Regulatory pathways for CGT and ATMP products

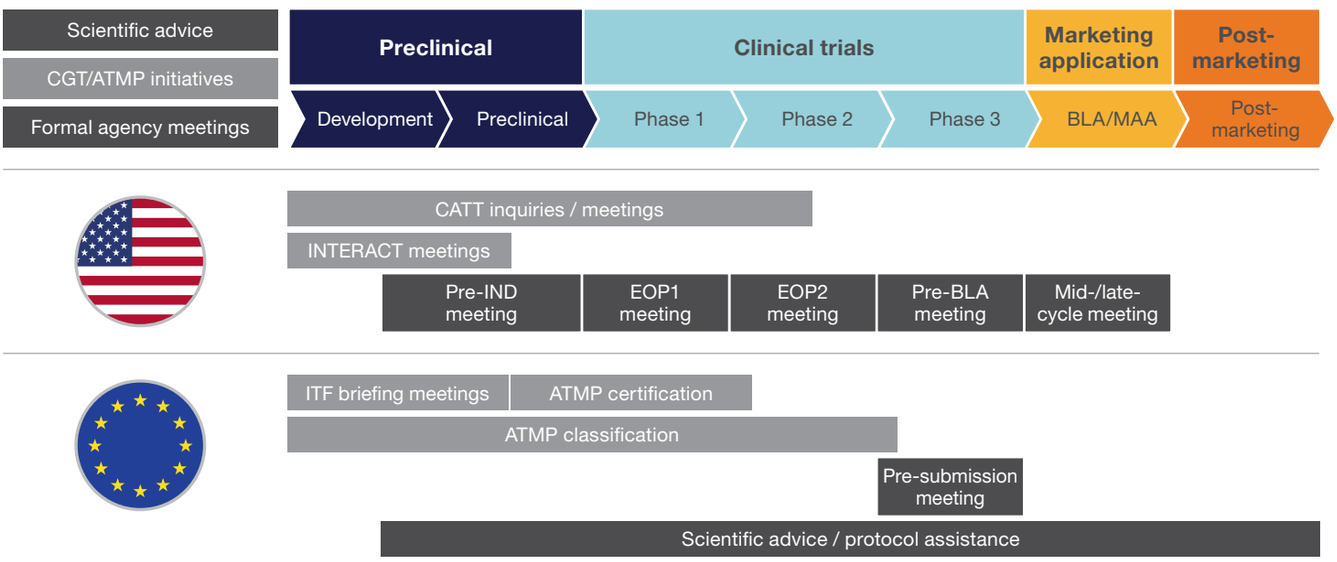
Three tips for success

According to the FDA, federal oversight of biologics began in 1902 under the Biologics Control Act, which provided government control over medical products made from living sources. Since that time, regulatory authorities around the world have made it their mission to provide guidance for manufacturing safe and effective therapeutic products.

As therapeutic strategies and technologies evolve, so do regulatory frameworks and guidance. Cell and gene therapy (CGT) is one of the fastest-growing therapeutic areas today, and for a good reason. These therapies provide hope to patients with the opportunity for cures instead of lifelong treatments. Regulatory agencies have responded to this promise and urgency by developing expedited approval pathways, such as Regenerative Medicine Advanced Therapy (RMAT) designation in the US and PRIME designations for Advanced Therapy Medicinal Products (ATMPs) in the EU. Expedited pathways have led to compressed CGT product development timelines, which present unique challenges and opportunities for developers. In addition, guidance and regulatory standards continue to tighten and evolve as the industry matures, so CGT developers must engage with regulatory authorities effectively. This infographic will review three tips for regulatory success.

Tip #1: Engage early and often

Opportunities to engage with regulatory agencies along expedited pathways are provided through formal meetings, special initiatives, and parallel scientific advice mechanisms.



Special initiatives provide unique regulatory engagement opportunities for CGT/ATMP developers.

INitial Targeted Engagement for Regulatory Advice on CBER producTs (INTERACT)	CBER Advanced Technologies Team (CATT)
Preliminary informal consultation for innovative investigational products at an early stage of development	Discussion on advanced manufacturing technologies to be implemented in CBER-regulated biologics products

Innovation Task Force (ITF) briefing meeting	ATMP classification	ATMP certification
Informal meetings to discuss scientific, technical, and regulatory issues for innovative drug development	Procedure to allow clarification if a specific product meets the criteria for ATMP definition	Opportunity for SMEs to check if the quality and non-clinical data generated comply with the standards for evaluating an MAA

EOP = End of Phase

Tip #2: Surround yourself with regulatory experts who can…

Assess	Coordinate	Execute	Deliver
Gap analysis and action plan for CGT regulatory CMC content for various development phases and commercial applications	Discussions, input, and deliverables from many stakeholders	Writing, reviewing, and revising CMC dossier before application submission	Regulatory agency meetings and interactions for pre-filing, reviews, pre-licensure, and GMP inspections

Tip #3: Leverage strong foundations

Careful site selection can help reach regulatory milestones faster, reduce risk, and build for commercial success.

When looking to get to the clinic and market quickly, building out capabilities and hiring for specific expertise takes too long. That's why it's often essential to leverage solid foundations that already exist. Here are some critical criteria to remember when selecting your manufacturing site.

- Experienced leadership team with a strong track record
- Scale and capacity flexibility
- Tech transfer and manufacturing success rate
- Robust materials management
- Facility design features that fit your drug substance/product
- Effective contamination and cross-contamination controls in place (cleaning/disinfection programs)
- Strong quality systems
- Proven regulatory and compliance track record

Thermo Fisher Scientific viral vector services

The experience and expertise you need to navigate the evolving regulatory landscape.

Experience and expertise	Strong foundation of proven success	Accelerate product access for patients
• >20 years viral vector development and manufacturing experience • Four late-phase/commercial manufacturing facilities • More than 50 drug substance and eight product suites • Experience with AAV (natural and novel stereotypes), LV, adenoviral, herpesviral, retroviral, and viral vaccines	• Two commercially licensed products • Multiple regulatory fillings in the pipeline • More than 650 viral vector cGMP clinical and commercial lots manufactured • More than 160 VV products produced • Expansive global network offering 650,000 ft^2 capacity	• 2,000 team members worldwide with 200 PhD level scientists • Leading VVS regulatory experts • Ten manufacturing platforms • All the advantages of Thermo Fisher's technology, cell lines, equipment, products, and logistics

Navigate the regulatory landscape and get your viral vector product to market faster with Thermo Fisher Scientific. Find out more.

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