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Highlight report from the 13th Industry stakeholder platform on research and development support

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Role	Name
Co-chairs:	Michael Berntgen and Iordanis Gravanis
Participants:	Industry: AESGP Christelle Anquez-Traxler; ARM Marcello Milano; EFPIA Gesine Bejeuhr, Sini Eskola, Jyo Krishnan, Nadege Le Roux, Laura Oliveira, Cathelijne de Gram, Almath Spooner; EUCOPE Mariska Mulders, Shekhar Natarajan, Maren von Fritschen, Seán Byrne, João Duarte, Lucia d'Apote; EuropaBio Lorcan Allen, Alexa Hunter, Bettina Dopper, Pedro Franco. EMA: Stiina Aarum, Ralph Bax, Michael Berntgen, Kevin Cunningham, Falk Ehmann, Maria Filancia, Iordanis Gravanis, Kristina Larsson, Jane Moseley, Chrissi Pallidis, Marie-Helene Pinheiro, Paola Samassa, Enrico Tognana, Tarita Toufexi, Patrice Verpillat, Thorsten Vetter. European Commission: Helene Casaert, Belen Torres, Izabela Taborska. EMA scientific committees and European regulatory network: Brian Aylward.

This was the thirteenth meeting between regulators and representatives of industry stakeholders to address topics of evidence generation along the medicine's life cycle and related product-development support activities, such as scientific advice and qualification, as well as specifics for paediatric and orphan medicines. The aim of the platform is to provide an opportunity for both general updates and more focused discussions on specific processes or issues to support continuous improvement, and generally to foster a constructive dialogue with industry stakeholders.

As part of the introduction a review took place of the status of follow-up actions from the last platform meeting. Significant progress was made in accordance with the planned deliverables and timelines, and follow-up discussions took place at the 13th meeting, where required.

Experience with Portfolio and Technology meetings (PTMs)

EMA gave an overview of the initial feedback on the Portfolio and Technology meetings (PTMs) held in 2024. PTMs represent a new interaction platform with developers launched by EMA at the end of 2023. The aim of this platform is three-fold: identify issues impacting the progress of product portfolios, capture

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
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new and disruptive technology already in use, and anticipate the scientific and regulatory expertise needed to assess future applications. Views were collected after each PTM with a survey specifically designed to capture industry feedback on the Agency early engagements that foster innovation. Industry sentiment was also collected during the different phases of a PTM preparation. Industry showed a general appreciation for this platform indicating its added value but pointing at the same time towards some opportunities for further strengthening. Some of the suggestions were already onboarded in the call for PTMs to be held during the first half of 2025. The second call for PTMs to be held during the second half of next year will open in Q1-25.

FOLLOW-UP:

- Follow-up reporting on the second year of experience with PTMs by the end of 2025, alongside dedicated feedback from the associations
- Considering that horizon scanning methodologies are being developed further by both developers and regulators and relevance of such information increases, initiate an exchange between EMA and industry associations at the platform meetings in 2025

Development support offering for programme-specific evidence planning

EMA provided a number of updates: the 2024 scientific advice volume of procedures, which was 10% higher than the number of procedures started in 2023; the number of scientific advice discussion meetings in 2024 which remained in the same low levels (slightly less than 10% of procedures) since the beginning of the COVID-19 pandemic; the experience to date with the Scientific Advice Working Party/Clinical Trials Coordination Group (SAWP/CTCG) pilot which provides the unique opportunity for applicants to clarify both clinical trial and marketing authorisation requirements in a single interaction; the status of the PRIME pilots on expedited scientific advice, submission-readiness meetings and development tracker and the planned analysis of their outcomes once the pilots conclude in March 2025; the progress to date with the action plan on future-proofing the Qualification of Novel Methodologies; and finally, ongoing activities related to combination products comprising a medicine and a medical device and/or in vitro (including companion) diagnostic.

The proposal to make the decision whether to have a discussion meeting on the basis of a draft advice letter and with an extension of the scientific advice procedure by one month, mentioned in the previous platform meeting, is no longer being pursued. The option for shorter (1 hour in duration) discussion meetings to allow more of these to be held in the finite duration of a SAWP meeting is still being considered but the focus should be more on efficient use of time and less on the actual meeting duration. As the discussion on optimising the use of discussion meetings in scientific advice is continuing, the SAWP will attempt to introduce in some scientific advice procedures additional exchanges in the form of List of Issues to be addressed in writing in the first instance with a view to deciding whether to also hold an additional discussion meeting based on the responses received.

The SAWP/CTCG pilot has seen a moderate uptake to date despite the promise it holds for alignment within the EU development support ecosystem. The reasons are not clear, but emphasis was placed on combined studies/combination product developments being out of scope, on the lack of possibility to involve ethics committees and notified bodies for medical devices and on paediatric developments and the possibility to engage the PDCO in these cases. EMA reiterated that paediatric developments are well within the scope of the pilot and, given the focus placed on it from the regulators' side, the involvement of the PDCO in pilot cases will be ensured.

EMA also took the opportunity to announce the introduction of a Development Support Coordinator (DSC) role which will be piloted in 2025. The DSC will replace the PRIME scientific coordinator as primary point of contact for a subset of PRIME designated products. Having relevant therapeutic area expertise, the DSC will handle scientific advice requests and ensure adequate coordination with other development support regulatory interactions. Establishment of the role follows the principle of stewardship (also called 'lighthouse' or 'one-stop-shop') agreed in conclusion to the 2019-20 Focus Group on integrated development support.

FOLLOW-UP:

- On the basis of the feedback also from the sounding board, continue the optimisation initiatives to enhance the use and efficiency of discussion meetings
- Complete the review of the new features in PRIME to inform the closing report of the pilot
- Follow-up review of the experience with the SAWP-CTCG pilots once more experience is gained
- Industry associations to clarify the specifics around SAWP-CTCG scientific advice regarding involvement of PDCO
- Progress the Development Support Coordinator concept together with the sounding board

Supporting paediatric developments

Paediatric Scientific Advice

Following the discussions at the previous platform meeting, EMA had discussed with the sounding boards (scientific advice and paediatrics, resp.) a proposed guidance update on paediatric scientific advice. The updated guidance, finally published in December 2024, clarifies that any questions except those under the strict remit of the PDCO can be asked in a scientific advice request.

EMA further announced that a closer collaboration between the scientific advice and paediatric medicines EMA offices will be set up allowing scientific advice officers to consult their paediatric counterparts on the appropriateness of the questions asked, soliciting input on scientific aspects of the development vis-à-vis any existing PIP or other PIP precedents in the specific context and deciding on the possible need to involve PDCO Rapporteurs (existing PIP) or PDCO members with relevant clinical expertise (ahead of any PIP agreement) in the procedure.

FOLLOW-UP:

- Industry associations to advertise the Scientific Advise guidance update (further enhanced on the interaction with PDCO) and collect feedback on companies' experience in seeking scientific advice and how they saw the SAWP/PDCO interaction
- EMA to strengthen internally the involvement of paediatric expertise in scientific advice requests with a paediatric component, including additional PDCO involvement beyond the current default criteria, as required

Experience from the Stepwise PIP Pilot

The stepwise PIP pilot, initiated in February 2023, was presented from both the EMA/PDCO and industry perspectives. The pilot aimed to conclude after the adoption of eight opinions by the PDCO. As of now, 15 sPIPs have been submitted, with seven opinions adopted and the eighth expected in January 2025. Most submissions focused on rare paediatric genetic disorders, followed by oncology.

Most elements in clinical studies not defined at the initial opinion stage included the sample size and dose, followed by endpoints, statistical analysis, duration of study and control. All PIPs include conditions on how these elements will be defined once further data are generated. The pilot was well received by the EMA and PDCO and the pre-submission meetings offered to applicants were found to be useful. It was clarified that the stepwise PIP is designed for situations where many elements cannot be defined initially (e.g., studies in paediatric subsets), but its principles are also applied to other PIPs (e.g., when the dose cannot be defined at the initial submission).

From the industry perspective, feedback on pre-submission interactions with the EMA was generally positive, highlighting approachability, discussion, and flexibility. The current implementation was appreciated as it did not require separate procedural requirements or timetables, making the sPIP another pathway to a full PIP. Clear guidance on which PIPs are applicable to the sPIP approach was suggested, with potential criteria expansion beyond the pilot's scope.

Success factors for the pilot could include shorter clock stops, reducing the overall duration from PIP submission to opinion, and the possibility of pre-submission meetings or scientific advice during the PIP lifecycle before modifications are needed as data are generated. Proposed long-term KPIs include a lower percentage of companies submitting PIPs late, a higher number of aligned programs with the FDA, and fewer sPIP modifications compared to standard PIP modifications.

In conclusion, the sPIP pilot was well-received, with industry eager to integrate sPIP into business practices. The industry supports PDCO's flexible approach to stepwise elements in "standard" PIPs and is willing to collaborate on how the system will function under revised legislation and any anticipated adaptations.

FOLLOW-UP:

- EMA to prepare a report on the pilot once Opinions for all eight applications are available, and consult on the report with the sounding board
- Building on the experience from both sides (developer and regulators) prepare a joint journal publication
- Establish a monitoring for follow-up on agreed sPIP applications in terms of life-cycle management, including establishment of performance indicators based on the initial proposals

Focus group to explore opportunities for the use of Real-World Data (RWD) and the generation of Real-World Evidence (RWE)

An update has been shared following the set-up of this new focus group in Q2 2024. A kick-off meeting took place in September, focusing on the objectives and work planning. Topics to be discussed during the next meeting were presented, based on feedback received from industry associations.

FOLLOW-UP:

- Follow-up reporting on the progress of the focus group work at the next platform meeting

Operational update

Processing Scientific Advice requests regarding New Fee Regulation

EMA provided a comprehensive update on the forthcoming Fee Regulation (EU 2024/568), set to be implemented from 1st January 2025. The presentation highlighted the implications of the new Fee Regulation on development support processes (orphan medicines, paediatric medicines and scientific advice), with particular emphasis on the scientific advice process. A detailed explanation was provided on the operational framework for pre-payment of fees for scientific advice.

EMA addressed questions submitted by participants ahead of the meeting, ensuring clarity on the updates. A list of useful links and contact points was shared to support stakeholders in navigating the changes effectively.

FOLLOW-UP:

- Industry associations to disseminate the presented information widely among members to ensure awareness and preparedness for the upcoming changes.

Onboarding of paediatric procedures onto IRIS

EMA provided an update on the status of system improvement suggestions submitted by industry representatives involved in the development of IRIS Paediatrics, namely the ability for applicants to download all structured data submitted to EMA, and update of the application form for annual reports.

EMA addressed questions from participants during the session. Furthermore, it was agreed to analyse some industry proposals following submission of further details, to evaluate the proposed improvements effectively.

FOLLOW-UP:

- EMA to review the late-breaking feedback received from the industry associations (including further details to be provided) and decide on any necessary actions.