Roundtable Session 1 – Table 1 - Submission Data Packages: What is our common vision of IND and BLA today?

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In the rapidly evolving landscape of biopharmaceuticals, the submission of data packages for Investigational New Drug (IND) and Biologics License Application (BLA) filings is critical for regulatory approval and market entry. This roundtable session aims to explore the current standards, challenges, and future directions in the preparation and submission of these data packages. The importance of higher order structure characterization in the preparation of these data packages will also be considered. Participants will discuss best practices, regulatory expectations, and innovative approaches to streamline the submission process, ensuring compliance and efficiency. The session will also delve into the harmonization of data requirements across different regulatory bodies and the role of digital tools in enhancing data integrity and submission quality.

1. What are the most common challenges faced during the preparation of IND and BLA submission data packages?

- **Complexity of data integration**: One of the biggest challenges is compiling and integrating data from multiple sources. Ensuring consistency and completeness across these data sets can be scientifically challenging and time-consuming.
- **Pharma companies are hesitant to include HOS data in CMC filing**. Pharmaceutical companies are hesitant to include higher-order structure data in their CMC filings. There is a lack of alignment across the industry regarding the type and extent of HOS data that should be included in these submissions.
- **Regulatory changes**: Regulatory requirements can change during the drug development lifecycle, making it difficult to keep submissions current and in line with the latest guidelines.
- **Quality control**: Ensuring the accuracy and completeness of large volumes of data and documentation across multiple departments is crucial but challenging.
- **Higher order structure characterization**: For biologics, the characterization of higher-order structures is critical to demonstrate product consistency and stability, which requires sophisticated analytical tools and expertise.
- **Cross-agency harmonization**: Differences in regulatory requirements between agencies like the FDA, EMA, and PMDA can create delays or inconsistencies in global filings.

2. Is there general agreement in overall submission content and phase appropriate deployment?

- There is broad alignment on the general content required for submissions, particularly with well-established guidelines from ICH (International Council for Harmonisation) and FDA regulations. However, the phase-appropriate deployment can vary. For early-phase INDs, agencies are more flexible in terms of data volume and depth, while later phases (and especially BLAs) require far more comprehensive data.
- The challenge lies in interpreting what constitutes "appropriate" data at each phase of development, which can differ between companies and regulatory authorities.

3. How can we standardize data requirements across different regulatory agencies to streamline the submission process?

- **Adopt ICH guidelines globally**: Encouraging all regulatory agencies to adhere strictly to ICH guidelines could help harmonize requirements. While the FDA, EMA, and PMDA are aligned, other regional bodies sometimes have different expectations.
- **Use of common technical documents (CTD)**: The CTD format is widely accepted, but continued refinement and global harmonization of this format would help.
- **Regulatory collaboration**: Increasing collaboration between agencies, such as mutual recognition agreements (MRAs) and joint reviews, can help reduce discrepancies in requirements and submissions.

4. What role do digital tools and technologies play in improving the quality and efficiency of submission data packages?

- **Data management platforms**: Modern digital platforms can consolidate data from multiple sources and ensure that data is accessible, up-to-date, and consistent across the organization.
- **Automated quality control**: Digital tools, including AI-driven data validation, can identify discrepancies in real time, improving the overall accuracy and integrity of submissions.
- **eCTD**: The electronic Common Technical Document (eCTD) is becoming the standard for regulatory submissions, improving efficiency, submission quality, and regulatory review timelines.
- **Advanced analytics**: Analytical tools can now process large datasets and provide insights into potential gaps or issues in the data package before submission.

5. What are the emerging trends and future directions?

- **Use of AI and machine learning**: AI is increasingly being explored for streamlining data preparation, automating data integrity checks, and enhancing predictive modeling for submission outcomes.
- **Greater emphasis on CMC data**: As biologics and advanced therapies (e.g., cell and gene therapies) become more common, the importance of CMC data and higher-order structure characterization is increasing.

- **Global harmonization efforts**: Continued efforts to harmonize global regulatory requirements, especially for emerging markets, are expected to grow. MRAs between agencies may become more common.