EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Webinar on the use of platform approaches in the non-clinical and clinical domains - Report

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Executive summary

During this European Medicines Agency webinar, regulators and medicines developers explored the use of platform approaches in the non-clinical and clinical development of medicines.

The use of prior knowledge is not new, but the proposal for the new European Union Pharmaceutical Legislation now formally introduces the concepts of platform technologies and platform marketing authorisation. The discussions highlighted how platform approaches can reduce redundant studies, streamline regulatory assessments and accelerate patient access while maintaining robust benefit–risk evaluation.

Veterinary experience with vaccine platform technology master files provided concrete evidence of feasibility, showing reduced assessment timelines and regulatory burden. Developers emphasised the scientific and economic value of platform approaches, especially for ultra-rare diseases and modulable technologies such as RNA, vector-based vaccines and antisense oligonucleotides.

Key challenges identified included the need for clear definitions, transparent evidentiary standards, effective lifecycle management of platforms, and mechanisms to enable data sharing while protecting intellectual property—particularly when platform technology master files owners and marketing authorisation holder are distinct.

Overall, participants agreed that continuous dialogue, practical guidance and shared learning will be critical to ensure platform approaches deliver measurable benefits in regulatory efficiency, innovation, and patient access across the European Union.

This report includes summaries of the talks provided by each speaker, an extraction of discussions held during the Question and Answer (Q&A) session and panel discussion, and key take aways. The report also contains a Question and Answer section with questions from the audience submitted via the online platform Slido that were not addressed during the webinar. In this last section, responses have been provided by panellists in writing after the webinar.

Introduction

The proposal for the European Union (EU) Pharmaceutical Legislation introduced the concepts of platform technology and platform marketing authorisation. These platform approaches allow to leverage data already assessed by regulatory authorities and offer greater flexibility in the approval of certain categories of medicinal products, such as individualised therapies. While platform approaches used for the manufacturing of medicinal products had been explored in previous EU multi-stakeholder discussions, their use in the non-clinical and clinical domains had not yet been addressed.

The European Medicines Agency (EMA) organised a webinar bringing together regulators and medicines developers to discuss the use of platform approaches in the non-clinical and clinical domains. The aim of this webinar was to explore the possible applications of platform approaches, identify challenges and opportunities to support more efficient development and evaluation of medicines, and prepare developers and regulators for the implementation of the new EU Pharmaceutical Legislation.

Presentations addressed existing EU regulatory frameworks for the development and marketing of individualised therapies (n=1) and for the use of prior knowledge in Marketing Authorisation Application (MAA) dossiers, veterinary experience with vaccine platform technology master files (vPTMFs) and the new EU Pharmaceutical Legislation introducing platform marketing authorisations and Platform Technology Master Files (PTMFs). Stakeholder perspectives highlighted scientific, operational and access-related challenges. Finally, speakers were invited to address questions from the audience during a panel discussion.

The webinar was broadcasted to approximately 1100 participants. The agenda, video recording and speakers' presentations are available online.

Welcome and opening remarks

Steffen Thirstrup (EMA) welcomed participants to the webinar on platform approaches. The concept of prior knowledge is not new, but the proposal for the new EU Pharmaceutical Legislation has now codified the concepts of platform technologies and platform marketing authorisation. The EMA chief medical officer highlighted the opportunity offered by these platform approaches to systematically capture and build on prior knowledge, creating a robust scientific and regulatory foundation for medicines development, in particular in the context of individualised therapy. The webinar aims to bring regulators and developers together to explore how the use of platform approaches can be facilitated and ensure the establishment of a framework that will accelerate patient access to novel, promising medicines to patients. Thanks were extended to the webinar contributors, including EMA colleagues, the European regulatory network and stakeholders.

Session 1: Leveraging platform approaches in the development of therapies in the EU

Current regulatory routes for individualised therapies in the EU

Ilona Reischl-Kock (Austrian Agency for Health and Food Safety [AGES], Chair of EMA Committee for Advanced Therapies [CAT]) outlined the regulatory pathways currently available for the development of individualised therapies and patient access in the EU, distinguishing the EU pharmaceutical framework from national exemptions. Most individualised medicinal products developed via the EU pharmaceutical framework may use prior knowledge thanks to a systematic manufacturing process and/or systematic target mechanism, such as autologous cell therapies using the same manufacturing process and testing steps, or a patient-specific RNA sequence delivered through an identical vector system. The presentation highlighted the importance of the systematisation of manufacturing approaches for the conduct of clinical trials and stressed that clinical trials on individualised therapies are possible as long as a certain degree of systematisation exists. While prior knowledge can replace new studies in certain areas, its acceptability depends on clarity of justification based on data and full assessor access to those data. Developers were advised to seek regulatory and scientific advice early, using the available national and EMA support tools, since individualised platforms remain a relatively new field that require case-specific regulatory interpretation.

Prior Knowledge in Chemistry, Manufacturing and Controls (CMC) Development

Ragini Shivji (EMA) provided a description of the regulatory experience using prior knowledge in the CMC domain. A number of guidance documents have been developed over the years to define the concept of prior knowledge in the CMC domain and to highlight how it can be used for certain parts of the MAA. Prior knowledge includes internal stakeholders experience gained from the development of similar medicines, robust published evidence and established scientific principles. The presentation emphasised that effective use of prior knowledge requires clear differentiation between platform-level data and product-specific evidence, and thorough justification of the applicability of the prior knowledge to subsequent developments. By reducing data generation to product-specific elements, use of prior knowledge allows to streamline development for stakeholders, streamline and reduce assessment time for assessors, ultimately accelerating patient access while maintaining robust data package. Developers were encouraged to seek Scientific Advice to obtain feedback on the strategy for use of prior knowledge, ensure that the data is inserted in the appropriate MAA location and that the appropriate level of granularity is provided.

Veterinary Vaccine Platform Technology Master Files (vPTMFs)

Javier Pozo Gonzalez (EMA) presented the experience from the veterinary sector with vPTMFs, a concept introduced in the EU Pharmaceutical Legislation for veterinary use that came into force in 2022. For veterinary medicines, platform technology refers to a collection of technologies using a common backbone, i.e. a vector or a carrier, that can be adapted by inserting different antigens or genes to produce new vaccines. A vPTMF allows developers to consolidate fixed platform elements common to all vaccines using this platform – mainly quality data, but also safety and efficacy data depending on the platform - into a single file. For new platform technologies not yet used in authorised vaccines, a vPTMF is certified in parallel with the submission of a new MAA. For platform technologies already used in authorised vaccines, vPTMF certification may be conducted either in parallel with a new MAA or as a separate vPTMF certification procedure. Once certified, the vPTMF can be referenced in

subsequent MAA without resubmitting redundant information. This system has already supported multiple approvals, demonstrating measurable reductions in regulatory questions and assessment timelines, and allowing rapid adaptation of vaccines to emerging pathogens. Future steps include applying the vPTMF to other types of vaccines, such as RNA and DNA vaccines, gain experience on the lifecycle management of the vPTMF and work towards a more harmonised international framework.

Platform approaches in the new EU Pharmaceutical Legislation

Florian Schmidt (European Commission) presented platform approaches as outlined in the new EU Pharmaceutical Legislation. The platform marketing authorisation permits elements of a product to remain fixed while others may vary within a single marketing authorisation dossier. This structure is particularly relevant for therapies targeting different variants of a pathogens and for individualised therapies and may become a crucial enabler for accommodating those therapies under the established marketing authorisation system. The PTMF provides a mean of certifying comprehensive, well-characterised, reproducible and standardised technological elements that can be reused across multiple products, such as delivery vectors and manufacturing processes. Certification may occur alongside the initial marketing authorisation or later, and recertification is only needed when substantive changes occur. Although certification provides regulatory clarity and validation, the full PTMF must still accompany each MAA to facilitate the assessor review of subsequent linked marketing authorisations. The new EU Pharmaceutical Legislation accommodates platforms developed and owned by non-commercial entities, for example, academics who would remain responsible for the lifecycle of the PTMF. However, the marketing authorisation holder (MAH) still remains responsible for the medicinal product. Detailed requirements will be defined in the revised Annex II during a transition period and until the new legislation will start to apply, extending to 2028.

Q&A

Ownership of the PTMF - The new EU Pharmaceutical Legislation does not set any limitation in the ownership of the PTMF. During the early stage of implementations, most scenarios where PTMF owner and MAH are the same entity are expected. However, the owner of the PTMF may differ from the marketing authorisation holder of the product(s) referring to the PTMF, multiple entities may share the ownership of a PTMF and ownership is open to academia.

Lessons learned from vPTMFs - EMA certified 4 vPTMFs since the recent implementation of veterinary vaccine platforms. Initial feedback from both industry and regulators is positive, highlighting reduced administrative burden and streamlined regulatory assessment process. One challenge lies in various levels of familiarity and understanding with these approaches across stakeholders, and discussions with companies are fundamental. Another challenge lies in distinguishing core elements from product-specific elements. Companies need to carefully consider what to include in the master file, according to their pipeline, in order to optimise the use of the scheme and avoid updates across the lifecycle.

Use of prior knowledge versus PTMF - Prior knowledge has already been used by developers and accepted by regulators. If a specific technology is not eligible for a PTMF, developers will still have the possibility to use prior knowledge. In this case, including existing data into a specific product's dossier and justifying the re-use of those data for this product development will be required.

Platform's lifecycle management - In comparison to an Active Substance Master File (ASMF), which entails distinct, established datasets, PTMFs will be more heterogeneous. It will be crucial for the PTMF holder to clarify which elements are variable, product-specific and which other elements can be

accepted as a core package to ensure that the platform technology remains viable throughout its lifecycle.

EU regulators views on the use AI-enabled discovery platforms in medicines development - Medicines regulators acknowledge that selection of lead candidates may benefit from the use of AI. However, discovery stage related to the selection of lead candidates is not covered by the EU Pharmaceutical legislation and medicines regulators only intervene in the next steps, to assess the data supporting the benefit-risk assessment of selected lead candidates. Use of AI shall comply with the relevant EU and national legislations.

Session 2: Non-clinical and clinical case studies

Stakeholder perspectives on the implementation of platform technologies in the EU regulation: results from a focus group study

Stephanie Oskam (Utrecht University, Medicines Evaluation Board [MEB]) presented the findings of a regulatory-science study examining stakeholder views on platform technologies. The study showed that platform technologies can substantially improve feasibility in ultra-rare disease development by reducing costs, limiting redundant animal studies, and enabling structured reuse of data. Stakeholders nonetheless stressed the need for clearer definitions of platform boundaries, transparent evidentiary standards in small-data settings and update procedures that avoid generating disproportionate regulatory workload.

Case 1 (academia)

Annemieke M. Aartsma-Rus (Leiden University Medical Center) presented the use of platform approaches for antisense oligonucleotides (ASOs). Standardising toxicology strategies, dosing principles and safety-monitoring frameworks across related ASO developments could create major efficiency gains. However, practical constraints include high assay-validation costs, lack of access to failed-development data from safety or efficiency aspects, insufficient natural history information in ultra-rare diseases and the absence of the shared infrastructure required for coordinated academic development.

Case 2 (large pharmaceutical company)

Mihai Bilanin (European Federation of Pharmaceutical Industries and Associations [EFPIA], Vaccines Europe) highlighted the benefits and requirements of maintaining technological platforms - particularly RNA and vector-based systems - across multiple products. Consistent manufacturing, analytical and non-clinical elements can support meaningful reuse of prior knowledge, thereby reducing study duplication and accelerating early development. The presentation also underscored the need for clear lifecycle-management guidance, firm delineation between platform-level and product-specific requirements, a framework supporting innovation by the non-duplication of information between the PTMF and MAA and by its applicability to clinical trial applications (CTA)/investigational medicinal product dossiers (IMPD), and international regulatory alignment to ensure predictable and efficient platform use.

Case 3 (small and mid-sized enterprises)

Alexander Natz (European Confederation of Pharmaceutical Entrepreneurs [EUCOPE]) provided an overview of platform approaches from the perspective of small and mid-sized developers.

Platforms were identified as a mean to increase the economic viability of highly individualised therapies. However, fragmented national reimbursement systems continue to restrict real-world patient access. The presentation underlined the need for contractual and regulatory tools that enable shared platform use when platform ownership and product development are separate, while safeguarding confidentiality and intellectual property.

Session 3: Panel discussion

Panellists: Annemieke M. Aartsma-Rus (Leiden University Medical Center), Mihai Bilanin (EFPIA, Vaccines Europe), Thomas Girard (EMA), Alexander Natz (EUCOPE), Stephanie Oskam (Utrecht University, CBG-MEB), Marjon Pasmooij (CBG-MEB, Utrecht University), Javier Pozo Gonzalez (EMA), Ilona Reischl-Kok (AGES-MEA, CAT chair), Ragini Shivji (EMA), Florian Schmidt (European Commission).

Definition of platform - The term "platform" is used in different contexts and covered different concepts, e.g. discovery platform, platform clinical trials, platform technologies or platform marketing authorisations. Platforms that are not strictly related to the development, manufacturing and quality testing of investigational medicines, such as platform intended for discovery purposes, do not fall within the scope of the EU Pharmaceutical Legislation. Regulators further highlighted the importance to distinguish prior knowledge from platform technologies. Although prior knowledge and platform technologies may co-exist, leveraging prior knowledge will not necessarily require the establishment of a PTMF.

Opportunities offered by platform approaches from an academic and SME perspective, in particular for complex or ultra-rare therapies - Two key limitations of named patient programs were highlighted. Data collection is not systematised outside the context of clinical trials and requirements for named patient use lie in the remit of EU Member States, hampering cross-border scale up of those programs. In addition, small entities may not afford the cost of marketing authorisation maintenance. From the academic and SME perspectives, platform approaches, potentially owned by academics, represent an opportunity for flexibility, allowing collaboration across stakeholders with the view of systematising the data collected and scaled-up access to ultra-rare or complex therapies beyond national borders.

Measuring the impact of PTMFs – Beyond the mere number of PTMFs, it will be important to measure the benefits of these platforms for stakeholders in the context of medicines development and building of MAA dossiers. On the regulatory side, the expected benefit would be a streamlined and faster assessment of MAA dossiers.

Requirements to resubmit data on platform technologies with every new MAA - It was discussed whether stakeholders shall re-submit the full set of data included in the PTMF. It was clarified that indeed the full dataset should be submitted - regulators must have access to the full data package to assess dossier and the MAH needs to retain full responsibility on product development. The industry position was reiterated that mechanisms can be found to allow non-duplication of platform data while ensuring that the totality of MAA data available for review by regulatory authorities remains unchanged.

Confidentiality agreements between PTMF owner and MAH - It was noted that the regulators need to have access to full dataset and the MAH needs to be able to have knowledge to take the full responsibility on the quality, efficacy and safety of the finished product. This may lead to situations where the PTMF owner would also need to be the MAH. It was raised by industry that as well as full data access for the regulators, access of relevant data for the MAH without infringing intellectual

property may be required. Industry suggested interactions with stakeholders will be needed to further develop tools to address this issue in the update of annex II and additional guidance.

PTMF lifecycle management – The established variation route might be used to regulate the lifecycle management process. Interactions with stakeholders will be important to ensure the most efficient implementation of the PTMF.

Link between PTMF and MAA - On the veterinary side, MAA and PTMF are linked and the two dossiers are assessed by the same rapporteur and co-rapporteur. On the human side as well, the PTMF should be connected to a MAA, and could be submitted either during the MAA or after for the purpose of obtaining a certified PTMF.

Key takeaways and next steps

- Clarify opportunities of regulatory tools available to developers.
- Agree on definitions of key concepts to ensure a clear understanding of the framework as basis for further discussions, including platform clinical trials, platform marketing authorisation, platform technology (CMC), platform technology (Non-Clinical and Clinical), PTMF.
- The proposal for the new EU Pharmaceutical Legislation is purposely not too technically prescriptive on platform approaches as it may encompass heterogenous situations. Guidance will be key to clarify technical aspects such as PTMF ownership and accountability, sharing of data and lifecycle management.
- Continuous dialogue and engagement between regulators and stakeholders is needed to ensure that PTMFs are fit to the various use cases, used where needed only and ultimately if it is expected to foster streamlined regulatory MAA submissions and regulatory MAA assessments. Guidance might be updated as experience is gained.
- The EMA/HMA European Platform for Regulatory Science Research can be used as a forum to discuss scientific evidence needed among stakeholders.

Slido Q&A

Regulatory tools and definitions

- **What is the difference between prior knowledge and data extrapolation**

  Prior knowledge is a concept commonly used in the quality and manufacturing domains and refers to developers knowledge gathered from previous development and manufacturing experience **-** e.g. experience based on similar compounds, products and processes - as well as reference to scientific and technical publications or application of established scientific principles.

  Extrapolation refers to the extension of prior knowledge to novel situations or products.

- **What is the EMA Qualification of Novel Methodologies and how does it relate to PTMF certification?**

  EMA Qualification of Novel Methodologies (QoNM) and PTMF certification are two distinct regulatory tools.

QoNM is a voluntary advice mechanism established to support the development, evaluation and regulatory endorsement of innovative methodologies in specific contexts of use. Methodologies eligible to QoNM refer to approaches allowing to generate evidence supporting benefit-risk assessment and decision making of medicinal products (e.g., a novel biomarker or an in vitro test intended for safety assessment). Qualification of methodologies under the QoNM is not linked to specific product developments, CTAs or MAAs. Instead, these methodologies should have a broad applicability in the sense that they can be used in different medicines development programmes by different developers. For more information on QoNM, please refer to Qualification of novel methodologies for medicine development | European Medicines Agency (EMA)

PTMF is a regulatory tool aimed at facilitating MAAs by leveraging data already assessed by regulatory authorities. Platform technologies eligible for PTMF refer to a well-characterised, reproducible and standardised set of technologies that have the potential to be incorporated in, or used by, more than one medicinal product. Applications for PTMF eligibility are submitted as part of a MAA or subsequently.

- **Do you expect certain innovative platform technologies will not only qualify for the new concepts of platform marketing authorisation or platform master file but also as a regulatory sandbox? If so, which pathway is to be followed?**

  The regulatory sandbox is an instrument to test innovative aspects of medicines and medicine developments that do not yet fit the regulatory framework for medicines, but would positively contribute to it. For platform technologies we have already passed this stage, as the new EU Pharmaceutical Legislation includes specific provisions to accommodate platform approaches. This being said, the network is open receiving proposals for regulatory sandboxes meeting the legislative requirements.

Regulatory routes for individualised therapies

- **Is a clinical study for n=1 not possible? Or even preferred as a named patient often does not allow gathering efficacy and safety data that can be used in a marketing authorisation?**

  Clinical trials for individualised therapies are possible as long as a certain degree of systematisation in the collection of data exists.

- **One of major limitation in the current legislation is that data generated by special provisions (named patient use, compassionate use or hospital exemption) have limited use for registration purposes. Would the use of platform technologies allow registration?**

  Platform technologies aim at facilitating the re-use of data in the context of MAA – it does not define the level of evidence needed to support the establishment of the quality, safety and efficacy of a MA.

- **What is the reasoning for and applicability of testing individual products in a single clinical trial - even if targeting different sites on one gene? Wouldn't that imply additional risks, given the results could be very divergent?**

  It is important to clearly distinguish the concepts of platform technologies and PTMF from the complex clinical trials, including platform clinical trials. For more information on platform clinical trials, please refer to Complex clinical trials – Questions and answers.

PTMF - general questions

- **Will a human version of the PTMF use the same principles as for the veterinarian one? If not, why and what differences are foreseen.**

  The implementation of the human PTMF is ongoing and we cannot comment as of now, but learnings from the vPTMF will certainly be taken into account when shaping operational details, in line with the new EU Pharmaceutical Legislation for human use.

  From an industry perspective, while the vPTMF has been a welcomed innovation, there are areas of improvement that could be applied also for the human version, such as:

  - industry proposal for the human version to (same as vPTMF) not duplicate the same PTMF information in the MAA as mechanisms can be found to fulfil the MAA and MAH requirements
  - allow PTMF applicability also to EU CTAs (for its IMPD part)
  - introduction of a 3rd party Intellectual Protection mechanism, (like a Restricted and Applicant Part(s), similar to the ASMF), as it can foster opportunities for joint co-development.
  - update its related MAAs only if there is a potential product impact (reducing "administrative information/certificate" only updates due to recertification of PTMF for minor changes).

- **It was mentioned that for veterinary vPTMF, the experience has shown that the process has sped up additional assessments. Could it be quantified more specifically?**

  Given the limited experience to date, it is not yet possible to quantify the impact of vPTMF use more specifically. Initial feedback from both regulators and applicants indicates that the use of a certified vPTMF in support of a new MAA contributed to a more streamlined assessment, with fewer questions following the initial assessment, reduced time needed for applicants to prepare responses, and an overall shortening of the time from submission to approval.

- **Can the PTMF pathway described for vaccines be applied to gene therapy products using viral vectors as delivery system (i.e. AAV-mediated gene therapy products)?**

  The PTMF provisions in the EU Pharmaceutical Legislation for veterinary use are only applicable to vaccines and cannot be applied to other types of veterinary medicinal products as of now. In the new EU Pharmaceutical Legislation for human use, PTMF provisions are not restricted to vaccines and can indeed be applied beyond these medicinal products.

- **You referred to application to individualised patients (n=1) on a single gene. Would you consider, similar to vaccine examples you presented, master file for common technology and platform master file with different therapeutic genes?**

  The implementation of the human PTMF is ongoing and we cannot comment as of now.

- **Can multiple platform sets/modules be leveraged for a single MAA?**

  In principle, the proposed new EU Pharmaceutical Legislation does not prohibit the use of multiple PTMFs for a single MAA. However, we will first need to gain experience with this concept and ensure that the use of platform technologies simplifies medicinal product development.

PTMFs and clinical trials

- **For platforms do you advise to request Scientific Advice for each new therapy under the platform, or do we leverage the original discussion points on the platform feedback?**

  The need to request Scientific Advice should be assessed on a case-by-case basis by each medicine developer. As experience with a given platform technology increases, developers may progressively gain confidence in relying on previously agreed principles without necessarily seeking Scientific Advice for each new therapy.

- **The PTMF needs to be established during clinical development, therefore it should be established in collaboration with a national regulatory agency, correct? How is this implemented?**

  Platform technologies need to be distinguished from platform clinical trials. It is recommended to seek national or EMA Scientific Advice to obtain feedback on the establishment of platform technologies and PTMF as part of a MAA, or obtain guidance on the best clinical trial design to generate robust information in the context of clinical trials.

- **Would the platform approaches also be applicable to ATMPs, both in non-clinical and clinical settings and for MAA?**

  Yes, platform approaches apply in the same way to ATMPs as to other medicinal products.

Lifecycle management of PTMFs

- **What do you see as main challenges of the lifecycle of a platform technologies master files?**

  Key aspects to consider for the lifecycle management of a PTMF include a clear, data-supported delineation by the PTMF holder between those data that are contained within the platform (i.e., fixed part, proposed to reside only in the PTMF) and product-specific elements (i.e., variable part, proposed to reside and be justified only in the MAA). This will allow for the assessment of the impact of PTMF modifications on the linked products and a more predictable regulatory route allowing for modifications in the PTMF and linked marketing authorisations.

  From an industry perspective, a proposal for a simplification of the framework include:

  - non-duplication of information in the PTMF and in its related MAAs;
  - ensuring PTMF content and format alignment to the MAA standard (e.g., CTD, Annex II), so that any section of PTMF can be used interchangeably with the MAA;
  - updating related MAAs only if there is a potential product impact (reducing "administrative information" only updates).

- **Not all updates to a "platform" will be equally applicable, or ideal for each product which is derived from the platform. How do you plan to solve this challenge, if developers prefer to keep one master platform and avoid resubmission?**

  It is anticipated that a platform by its very nature comprises a set of data that are applicable to all linked products.

Data sharing and collaboration

- **How can we foster quality data generation and ensure sharing and re-use of data / building knowledge?**

  Increased interactions between all stakeholders, including academic and industry stakeholders, is needed. Certain areas of development would particularly benefit from such collaborations and data sharing. Academic developers need support and education to allow collection of quality data. The possibility to re-use and share data should be also considered at the consenting stage with the patients.

- **How can platform approaches support the use of prior knowledge also between different developers?**

  Developers are encouraged to share and publish data as far as feasible, to further knowledge of all stakeholders in a specific area. Notably, platform approaches will allow to scale up the generation of information for compounds falling under the same platform approach. While these data will be likely collected for a limited set of patients per compound, multiple compounds will be tested in parallel. This will be important not only to optimise further compounds under the same platform approach, but will allow to facilitate the development of compounds of the approach applying to larger cohorts outside of a platform setting.

  From an industry perspective, including an Intellectual Property protection mechanism in the upcoming delegated acts of the new EU Pharmaceutical Legislation (like a closed and open PTMF part, similar to the ASMF) can foster innovation by allowing leverage of proprietary prior knowledge, thus increasing the opportunities for joint co-development of innovative medicinal products.

- **With platform marketing authorisations, approvals may expand rapidly, yet capacity and funding will likely remain scarce. Is there any discussion at regulatory or cross-stakeholder level about how prioritisation between eligible patients should be governed?**

  We acknowledge the importance of the topic, which should be tackled with a broad stakeholder perspective beyond regulators. Notably, the 1 Mutation 1 Medicine (1M1M) initiative has already published a best practice framework on eligibility assessment for individualised ASO treatments for patients with severely debilitating progressive neurological diseases (https://pubmed.ncbi.nlm.nih.gov/40727088/ and https://pubmed.ncbi.nlm.nih.gov/34591693/) .

Legislation

- **What implementing and delegated Acts do you foresee for a successful implementation on non-clinical and clinical platform framework?**

  No implementing and delegated acts is foreseen for the implementation of non-clinical and clinical platforms.

- **When can we see the finalised legal text for the EU Pharmaceutical Legislation?**

  The finalised EU Pharmaceutical Legislation is expected Q4 2026. Publication of the final text will be followed by a 2-year implementation period before full entry into force.

- **The word "platform" is used in several different contexts. Hence it would be helpful if the legislative text add some specifics to the term used for the scope of today's discussion. Are there such plans for the definition?**

  The legislation defines 'platform technology' and 'platform technology master file' in relation to the possibility of EMA certification. Additionally, the meaning of a 'platform marketing authorisation' is also specified in the new EU Pharmaceutical Legislation.

EU/FDA

- **Is this platform approach shared with other regulations like FDA or is it uniquely used in EU?**

  The new EU Pharmaceutical Legislation is applicable in the EU only. Exchanges amongst international regulators, including the FDA, will allow better alignment of the regulation of platforms in different jurisdictions.

Interplay with MDR/IVDR frameworks

- **Can platform approaches also be applied to data supporting compatibility with drug-delivery devices and "reuse" of Notified Body opinion report for multiple MAAs.**

  The concept of platform approaches as proposed by the new EU Pharmaceutical Legislation will apply to medicinal products only.

Non-clinical testing

- **Would it be possible that in the future, platform technology (i.e. for genetic therapies like RNA) could allow for reduced animal studies, due to difference in genetics? This in the scope of ethical use of test animals and animal suffering.**

  Platform approaches and more generally use of prior knowledge allows to optimise non-clinical programs in line with the 3Rs principles (reducing, refining, and replacing of animals).