MEB Science Day 2025 – Platform Technologies: opportunities and challenges

Platform technologies have recently gained increased attention due to their potential to advance medicines development. A growing number of vaccines, antisense oligonucleotides and advanced therapy medicinal products (ATMPs) are being produced using platform technologies. Moreover, in the 2023 proposal for the EU pharmaceutical legislation for human medicines platform technology has been included as a novel approach to approve medicines with a fixed and variable component.

The opportunities and challenges of platform technologies were therefore the main theme of this year's MEB Science Day. The event, which took place at TivoliVredenburg in Utrecht on 19 May 2025, was chaired by prof. Eric Boersma, chair of the MEB's Science Committee and professor of Clinical epidemiology of cardiovascular diseases at Erasmus MC, University Medical Centre, Rotterdam. In his opening remarks, he introduced this year's theme: Platform Technologies: opportunities and challenges. Before exploring key questions such as: *What defines a platform? What are the benefits? Are platforms sufficiently adaptable?*, the event began with the release and presentation of the new MEB Science Policy 2025-2029.

MEB Science Policy 2025-2029

The release of the MEB Science Policy 2025-2029 sets a clear direction for the agency's scientific priorities for the coming years. The MEB aims to keep improving and innovating regulatory science by publishing a new policy every five years. Dr. Marjon Pasmooij, Head of the Science Department at the MEB, presented the key points of the newly released policy.

The 2025-2029 policy presents four main themes the MEB commits to work on:

1. Replacement, reduction and refinement of animal studies (3Rs);
2. Data-driven assessment;
3. Personalised medicine;
4. New pharmaceutical legislation.

The first three themes are a continuation from the previous policy (2020-2024). The fourth theme has been newly added in response to the European Commission's (EC) 2023 proposal to revise the European Union (EU) pharmaceutical legislation.

Dr. Pasmooij elaborated on each of these themes. First, the 3Rs theme focuses on evaluating the added value of animal research in medicine development. In addition, it concentrates on contributing to reducing the number of animals used in research by assessing the applicability of alternative models such as organoids.

Second, the data-driven assessment theme promotes the integration of real-world data, patient experience and participation, model-informed decision-making, and structured data systems such as the European Medicines Regulatory Database. All together this supports more informed regulatory decisions.

The third theme, personalised medicine, focuses on tailoring treatments to individual patients, especially those with rare diseases. This is achieved by supporting the development and regulatory evaluation of advanced therapy medicinal products (ATMPs), the use of biomarkers, and the integration of technological innovations.

The fourth and final theme reflects the once-in-a-generation revision of the EU pharmaceutical legislation. The MEB will conduct system-level research to support the optimal implementation of topics such as platform technologies and drug repurposing.

To conclude, Dr. Pasmooij underlined the importance of collaboration. The MEB is currently connected to 26 PhD students affiliated with various Dutch universities. She highlighted the agency's involvement in national and European initiatives, such as the European Platform for Regulatory Science Research. These collaborations are essential to maintain a forward-looking, evidence-based regulatory system.

Introduction to the topic of Platform Technology

As platform technology is a concept in the proposed revision of the EU pharmaceutical legislation, it was fittingly chosen as the central theme of this year's Science Day. The second presentation of the day offered an introduction to the topic by Dr. Falk Ehmann, Head of Innovation and Development Accelerator & Regulatory Science and Innovation Task Force at European Medicines Agency (EMA). He provided a broad overview of the concept including potential definitions, anticipated benefits, and challenges within the regulatory and scientific landscape.

As Dr. Ehmann explained, there is currently no universally accepted definition of 'platform technology'. Several key stakeholders interpret the term differently. For example, the United States Food and Drug Administration (FDA) primarily links the concept to medicine manufacturing, focusing on reproducible technologies that can be reused across products. In contrast, the current EC's proposal refers to platform technology as an application for a marketing authorisation for a medicine with both a pre-defined fixed and variable component. This platform is especially relevant to treat different variants of a pathogen or for ultra-rare diseases.

Dr. Ehmann emphasized why we need platforms and the wide range of opportunities that platform technologies offer across the medicine lifecycle. They can streamline development, reduce duplication, accelerate responses to emerging health threats, and improve the scalability of innovation. For patients and healthcare systems, this could

mean faster access to new therapies and more affordable treatment options. For developers, platform technologies enable the reuse of data and infrastructure potentially across multiple products, reducing time, cost, and regulatory burden.

Drawing on EMA data, Dr. Ehmann noted that 34 relevant platform technology related procedures were identified between 2017 and 2024. Within these procedures, platform technologies were used most frequently in manufacturing, followed by non-clinical and clinical development, with Covid-19 being the most common targeted disease. Approximately 30% of platform technologies have an orphan designation, underscoring their role in rare disease treatment. The most frequent platform technologies were viral vector platforms (particularly adeno-associated viruses), followed by gene therapy platforms. Looking ahead, Dr. Ehmann remarked that platform technologies are expanding into areas such as mRNA-based cancer vaccines, cell therapy manufacturing platforms, and artificial intelligence (AI)-driven drug design platforms.

However, Dr. Ehmann also acknowledged the challenges of implementing such technologies. These include the complexity of life cycle management across medicines sharing a platform, regulatory and legal issues surrounding marketing authorisation applications involving multiple platforms, the question of data requirements across indications, and concerns about managing intellectual property.

Dr. Ehmann stressed that platform technology driven development should not be seen as a shortcut, but as a tailored, science-driven process. He encouraged the regulatory community to learn from existing regulatory tools and frameworks that share similarities with the platform concept, such as prior knowledge, the veterinary platform concept and biosimilar framework, to support efficient integration of platform technologies. He concluded that while platform technologies hold transformative potential, realising their benefits will require international alignment and cross-sector collaboration.

Learnings and reflections based on stakeholders' views presented at the EMA Quality Innovation Group Listen & Learn Focus Group meeting on Platform Technologies

In November 2024, the fourth Listen & Learn Focus Group (LLFG) meeting from the EMA's Quality Innovation Group (QIG) took place. Dr. Marcel Hoefnagel, senior quality assessor at the MEB & Chair of the QIG at EMA, shared insights from this meeting regarding platform technologies. The goal of this LLFG was to open a dialogue with stakeholders and encourage them to share their perspectives on the use of platform technologies in the quality domain. These stakeholders were given the opportunity to present case studies that use prior knowledge to support platform-based manufacturing approaches. The meeting also aimed to identify general scientific challenges associated with the use of platforms and possible solutions.

Dr. Hoefnagel explained that three end-to-end platform scenarios were anticipated for discussion. The first involved a platform based on utilising prior knowledge to manufacture medicines, each receiving their own marketing authorisation. The second scenario involved platform technology resulting in a single marketing authorisation rather than multiple marketing authorisations. In that scenario the focus lied on pandemic preparedness. A platform produces medicines against pathogens which are causing significant transboundary health risks. Lastly, the third scenario was a platform for the manufacturing of personalised or individualised medicines. In this scenario, one patient or group of patients leads to one product with one platform technology marketing authorisation. All three of these scenarios have the common desire to increase efficiency by reusing established data and processes, and accelerate patient access.

During the LLFG, several case studies on different platform technologies were presented by industry and academic stakeholders. One example focused on the use of standardised manufacturing process for viral vectors to accelerate vaccine development. Another involved mRNA-lipid nanoparticle platform technology, where shared scientific principles and manufacturing processes across products led to streamlined development. Some stakeholders also highlighted how modular chemistry, manufacturing and controls (CMC) platforms are particularly valuable for rare genetic diseases, where patient populations are small.

Dr. Hoefnagel shared a number of discussion topics from the meeting. One topic was the definition of a platform and how it differs from the use of prior knowledge. A platform could potentially streamline prior knowledge. Stakeholders agreed that platforms can help accelerate the development of more robust control strategies. Furthermore, the group debated whether a platform must be end-to-end, or whether modular approaches - in which individual steps in the manufacturing process are reused - could also qualify as platform technologies. Opinions varied on what is required to consider a platform established. Some stakeholders argued that one product might be sufficient, while others believed that a minimum of two is necessary. In either cases, it was noted that the relevance and robustness of the supporting data must be well-justified. Another important topic that Dr. Hoefnagel raised, focused on how much deviation is allowed before a platform stops being a platform. When discussing lifecycle management, stakeholders considered how changes to a shared platform might affect multiple medicines, and how such changes should be handled. Stakeholders also reflected on whether the platform concept might unintentionally stifle innovation.

In closing, Dr. Hoefnagel highlighted that platform technologies can accelerate product development. He noted that diverse interpretations of what constitutes a platform exist, and he stressed that for ultra-rare indications, it may be scientifically acceptable to leverage development data across different indications to demonstrate the robustness of the manufacturing approach.

Conceptualising opportunities and challenges for the parallel development of multiple Advanced Therapy Medicinal Products utilising a Platform Approach

Fondazione Telethon is a non-profit organisation dedicated to advancing treatments for rare diseases. Sean Russell, Head of Regulatory Affairs at Fondazione Telethon, presented a compelling case for leveraging platform technologies in the development of ATMPs for rare conditions. There are over 7,000 known rare diseases (of which 80% are genetic) and it is estimated that these diseases affect 263 to 446 million people worldwide. Yet for 95% of these rare diseases there is currently no specific treatment available. This highlights the urgent need for more efficient and scalable development models.

While ATMPs hold great promise, they are extremely costly to develop. It is roughly estimated, by AI, that approximately 30% of an ATMP's sale price goes toward recouping R&D costs. Against this backdrop, Russell proposed that platform-based development could offer a more sustainable and optimised path forward.

He presented a model in which multiple ATMPs can be developed in parallel using a modular platform. In this approach, shared components are reused across products. For example, in the development of products based on lentiviral vector transduced CD34+ haematopoietic stem cells, fixed elements - such as the cell line, vector (and its stability), and cryopreserved cell stability - remain the same. In contrast, product-specific components, such as the varying transgene, make it possible to target different rare diseases. This structure of having fixed elements, enables the developer to avoid repeating full sets of studies for each product and instead rely on smaller, more targeted experiments to confirm any differences. As a result, duplication is reduced, timelines are shortened, and per-product costs are decreased.

A full data package would be submitted for the lead indication, while subsequent products can reference the existing platform dossier and submit reduced, targeted data. This "plug-and-play" scenario allows for faster development while maintaining regulatory robustness. Russell emphasised that such platforms should not be static but must be actively maintained and updated as new scientific knowledge becomes available.

Finally, Russell concluded by highlighting both the opportunities and challenges of this platform approach. On the one hand, platforms can significantly reduce R&D costs per product and per disease. He noted that the accumulation of knowledge across products can meaningfully inform the potential risks and benefits when translating research into clinical trials, ultimately benefiting patients. In his view, the greatest opportunity for platform technology is bringing a significant benefit to patients in the clinical development setting. Russell argued that waiting for commercially authorised products to complete the entire development process before leveraging that data and knowledge would be a waste. Platforms based on well-understood modules, combined with product-specific data, have the potential to expand patient access both in clinical trials and in

commercial use. On the other hand, several challenges remain: the lack of a clear definition of platform, the absence of a regulatory framework for maintaining and referencing platform dossiers, the need to provide sufficient supporting data to define the platform, and the difficulty of retrofitting existing programmes into a platform model.

After the first four presentations, the spotlight shifted to seventeen PhD students, each given a brief moment to pitch their research posters. This pitch session offered the audience a glimpse into the broad range of scientific projects the MEB is involved in. Each project could be further explored in more detail during the poster session in the break.

Non-clinical aspects of non-coding RNAs

Following the poster session, Britt Duijndam, non-clinical assessor at the MEB, explored the potential of platform approaches in the non-clinical development of oligonucleotide-based therapeutics (ONTs), specifically for non-coding ONTs. While platform approaches are already established for coding ONTs, like mRNA vaccines, she noted that they are more challenging to implement for non-coding variants such as antisense oligonucleotides (ASOs) and small interfering RNAs (siRNAs).

A key aspect of platform approaches is the ability to leverage data from similar products. However, for non-coding ONTs, the central challenge lies in defining what qualifies as "similar" and what constitutes a significant change. Is it a modification to the phosphate linker, the ribose, the sequence, or a conjugation to a receptor ligand such as GaINAc, which facilitates uptake in the liver? Depending on the specific endpoint, each of these factors may influence whether data from earlier products can be meaningfully extrapolated.

An ongoing project at the MEB, conducted in support of the ICH S13 guideline drafting group, created an overview of non-clinical safety testing strategies for ONTs, including those potentially eligible for platform approaches. For instance, Duijndam stated that platform approaches may be scientifically justified to assess the genotoxic potential of ONTs. If a group of ONTs share a known, previously tested chemical modification, existing genotoxicity data may be reused. Nevertheless, the project found that in practice only a small portion of ONT products currently omit genotoxicity testing or apply a reduced testing strategy, even when scientific justifications may exist.

Duijndam also discussed another example of how data from one ONT may be leveraged for another variant in the context of embryofoetal development (EFD) studies. She explained that even when certain modifications are present, these do not necessarily have a negative effect on EFD endpoints. In such cases, developers may justify extrapolating existing data.

Despite concerns, such as sequence- and chemistry-dependent effects, Duijndam presented that platform-like approaches can be applied to non-coding ONTs, depending on the (toxicological) endpoints being assessed. These approaches mark a gradual shift away from a checkbox-based approach and could justify leaner animal study programmes, in line with the 3Rs principle. She concluded by emphasizing the regulator's need for more data to substantiate similarity claims and to foster the development of alternative, non-animal testing methods in non-clinical development.

A scalable platform approach for preclinical and clinical development of patient-customized ASOs for brain diseases

The final presentation of the day was given by Prof. Matthis Synofzik, Head of the Research Division Translational Genomics of Neurodegenerative Diseases at the University of Tübingen. He discussed the use of scalable platform approaches in the preclinical and clinical development of patient-customized ASOs targeting rare neurological diseases (RNDs). Although there is an increase in RND drug development, most of the thousands of mainly genetic, ultrarare RNDs still lack treatment options. Traditional drug development and regulatory paradigms in medicine are not suited for ultra-rare diseases. For instance, clinical trials are unfeasible due to a lack of eligible patients. Prof. Synofzik stressed the urgent need for a systematic, scalable approach that can be applied across similar RNDs by targeting a shared biological principle. This would advance and streamline the development of ultra-individualized treatments.

He introduced an academically driven European platform, coordinated by universities in Tübingen, Heidelberg, and Leiden. This initiative aims to develop and implement patient-specific ASO therapies in a sustainable, scalable, time-critical and safe manner for eligible patients with severely debilitating or life-threatening rare neurological diseases. The platform is known as 1M1M – 'one mutation, one medicine'. It focusses on targeting a shared biological principle: splice modulation. This enables the same core strategy to be applied across a broad range of diseases and mutation types, including cryptic splice mutations, canonical splice defects, and frameshift mutations.

Prof. Synofzik explained that this platform compromises of two intertwined platforms: a platform drug embedded in a platform process. The platform drug consists of a fixed, the oligonucleotide backbone, and pre-defined variable component, the specific ASO sequence. The platform process it is embedded in, enables multiple ASOs to be developed simultaneously using the same biological principle: splice modulation. This modular approach, where only the sequence is adapted while maintaining the same chemistry and delivery method, makes the system highly scalable.

Prof. Synofzik also presented a platform trial design which allows for simultaneous assessment of the efficacy and safety of multiple patient-specific ASOs. Each trial arm

represents a different ASO, but all share harmonized endpoints. This design could generate regulatory acceptable evidence for very small patient populations, accelerating treatment access even in N-of-1 or N-of-few settings.

In closing, Prof. Synofzik underscored that this platform approach is not only medically or translationally promising, but also regulatorily relevant. He proposed that it could serve as a blueprint for the regulatory assessment of RNA therapy platforms. As RNA therapy platforms are promising, due to their inherently programmable nature, he expects the EMA to receive a growing number of applications for this type of platform. He further argued that the platform could extend beyond drug products to include platform processes within marketing authorisations. This would leverage the advantage of putting different products under a single marketing authorization, significantly accelerating access to therapies for ultra-rare diseases and reducing regulatory burden. Finally, Prof. Synofzik stated that a master protocol for a platform trial with very small group arms would allow for the generation of regulatory evidence of efficacy and safety for marketing authorisation even for ultra-rare diseases currently beyond the reach of standard pathways.

Panel Discussion

Following six thought-provoking presentations, the day concluded with an interactive panel discussion that invited the audience to further ask questions about the regulatory, scientific and practical implications of applying platform technologies. This discussion was chaired by Dr. Viktoriia Starokozhko, clinical assessor at the MEB & Member of the Scientific Advice Working Party at the EMA. The panel included the five speakers who had earlier presented on platform technology, Dr. Falk Ehmann, Dr. Marcel Hoefnagel, Sean Russell, Britt Duijndam, and Prof. Matthis Synofzik, alongside Dr. Emmely de Vries, senior clinical assessor at the MEB & Member of the Committee for Advanced Therapies at the EMA. Audience members could participate by submitting questions through Slido or by raising their hand to receive the square, throwable microphone cube, creating a lively and engaging atmosphere.

Several key topics were discussed, including the issue of data reusability and cross-referencing. While many acknowledged its potential to accelerate development and reduce duplication, some also raised critical questions about boundaries and justifications. What types of data can be reused? Under what conditions? And with what scientific rationale? These questions tied into broader questions about what exactly constitutes a platform, and how the definition of platform technology may apply depending on the type of product, disease area, or development context.

One panellist stressed the importance of early engagement and exchange with regulators to explore flexible pathways including platform technologies within regulatory

frameworks. The need for international alignment on definitions and regulatory expectations was also discussed. Harmonisation, especially in areas such as documentation standards, was seen as essential to ensure optimal implementation of platform technology.

Existing regulatory tools such as prior knowledge and the active substance master file were frequently mentioned as building blocks for platform-based development. A panellist referenced to the FDA's platform designation and suggested that the EU should diverge from the FDA's definition to offer clearer, more useful pathways and create a success story.

During the discussion the potential use of master files for platform technology was also debated. It was seen as a way to reference core platform data across multiple products, offering efficiencies in both evaluation and lifecycle management. Several panellists noted that different products could potentially be authorised under the same master file, depending on how variable components are defined. Also a need for modular platform approaches was underscored. Challenges with regards to the implementation and life-cycle management of such platforms were highlighted.

The use of regulatory sandboxes to pilot platform approaches was mentioned. In such a sandbox setting, developers and regulators could collaboratively test how much data is needed under different scenarios, test the implementation of master files, evaluate what benefit-risk thresholds are acceptable, and experiment how to responsibly use N=1 or N-of-few trial designs. These approaches would allow flexibility for highly individualised or ultra-rare treatments, provided the scientific rationale is solid. However, the panel also noted the challenge of using such a sandbox and the importance of precisely defining the scope of sandbox applications.

The sandbox discussion also opened up a discussion on marketing authorisations. Should the authorisation apply to the platform as a whole, to the product, or to the process? There was interest in exploring options where different products could fall under a single marketing authorisation, assuming core platform elements remain consistent. This could offer major benefits in terms of regulatory efficiency and speed of access, but also introduces challenges related to lifecycle management, such as what happens when one element of a platform is modified. Provisions on platform marketing authorisation applications in the draft new pharmaceutical legislation were highlighted.

The issue of data sharing was also raised. Panellists reflected on how and when data must be shared to support safety and ensure responsible platform-based development. As one panellist stated: "If you don't have all the data, you can't be fully responsible, but you should share enough to be responsible."

Toward the end of the discussion, Dr. Starokozhko invited panellists to reflect on what the biggest challenges of platform technology are. Responses varied, and besides the already

mentioned challenges, one extra challenge mentioned was the need to integrate and/or establish a fair and transparent reimbursement system.

This year's fascinating MEB Science Day, was wrapped up by Prof. Ton de Boer, chair of the MEB & Emeritus Professor of Pharmacotherapy, Utrecht University. He, just as the other participants, enjoyed learning about the innovative development, platform technology. He shortly reflected on the various presentations and highlighted the valuable contribution the PhD students had to the MEB Science Day. He found it fascinating to learn more about several projects ongoing within the MEB. He closed off by stating that platform technologies hold many advantages, including lowering costs, reducing duplication efforts, speeding up drug development and accelerating patient access.

Prof. Boersma closed the day by once again thanking the presenters, the participants, Dr. Marjon Pasmooij and Sietske Oosterhoff, secretary of the science department at the MEB, who all made this day possible. It was a successful edition of the MEB Science Day, and the organisation looks forward to seeing everybody again at next year's edition.

Post-meeting note

The MEB will continue on working on the topic of platform technologies in the coming years as part of our MEB Science Policy 2025-2029. In collaboration with FAST-NL and Utrecht University, focus groups have been conducted to explore the perspectives of different stakeholders on the definition, utilization and regulatory and clinical acceptability of platform technology. The insights gained from this study will contribute to the upcoming discussions on the implementation of platform technology, which will become especially relevant once the new pharmaceutical legislation is adopted (expected by the end of 2025 at the earliest).

In addition to this report, you can also take a look at the presentations, the 'Top 5 Opportunities and Challenges for Platform Technologies' and a short summary of the report from the MEB Science Day.