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Highlight report from the 16th Industry Stakeholder Platform on Research and Development Support

2 July 2026

Role	Name
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This was the sixteenth 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 16th meeting, where required.

Strengthening the delivery of scientific advice to efficiently support development programmes

Update on recent developments

Volumes of scientific advice applications in the first half of 2026 reached record levels. As a result some delays and postponements in the start of procedures were experienced, while the number of discussion meetings (including explanatory teleconferences) remained similar to those of previous years. Industry representatives suggested that further guidance is published on the procedural option of explanatory teleconferences.

The update of the scientific advice briefing document template is progressing with further discussions on the patient representative-specific annex and consideration by the Scientific Advice Working Party (SAWP) of an industry proposal for changes towards harmonisation of document requirements between international regulators. The SAWP will consider this proposal as part of an initiative aimed at re-thinking the way that scientific advice responses are being drafted to enhance clarity of the advice given. The scientific advice sounding board will be consulted.

EMA announced that, following preliminary internal discussions, a pre-payment model for scientific advice applications at the time of submission (and not at validation, as done since the beginning of 2025) is under consideration. The planned implementation for this change is expected by the end of 2026. EMA further re-confirmed that direct debit will also be enabled for the payment of regulatory invoices.

On the topic of broad scientific advice, EMA reported on preliminary internal reflections regarding the possibility of engaging other EU Agencies in scientific advice, in line with provision of the New Pharmaceutical Legislation (NPL). EMA will re-visit the scope of broad scientific advice along with the process and relevant public guidance as part of the NPL implementation.

Finally, under the SAWP/CTCG (Clinical Trials Coordination Group) pilot, there have been 19 applications until mid-2026. The pilot continues until further notice. Industry suggested that the involvement of CTCG becomes part of regular operations in relevant cases and that the scope of the SAWP/CTCG process be expanded in a new pilot involving also ethics committees and combinations products.

Progress of the Focus Group on agility in scientific advice

A Focus Group with representatives from industry, SAWP and EMA has been constituted to explore the possibility of an agile scientific advice process designed to support time-critical drug development while maintaining the robustness and predictability of the EU regulatory system. Deliverables of the Focus Group are to define scope, submission requirements, assessment resource allocation, timelines and

outcome of the agile process, to decide on feasibility of agile process implementation and, depending on it, on the conduct of a pilot. Initial TCs have been held, and the group has reached in principle agreement on the need for a more agile scientific advice tool and the value of testing the concept through a pilot phase.

The proposed Agile Scientific Advice model proposed is intended for focused, well-defined scientific questions requiring rapid feedback, possibly in areas of unmet medical need or where accelerated SA timelines could have a meaningful impact on development decisions. To ensure feasibility and sustainability from a resource perspective, the process could be limited to a small number of questions, generally within a single discipline, based on justification demonstrating both medical need and the necessity for agility. Complex, precedent-setting or multidisciplinary topics would remain within the standard Scientific Advice framework.

Discussions have also initially explored the operational design of the procedure. The next phase of work will refine the scope, operating model, pilot design and success metrics, with the objective of launching a pilot in Q1 2027.

Pilot on scientific advice specific Model-informed Drug Development (MIDD-SA)

EMA launched in May 2026 a pilot for a scientific advice procedure tailored to development programs with a strong MIDD element, i.e. programs in which MIDD evidence is expected to play a key role in regulatory decision-making. The pilot is part of ICH M15 guideline implementation activities and also a follow-up action to the HMA/EMA multi-stakeholder workshop on reporting and qualification of mechanistic models for regulatory assessment. A discussion meeting will be offered in all relevant cases, unless the applicant and the SAWP both decide to cancel it. For more information and for instruction as to how to apply to the MIDD-SA pilot, please refer to the relevant guidance on the EMA website.

Update on activities of the COMBINE workstream 5 focusing on scientific advice for combination developments

EMA provided an update on the progress of Project 5 under the COMBINE program. The objective of the project is to clarify the advice needs for sponsors of combinations products (comprising medicinal products and medical devices/in vitro diagnostics) and the needs for exchange of best practices among relevant regulators/decision-makers. The project further aims to analyse where needs are covered by existing advice formats/assessors' fora and where there are gaps and finally to propose how these gaps could be addressed. To date, the project has progressed through the need clarification and gap analysis phases, for which project group meetings, a stakeholder workshop and surveys to sponsors and authorities/ ethics committees/ notified bodies have been employed. A draft report is in preparation while a second stakeholder workshop is being planned for the end of September 2026. The project will go into its last phase of proposal development to cover the identified needs and gaps between October 2026 and Q1 2027. This phase is also likely to include a stakeholder meeting.

Follow-up:

Update on recent developments

- EMA to communicate on the progress with re-thinking the way advice is being drafted and subsequently following up on the implementation of the revised briefing document template.
- EMA to reflect on opportunities for communication in case of an unforeseen postponement of a scientific advice start.

- EMA to consider adding the concept of "explanatory TC" to the publicly available guidance, once further consolidated.
- EMA to implement pre-payment at time of scientific advice submission.

Progress of the Focus Group on agility in scientific advice

- Focus group to progress the development of the agile scientific advice framework, for a closing report at the upcoming R&D stakeholder platform for subsequent launch of the pilot.

Pilot on scientific advice specific Model-informed Drug Development (MIDD-SA)

- Industry associations to cascade information on the pilot on scientific advice specific for Model-informed Drug Development.
- EMA to launch a call for a dedicated MIDD sounding board and follow-up discussion on the new MIDD-SA pilot in the dedicated sounding board to clarify open questions from developers on the concept.

Update on activities of the COMBINE workstream 5 focusing on scientific advice for combination developments.

- Feedback from the stakeholder workshop and on the upcoming pilot at the next R&D stakeholder platform.

Survey on experience and expectations with parallel EMA/FDA development support

EMA and Industry stakeholders presented the interim results of the survey on EMA-FDA support to global development plans, conducted to better understand developers' needs, expectations, and challenges when using parallel regulatory support mechanisms. The survey highlights strong industry support for EMA-FDA collaboration in supporting global medicine development. Stakeholders recognised the value of parallel regulatory interactions in facilitating global development strategies and regulatory alignment, while also identifying opportunities to improve efficiency, transparency, communication, and coordination across existing support mechanisms.

Follow-up:

- EMA to further complete the analysis and also continue to engage with FDA on the feedback received from developers and possible changes / improvement to parallel support mechanisms.
- Discussion of the survey outcome with the sounding board, to agree on follow-up activities as a result of the survey.

Continuous evolution of the PRIME scheme

EMA provided an update on the ongoing activities of the PRIME scheme, including the Product Development Coordinator (PDC) pilot, progress with implementation of recommendations of the recent PRIME pilot feature report, and preparations for the 10-year analysis of PRIME. EMA reported a year-on-year increase in PRIME applications since 2022 and highlighted the expansion of the PDC pilot, which was launched in July 2025 to strengthen product-specific support and facilitate interactions across the regulatory network. The pilot will be expanded during the second year, while experience gained during the first 12 months will be used to further refine the role and associated performance indicators.

Updates were provided on the implementation of PRIME pilot recommendations. Activities are progressing across several areas, including refinement of the product development tracker, enhancement of feedback mechanisms following tracker submissions, further promotion of the tracker as a central tool supporting iterative scientific dialogue, clarification and optimisation of expedited scientific advice, and improvements to submission readiness activities. EMA outlined a combination of short-, medium- and longer-term actions planned for implementation, and continued consultation with industry stakeholders foreseen.

EMA further outlined the scope and timelines of the planned 10-year analysis, which will assess PRIME eligibility patterns, development outcomes, regulatory interactions and the impact of PRIME support on marketing authorisation applications. Input from industry stakeholders will continue to be sought through the sounding board and other engagement forums during the finalisation of the analysis.

Industry representatives welcomed the progress made and emphasised the importance of maintaining continuity of knowledge throughout PRIME development, strengthening two-way communication between developers and regulators, and ensuring that the PDC role delivers added scientific and regulatory value while supporting efficient interactions across the network. Industry also encouraged the use of outcome- and impact-focused measures alongside operational KPIs when evaluating the PDC pilot and highlighted the opportunity of the upcoming 10-year PRIME analysis to identify lessons learned and to inform future implementation of the NPL.

Follow-up:

- EMA to progress the developments and measurement of the KPIs for the PDC, also in cooperation with the sounding board.
- Industry perspectives on the future opportunities with PRIME to be considered in the upcoming discussions on the PRIME implementation under the NPL.

Progress update with the action plan on modernising the Qualification of Novel Methodologies

EMA presented an update on the progress of implementing the action plan to future-proof the Qualification of Novel Methodologies (QoNM). The updated procedural guidance to applicants has been published, reflecting the broad input from stakeholders and working groups internally. Key new elements include:

- Detailed chapter defining the 'Scope of QoNM'
- 'QoNM early-interaction support' with scoping meetings before submission of a formal request which will help clarify the best available regulatory support for methodology developments and improving the quality of briefing packages
- Optional stakeholder consultation if external expertise is needed (e.g. for highly innovative methodologies) but external experts cannot be involved as QT members due to conflict of interest considerations
- Publication of high-level information on Qualification Advice
- Periodic informal dialogue/trialogue (QO holder, MP developer, EMA), possible upon request
- Considerations on generating evidence for qualification
- Lifecycle management considerations

Work on the upgrade of the QoNM webpage is ongoing, with a targeted delivery by September 2026.

Briefing document (BD) templates are being drafted and will be consulted with the sounding board. Targeted publication of a general BD template and the template for registry / RWE data source qualification by Q3 2026, for qualification of (digital) clinical outcome assessments by end of 2026, for qualification of modelling and simulation related methodologies in 2027.

Guidance on use of real-world data: questions and answers have been made available on the EMA real-world-evidence webpage in May 2026. Inclusion of aspects related to RWE data sources/registry qualification in this Q&A resource will be considered in 2027. Q&As on (digital) clinical outcome assessments and a Q&A on modelling and simulation-based methodologies are planned for end 2026.

A stakeholder communication and engagement plan is being drafted and will be consulted with the sounding board, targeting reach-out to different stakeholder groups e.g. by way of a webinar to inform on the new elements of the QoNM process and the opportunities provided by qualifying novel methodologies.

Follow-up:

- EMA to progress, also together with the sounding board, with the communication and engagement plan on the modernisation of the QoNM framework, including a webinar later in 2026.
- Core group to consider utility / need for additional Q&As on other types of methodologies.
- EMA to develop further AI based knowledge management tools to monitor the impact of qualifications.

Role and activities of the R&D stakeholder platform in the implementation of the New Pharmaceutical Legislation (NPL)

An update was provided on the NPL implementation programme, including the governance structure and the proposed industry engagement approach. It was explained that the Industry Stakeholder Group (ISG) will serve as the strategic forum for engagement, while the stakeholder platforms will provide topic-specific discussions and facilitate alignment on cross-cutting implementation topics. An overview of the topics allocated to each platform was also presented. For the R&D Stakeholder Platform, discussions are expected to focus primarily on topics under the Development Support and ERA & 3Rs delivery streams. An initial overview of the related implementation projects was shared, with more detailed discussions to follow as implementation progresses.

Industry representatives acknowledged the proposed engagement approach and emphasised the importance of receiving more detailed implementation plans as they become available to support preparedness and facilitate timely discussions on priority implementation topics.

Follow-up:

- EMA to maintain transparency in presenting the roadmap for implementation activities and provide further granularity in the planning for the topics relevant for the R&D stakeholder platform.
- Future R&D stakeholder platform meetings to cover relevant topics of operational / technical nature, including use of sounding boards and focus groups as appropriate.
- EMA to review the list of preliminary questions on certain topics relevant for development support and ERA, respectively, in the context of implementation planning for the NPL.

Progressing the support to paediatric developments

An update was presented following the review of the stepwise PIP pilot and publication of the report showing that the approach is both feasible and useful. Based on this experience, the stepwise PIP approach, originally developed through the R&D stakeholder platform in a dedicated focus group, is now embedded into standard practice. Applicants can use this approach where scientifically justified, but expectations remain clear:

- the initial PIP must still contain a paediatric strategy.
- milestones and future steps must be well defined and justified.

Regarding the topic of "mechanism of action-based PIPs in paediatric oncology and beyond" EMA highlighted that the NPL will introduce the Mechanism of Action (MoA) concept. Also, the public consultation on a concept paper related to further guidance on proof-of-concept data to support the development of anti-cancer products in paediatric patients has just ended, with a subsequent workshop on advancing proof-of-concept data in paediatric oncology drug development to be held on 21 September. Furthermore, two short communication publications will be submitted shortly:

- Translating non-clinical proof-of-concept into paediatric development decisions: the case of HER2-targeting antibody-drug conjugates
- Integrating non-clinical and clinical evidence for prioritisation in paediatric oncology drug development: regulatory experience with PARP inhibitors

The upcoming workshop will allow to gather stakeholder feedback on key questions, data needs, and perceived challenges to practical application, with a view to further strengthen the evidence-base supporting paediatric oncology development; and to leverage learnings from oncology and discuss challenges in MoA based paediatric developments beyond oncology, and what is needed to support such developments as foreseen under the NPL across other therapeutic areas.

Industry complemented these activities by recent discussions at multi-stakeholder workshops. In addition, they provided proposals for paediatric topics to be covered as part of the implementation of the NPL.

Follow-up:

- Industry association to cascade information on the workshop on advancing proof-of-concepts data in paediatric oncology drug development scheduled for 21st September.
- EMA to progress short communications on translating non-clinical proof-of-concept in paediatric development and on integrating nonclinical and clinical evidence for prioritisation.
- EMA to review the priorities by industry for paediatric aspects in the context of implementation planning for the NPL.

Initial considerations on the new concept of a 'Regulatory sandbox'

EMA presented the concept of regulatory sandbox based on the provisions of the NPL and shared information on the upcoming joint EC/EMA multi-stakeholder workshop on regulatory sandbox to be held on 21 September at EMA premises.

Industry stakeholders flagged that they are also considering the sandbox provisions mentioned in other legislations (e.g., AI act, draft Biotech act, revision of medical device regulation…). Moreover, they highlighted the activities of the IHI consortium BRIDGE and mentioned that it will be important to have a broader stakeholder perspective on regulatory sandboxes and innovative technologies/products, including small and medium enterprises and academia.

Follow-up:

- Industry association invited to provide by 4th September proposals for questions and topics to be addressed in the workshop in September.
- Industry association to cascade information on the EC/EMA multi-stakeholder workshop on regulatory sandboxes, scheduled for 21 September.

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

As a follow-up of the previous meeting, feedback about the status of the deliverable agreed (Q&A on DARWIN EU) was given: the document is under final review by EMA, after having received and implemented comments from industry associations. It should be published after the summer period. As there is no other deliverable planned as part of the focus group, a discussion will take place before the end of the year, and the conversion to a sounding board is highly probable.

Follow-up:

- Closing report of the Focus Group at the R&D platform meeting at the end of 2026.

Development of a new guidance for reporting on indirect comparisons

EMA presented the ongoing development of a Q&A guidance on indirect treatment comparisons (ITCs). The focus of this Q&A is on the expected reporting of ITCs in submissions, not on evidentiary standards or permissive context. The work is a collaboration across MWP, COMP and CHMP and is also referenced in the MWP workplan. The initiative responds to the increasing use of indirect comparisons in regulatory submissions and to the observed heterogeneous and non-transparent reporting.

The presentation also described a supporting research project on the use and reporting of ITCs in all orphan maintenance procedures initiated between 2023 and 2025. This work reviewed the type of indirect comparison methods used, the completeness of ITC-specific reporting elements, the issuance of Lists of Questions and procedural outcomes, with the aim of characterising current practice and identifying potential areas for improvement. The findings showed heterogeneous reporting standards, ranging from brief summaries with solely point estimates to extensive contractor-prepared reports, and indicated that requests for further clarification were commonly linked to transparency and reporting limitations. In this sense, EMA noted that the Q&A and the planned publication on this review will strive to improve reporting standards in future submissions.

From the developer perspective, industry highlighted that ITCs for orphan medicinal products remain challenging for multiple reasons, including among others the small and heterogeneous populations, but also issues with identifying treatment effect modifiers. Industry welcomed reporting guidance, while calling for fit-for-purpose evidence approaches in rare diseases, flexibility in methodology and interpretation, and the introduction of a structured framework to whether robust ITCs are feasible.

Follow-up:

- EMA to progress the development of a Q&A on indirect comparisons.
- Progress the publication of the experience on ICT use in orphan maintenance procedures.

Progress update on the launch of the Voluntary Data Submission (VDS) pilot for 3Rs methodologies

EMA provided an update on the Voluntary Data Submission (VDS) pilot, an initiative intended to build shared understanding and facilitate broader regulatory acceptance of new approach methodologies (NAMs). A summary of previous interactions with stakeholders was presented, highlighting the strong interest and engagement from industry and CRO trade associations. In addition, EMA outlined the scope of the pilot within four regulatory applicability areas (biologics risk assessment, developmental and reproductive toxicity risk assessment, safety pharmacology, hepatotoxicity and drug-induced liver injury) as well as the process for data submission and expected assessment timelines by the NAMs operational expert group. The outcome of the pilot will include written feedback to applicants on readiness of the overall approach or NAM including regulatory considerations on the adequacy of the data and potential next steps as well as an EMA lessons-learned report.

Follow-up:

- EMA to launch the VDS pilot in Q3 2026, which should then be cascaded by the industry associations.
- Reporting on lessons learned at a future R&D platform meeting, once the assessment of submissions under the pilot is complete.

Follow up discussion on the identification of product-specific bioequivalence guidelines

EMA provided an update on implementation activities related to ICH M13A. The list of expected product-specific bioequivalence guidelines (PSBGLs) in 2026 has been published on EMA's product-specific bioequivalence guidance webpage. Furthermore, all Q&As in section 4 were reviewed, including updates of references and citations, and rephrasing standardised texts, e.g. regarding high-risk products.

In terms of future planning for PSBGLs, EMA presented a concrete proposal for an annual survey and later notification of confirmed topics.

Follow-up:

- Industry associations to raise awareness and provide input into the selection of product-specific bioequivalence guidelines with the revision of the MWP workplan.
- EMA to consider proposed additional refinements for the templates proposed by industry.