Early Phase Cell Therapy Product Development:

Potency Assays

Introduction

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What is Potency?

"The specific ability or capacity of the product, as indicated by appropriate laboratory tests or by adequately controlled clinical data obtained through the administration of the product in the manner intended, to effect a given result."

2011 FDA Guidance for Industry: Potency Tests for Cellular and Gene Therapy Products

FDA Requirements for Potency Assays

A validated potency assay with pre-defined acceptance/rejection criteria is required in the Biologics License Application (BLA) and must:

- Measure a relevant biological activity (mechanism of action) of the product
- Use appropriate reference standards and/or controls
- Be quantitative
- Establish accuracy, precision, specificity and range of test methods

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21st Century Cures Act

The 21st Century Cures Act is a United States law enacted by the 114th United States Congress in December 2016. It authorized $6.3 billion in funding, mostly for the National Institutes of Health.

FDA drug approval process

The 21st Century Cures Act modified the FDA drug approval process by mandating new rules that direct the FDA to approve drugs and devices with greater urgency.

- RMAT (Regenerative Medicine Advanced Therapy)
- Fast Track
- Breakthrough Therapy
- Priority Review
- Accelerated Approval

When to Apply Potency Assays?

- Product characterization
- Comparability testing
- Assessing manufacturing changes
- Stability studies
- Lot release testing

Webinar Objectives

1. Acquire knowledge of the regulatory expectations for potency assay rigor in early or late phase clinical trials.
2. Observe relevant examples of potency assay application for a representative cell therapy product.
3. Determine when and what test methods to apply during cell therapy product characterization and validation.

Regulatory Publications

Source	Title
USP <1030>	Biological Assays Chapters-Overview and Glossary
USP <111>	Design and Analysis of Biological Assays
USP <1032>	Design and Development of Biological Assays
USP <1033>	Biological Assay Validation
USP <1034>	Analysis of Biological Assays
FDA Guidance for Industry	Potency Tests for Cellular and Gene Therapy Products