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.

The day also marked the launch of the MEB Science Policy 2025-2029, presented by Dr. Marjon Pasmooij (MEB). The policy outlines four key themes that the MEB will focus on in the coming years: replacement, reduction and refinement of animal studies (3Rs), data-driven assessment, personalised medicine, and the new pharmaceutical legislation.

Dr. Falk Ehmann (EMA) introduced the concept of platform technologies, highlighting the absence of a single definition. He emphasized the potential of platform technology to streamline development, reduce duplication, accelerate responses to emerging health threats, and improve the scalability of innovation. Furthermore, Dr. Ehmann addressed key challenges, including lifecycle management, regulatory complexities, and data requirements. He encouraged drawing on existing regulatory tools to support the integration of platform technology.

Dr. Marcel Hoefnagel (MEB/EMA) shared insights from the EMA Quality Innovation Group's fourth Listen & Learn Focus Group (LLFG) meeting on platform technologies. Three end-to-end platform scenarios were explored, ranging from using prior knowledge to personalised medicine. Key discussion points that arose during the meeting included defining a platform, modular platform approaches, and acceptable levels of deviation within a platform.

Sean Russell (Fondazione Telethon) presented a modular platform approach for the development of multiple advanced therapy medicinal products (ATMPs) targeting rare diseases. By reusing fixed elements across products and submitting reduced data packages, this approach can lower costs, shorten timelines, and provide a significant benefit to patients, especially in the clinical development setting.

Britt Duijndam (MEB) explored the potential of platform approaches in the non-clinical development of oligonucleotide-based therapeutics (ONTs), specifically for non-coding ONTs. She outlined cases where existing safety data could be reused across similar ONT variants, depending on the endpoints assessed. She also emphasized the need for more data to support similarity claims and to advance the reduction of animal testing.

Prof. Matthis Synofzik (University of Tübingen) explained the 1M1M platform: 'one mutation, one medicine'. This initiative aims to develop and implement patient-specific antisense oligonucleotide (ASO) therapies for rare neurological diseases in a sustainable, scalable, time-critical and safe manner by targeting a shared biological principle: splice modulation. He also briefly touched upon a platform trial design which allows for simultaneous assessment of the efficacy and safety of multiple patient-specific ASOs.

The panel discussion, moderated by Dr. Viktoriia Starokozhko (MEB), discussed data reusability, platform ambiguity, and the need for international regulatory harmonisation. Additional topics included the use of master files, regulatory sandboxes to pilot new technologies or frameworks, and the possibility of shared marketing authorisations.

This year's insightful MEB Science Day concluded with closing remarks by Prof. Ton de Boer (MEB/UU), who highlighted the potential of platform technologies to reduce costs, avoid duplication, and accelerate access to innovative medicines.

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 summary report, you can also take a look at the presentations, the 'Top 5 Opportunities and Challenges for Platform Technologies' and a more detailed report from the MEB Science Day.