple asset types, including:

- Adenovirus/AAV
- Lentivirus
- Virus-based immunotherapies
- Non-viral vectors
- CAR T cells
- Cell-based medicinal products



SITE AND PATIENT STRATEGY

With our experience in cell and gene therapy comes a comprehensive understanding of and access to the sites and patients needed to deliver these studies on time. Innovative and emerging therapies often mean you will face additional local requirements for approvals and for handling of samples and the investigational product. We will help you anticipate and manage or altogether avoid these challenges. Additionally, existing perceptions of gene therapy and the need for long-term follow-up require a well thought out and gentle approach to patient engagement and education. We offer solutions that provide a high-touch clinical trial experience for sites, patients and caregivers participating in these innovative studies.

Regulatory Expertise

The regulatory environment for cell and gene therapies is complex and rapidly advancing. Staying current with evolving regulatory intelligence and having frequent and early interactions with regulatory agencies are critical to ensuring the most efficient path to approval and market access.

Our global team of regulatory experts provides full end-to-end regulatory support, from program design through execution. We deliver regulatory expertise for cell and gene therapy studies, with services including:

- **Early Engagement Activities:** Scientific advice, RMAT and ATMP classifications, orphan drug designation, pediatric plans
- **Genetically Modified Organisms (GMO) Expertise:** Authoring of environmental submission dossiers, interaction with country specific agencies
- **Clinical Trial Applications:** IND and IMPD authoring and gap analysis, protocol review, global submissions
- **Consultancy:** Strategic regulatory consultancy, clinical/non-clinical consultancy



COMPREHENSIVE LABORATORY EXPERIENCE

PPD® Laboratories has extensive experience with a wide variety of cell and gene therapies including plasmids, linear and encapsulated nucleic acid based products, CAR T, CRISPR/Cas9 and viral vectors.

Market Access and Value Demonstration

More cell and gene therapy products are maturing towards approval. Out-of-the-box thinking is required to successfully navigate cell and gene therapies through global systems that have not anticipated these types of interventions. Limited precedent challenges reimbursement “fit” for authorities. Our evidence and peri-/post-approval group, Evidera, is a global leader with significant expertise in value demonstration and reimbursement strategy for these new therapies. This experience is used to define development plans relevant to payers, providers and patients and to uptake risk and refine value demonstration throughout the lifecycle.

With experience across **more than 200 cell and gene therapy projects**, we offer leading expertise in:



Market access



Outcomes research



Reimbursement and pricing strategy



Real-world evidence

The Right Partner for Biotech to Deliver Successful Cell and Gene Therapy Studies

Our experience gives us a thorough understanding of the special needs surrounding cell and gene therapy trials, from country-specific regulatory requirements, to identification of experienced sites and enrollment of qualified and engaged patients. PPD Biotech forges close partnerships with clients to implement novel solutions, delivering new treatments to patients in these emerging modalities.