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Highlight report from the 11th Industry stakeholder platform on research and development support

4 December 2023

Role	Name
Chair:	Michael Berntgen
Present:	Industry: **AESGP** Klavdija Kmetič **ARM** Sibylle Herzer, Martin O'Kane, Etienne Regulier, Elisabetta Zanon **EFPIA** Gesine Bejeuhr, Lucia D'Apote, Inka Heikkinen, Francois Hebraud, Jyothsna Krishnan, Mireille Muller, Claudia Popp, Isabelle Stoeckert **EUCOPE** Andrew Gray, Axel Korth, Mariska Mulder, Shekhar Natarajan, Lars Hyveled-Nielsen **EuropaBio** Pedro Franco, Alexa Hunter, Marcello Milano, Valentin Plouchard **Medicines for Europe** Nivedita Valentine Pharmanovia, Raluca Radu, Martin Schiestl **MPP Association** Shayesteh Fürst-Ladani, Andreas Emmendörffer, Fatima Bennai-Sanfourche, Christoph Joosten **Vaccines Europe** Stephane Callewart. EMA: Stiina Aarum, Ralph Bax, Michael Berntgen, Christelle Bouygues, Francesca Cerreta, Kevin Cunningham, Corinne De Vries, Falk Ehmann, Maria Filancia, Iordanis Gravanis, Anna Gross, Kristina Larsson, Thorsten Olski, Chrissi Pallidis, Marie-Helene Pinheiro, Enrico Tognana, Tarita Toufexi, Thorsten Vetter, Ana Zanoletty, Claudia Vincenzi. European Commission: Valentina Barbuto, Marco Capellino, Isabelle Clamou, Fabio D'Atri, Lilia Luchianov, Olga Tkachenko, Jose Valverde Albacete. EMA scientific committees and working parties: Brian Aylward, Kieran Breen, Jörg Engelbergs, Hilke Zander. HTA bodies: Paul De Boissieu, Stephanie Said, Anne Willemsen. Notified bodies: Jonathan Sutch, Petra van Leeuwen.

This was the eleventh 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.

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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 11th meeting, where required.

Development support offering for programme-specific evidence planning

Insights into recent trends with the use of central scientific advice in development programmes was provided through a targeted cross-association survey among their members. 20 companies responded to the survey, which allowed to identify attitudes and challenges for building central scientific advice into programmes or potentially using alternatives. It was stressed that none of these alternatives are to replace central scientific advice. Ultimately, the reasons for a transient reduction in scientific advice requests observed in the first half of 2023 are unknown and probably unspecific. Key areas for improvement identified in the survey were low availability of discussion meetings, perceived limitations to paediatric scientific advice and the limited options for scientific advice for combination products. Other challenges revolved around consistency of advice throughout the lifecycle and the various development support platforms.

Looking at discussion meetings, the percentage of scientific advice requests including a discussion meeting is around 10-12% in recent years. There are practical limitations to the number of discussion meetings that can be held during a Scientific Advice Working Party (SAWP) plenary meeting. Nevertheless, there is an opportunity to review current practices together with the SAWP.

In terms of scientific advice on paediatric developments, industry representatives considered that the October 2022 revision of the scientific advice guidance, which attempted to clarify the remits of SAWP and PDCO in guiding paediatric medicines development, is difficult to interpret and discourages paediatric advice requests to the SAWP. EMA provided examples of appropriate and inappropriate questions to the SAWP (Insight from EMA on recent scientific advice, slide 6). It was agreed to perform a more in-depth review also with industry participants, which will then allow better communication.

EMA furthermore provided an update on the implementation of pilot initiatives based on first 5 years' experience with PRIME. First experiences were shared regarding the possibility for expedited scientific advice requests, development tracker submissions as well as the first submission readiness meeting. All elements are subject to pilots launched in March 2023 and which will be subject to surveys to gather structured feedback.

FOLLOW-UP:

- On the basis of the proposals regarding the offering of Discussion Meetings in scientific advice procedures, review of the current practices together with SAWP and enabling a discussion with the sounding board
- Follow-up discussion on the use of scientific advice for paediatric development programmes to enhance guidance and provide clarifications, where applicable, in consultation with both concerned sounding boards (scientific advice and paediatrics)

Action plan to strengthen the tool for qualification of novel methodologies

EMA presented the highlights from the summary report and recommendations derived from the multi-stakeholder workshop on futureproofing the Qualification of Novel Methodologies (QoNM) platform in

April 2023. The draft report has been shared and discussed with the industry sounding board and will be published after the stakeholder platform meeting in December 2023.

Industry welcomed the workshop report and recommendations emphasising that qualifications are enablers to innovation and foster an innovation friendly ecosystem by allowing scientific debate on new tools and technologies. Commitment to support the implementation of the workshop recommendations to futureproof the QoNM procedure was expressed.

Post-meeting note: The report was published on 15 December 2023 (summary report).

FOLLOW-UP:

- Follow-up discussion with the scientific advice sounding board (with a further extended membership, if necessary) on the delivery of the resulting action plan

Introduction to the revamped Business Pipeline meetings

The scope and objectives of Portfolio and Technology meetings (PTMs), an evolution of the interactions previously known as Business Pipeline meetings (BPMs), were presented. As before, the discussion in PTM's will focus on product portfolios. The aim of this platform is three-fold: identify issues impacting the progress of product portfolios, capture new and disruptive technology already in use, and anticipate the scientific and regulatory expertise needed to assess future applications. There will be annual call for expression of interest published on the Agency website to attend a PTM in the subsequent year. The call for 2024 PTMs closed on 10th of November 2023.

FOLLOW-UP:

- Feedback to be obtained on the new arrangements, directly from companies subject to PTMs in 2024 as well as an annual review facilitated by the R&D platform

Industry perspective on future-proofing the regulatory system

Industry presented a perspective on the regulatory sandbox in the context of future-proofing the regulatory system. The exchange allowed building a shared understanding on potential examples for future sandboxes and how the sandbox concept would fit into the current regulatory toolbox supporting innovation.

Recent and upcoming developments of the IRIS platform for R&D processes

EMA provided an overview of recent and upcoming developments of the IRIS platform for R&D processes. Regarding PRIME, the application for PRIME eligibility as well transfer and withdrawal of PRIME regulatory entitlements went live on 10 July 2023. Subsequently, the application for PRIME meeting requests and periodic update submissions were launched on 3 October 2023. Highlights were provided regarding specific sections in the PRIME meeting request and periodic update submission forms. Also, updates were provided on information available publicly such as quarterly system demo, PRIME webpage and guidance for applicants. A short survey on user experience will be launched in the upcoming period.

With regards to paediatric processes the development roadmap was presented with a tentative go-live in May 2024. Furthermore, EMA informed industry associations on the upcoming quarterly system

demo for the initial paediatric investigation plan and the launch of call for expression of interest to act as volunteers for webinars to provide input to the onboarding of paediatric procedures.

FOLLOW-UP:

- Trade associations to cascade information about the latest IRIS developments concerning PRIME procedures
- Trade association to cascade information on the demo webinar concerning the plans for onboarding of paediatric procedures on 19.12.2023
- EMA to launch the call for expression of interest to act as volunteer for webinars to provide input to the onboarding of paediatric procedures

Progressing agility in paediatric processes

EMA presented the experience over 9 months following the launch of the stepwise PIP (sPIP) pilot in February 2023. So far 16 enquiries relating to the pilot were received and five sPIP applications have been discussed by the PDCO. The medicines are to treat diseases mainly in the metabolic and neurology therapeutic areas and the majority were at very early stages in development. The main reasons sponsors were interested in the sPIP pilot were due to uncertainties related to early development and challenges with aspects of clinical development in ultra-rare conditions such as dose finding or endpoints.

Recent activities of the sounding board on paediatric matters were summarised. These included finalisation of the sPIP guidance, finalisation of the Key Elements form as well as input to the summary report/scientific document template (Part B-F). The input to the scientific document template was discussed in more detail with clear feedback where the draft could benefit from further clarification. This resulted in a template with clear guidance for a streamlined PIP development and assessment and better mutual understanding. It was suggested that the dialogue is continued to resolve the remaining open questions. Applicants are recommended to use the new scientific template on a voluntary basis and the experience from this will be evaluated prior to the date when use of the new template becomes mandatory.

FOLLOW-UP:

- Trade associations to continue promoting the sPIP pilot to ensure well-balanced and comprehensive learning, and later reporting about the experience at the R&D platform
- EMA to review the additional comments on the template and consider if refinement in the templates is needed

Collaboration at the regulatory / HTA interface

Industry associations presented on the preparation at companies' level for the HTA Regulation implementation. Leverage of the vast joint experience in EMA-HTA interaction for a successful implementation was highlighted. EU HTA will impact product teams in industry as of 2024, such as changing EU input to product teams, navigating PICO processes, planning for compliance, assessment of EU regulatory filing strategies as well as interactions with regulators and information sharing with the HTACG. It was noted that companies have started preparing and first questions are coming in on key aspects such as the interplay between centralised procedure and joint clinical assessment. The publication of the Implementing Acts and further guidance is therefore awaited.

FOLLOW-UP:

- Industry to contribute to the public consultation on the Implementing Act for Joint Clinical Assessment, once initiated
- Trade associations to consider further initiatives to disseminate information about the new JCA framework, including good preparatory practices

Evidence planning for combination developments comprising medicinal products with medical devices and/or companion diagnostics

EMA presented the agreed follow-up actions and next steps following the discussions and conclusions of the relevant focus group. Insights into scientific questions and expertise needs for scientific dialogue will be concluded in a scientific publication. Building on the experience from the existing pilot for advice by expert panels on high-risk MDs, the complementarity between SAWP (MP focus) and expert panels (MD focus) will be further explored. Possibilities of involving NCAs with device competence in EMA scientific advice on development proposals will be further looked at and EMA will continue collaboration with the EC as Chair of the Medical Device Coordination Group (MDCG). Regarding the options and specific actions within ACT EU and COMBINE, EMA will continue contributing to the project. Finally, the results of the Notified Bodies Coordination Group for medical devices (NBCG-Med) position paper will be reviewed and eventual further actions and options for collaboration with NBCG-Med and the Notified Body Oversight (NBO) subgroup considered.

Industry presented their perspective on challenges with the development and approval of medicinal products (co-)developed with medical device and IVDs (CDx) and welcomed the initiatives taken. Industry reiterated that a joint discussion platform for questions regarding (co-)development strategy for DDC/Drug-CDx combinations is warranted to help navigate through the very complex environment. Furthermore, two position papers were presented, one about the challenges in conducting clinical trials involving medicinal products with medical devices or IVDs (combined trials) and another one on regulatory pathways for connected combined products (CCPs).

Finally, progress with the COMBINE project was presented by the EC summarising background, scope, timelines and actors involved. Information about the project is published on the Commission website. An analysis including proposals for solutions will be presented in January 2024. Prioritisation, selection, and development of solutions will be discussed after the analysis phase. The link of the COMBINE project to ACT EU via the Multi-stakeholder platform (MSP) was confirmed, noting that the activity and progress of the project will be reported at the MSP annual meeting.

FOLLOW-UP:

- EMA to coordinate progress of the agreed follow up (scientific publication; opportunity for bridge to expert panel advice; involvement of NCA MD expertise)
- EMA and industry to continue contributing to the COMBINE project
- EMA to review the recommendations regarding combined trials presented by industry at the meeting, particularly the interface with CTIS and related guidance