Opportunities and Challenges for Platform Technologies based on the MEB Science Day 2025

Top 5 Opportunities

- Accelerating medicines development and approval
- Cost reduction and scalability
- Enabling personalised medicine
- Facilitating data reuse and regulatory alignment
- Promoting scientific synergy through modular design

Top 5 Challenges

- Lack of a clear and unified definition
- Complexity of lifecycle management
- Unclear regulatory pathways for platform-based authorisations
- High bar for scientific justification of data reuse
- Risk of limiting innovation

Top 5 Opportunities

1. Accelerating medicines development and approval

Platform technologies allow the reuse of data, processes, and infrastructure, reducing duplication and shortening timelines. This is especially valuable in pandemic preparedness and the treatment of ultra-rare diseases.

2. Cost reduction and scalability

By using shared components (e.g., viral vectors or antisense oligonucleotide backbones) across multiple products, development becomes more efficient and cost-effective, particularly for advanced therapy medicinal products (ATMPs).

3. Enabling personalised medicine

Platforms support the parallel or individualised development of therapies tailored to patients' specific genetic or clinical profiles, such as custom antisense oligonucleotides for neurological diseases.

4. Facilitating data reuse and regulatory alignment

Through tools like master files and modular dossiers, platform technologies can improve data sharing and consistency. Harmonisation between regulators will help streamline evaluations and life cycle management.

5. Promoting scientific synergy through modular design

Shared scientific principles can be applied across diseases and product types, allowing knowledge to be translated more rapidly into new applications.

Top 5 Challenges

1. Lack of a clear and unified definition

Diverging definitions across stakeholders (e.g., EMA, FDA, EC) create uncertainty. Without a shared understanding, implementation in regulation and development remains inconsistent.

2. Complexity of lifecycle management

Changes to a shared platform component can affect multiple products, raising challenges around tracking, evaluating, and approving modifications across a platform's portfolio.

3. Unclear regulatory pathways for platform-based authorisations

Should the authorisation cover the product, the platform, or the process? There is currently no well-defined framework to guide these decisions, leading to uncertainty for developers.

4. High bar for scientific justification of data reuse

To reuse data across products, developers must robustly demonstrate similarity and justify extrapolation – particularly difficult in non-coding RNA products or personalised therapies.

5. Risk of limiting innovation

If platform frameworks are too rigid or narrow, they may discourage novel approaches that do not fit into existing models. Flexibility and adaptability must be built into any platform concept.