

Platform technologies

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What Are Platform Technologies?

“Drug development platform (technology) refers to an array of foundational tools, methodologies, and systems designed to facilitate the efficient creation and production of pharmaceutical drugs.”

Pharmaceutics; 2024 Jul 10;16(7):918.doi: [10.3390/pharmaceutics16070918](https://doi.org/10.3390/pharmaceutics16070918)

Well-understood and reproducible technology, which may include a nucleic acid sequence, **molecular structure, mechanism of action, delivery method**, vector, or a combination of any such technologies...

Prerequisite: The Platform is...

- **essential to the structure or function of a medicine;**
- can be used by **more than one medicine sharing common structural elements;**
- facilitates the **manufacture or development** of more than one medicine

Based on US FD&C Act

History and development of the platform technology concept

History:

1. ICH guideline Q11 (2012)
2. Regulation for Veterinary Medicines (2019)
3. US FD&C Act (2018) → DRAFT FDA guidance
4. New Pharmaceutical Legislation (NPL) (2023)
 - EMA concept paper
 - Current NPL provisions Art. 15.2

New pharma legislation for human MP

- "...a marketing authorisation may... be granted for a medicinal product comprised of **a fixed component and a variable component that is pre-defined...** to target different variants of an infectious agent **or**, to **tailor** the medicinal product to characteristics of an **individual patient or a group of patients** ('platform technology')."
- **Adaptation to single patients (individualised treatment)**
 - To reduce the regulatory burden and providing a flexible regulatory framework to support innovation and competitiveness
- **Adaptation to an infectious agent**
 - To increase preparedness and responsiveness against health threats, in particular the emergence of antimicrobial resistance.

The bigger picture...

Regulatory tools

Concept of prior knowledge

Extrapolation
(Pediatric, Orphans,)

Modelling and Simulation

Types of platforms

CMC Platforms

Non-clinical Platforms

Clinical Platforms

Technology Platform

Platform MAAs (Marketing Authorization Applications)

Guidance and legislation

FDA DRAFT guidance

ICH relevant guidance
(Q11) (prior knowledge)

Veterinary medicines
framework (2019)

New pharmaceutical legislation
(under Council review)

Regulatory concepts and frameworks

Human medicines
framework

Single- vs multiple
Marketing Authorisations

Biosimilar framework

Master File concept

Network concept paper for the new pharma legislation

Under the concept of marketing authorisations based on platforms proposed in this CP, three scenarios were foreseen:



Platforms for the manufacture of some personalised medicines

- Same indication
- Fixed and pre-defined variable component
- Adaptations in e.g. level of AS, starting materials, raw materials, manufacturing process,

Intended to allow a difference in products under a **single MA**



Platform for medicinal products against agents which are or have a potential to cause serious cross-border threats to health

- Indication for several variants/related strains of an infectious agent
- Vaccines, mAbs...
- Standardized manufacturing process accommodating a fixed and variable component



Platform for medicinal products manufactured using prior knowledge

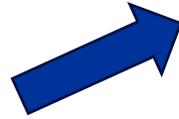
- Indication-independent
- Standardized manufacturing process accommodating a fixed and variable component
- Potential **wider applicability (expanding NC/C)**

Intended to rely on some **prior assessment** by the authorities to avoid duplication of assessment

Why Platform Technologies – opportunities...

Why Platform Technologies?

- Optimised product development cycles
- Resource savings through “reusable” components
- Accelerated response to emerging health threats
- Greater innovation scalability



Benefits to Patients and Healthcare Systems

- Rapid access to innovation (e.g., pandemic)
- Personalized and targeted therapies
- Cross-indication scalability
- Extension and affordability of treatment options



Benefits to Developers

- Shared data and infrastructure
- Faster time to market and patient
- Opportunities for lifecycle and portfolio management
- Business cases for neglected areas

Experience and examples of Platform technologies:

Analysis of the Platform Technologies:

- EMA procedures of the last 6 years 2017-2024
- A total of 34 relevant PT were identified
- Most frequently used PT on CMC/manufacturing followed by NC and Clinical
- Covid-19 most common targeted disease
- 30% of the PT analysed have ORPHAN designation
- Most frequent PTs were Viral vector (particularly AAV), followed by gene therapy platforms

Potential applications / examples:

- AAV gene therapy platforms
- mRNA vaccines (e.g., COVID-19)
- Cell therapy manufacturing platforms (CAR-T)
- Digital platforms for drug design (AI/ML)
- Carriers/delivery methods eg Nanoparticles
- New Approach Methodologies (NAMs)

Stakeholder questions related to platform technologies



Can **multiple RNA-based therapeutics** that target patient (group) specific mutations of the same disease result in a **single MA**?



When is it **possible to use clinical and nonclinical data** from an established **virus vector-platform** for the MA of a novel vaccine, given that both have the same vector but contain a **different antigen**?



When can **data from a proprietary gene editing platform** be used for the **MA of a novel vaccine**, given that they utilize the **same vector backbone**?



Does the Agency agree that the **pooled safety data from Study a, b and c with supportive** safety data from the US Phase III Study X (all of which are clinical studies on MPs with the same backbone vector) will be sufficient for the evaluation of the product safety profile?

Challenges for the Implementation of Platform Technologies

- Life cycle management
 - Platform variation and comparability
 - Legal/regulatory complexity of MAA including multiple platforms
- Case by case individual b/r assessment
 - Data requirements across indications
- IT infrastructure / confidentiality
- IP and market exclusivity

Can we learn from other frameworks?

From the scientific viewpoint, *platform technology* driven development programmes should not be seen as 'abridged' but rather tailored and science-driven...

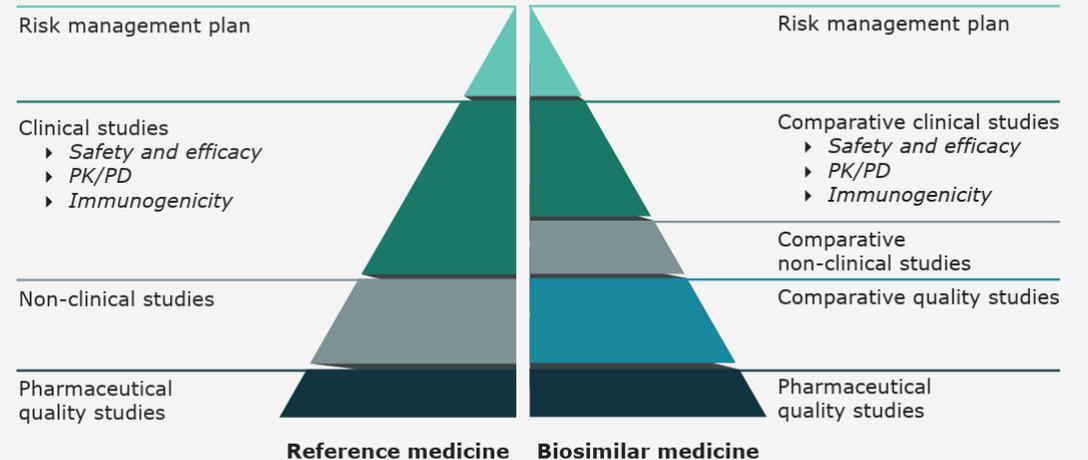
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Extrapolation of indications

Guideline on Similar Biological Medicinal Products containing Biotechnology-Derived Proteins as Active Substance: Non-Clinical and Clinical Issues:

- „In case the originally authorised medicinal product has more than one indication, the efficacy and safety of the medicinal product claimed to be similar has to be justified or, if necessary, demonstrated **separately for each of the claimed indications.**“
- „In certain cases it may be possible to **extrapolate** therapeutic similarity shown in one indication to other indications of the reference medicinal product.“
- „Justification will depend on e.g., clinical experience, available literature data, **whether or not the same mechanisms of action or the same receptor(s) are involved in all indications.**“
- Distribution, density, avidity and other characteristics of these receptors per indication?
- „Possible safety issues in different subpopulations should also be addressed.“

Figure 4. Comparison of data requirements for approval of a biosimilar versus the reference medicine



▶ MAbs. 2009 Sep-Oct;1(5):394-416. doi: [10.4161/mabs.1.5.9630](https://doi.org/10.4161/mabs.1.5.9630)

European Medicines Agency workshop on biosimilar monoclonal antibodies July 2, 2009, London, UK

Conclusions & Way Forward

- Platform technologies offer transformative potential
- Explore and learn from adaptive regulatory tools:
 - Extrapolation
 - Modelling and simulation
 - Orphans / rare diseases
 - Prior knowledge (CMC)
- Learn from existing regulatory frameworks:
 - Veterinary Platform concept
 - Biosimilar framework
- Cross-sector collaboration and International alignment
 - EU New Pharmaceutical Legislation (NPL), Biotech ACT, Life Sciences ACT
 - FDA guidance
 - ICH



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Thank you

