Roundtable Session 2 – Table 1 – Prior Knowledge and Platform Data in CGT: Opportunities, Challenges, and Regulatory Expectations

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Abstract

Cell and gene therapies are transforming the treatment landscape across a broad range of diseases. However, the complexity of these products, combined with small patient populations and accelerated development timelines, presents unique challenges in generating robust product-specific datasets. As the field continues to mature, sponsors and regulators are increasingly exploring how prior knowledge, platform technologies, historical manufacturing experience, and cross-product data can be leveraged to streamline development, reduce redundant studies, and accelerate patient access.

Platform approaches offer significant opportunities across multiple aspects of CMC development, including analytical method development and validation, product characterization, process development and validation, comparability assessments, control strategy design, and lifecycle management. At the same time, important questions remain regarding the scientific justification for applying data across products, the boundaries of platform applicability, and the level of evidence required to support regulatory decision-making.

This roundtable will bring together industry leaders, regulators, and CMC experts to discuss current perspectives on the use of prior knowledge and platform data throughout the product lifecycle. Through practical case studies and regulatory experience, participants will explore opportunities to enhance development efficiency, reduce unnecessary testing, and strengthen manufacturing and control strategies while maintaining appropriate product-specific understanding and regulatory confidence.

Attendees will gain practical insights into developing and defending platform-based CMC strategies, identifying areas where prior knowledge can deliver the greatest regulatory benefit, and applying these approaches to support more efficient and scientifically robust product development.

Discussion questions

1. *Where can platform approaches deliver the greatest value across the lifecycle of cell and gene therapies?* Which CMC activities are most amenable to platform-based approaches? Where have platform strategies demonstrated the greatest impact on development timelines, regulatory interactions, or lifecycle management?

2. *What has been your experience using platform claims in regulatory submissions?* Which platform-based approaches have been successfully accepted in CTA/IND, MAA/BLA, or post-approval submissions? Which approaches faced regulatory challenges? Have experiences differed between the FDA, EMA, and other health authorities?

3. *What are regulators' current expectations for leveraging platform data?* What evidence is needed to justify extrapolation from one product, process, or manufacturing platform to

another? How should sponsors define the boundaries of a platform? Do expectations differ across ATMP modalities?

4. *How can companies build platform strategies proactively rather than retrospectively?* What data should be generated and retained early in development to maximize future platform applicability? How can organizations establish and maintain platform knowledge that remains scientifically relevant and regulatory credible over time?

5. *What are the scientific and regulatory limits of platform approaches?* At what point does product-specific knowledge outweigh platform knowledge? Which CMC elements are least suitable for leveraging prior knowledge or platform data?

Summary of discussion

The discussion focused on how prior knowledge, historical data and platform approaches can support more efficient CMC development for cell and gene therapies. Participants recognised the particular pressure created by limited batch numbers, small patient populations and short development timelines. The overall view was that platform knowledge can materially accelerate development, but only when its scope is clearly defined and its relevance to the specific product is scientifically demonstrated.

1. Where platform approaches provide the greatest value

The greatest immediate value was associated with early clinical development, particularly Phase 1, when speed to clinic is critical and product-specific datasets are necessarily limited. Early-phase specifications may be broader and development strategies more flexible, creating greater opportunity to use established methods, processes and control concepts while product-specific knowledge is built.

Platform approaches can be applied across several CMC areas, including analytical methods, raw materials, manufacturing operations, process parameters, product characterisation, reference materials, stability strategies and elements of the control strategy. Reusing established approaches may reduce duplicated work, focus resources on genuinely product-specific risks and improve consistency between programmes.

External manufacturing partners may provide additional leverage where they have experience across multiple products using comparable technologies. Their established facilities, methods, templates and operational knowledge can shorten start-up activities. However, the value obtained depends on transparency, access to the underlying data and the sponsor's ability to understand and defend the platform rather than relying on the partner's experience as a stand-alone justification.

2. Regulatory use and evidentiary expectations

Experience with platform claims was described as variable. Acceptance depends less on the use of the term "platform" than on the strength of the scientific argument. A submission should explain which elements are common, why the prior data are relevant, what differences exist, and how those differences affect product quality and patient risk.

Participants noted that expectations may differ by authority, reviewer and development stage. Early dialogue can help clarify whether a proposed use of prior knowledge is acceptable and what bridging or confirmatory information will be required. Sponsors should avoid assuming

that an approach accepted previously, or in one region, will automatically be accepted for another product or submission, as requirements evolve over time.

A platform claim is strengthened by a well-characterised history across representative batches or products, consistent performance, predefined acceptance criteria and clear links between prior knowledge and the current product. Where changes are introduced, the package should show that the platform remains applicable through risk assessment, comparability data or targeted product-specific studies.

3. Defining the platform and its boundaries

The platform should be defined before it is invoked. This includes the shared product or process features, unit operations, materials, equipment, analytical principles and operating ranges, together with the attributes that may vary. The definition should be specific enough to be testable and should identify conditions under which extrapolation is no longer justified.

Product-specific knowledge takes precedence when differences could influence identity, potency, purity, safety, stability or clinical performance. Biological variability, starting material, vector or construct design, cell source, mechanism of action and patient-specific manufacturing may limit the extent to which knowledge can be transferred. Analytical methods may also require product-specific suitability or validation even when the underlying method is established.

The discussion distinguished genuine platform evidence from simple organisational standardisation. Common procedures and templates are useful, but they do not by themselves establish that data can be extrapolated across products. The regulatory argument must remain grounded in scientific similarity and an understanding of residual uncertainty.

4. Building a proactive and durable platform strategy

Participants favoured building the platform prospectively rather than assembling a justification retrospectively for a submission. Organisations should decide early which data will be generated consistently across programmes, how metadata and deviations will be captured, and which parameters and outcomes will be trended. Both successful and unsuccessful experience can help define the true limits of the platform.

Knowledge should be retained in a structured, accessible form, including development rationale, method and process history, changes, deviations, comparability conclusions and regulatory feedback. Tacit operational knowledge also matters and may not be fully captured on paper. Continued access to experienced staff, cross-programme reviews and effective transfer between sponsors and manufacturing partners remain important.

Agreements with external partners should address data access, the right to reference or use relevant platform information, change notification and the responsibilities for maintaining the platform package. These issues are easier to resolve at contracting and programme set-up than after the data are needed for a regulatory interaction.

Overall conclusions

Platform approaches are most credible when they are treated as a defined body of evidence rather than as a label. They can provide substantial efficiencies, especially in early development and across repeated manufacturing or analytical activities, but they do not remove the need for product-specific understanding. The most effective strategy is to define the platform and its

limits early, collect comparable data systematically, preserve both documented and tacit knowledge, and engage regulators proactively on the proposed use of prior knowledge.