Roundtable Session 2 – Table 1 –

Submission Data Packages: What is our common vision of IND and BLA today?

Facilitator: Mingyue Li, *Pfizer, Inc.*

Scribe: Mingzhang (Maple) Wang, Merck & Co.

Abstract:

Sponsor companies need to get approval from Food and Drug Administration (FDA) to market a new drug in the United States by authoring the regulatory filings. The Investigational New Drug Application (IND) and Biologics License Application (BLA) are two major submissions for clinical trial administration and marketing approval, respectively.

In this roundtable session, we will discuss the common vision of IND and BLA today as we see more novel modalities, such as cell and gene therapy. The determined vision should guide us to prepare HOS data packages that provides demonstrative evidence to prove that the drug product is safe and effective for the proposed use.

Discussion Questions:

What is the common vision of IND and BLA from HOS perspectives today?

What guidance/strategy are you using to decide what data should be included in the filings?

For newer modalities, such as gene therapy and mRNA vaccine, what strategy are you using when prepare the data package for submission?

Notes:

For particle characterizations, such as gene therapy, DLS, SEC-MALS, AUC, and zeta potential measurement are often used. Single particle measurements, such as TEM, may have bias of operators leading to problem with method validation.

For mRNA-LNPs, size distribution by AF4, IR of lipids, composition, loading, encapsulation are key aspects to consider.

In BLA extended characterization of mAb and mAb-like molecules, many attendees recommended on using DSC, CD, and IR in the format of overlay or in combination with statistical analysis and spectral deconvolution in filling.

SLS and DLS are release assays for vaccines. There is discussion about setting specifications.

We also touched base on orthogonal techniques may reach different conclusions due to differences in relative sensitivity.

CMC plays a key role in filing strategy and are experienced. Sometimes CMC is hesitant about including new technologies. If a technique is specific to one or two vendors, then that might be a concern. More publications from vendor will be good proofs. CMC prefers to include less, fewer but gold standard methods.

To introduce a new technique, start in early development, have sufficient data, replacing an old method is challenging. It is easier for a new method to make its way. Having bridging data, low-cost, and easy to implement in QC are nice features.

Vendors have experience with CD, IR and Raman on peptides and biosimilars. Instrument company couldn't get samples sometimes, NIST LNP standard in addition to NIST mAb will be a good standard for collaborations, and we need to ask about the timeline.

ChatGPT might be helpful to generate templates.