16 December 2024

Submission of comments on 'European Platform for Regulatory Science Research Concept paper – DRAFT' (EMA/530334/2024)

Comments from:

ACRO (Association of Clinical Research Organizations)

Please note that these comments and the identity of the sender will be published unless a specific justified objection is received.

When completed, this form should be sent to the European Medicines Agency electronically, in Word format (not PDF).

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1. General comments

Stakeholder number *(To be completed by the Agency)*	General comment (if any)	Outcome (if applicable) *(To be completed by the Agency)*
	In this draft concept paper, EMA and HMA present a proposal for creating a "European Platform for Regulatory Science Research." To facilitate understanding of the topics that would be covered by this Platform, the concept paper provides helpful definitions of both "regulatory science" and "regulatory science research." In lines 63-64, the document defines "regulatory science" as *"A range of scientific disciplines that are applied to the quality, safety and efficacy assessment of medicinal products and that inform regulatory decision making throughout the lifecycle of a medicine. It also contributes to developing regulatory standards and tools."* The document defines the role of "regulatory science research" as: ▪ *To investigate and create new tools for developing and evaluating medicinal products* ▪ *To investigate and create new methods to generate and optimize evidence generation for decision-making* ▪ *To investigate and systematically identify and address gaps in evolving regulatory activities and system* Moreover, the draft concept paper outlines the objectives of this proposed Platform:	

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	*"advancing and accelerating regulatory science research, addressing regulatory science research questions and increasing the quality and impact of research to improve regulatory practices and medicines development, by promoting dialogue and fostering collaboration among **academic regulatory science researchers** and with regulators across Europe and beyond* (lines 43 to 47, emphasis added)." Similarly, the concept paper outlines the <u>scope</u> of the Platform as follows: *The platform will provide a unique systematic approach, acting as mechanism among **academic and non-for-profit** **[sic]** **research organisations** and regulators working on regulatory science research to discuss and collaborate on important regulatory science research needs with regulators, and optimise research outcomes and impact on regulatory practices, standards, medicines development and use* (lines 115 to 118, emphasis added). What is notable about these two statements on the objectives and the scope of the proposed Platform is the explicit mention of academic regulatory science researchers and the omission of industry stakeholders.	

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	Indeed, industry is only mentioned twice in this draft concept paper. The first discussion of industry is in lines 215-224. The concept paper proposes a steering group for this Platform to identify priority tasks and topics for discussion, draft workplans (meeting topics and frequency), and define ways of working on priority topics and further note that patient, healthcare professional, industry, and health technology assessment (HTA) representatives, and EU and national research funders will be invited to participate in steering group meetings as observers. The EMA is widely recognized (and highly regarded) for its strong commitment to multi-stakeholder input and consultation. Because of this, it seems odd that industry would only be invited to participate in the steering group as "observers." The second mention of industry is in lines 253-263. The concept paper states: *It is acknowledged that researchers not only from the academic sector and within regulatory agencies conduct relevant regulatory science research. Also, other stakeholders, such as in the for-profit-sector and healthcare professional or patient organizations, are engaged in such activities. Additional types of interested parties (see below) may become involved in the platform participant group over time, also from outside the EU,*	

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	such as international partner organisations or authorities: ▪ *National and EU-level research funders* ▪ *Industry trade organizations* ▪ *Patient organisations* ▪ *Healthcare professional organisations* ▪ *HTA bodies* ▪ *Policy makers* Because of the EMA's widely recognized (and highly regarded) commitment to multi-stakeholder input and consultation, the statement that industry and other stakeholders *"may become involved in the platform participant group over time*" seems non-committal and anemic. As the concept paper notes, regulatory science research *"encompasses basic and applied biomedical and social sciences and contributes to the evaluation of existing, and development of new, regulatory standards, tools, methods, and principles used for developing medicines, as well informing requirements for their evaluation."* Industry stakeholders are directly impacted by the "tools, methods, and principles used for developing medicines" and the "requirements for their evaluation." Because of this – and because industry conducts the majority of clinical trials globally – industry acts as a vital "laboratory" or "practicum" for regulatory science theory	

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	(regulatory tools, methods, and principles). We are in a pivotal and exciting time for clinical research where unproductive silos are being dismantled: Real-world evidence is being increasingly incorporated into randomized clinical trials. Randomized clinical trials are being increasingly integrated into routine clinical practice. It would therefore be a striking step backward to see an artificial wall constructed between academia and industry on regulatory science research. ACRO, therefore, respectfully requests that industry stakeholders (sponsors, CROs, and clinical technology companies) are included in this initiative as active participants rather than mere observers and also that the statement that industry and other stakeholders *"may become involved in the platform participant group over time"* be replaced by an explicit commitment for industry involvement from the beginning of the Platform's work.	

2. Specific comments on text

Line number(s) of the relevant text *(e.g. Lines 20-23)*	Stakeholder number *(To be completed by the Agency)*	Comment and rationale; proposed changes *(If changes to the wording are suggested, they should be highlighted using 'track changes')*	Outcome *(To be completed by the Agency)*
Lines 215-224		**Comment:** The concept paper proposes a steering group for this Platform to identify priority tasks and topics for discussion, draft workplans (meeting topics and frequency), and define ways of working on priority topics and further note that patient, healthcare professional, industry, and health technology assessment (HTA) representatives, and EU and national research funders will be invited to participate in steering group meetings as **observers**. **Proposed change (if any):** We ask the Agency to revise this section so that it reads that— The concept paper proposes a steering group for this Platform to identify priority tasks and topics for discussion, draft workplans (meeting topics and frequency), and define ways of working on priority topics and further note that patient, healthcare professional, industry, and health technology assessment (HTA) representatives, and EU and national research funders will be invited to participate in steering group meetings as **active participants**.	
Lines 253-263		**Comment:** The concept paper currently reads that "Additional types of	

Line number(s) of the relevant text *(e.g. Lines 20-23)*	Stakeholder number *(To be completed by the Agency)*	Comment and rationale; proposed changes *(If changes to the wording are suggested, they should be highlighted using 'track changes')*	Outcome *(To be completed by the Agency)*
		interested parties (see below) may become involved in the platform participant group **<u>over time</u>**, also from outside the EU, such as international partner organisations or authorities . . . " **Proposed change (if any):** We ask the Agency to revise this to read that— "Additional types of interested parties (see below) may become involved in the platform participant group **<u>immediately when the initiative is launched</u>**, also from outside the EU, such as international partner organisations or authorities . . . "	
		Thank you for the opportunity to provide comment on this concept paper, and please do not hesitate to contact ACRO (knoonan@acrohealth.org) if we answer any questions or provide additional details.	

Please add more rows if needed.