**Roundtable Session – Table 6 - New EU Pharma Legislation (NPL): Analyzing the Benefits and Challenges of the New Modular Framework**

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**Abstract:**

The recent overhaul of the EU Pharmaceutical Legislation marks a shift for Chemistry, Manufacturing, and Controls (CMC) strategy, moving from rigid submissions toward a modular, data-driven framework. This roundtable will discuss the practical implications of the pathways that may (or not?) facilitate biological and complex product development (e.g., Platform Technology Master Files, Additional Quality Master Files, Decentralised Manufacturing, Sandbox).

**Discussion Questions:**

1. **Platform Technology Master File (PTMF):** What type of project could benefit from PTMF? In practice how should this work? What would be the challenges?
2. Additional **Quality Master File (AQMF):** What type of project could benefit from QMF? How will QMF be managed (from industry and authority perspectives)? How to ensure alignment between QMF and specific dossier? How to deal with comparability?
3. **Decentralised Manufacturing (DM):** What type of project could benefit from DM? Challenges (for those that can apply or not apply the tool)?
4. **Sandbox:** What type of project could benefit from Sandbox? What are the anticipated challenges?

**Notes:**

**1 & 2. Master Files (PTMF & QMF) and Platform Marketing Authorization**

- **Clarity on Concepts and Terminology:** A recurring theme in the discussion was the need for clarity regarding the distinctions between PTMF, QMF and platform marketing authorisation.
    - **Platform Technology Master File (PTMF):** The concept of platform technology master files allows the reliance on data— encompassing aspects of quality, non-clinical, and clinical information—that has been pre-evaluated in the marketing authorisation application of a medicinal product.
    - Additional **Quality Master File (AQMF):** additional quality master files i.e. for active substances other than chemical active substances, or for other substances

present or used in the manufacture of a medicinal product

- **Platform Marketing Authorisation (Platform MA):** An authorisation for a medicinal product comprised of a fixed component and a variable component, justified for therapeutic purposes (e.g., to target different variants of an infectious agent or tailor the product to specific patient characteristics).

- **Industry Sentiment & Implementation:** The Platform MA and new Master File (AQMF/PTMF) concepts are supported by industry groups (e.g., EFPIA) and is generally viewed positively. However, there are significant questions regarding its practical elaboration, which will likely need to be defined through Implementing/Delegated Acts.

- **Operational Mechanics:** Participants questioned how the mechanism would function in practice.
  - Similar to the US Drug Master File (DMF)? Applicable accross product lifecycle? Across different MA? Across different MAH? Similar to the Plasma Master File (PMF)?
  - It was noted that the Master File owner could be different from the Marketing Authorisation Holder (MAH), which would be highly beneficial for Contract Development and Manufacturing Organizations (CDMOs) allowing the file to be used across different MAs from different MAHs.
  - Could such Master Files have Open and Closed Parts?

- **Scope of PTMF:**
  - Could it include analytical methods (e.g., basic description and validation)? How would product specific information be submitted?
  - Could it cover synthesis, including elements beyond quality such as toxicology and non-clinical data?

- **Scope of AQMF:**
  - The exact scope of the AQMF remains unclear and could potentially be very broad. It was questioned whether biological active substances are excluded from this pathway.
  - Benefit anticipated for novel excipients, and media components.

- **Anticipated Challenges:**
    - *Change Management:* If the PTMF serves as a repository of information (methods/processes), making changes to it could have a far-reaching impact. How will the impact on different products be assessed? Will the assessment be regulatory (via worksharing) or scientific (like a PACMP)?
    - *Variation Categories:* Changes to the PTMF and QMF are currently not defined in variation guidance.
    - It is unclear how , after a change to a Master File, the MAH should assess the impact on the MAA and if this represents a separate variation with specific data requirements (i.e. how to determine if such a change could be a type 1A or higher). Should there be two distinct types of PTMFs (one with product-specific impact and one without)?
    - *Standalone vs. Linked:* The group debated whether the PTMF could be a standalone certification process (like a PMF) or if its assessment would only be triggered by an MA or variation submission (like an ASMF). The table expressed a preference for a standalone certification process.
    - *Confidentiality:* Handling open and closed parts (similar to US Facility MFs or Container Closure MFs) remains a complex hurdle, as these concepts are not clear in the current proposal.

**3. Decentralised Manufacturing (DM) & Sandbox**

- *Note: While Decentralised Manufacturing and Regulatory Sandboxes were on the agenda, the primary discussion at the table focused heavily on the mechanics and complexities of Master Files and product classifications.*

**4. Other Topics: Bio-Hybrids**

- **Classification:** Bio-hybrids are emerging as a new class of products. This acts as an addition to Biosimilars, which are expected to be more narrowly defined under the new legislation.
- **Regulatory Rationale:** Participants discussed the regulatory thinking behind this class, drawing analogies to generics and hybrids (Article 10(1) and 10(3). Is it intended to act as a "wide box" to capture complex products that do not strictly fit the biosimilar definition?
- **Future Scope:** A question was raised regarding whether this Bio-Hybrid pathway could eventually serve as an option for Advanced Therapy Medicinal Products (ATMPs).