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Highlight report from the 14th Industry Stakeholder Platform on Research and Development Support

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Role	Name
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This was the fourteen 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 14th meeting, where required.

Development support through scientific advice

EMA highlighted the positive impact of the November 2024 public guidance update on paediatric scientific advice with all but one respective scientific advice requests processed without validation issues since then. Regarding the new pre-payment requirements since January 2025, EMA noted that there have been multiple instances of applicants missing pre-payment deadlines. EMA has exercised flexibility in the transition period, but as of July 2025 scientific advice applications for which pre-payment has not been made by the pre-set deadline will be postponed to start in the following month. A clarification has been introduced on the EMA scientific advice submission webpage.

EMA provided an update on the status of the Scientific Advice Working Party (SAWP)/Clinical Trials Coordination Group (CTCG) scientific advice pilot to date. Nine applications have been submitted to date and seven of these have been completed. Applicants' feedback is positive as they consider that the SAWP/CTCG interaction allows harmonization of clinical trial and marketing authorization requirements, identification of issues in advance and creates expectations for fewer request for information during clinical trial application assessment. On the other hand, concerns were expressed by industry representatives regarding lack of participation in the pilot of all Member States concerned in the intended clinical trial application and, generally, in relation to the European Medicines Regulatory Network (EMRN) awareness at the time of clinical trial application of the prior advice given. Moreover, requests were made by them for involvement of ethics committees and for extension of the pilot scope to combined studies and combination products, while it was also clarified that phase 2 studies are not excluded from the pilot currently.

On the topic of more interactive and agile scientific advice, industry representatives presented a well-developed proposal for shorter timelines and lighter process for scientific advice requests restricted to few questions, involving a single area of advice and not requiring referral to other committees and working parties. EMA acknowledged that changes to development plans are sometimes observed in the course of scientific advice procedures, a fact which attests to the need for more prompt regulatory feedback. However, EMA also expressed feasibility concerns relating to the industry proposal in the current environment and with the existing IT tools. Nevertheless, EMA committed to bring the proposal to the SAWP towards possibly tripartite discussions between SAWP, EMA and industry associations for further discussions on the topic.

Finally, EMA announced an upcoming update to the scientific advice briefing document template with the main purpose of improving its use by patient representatives and enhancing their input into scientific advice. Other changes revolve mainly around special populations.

Follow-up:

- EMA to bring the proposal from developers for introducing agility into scientific advice to the SAWP, with the aim of initiating tripartite discussions to further refine the proposal and co-create a pilot framework.
- Once the update of the briefing document is progressed, sharing of the draft with the sounding board for comments.
- For the SAWP-CTCG pilot, feedback from developers to discuss further with the EMRN and address within the final pilot report, in terms of 1) clarification in guidance and 2) structural optimisation.

Horizon scanning activities by different stakeholders

EMA presented its horizon scanning activities that aim at identifying early signals of novel developments that might pose regulatory challenges or provide opportunities for public health. Such activities include monitoring and screening of different data sources (scientific literature, funding calls, news, conference reports, third-party reports, EMA internal data) to identify relevant signals and drafting of deep-dive reports on selected topics. EMA works on horizon scanning together with the European Innovation Network (EU-IN), a HMA/EMA group that brings together representatives of innovation offices of national competent authorities and EMA. Each report not only describes the state-of-art of the specific development or topic but also regulatory challenges and recommendations. Examples of recently published reports are New Approach Methodologies, Nanotechnology-based medicinal products for human use, and Alzheimer's disease. Ultimately, horizon scanning contributes to fostering innovation, increasing preparedness and future-proofing the European Medicines Regulatory Network.

EMA also reported from a recent webinar to discuss horizon scanning methodologies together with other EU agencies and non-profit institutions performing horizon scanning. The workshop aimed at sharing best practices and fostering collaboration among institutions to ultimately increase the impact of horizon scanning activities.

Concerning the more immediate future, EMA highlighted its forecast activities and business intelligence gathering on marketing-authorisation applications. With a three years' time window, these activities are designed to enables accurate budgeting, properly plan workload and expertise, facilitate development and fostering innovation in medicine's developments.

Similarly, industry shared their view on horizon scanning which is performed at different levels both by companies and trade associations. The relevance of these activities was recognized noting the different scopes of horizon scanning between regulators and developers.

Follow-up:

- EMA to disseminate the report from the recent webinar on horizon scanning, once available. On this basis maintaining the dialogue on the topic.

Experience with the new features of the PRIME scheme

Following the conclusion of the 2-year pilot phase of the new PRIME scheme features (Regulatory Roadmap and Development Tracker, Expedited Scientific Advice (SA), and Submission Readiness Meeting (SRM)) EMA presented preliminary analyses of the pilot, including Rapporteurs' and PRIME product developers' experience with the features.

64 development trackers were submitted during the pilot, received as part of periodic development updates (31) and to support kick-off (23) and submission readiness (10) meetings. Rapporteur teams found the trackers effective for tracking development issues and regulatory submissions. Both assessors and developers suggested technical improvements. Developers requested more detailed instructions and a more user-friendly online tool. Assessors also suggested simplifying the table and solutions to simplify the dissemination of the tracker among assessment teams.

Based on 12 expedited SA requests submitted in the pilot, the expedited procedure demonstrated a reduction in the overall timeline from scientific document submission to the final advice letter, with an average duration of 50 days compared to 79 days for a standard SA. Both developers and assessors generally reported a positive view of the process. Developers suggested improvements, such as providing more agile advice outside published timelines and applying expedited SA to initial advice. Assessors emphasized the importance of preserving active review time to prevent detriment to the advice, and

recommending increasing predictability of expedited SA requests to facilitate the Rapporteur team participation.

10 SRMs were held during the pilot, and experience of the meeting was generally very positive in terms of scope and timing, and was found to be effective in identifying outstanding risks for the subsequent MAA, and for MAA preparation and assessment. In terms of further refinement, developers suggested more flexible timing for the meeting, and broader involvement of regulators and downstream decision makers. Assessors recommended a stronger focus on scientific advice issues and deviations.

In addition, Industry associations provide the results of surveys circulated to Industry developers irrespective of PRIME experience, to gauge perceptions of the scheme. The surveys highlighted that PRIME remains a core tool for regulatory support for many developers, and suggested improvements including broadening the PRIME eligibility criteria, further enhancing the early and continued engagement with Rapporteurs, and increasing global collaboration on development support.

Follow-up:

- EMA to finalise the report on the experience with the new features of the PRIME scheme and refine current working practices and tools on this basis. The sounding board will be consulted in this context.
- Industry to finalise the analysis of their survey and finetune recommendations from developer's perspective.
- Initiate planning for the 10-year anniversary and how to measure impact of PRIME, eg through case reviews.

Piloting of the product development coordinator for PRIME products

EMA announced the start of a pilot introducing a new role of a "Product Development Coordinator" (PDC), initially introduced for a subset of PRIME designated products. Establishing such role reflects previous calls from industry representatives for stewardship through development support activities. The PDC will replace the PRIME scientific coordinator as primary point of contact for PRIME designation holders and, with a background and expertise in the therapeutic area of the product, the PDC is expected to not only help navigate PRIME designation holders through the various regulatory interaction possibilities and respond to ad-hoc queries, but to also better support with discussions on evidence planning. The longer-term vision is for the PDC to be managing different types of regulatory interactions (scientific advice, paediatric investigation plan, orphan designation), but initially PDCs will only handle scientific advice requests for their assigned PRIME products. EMA suggested to measure the impact, added value and resource implication of the new role by establishing relevant performance indicators also together with developers.

Follow-up:

- Identification of performance indicators to measure impact of the single development support contact at EMA for developers, through discussions at the sounding board
- Communication by EMA to concerned developers if their PRIME product has a change in contact point

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

The focus group was established to share knowledge and experience with use of RWD and generation of RWE to advance integration of relevant and reliable RWE in regulatory decision making. The operation of the focus group is working well, with great engagement and attendance from the industry groups, and active exchanges and experience sharing.

So far in 2025, two meetings were held and focused on feasibility assessments in performing RWD studies, and on the HMA-EMA Catalogues of RWD sources and RWD studies as tools for data discoverability and transparency, and trialogue between industry, registry holders and EMA. Future meetings this year may focus on experience and role of RWE across medicinal products' lifecycle and data quality in study planning.

Industry representatives expressed their appreciation for the establishment of the focus group and the bidirectional dialogue. There is momentum on the topics discussed and the feedback supports especially the smaller companies. Industry is happy to share experience as regards RWE generation (by industry, and by regulators), as well as on special topics such as single arm trials/external comparator arms, given ongoing guidance development. Both EMA and industry expressed gratitude for and willingness for continued cooperation.

Follow-up:

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

Moving the stepwise Paediatric Investigation Plan (sPIP) concept from pilot to regular operations

An update on the stepwise Paediatric Investigation Plan (sPIP) initiative was presented by both EMA and industry representatives. The pilot phase concluded in January 2025, following the adoption of eight opinions by the Paediatric Committee (PDCO). The presentation provided an overview of the underlying principles of the stepwise PIP approach, along with data gathered during the pilot phase.

Other elements of the presentation included insights into the lifecycle of the sPIP and the experience gained from the first modification of such a procedure. This modification involved substantial updates to various aspects of a clinical study, informed by the results of a recently completed dose-finding study. These updates suggest that the stepwise approach is functioning as intended.

The lessons learned from the pilot are expected to inform the development of the procedure, which is currently proposed in the new pharmaceutical legislation. From the industry perspective, feedback on pre-submission interactions with the EMA was largely positive, with stakeholders highlighting the EMA's approachability, openness to discussion, and flexibility. The stepwise elements of the process were highly valued by industry, which expressed strong interest in continuing this practice. It was recommended that the guidance be clarified to explicitly state that stepwise elements may also be applied to PIPs that are not formally designated as sPIPs, when appropriate.

During the discussion, it was also suggested that the guidance should more strongly emphasise the importance of pre-submission meetings, particularly in the context of proposed modifications. Ongoing collaboration through the sounding board framework will continue to support the monitoring and development of the sPIP lifecycle.

Follow-up:

- EMA to publish the final report on the pilot

- Continue the development of a joint scientific publication on the experience
- Facilitate a monitoring of the follow-up on agreed sPIP applications in terms of life-cycle management through the sounding board

Early engagement meetings that foster innovation

Industry stakeholders attending in 2024 one or more early engagement meetings to foster innovation (Innovation Task Force briefing meetings (ITF BMs); Portfolio and technology meetings (PTMs); Quality Innovation Group (QIG) meetings (Listen and learn focus group and 1:1 meetings); Small and medium-sized enterprises briefing meetings (SMEs BMs) were surveyed.

Overall applicants shared positive feedback on the early engagement mechanisms, emphasizing their value and relevance to their needs. It was noted that the main areas flagged for improvement were: clarify/update published guidance to boost awareness and ensure preparedness; streamline timelines and organisational aspects; ensure more dialogue with relevant experts during the meeting. An update on how each recommendation is being implemented by the relevant EMA teams was presented.

Follow-up:

- EMA to progress the improvement of the various platform offerings and related communications, based on the opportunities identified through the survey
- Considering the additional proposals from developers, relevant teams will further reflect on these and EMA will follow up as needed

Delivery of the action plan to strengthen qualification of novel methodologies

EMA provided an update on the delivery of the action plan. Discussions progressed on the scope of Qualification of Novel Methodologies (QoNM), lifecycle management, early-interaction support and process improvement, external expert input during review for highly innovative methodologies, as well as ways to increase EMRN expert awareness/involvement. In terms of tools, work on general procedural guidance, briefing document templates as well as the webpage is progressing. Turning to Q&As, the update of Q&A on Qualification of methods related to DHT/AI/ML is planned to be published in 1Q26; the Q&A on Qualification of registries/data sources and the Q&A on Qualification of Clinical Outcome Assessments are both aimed for consultation in 1H26; the drafting of the Q&A on Qualification of modelling & simulation related methods is starting in 4Q25. Regarding transparency, it was noted that industry trade organisations agreed to the publication of high-level information from Qualification Advice.

Industry confirmed their support to the execution of the agreed action plan and emphasised the importance of education on QoNM. Fitting qualifications into regulatory and healthcare frameworks is crucial to ensure a future-proof and agile system for decision making.

Follow-up:

- Follow-up reporting on the delivery at the next R&D industry stakeholder platform

Establishment of potential alternatives to animal testing in line with the 3Rs principles

Background was provided on the initiatives carried out by the 3RsWP to foster regulatory acceptance of new approach methodologies (NAMs). This includes the revision of the guideline on the principles of regulatory acceptance of 3Rs (replacement, reduction, refinement) testing approaches as described in the published concept paper. Part of the revision aims at introducing annexes on acceptance criteria for complex *in vitro* models for a specific context of use. However, to develop the guidance, it is important for EMA and assessors to build confidence and knowledge regarding these innovative methods.

In 2016, EMA introduced the mechanism of voluntary data submission (VDS) or "*safe harbour approach*" which allows applicants to share data on 3R testing approaches alongside the data required for an initial Marketing Authorisation Application (MAA). However, no data has been submitted using the VDS pathway until today. Therefore, EMA is proposing to reframe this pathway and presented the objective and benefits of the new proposal for VDS, in which the NAMs data submission is decoupled from the MAA. Anticipated benefits include gaining knowledge on more recent data and methods, rather than those that will be outdated by the time of the MAA, as well as ensuring no risk of delays in the MAA procedure. EMA will be organising an ad-hoc meeting with trade associations to further develop and co-create the VDS pilot project, which is planned for 2026.

Follow-up:

- Industry associations to disseminate information about the voluntary data submission framework in view of co-creation of the pilot
- Proposal to be presented at a future R&D industry stakeholder platform