

Advancing a Phase 3 Gene Therapy Program to BLA Readiness

The Challenge

A gene therapy company had generated strong Phase 3 clinical data and received FDA alignment to proceed with a Biologics License Application. The company needed to translate a successful clinical program into a complete, inspection-ready CMC package capable of supporting BLA filing and review.

Critical late-stage activities remained, including process performance qualification, control strategy development, analytical method validation, BLA gap closure, and generation of Module 3.

The Operating Model

Syner-G embedded senior CMC and regulatory expertise to assess readiness, close priority gaps, and coordinate the technical workstreams required for BLA generation.

Program at a Glance

- ✓ PHASE 3
POSITIVE DATA
- ✓ FDA
BLA ALIGNMENT
- ✓ PPQ
PROTOCOL SUPPORT
- ✓ METHODS
VALIDATION
- ✓ BLA
MODULE 3

The Syner-G Solution

Syner-G served as an embedded extension of the company, integrating process, analytical, quality, manufacturing, and Regulatory CMC expertise to strengthen overall BLA preparedness and support generation of a cohesive Module 3 package.

PPQ PROTOCOL DESIGN & IMPLEMENTATION

- Helped design the process performance qualification protocol and align the approach with the intended commercial manufacturing process.
- Supported protocol implementation, execution planning, acceptance criteria, and cross-functional coordination.
- Connected PPQ activities with validation, manufacturing, quality, and BLA documentation needs.

CONTROL STRATEGY DEVELOPMENT

- Developed an integrated control strategy linking process parameters, material controls, in-process controls, release testing, and product quality attributes.
- Assessed late-stage control strategy gaps and clarified the supporting evidence required for the BLA.
- Aligned control strategy content across validation, analytical, manufacturing, and regulatory documentation.

ANALYTICAL METHOD VALIDATION

- Guided analytical method validation planning for assays supporting release, stability, characterization, and process validation.
- Reviewed validation readiness, protocols, acceptance criteria, data packages, and documentation needed for BLA inclusion.

BLA READINESS & GAP ASSESSMENT

- Conducted a comprehensive BLA CMC gap assessment across process, analytical, validation, manufacturing, quality, and regulatory documentation.
- Prioritized gaps, established owners and closure plans, and integrated remaining activities into the BLA readiness plan.
- Assessed consistency and traceability across source records, validation deliverables, and the regulatory narrative.

MODULE 3 GENERATION

- Supported generation and integration of BLA Module 3 content across process, analytical, manufacturing, control strategy, validation, and quality sections.
- Established a structured review framework to drive completeness, consistency, traceability, and submission readiness.

Business Impact

Syner-G provided the integrated late-stage CMC and Regulatory CMC support needed to convert positive Phase 3 results and FDA filing alignment into an actionable, coordinated BLA readiness program.

01

PPQ protocol design and implementation supported

02

Commercial control strategy developed

03

Advanced analytical method validation

04

Priority BLA gaps identified and addressed

05

Supported integrated Module 3 generation

From positive Phase 3 data to BLA readiness

Integrated PPQ, control strategy, analytical validation, gap closure, and Module 3 generation for BLA preparedness.