European Federation of Pharmaceutical Industries and Associations

Vaccines Europe
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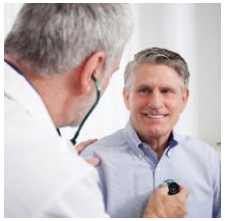
Platform Approaches in the Non-clinical and Clinical Domains in EU/EEA

EFPIA and Vaccines Europe – Human Medicines






Mihai Bilanin (GSK) *on behalf of* EFPIA and Vaccines Europe

EMA Webinar, 2nd of March 2026

About EFPIA

* The European Federation of Pharmaceutical Industries and Associations (EFPIA) represents the pharmaceutical industry operating in Europe. Direct membership of 36 national associations and 40 leading pharmaceutical companies and a growing number of small and medium-sized enterprises (SMEs).

EFPIA mission:

* To create an environment that enables our members to discover, innovate, develop and deliver new therapies and vaccines for people across Europe, as well as contribute to the European economy.

For more information, please visit https://efpia.eu/

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About Vaccines Europe

Vaccines Europe

Vaccines Europe is the specialised vaccines group within the European Federation of Pharmaceutical Industries and Associations (EFPIA)

- **Innovative research-based companies**

Our members represent innovative research-based global vaccine companies operating in Europe, accounting for a large share of human vaccines used worldwide, as well as European-based small and medium-sized enterprises

- **Our mission**

Foster innovation & value recognition of lifecourse immunisation in Europe to protect people against evolving health challenges

- **For more information**, please see https://www.vaccineseurope.eu/

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Industry Interest in Platform Approaches (MAs & PT/PTMF)

EFPIA and Vaccine Europe members:

- appreciate EMA' vision to accelerate the path from innovation to safe and effective medicines [1]
- see an opportunity to streamline medicines development through adoption of a platform approach
- agree with the general criteria that applicant should submit data that demonstrates that the platform technology is applicable to, has the potential to be incorporated in, or utilized by, more than one drug without an adverse effect on quality, manufacturing, or safety.

Platforms Approaches' Definitions and Scope – as we are expecting:

- to see the Definitions in the upcoming General Pharmaceutical Legislation text
- further clarity on the Scope of the new approaches of "Platform Marketing Authorization (PMA)" and of "Platform Technologies (PT)" and its "Platform Technology Master File (PTMF)"

Potential benefits of a platform approach in the non-clinical and clinical domains

- leverage the prior knowledge and understanding of the safety, efficacy, and performance of a platform and of its components
- facilitate the use of regulatory knowledge to enable more efficient review processes by allowing the assessor to focus on unique features of new products rather than repetition of what has been previously demonstrated and approved.

[1] *"A Fast Path from Innovation to Safe and Effective Medicines", Peter Arlett et. All, Clinical Pharmacology & Therapeutics, Oct 2025 – EMA Newsroom*

Case Study – general platform approach enabling acc. development

Case study on the use of platform technologies in clinical/non-clinical domains
Case example No. 1 *Choose one:* [X] Clinical domain [X] Non-clinical domain
Executive summary: The Company has developed several vaccines based, for e.g., on recombinant protein(s) formulated with an adjuvant system. The use of such technological platforms (including computational tools for antigen design, beside use of multiple antigen expression platforms for recombinant proteins (e.g. CHO cells, yeast, …) and several adjuvant systems (e.g. AS04, AS01, …)) has enabled accelerated development timelines with reduced costs.
Area of use and possible application: • Technical development leveraged by previous learnings and knowledge (e.g. compatibility, formulation,…) • Antigen design that has improved substantially through the use of computational design tools • Preclinical development leveraging results from previous studies with same components enabling improved clinical studies design (e.g. immunogenicity studies) or avoiding repeat of studies (e.g. biodistribution and toxicology studies) • Phase I clinical study design optimized by incorporating previous observations such as those related to dose-ranging (that pre-clinical models cannot reliably predict) and safety profiles.
Added value and impact: • Reduced, refinement and replacement (3R) of animal use • Reduced human exposure in new clinical studies • Reduced development time and costs, acceleration to First Time in Human studies.
Challenge/barrier description: Novel combinations still expected to require dedicated studies (complementary to platform studies).

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Case Study – mRNA platform approach enabling acc. development

Case study on the use of platform technologies in clinical/non-clinical domains
Case example No. 2 *Choose one:* [ ] Clinical domain [X] Non-clinical domain
Executive summary: The Company has leveraged five repeat dose toxicity studies to initiate 12 Phase 1 trials covering 6 different indications and five different LNPs. • This platform approach is based on the understanding that the LNP is the primary driver of safety and is used when the formulation differs only in the translated mRNA component (same lipid composition and ratio; lipid/mRNA ratio; within the maximum tolerable dose and same number of injections).
Area of use and possible application: • Supported quicker Phase 1 initiation of new candidate vaccines by leveraging repeat dose toxicology study data using the same LNP and a single biodistribution study per LNP using a model antigen. • Applicable to multivalent and combination vaccines as dose is based on total mRNA tested.
Added value and impact: • Reduced animal use. • Reduced time to IND/CTA submission leading to earlier FVFS, particularly when addressing a pandemic or other health emergency.
Challenge/barrier description: Novel antigens require dedicated toxicology studies, although biodistribution can still be leveraged based on the LNP.

Case Study – mRNA platform approach enabling acc. development

Case study on the use of platform technologies in clinical/non-clinical domains
Case example No. 3 *Choose one:* ☐ Clinical domain ☒ Non-clinical domain
Executive summary: Development of Comirnaty (Pfizer-BioNTech COVID-19 vaccine; BNT162b2) as well as subsequent approval of COVID-19 variant vaccines utilized a platform approach. For the original BNT162b2 vaccine, nonclinical safety data from other RNA-LNP formulations along with dosing phase data from an ongoing repeat-dose toxicity study with BNT162 formulations was submitted to allow for FIH initiation ~3-4 months after SARS-CoV-2 sequence availability. The full nonclinical safety and ADME packages for BNT162b2 were subsequently used to support approval of 6 COVID-19 variant vaccines (to date), which used the same RNA backbone, LNP, and similar manufacturing practices as the original BNT162b2 vaccine.
Area of use and possible application: Infectious disease vaccines Beyond vaccines, mRNA-LNP platforms have shown promise as cancer therapies; other potential areas under exploration are therapeutic antibodies and gene therapies
Added value and impact: The ability to use platform nonclinical toxicology and ADME data can eliminate conduct of nonclinical studies and accelerate initiation of clinical development, allowing substantial savings in time, resources, and animals. In addition, once established the mRNA-LNP platform is highly adaptable and can relatively easily be utilized to quickly develop a new vaccine.
Challenge/barrier description: Durability of immune response (for vaccines) How much can an established platform be changed before it is considered a new platform by health authorities When is the best time to evaluate biodistribution Limitations to IV administration of mRNA-LNP Misinformation about mRNA vaccines Changing regulatory and research funding environments.

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Opportunities and Challenges related to platform uses

- **These case studies show the potential application of platform approaches for clinical and non-clinical data. In order to maximise the opportunity in this area:**
  - introduction of a structured, predictable "platforms" framework in the upcoming EU legislation
  - the new tools facilitating efforts to enable faster patients' access to innovative medicinal products

Opportunities	Challenges if we operate within
Subsequent applications that rely on a platform approach should be (predictably) eligible for: ▪ reduction of some data requirements (CMC, non-clinical, and clinical) ▪ leveraging and rationalizing requirements for new products' demonstration of benefit/risk profile if a platform (clinical) safety profile is well characterized ▪ rolling submissions and review, once initial efficacy analysis are available given the high likelihood that the benefit/risk profile will align to the predicted one ▪ use of Platform' CTA (IMPD) and/or Platform Technologies Master Files (PTMF)	A regulatory' platform (data, requirements and/or qualification criteria) framework that: ▪ is not adaptable to new innovative approaches ▪ is misaligned in various, global jurisdictions ▪ doesn't allow any part of the drug development to qualify for a platform approach if scientifically justified and agreed with the regulators ▪ doesn't facilitate an efficient life-cycle management of updates/changes to platform data

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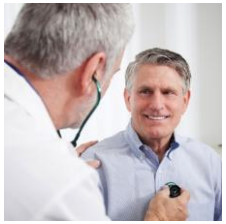
Our belief is that a new platform technology approach, flexible and applied to all medicinal products, will promote innovative approaches in product development and manufacturing and enable faster patients' access to innovative, high quality, efficacious and safe medicinal products






Thank you