



International Alliance for
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Statistical Considerations During the Review of Gene Therapy Products

Gene Therapy (GT) development has rapidly expanded over the last two decades, with products progressing to licensure. Statistical methods are used during product development and review from early clinical studies through licensure, and post-licensure to evaluate different aspects of GT manufacturing and testing including determination of lot release specifications, process control, and product comparability. GT products have several unique product characteristics, and it is important to identify appropriate statistical approaches for evaluation. Ultimately, understanding the relationship between the product biology and the statistical approach is paramount to support GT development

