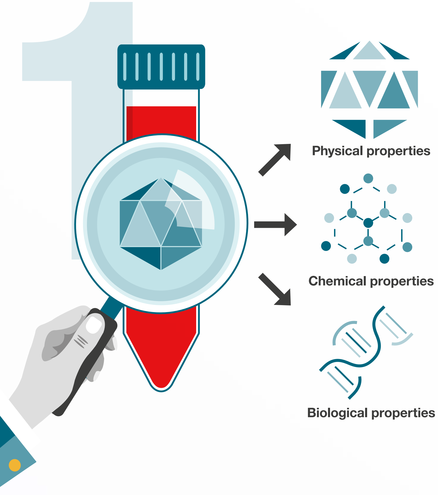
Gene Therapy Analytical Development

Vital tools in gene therapy product manufacturing

Analytical methods are vital tools in developing gene therapy products, providing critical information on product characterization, safety, efficacy, process control, regulatory compliance, and batch consistency. They contribute to the understanding, optimization, and successful translation of gene therapies from the laboratory to clinical application.

Gene therapy product manufacturing involves three types of analytical assays: characterization assays, in-process assays, and release assays. It is important to note that the regulatory oversight by the United States Food & Drug Administration (FDA) differs for these types of assays.

More information



Characterization assays

Characterization assays are used to analyze the gene therapy product and understand its physical, chemical, and biological properties. They help in determining the product's structure, composition, and functionality. While the FDA does not directly regulate characterization assays, it expects them to be conducted based on sound scientific principles to ensure the safety and effectiveness of the product.

Read the white paper to learn more

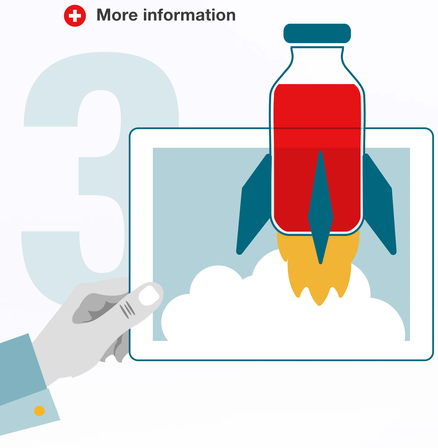
More information

In-process assays

In-process assays are performed during various stages of the manufacturing process to monitor and assess the product's quality and consistency. They help manufacturers make timely adjustments and ensure that the product is meeting the desired specifications. Similar to characterization assays, the FDA does not have direct regulatory oversight over in-process assays. However, it is expected that these assays are conducted using scientifically valid methods to maintain process control and product quality.

Read the white paper to learn more

More information



Release assays

Release assays are specifically regulated by the FDA under 21 CFR Part 610. These tests are conducted to determine whether a batch or lot of the gene therapy product meets predetermined specifications before it is released for use or distribution. The FDA requires that release testing establishes meaningful measures of sterility, identity, purity, and potency of the gene therapy product. These tests play a critical role in ensuring the safety and efficacy of the product before it reaches patients.

Release assays are typically considered higher risk compared to characterization and in-process assays due to their direct impact on patient safety and the regulatory scrutiny they receive.

Read the white paper to learn more

More information

Summary

Non-compendial methods are needed in gene therapy manufacturing due to their ability to:

- Incorporate innovation
- Address the unique requirements of gene therapies
- Provide comprehensive information
- Offer customization and optimization
- Gain regulatory acceptance
- Support research and development efforts

Non-compendial approaches for manufacturing analysis of innovative methods in gene therapy may support regulatory acceptance as long as they are scientifically valid and reliable. This regulatory acceptance encourages the use of new technologies, fostering a supportive environment for advancing gene therapy characterization and product development.

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