International Alliance for Biological Standardization

10th Annual Statistics Workshop: Science & Statistics – Elevating CMC through Partnership

November 12-14, 2024
IBBR, Rockville, USA

Statistical opportunities related to ICH Q2(R2) and Q14

With the publication of ICH Q2(R2) and Q14 comes numerous opportunities for CMC statisticians to collaborate with their laboratories, to help ensure fitness for use of their analytical procedures. Key among these are the introduction of confidence and prediction intervals as the basis for establishing conformance of a procedure to its performance requirements. This challenges the laboratory to address validation study risks through strategic design and analysis of their studies. While traditional approaches may be suitable for small molecule analytical methods, the inherent variability of procedures used to control biological products forces a company and reviewers to manage this more carefully through validation design. Additional concepts that should appeal to CMC statisticians are the use of validation results to develop CMC procedures during development, for release, and for comparability. Given the size and duration of most validations studies, the use of ongoing procedure performance verification has been proposed as a means to improve the understanding of procedure behavior. This talk will describe the elements of these guidelines that every CMC statistician should be aware of, and efforts to support the guidelines through the introduction of training materials.

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Session III: Enhancing Biologicals Development with Advanced Analytical Approaches

This session will explore the transformative journey of analytical methods in the biologics sector, highlighting the shift from traditional control strategies to cutting-edge approaches. We will delve into the modernization of analytical characterization, which is crucial for the rapid development and approval of gene therapies, cell therapies, and novel vaccines. The session will discuss the challenges and opportunities presented by the ICH Q2(R2) and Q14 guidelines, emphasizing the role of CMC statisticians in ensuring the fitness for use of analytical procedures. Additionally, we will examine a patient-centric approach to cell therapy manufacturing, focusing on the link between CAR-T product attributes and clinical outcomes. Through examples from recent successful submissions and clinical trials, the session aims to stimulate Quality by Design thinking and showcase the lifecycle management of analytical methods. Join us for a comprehensive discussion on enhancing biologic development with advanced analytical approaches and the role of statisticians in shaping the future of biologics.