

Roundtable Session 1 – Table 16 - Use of Machine Learning (ML) and Artificial Intelligence (AI) in Content Creation of Early Phase Development and Managing Submissions and Information Requests with Health Authorities

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Abstract:

The rapid rise of machine learning (ML) and artificial intelligence (AI) is changing how we generate, structure, and submit regulatory content. The most advanced organizations in digitalization and automation are now using generative and retrieval-augmented systems not only to draft internal reports and Module 3 text, but also to organize existing knowledge, summarize complex analytical data packages, predict stability, and even automatically generate responses to health authority information requests. At the same time, ML-driven tools are being embedded earlier in CMC development for risk assessment of critical quality attributes, process characterization, and tech transfer readiness, all of which flow directly into what ultimately gets written in early phase submissions, initial briefing packages and will ultimately make their way into commercial submissions.¹

Health authorities (e.g. FDA CDER/CBER) have publicly acknowledged both the opportunity and the risk: they are exploring how AI/ML can support regulatory decision-making and are asking for transparency, data integrity, and appropriate human oversight of AI-generated analyses and documents. Draft frameworks published in 2023–2025 emphasize model transparency, data provenance, change control, and fit-for-purpose use of AI to support submissions and manufacturing decisions.²

This roundtable aims to focus on practical use cases and discussions will include:

1. How AI/ML is being applied today to create or assemble submission-ready CMC content in early development.
2. How companies are managing accountability, authorship, and validation of AI-assisted text and data summaries.
3. How teams work with regulators when AI tools help create or summarize submission content or responses.
4. Attendees will share experiences, pain points, emerging internal governance models, and open questions for regulators as we move toward more AI-assisted regulatory interactions in the future.

References:

¹ U.S. Food and Drug Administration (FDA). Artificial Intelligence in Drug Manufacturing. Silver Spring (MD): FDA; 2023. Available from: <https://www.fda.gov/media/169682/download>

² Mirakhori F, Niazi SK. Harnessing the AI/ML in Drug and Biological Products Discovery and Development: The Regulatory Perspective. *Pharmaceuticals*. 2025;18(1):47.

Discussion Questions:

1. How do we make sure people stay accountable for what's written in submissions if using AI?
 - a. If AI tools help write or summarize parts of our regulatory documents, who is responsible for checking accuracy and final approval? Should we tell regulators when we've used AI to help create content?
2. How much can we rely on AI predictions or summaries in early development?

- a. In early studies, data is limited, and timelines are short. If AI helps predict stability, summarize manufacturing data, or flag process risks, how do we decide what's "good enough" to include in an early phase submission?
 - b. Should we treat AI results as internal guidance only, or are we comfortable referencing them in early submissions?
3. How can AI safely help with information requests from regulators?
 - a. Many companies are using AI to draft first responses to health authority questions after submitting an early phase submission.
 - b. What are the risks e.g. mistakes, outdated information, or inconsistent wording. How do you keep quality control over what's sent back?
4. Where should AI tools "live" in our quality systems?
 - a. If AI helps write CMC sections or summarize data, do we need to manage that tool like any other validated system (with audit trails and version control)?
 - b. Or is it just an optional "assistant" that doesn't fall under GxP rules yet?
5. What do we need from regulators next?
 - a. Would clearer FDA or EMA guidance help, e.g. rules for using AI-generated text in Module 3 or AI-based models for early development predictions?

Notes:

Overview: The session included six participants (including the facilitator and scribe). The discussion confirmed the premise established in the abstract: that AI/ML is rapidly changing how organizations generate and structure regulatory content. Of note, companies are at different stages in their adoption and application of these tools whether supported in-house or outsourced. Participants emphasized that the primary value drivers for AI adoption are the ability to streamline workflows more quickly and support data verification.

Applications in Content Creation and Data Management: Applications for AI in creating submission-ready content including the automation of comparability and qualification reports was discussed. A significant challenge identified was difficulty authoring without structured data. While AI tools can extract data from paper and handwritten documents, this introduces higher variability and risk compared to digital sources. To mitigate data structure issues, there is a need for minimum requirements to be put in place between Contract Manufacturing Organizations (CMOs) and sponsors.

Accountability, Risk Management, and Validation: Reflection on the need for appropriate human oversight and discussion on how to manage authorship and validation. There is a prevailing fear of hallucination among users. To manage this, it was suggested defining strict scopes and guardrails for smaller tasks that do not require extensive human interaction, while ensuring step-by-step human checks are maintained. A critical requirement for accountability is traceability back to a source document, ensuring data integrity. A specific method proposed for validation involved using AI models to check the outputs of other AI models.

Implementation Strategies: Evaluation of different governance and implementation models for AI tools including discussion on selection risks of buying off-the-shelf tools (which are supported) versus

developing proprietary tools (which present the biggest risk for the company) versus a combination of the two, resulting in customized solutions.

Managing Information Requests (IRs): Some organizations are using systems to help generate responses to health authority IRs based on internal databases.

Regulatory Guidance: The current state of guidance feels very high level citing the recent Jan 2026 FDA/EMA Guiding Principles of Good AI Practice in Drug Development.