

Navigating accelerated cell and gene therapy pathways

Compared to traditional pharmaceuticals, accelerated clinical trials pathways for advanced therapy medicinal products (ATMPs) allow less time for product development and characterization. However, expectations are still high when it comes to regulatory compliance.

In a recent Aspen Survey, 53% of respondents felt that regulatory submissions are the biggest bottleneck in biopharmaceutical development [1].

Explore the key regulatory challenges at each critical checkpoint in the accelerated pathway for cell and gene therapies.

Preclinical phases

RISK

Risk assessments

Create a risk assessment based on all process materials and components and revise at each stage of product development.

Product development

Start product development and characterization with full materials and component traceability. Ensure your supplier can take you from product development to commercialization.

Endpoints

Safety endpoints in disease models, including cytotoxicity and potency tests should be considered.

Clinical phases

Control strategy

Establish Critical Process Parameters and Critical Quality Attributes to set manufacturing specifications. Review your risk assessment ensuring a robust contamination control strategy.

Process design

Design your process with cGMP compliance in mind, thinking ahead on scalability, reproducible performance and increasing compliance requirements through clinical phases.

Testing and control

Extractables and leachables
Perform an extractables and leachables risk assessment with your supplier, noting the enhanced complexity of product contact with single-use-systems in cell therapy. *In silico* modeling may be applied to predict extractables and leachables outcomes.

Microbial quality control

The short shelf life of many ATMPs requires real-time release testing for sterility and mycoplasma.

GMP-compliant raw materials

Ensure all raw materials are suitable for GMP manufacturing.

Product specifications

Product specifications must be set by Phase III with validated analytical methods. Note that limited patient numbers at the time of filing leads to a conservative approach to setting process parameters and specifications.

Process validation

Process validation should be carried out to document that the manufacturing process can consistently produce results within specific parameters.

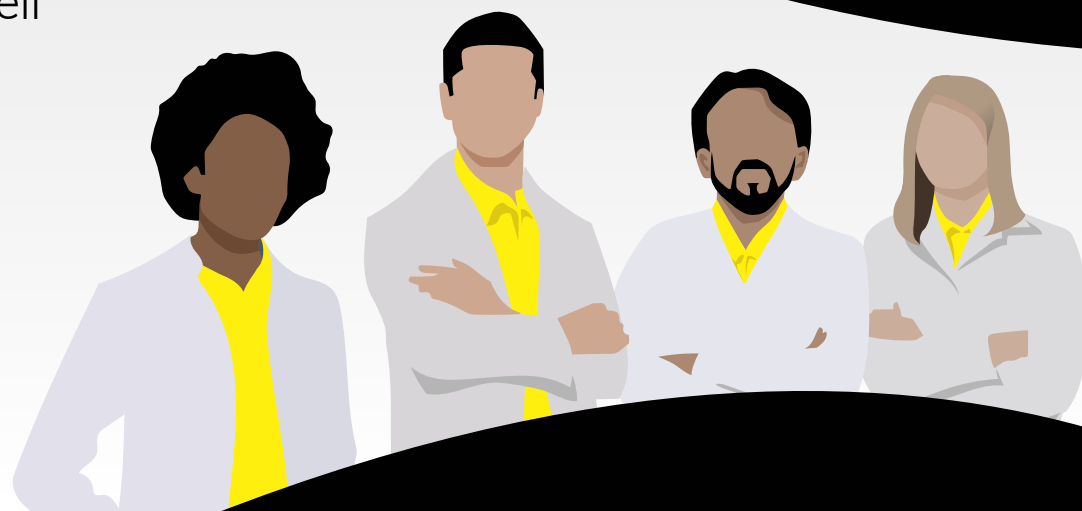
Supply chain management and qualification

Ensure reliable supply of critical materials supported with appropriate documentation for regulatory filing. Qualify your supplier's quality management systems and enter into a quality agreement.

**Biologics License Application/
Market Authorization Application review submitted**

Sartorius offers comprehensive support from research and development through GMP-grade production, providing phase-appropriate solutions to navigate the complexities of cell and gene therapy development.

Explore more Sartorius cell and gene therapy resources.



1. Aspen Alert. ASQ 818 2022 0114 - Where is the biggest bottleneck in biopharmaceutical development? <https://aspenalert.com/asq-818-2022-0114-of-the-following-where-is-the-biggest-bottleneck-in-biopharmaceutical-development-please-comment-on-your-reasons>