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Public consultation on European Medicines Agencies Network Strategy to 2025

Fields marked with * are mandatory.

Introduction

The purpose of this public consultation is to seek views from EMA's and HMA's stakeholders, partners and the general public on the proposed joint **European Medicines Agencies Network Strategy to 2025** and whether it meets stakeholders' needs. By highlighting where stakeholders see the need as greatest, there is an opportunity to help shape the strategy for the coming years, 2021-2025.

The views being sought on the proposed strategy refer both to the extent and nature of the broader strategic theme areas and goals. We also seek your views on whether the specific underlying objectives proposed are the most appropriate to achieve these goals.

The strategy will be aligned with the broader **Pharmaceutical Strategy for Europe** being developed by the European Commission and its actions will seek to provide synergies with actions developed under the Pharmaceutical Strategy where their subject matter overlaps. Wherever matters of policy or potential legislative change are referred to, these should be understood as supporting the development and implementation of the broader Pharmaceutical Strategy, where the ultimate responsibility for such matters will lie.

The questionnaire has been launched on 6 July 2020, to enable stakeholder feedback to be collected on the draft network strategy and will remain open throughout the consultation period until **4 September 2020**. In case of any queries, please contact: EMRN2025strategy@ema.europa.eu.

Completing the questionnaire

This questionnaire should be completed once you have read **the draft joint strategy document**. The survey is divided into a general section on the whole document and then focuses on each strategic theme area. You are invited to complete the sections which are most relevant to your areas of interest.

We thank you for taking the time to provide your input; your responses will help to shape and prioritise the future objectives of the European Medicines Agencies Network.

Data Protection

By participating in this survey, your submission will be assessed by EMA and HMA. EMA collects and stores your personal data for the purpose of this survey. Requests for contributions to be published in an anonymised form, can be sent to the data controller (S-DataController@ema.europa.eu).

* Name

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Stakeholder Information

* **Question 1: What stakeholder, partner or group do you represent:**

- ○ Individual member of the public
- ○ Patient or Consumer Organisation
- ○ Healthcare professional organisation
- ○ Learned society
- ○ Farming and animal owner organisation
- ○ Academic researcher
- ○ Healthcare professional

- ○ Veterinarian
- ○ European research infrastructure
- ○ Research funder
- ○ Other scientific organisation
- ○ EU Regulatory partner / EU Institution
- ○ Health technology assessment body
- ○ Payer
- ◉ Pharmaceutical industry
- ○ Non-EU regulator / Non-EU regulatory body
- ○ Other

* ***Please specify:***

Please select one option that best describes your organisation

- ☐ Individual company (non-SME)
- ☑ Trade association
- ☐ SME

* ***Name of organisation (if applicable):***

If not applicable, please insert "n/a"

European Federation of Pharmaceutical Industries and Associations (EFPIA)

Overall strategy

* **Question 2: Please indicate which area is relevant to your area of interest?**

Please select one or both options, as applicable

- ☑ Human
- ☐ Veterinary

Question 3: Having read the proposed strategy, how would you rate it in general terms?

Answer the following question on a scale of 1-5, where 5 indicates highly satisfied and 1 highly dissatisfied

	1. Highly Dissatisfied	2. Dissatisfied	3. Neutral	4. Satisfied	5. Highly satisfied
* What are your overall impressions of the EMAN Strategy to 2025?	○	○	○	◉	○

* **Question 4: Are there any significant elements missing in this strategy?**

Please note that the strategy aims to focus on major areas of interest for the next five years and it is not intended to cover all activities undertaken by the Network.

- ◉ Yes

No

If yes, please provide further details.

EFPIA welcomes the European medicines regulatory agencies network (EMRN) draft strategy to 2025 (EUNS 2025) and fully supports the establishment of an overarching strategic plan. Here EFPIA provides its views on activities that the innovative biopharmaceutical industry believes will have the most benefit for European citizens as well for the global community by ensuring that EMRN remains on the cutting-edge of innovation in healthcare. EFPIA represents 39 biopharmaceutical companies and a growing number of SMEs and includes direct membership of 36 national associations. We strongly support global regulators' exceptional efforts, including those of the EMRN, to facilitate the development of potential therapeutics to fight COVID-19.

EFPIA views the EUNS 2025 and the related EMA's Regulatory Science Strategy to 2025 (RSS 2025) as key enablers for bringing the promising next wave of innovation to patients who face the burden of unmet medical need. The EUNS 2025 is intended as a high-level strategic document rather than an all-encompassing plan. As such, this important strategic direction needs to be balanced with a strong sense of urgency to develop a prioritised implementation plan with clear actions and measurable deliverables. EFPIA understands that the overall implementation of the EUNS 2025 will be monitored annually; however, to measure progress, the EMRN should include assessable outcomes against each objective, that should then be shared with stakeholders. The same applies to the EMA and the HMA annual and multiannual programming documents and reports that should provide, through the inclusion of measurable outcomes, the link between the EUNS 2025 and the activities of the EMRN.

We would like to propose a prioritisation of the proposed objectives as well as a number of actions. We would also like to raise our concern regarding a number of objectives that we believe may not entirely contribute to the EMRN's stated mission. Some of the objectives appear to be in areas or involve actions in which EMRN may have a role but should not be leading. The focus of EUNS 2025 should be on activities that are within the EMRN's responsibility and that do not detract from its core roles. It is critical to understand how the various themes, with several cross cutting goals and objectives, will be advanced and aligned from a content perspective across the EC Pharmaceutical Strategy and RSS 2025 (i.e. dynamic regulatory advice from Theme 2/Goal 3/Objective 1 with Theme 3/Goal 1/Objectives 5&6). In the final EUNS 2025, we suggest adding clarity on the EMA and EU National Competent Authorities' (NCAs) roles in meeting the EUNS 2025 objectives and connecting this to implementation of the RSS 2025.

The EUNS 2025 references the importance of patients throughout different strategic themes. We would like to propose the addition of a new standalone goal to emphasize this essential patient focus. For example, adding considerations to advance patient-focused medicines development could be added under the Innovation theme.

Even before the COVID-19 outbreak, EFPIA members reflected on how the EU regulatory framework could best leverage the new opportunities science is offering. EFPIA's strategic regulatory science initiative, the so-called Regulatory Road to Innovation (RRI) (https://efpia.eu/media/541132/efpia_regulatory-road-to-innovation_leaflet.pdf), includes suggestions on what improvements can be implemented within the current legislative framework.

EFPIA believes that enabling future medical innovations to reach the patient require additional strategic actions and diligent prioritisation to adequately address them. Consistent with the RRI, EFPIA proposes the following as priority actions with measurable outcomes:

- Innovative clinical trials: Escalate this imperative from an EUNS 2025 objective to a theme or goal
- Iterative regulatory advice: Redesign a more flexible, integrated product support mechanism

- Real world evidence (RWE): Implement RWE use cases/pilot programme
- Digital transformation: Develop and implement an overarching EU Telematics Strategy within EUNS 2025

Reflecting the numerous, diverse elements in the EUNS 2025, we'd like to highlight the importance of a continuous dialogue with relevant stakeholders to strengthen the EMRN and encourage innovation. EFPIA would value the continuation of the EMA, HMA, and NCAs' stakeholder engagement throughout the implementation phase, e.g. EFPIA supports the reconvening of EMRN platform meetings including with medicine and vaccine developers to engage in comprehensive discussions on for how to move forward on implementing the actions. Importantly, we would like to propose that foundational enablers, such as telematics, governance, resourcing, expertise and capabilities development, success measures, ongoing stakeholder dialogue, and global cooperation be fully described and integrated within the final EUNS 2025.

Question 5: The following is to allow more detailed feedback on prioritisation of the joint EMA/HMA goals for each strategic theme, which will also help shape the future application of resources. Your further input is therefore highly appreciated. Please choose for each row the option which most closely reflects your opinion. For areas outside your interest or experience, please leave blank.

Should you wish to comment on any of the goals and their underlying objectives, there is an option to do so.

Strategic Theme area 1: Availability and accessibility of medicines

	Very important	Important	Moderately important	Less important	Not important
1) Strengthen the availability of medicines to protect the health of European citizens, via: efficient and targeted regulatory measures, made possible through an in-depth understanding the root causes of unavailability of patented and off-patent products; identification of possible challenges in implementing legislation, removal of national barriers, increased coordination of the EMRN, sharing and implementation of best practices including stakeholders and increased transparency are the essential steps towards this goal.	○	○	◉	○	○

2) Optimise the path from development, evaluation through to access for innovative and beneficial medicines through collaboration between medicines regulators and other decision makers in the areas of: evidence planning, including post-licensing evidence; engagement in review of evidence and methodologies, respecting remits of the various players; collaboration on horizon scanning. As a result of this work, medicines that address unmet medical needs should have broader and earlier access coverage.	○	◉	○	○	○

Strategic Theme area 2: Data analytics, digital tools and digital transformation

	Very important	Important	Moderately important	Less important	Not important
1) Enable access to and analysis of routine healthcare data and promote standardisation of targeted data	○	◉	○	○	○
2) Build sustainable capability and capacity within the Network including statistics, epidemiology, real world data and advanced analytics	○	◉	○	○	○
3) Promote dynamic regulation and policy learning in current regulatory framework	◉	○	○	○	○

4) Ensure that data security and ethical considerations are embedded in the governance of data within the Network	◉	○	○	○	○
5) Map the use and needs of data analytics for veterinary medicines and support a streamlined approach across borders within the EEA	○	○	○	○	○

Strategic Theme area 3: Innovation

	Very important	Important	Moderately important	Less important	Not important
1) Catalyse the integration of science and technology in medicines development and ensure that the network has sufficient competences to support innovators in various phases of medicines development.	◉	○	○	○	○
2) Foster collaborative evidence generation - improving the scientific quality of evaluations and ensuring generation of evidence useful to all actors in the lifecycle of medicines, including HTAs, and pricing and reimbursement authorities.	◉	○	○	○	○
3) Enable and leverage research and innovation in regulatory science	○	◉	○	○	○

4) Enhance collaboration with medical device experts, notified bodies and academic groups	○	◉	○	○	○

Strategic Theme area 4: Antimicrobial resistance and other emerging health threats

	Very important	Important	Moderately important	Less important	Not important
1) Provide high quality information on antimicrobial consumption and surveillance data on antimicrobial resistance in animals and humans in support of policy development.	○	○	○	◉	○
2) Contribute to responsible use of antibacterial agents and effective regulatory antimicrobial stewardship in human and veterinary sectors by putting in place strategies to improve their use by patients, healthcare professionals and national authorities	○	◉	○	○	○
3) Ensure regulatory tools are available that guarantee therapeutic options (with a focus on veterinary medicines) while minimising impact of antimicrobial resistance on public health and the environment	◉	○	○	○	○

4) Define pull incentives for new and old antibacterial agents, including investigating support for new business models and not-for-profit development	◉	○	○	○	○
5) Foster dialogue with developers of new antibacterial agents and alternatives to traditional antimicrobials, to streamline their development and provide adequate guidance in both human and veterinary medicine	○	○	◉	○	○
6) Improve regulatory preparedness for emerging health threats	○	◉	○	○	○

Strategic Theme area 5: Supply chain challenges

	Very important	Important	Moderately important	Less important	Not important
1) Enhance traceability, oversight and security in the human/veterinary medicine supply chain from manufacturing to importation and final use of active pharmaceutical ingredients (APIs)	○	◉	○	○	○
2) Enhance inspector capacity building at EU and international level to address the problem of APIs, new technologies and continuous manufacturing	○	◉	○	○	○

3) Reinforce the responsibility for product quality by harmonising and reinforcing guidance to facilitate a coherent approach to the standards by regulators and industries	○	○	◉	○	○
4) Encourage supply chain resilience and review long-term risks resulting from dependency on limited number of manufacturers and sites, to ensure continuity of supply and availability of medicinal products.	○	○	◉	○	○
5) Analyse the possible implications of new manufacturing technologies in order to regulate the new supply chains needed to manufacture and distribute new types of medicinal products for human and veterinary use.	◉	○	○	○	○

Strategic Theme area 6: Sustainability of the Network and operational excellence

	Very important	Important	Moderately important	Less important	Not important
1) Reinforce scientific and regulatory capacity and capability of the network	○	◉	○	○	○
2) Strive for operational excellence, building on the work done in the current strategy	○	○	◉	○	○

3) Achieve a sustainable financial and governance model for the network	◉	○	○	○	○
4) Develop a digital strategy to drive digital business transformation	◉	○	○	○	○
5) Enable quick, consistent and adequate response to public and animal health challenges	○	◉	○	○	○

Strategic focus areas

* Please indicate which Strategic Theme area(s) you would like provide input

Please select as many choices as applicable.

- ☑ 1. Availability and accessibility of medicines
- ☑ 2. Data analytics, digital tools and digital transformation
- ☑ 3. Innovation
- ☑ 4. Antimicrobial resistance and other emerging health threats
- ☑ 5. Supply chain challenges
- ☑ 6. Sustainability of the Network and operational excellence

Strategic Theme area 1: Availability and accessibility of medicines

Question 6: Do the objectives adequately address the challenges ahead?

- ○ Yes
- ◉ No

Comments on objectives of the strategic theme area:

Addressing the current challenges related to availability;
EFPIA believes that Goal 1/Objective 1 (Identify the specific root causes of shortages and develop strategies to improve prevention and management of shortages…), Goal 1/Objective 4 (Improve coordination of information and actions, including implementation of best practices, both for EU regulatory authorities, stakeholders and international partners) and (reworded proposal below for) Goal 1/Objective 5 (Ensure availability of critical medicines in the EU/EEA by supporting increase of production capacity to meet demand) will help to address the challenges outlined by the EMRN. Understanding the root causes of shortages is essential in defining solutions. This must involve all relevant stakeholders, including medicine developers.
EFPIA welcomes the suggestion in Goal 1/Objective 2 (Help to identify and suggest areas where changes to EU or national legislation could improve supply…) that EMRN supports legislative changes for the implementation of ePI. Other potential measures mentioned in the same objective to improve supply (e.g. a tool to track shortages, transparency of the supply chain and of stock levels) do not however require legislative changes, contrary to what the strategy suggests. It is important to identify and suggest other areas where changes to EU or national legislation could improve supply since many countries are implementing in parallel their own legislation without any coordination and risks of costs discards related to stockpiling.
See references:
- 'Preparing for a second potential COVID-19 crisis: joint statement by Medicines for Europe and EFPIA': https://www.efpia.eu/news-events/the-efpia-view/statements-press-releases/preparing-for-a-potential-second-covid-19-wave/; and
- EFPIA June 2020 report 'The root cause of unavailability and delay to innovative medicines':https://www.efpia.eu/media/554527/root-causes-unavailability-delay-cra-final-300620.pdf

Focusing EMRN's biosimilar role on approval standards and robust pharmacovigilance measures
EFPIA recognises the leading role EU regulators have played in pioneering the "biosimilar concept"; the principles of which have been replicated and adopted by regulators around the world. However, EFPIA does not consider that promotion of the availability and uptake of biosimilars in healthcare systems (Goal 1 /Objective 3) is a topic that should be prioritised by regulatory authorities. Differences in availability and uptake of biosimilars are rooted in Member States' medicines pricing and reimbursement and procurement systems and their corresponding impact on commercial decision-making and operational restrictions (e.g. manufacturing and logistical capacity), which are beyond the remit and responsibilities of EMRN. EFPIA supports the EMRN's efforts to promote the solid framework for biosimilars approval in the EU, which is based on scientifically appropriate approval standards and robust pharmacovigilance measures that put patient safety first.

Questioning fit of new metrics;
We feel that Goal 2/Objective 5 (New metrics for accessibility of medicines that better represents real patient access to newly authorised medicinal products in different markets) does not fit with the overall goal as, in our view, the goal focuses on evidence, its generation, and communication around how the evidence supports the decision that has been made. In addition, "Real patient access" is about "reimbursed access" which requires not only that a medicine is approved by regulatory authorities, but also that national and regional decision makers determine whether a product is accessible to patients. Thus, this is not considered a task of regulatory authorities.

Question 7: Are there any other challenges that should be addressed by the EMA/HMA network in this area?

- Yes

No

If yes, please specify

Please remember to specify if a particular comment relates specifically to the human or veterinary part.

Rewording Goal 1/Objective 5 to recognise need for improved transparency of demand;
Goal 1/Objective 5 should be reworded in order to recognise the need for improved understanding and transparency of Member States' demand and should aim at increasing production capacity for critical medicines in order to meet country patient needs rather than demand. The COVID-19 pandemic has led to an enormous increase in demand for some medicines, especially medicines used in ICU and COVID-19 treatment candidates. Apart from the increase in production by EFPIA member companies to meet this exceptional growth in demand, all the evidence suggests that there is also an underlying allocation challenge. The real issue is, not simply about producing and supplying more drugs, it is about getting the right drug to the right hospital in the right country at the right time, and having a clear picture of what is likely to be needed for any future waves. Practices such as parallel export and stockpiling are disruptive.

Optimising advice provision and decision-making;
For the Goal of "Optimising the path from development to evaluation & Developing better scientific evidence", the first four objectives listed do address the challenges. This issue was highlighted as a key priority by EFPIA in its response to the EMA's RSS 2025. Here, EFPIA stated there is a need "to better connect the different decision-making steps across the lifecycle of a medicine". There is a similar need to better link and integrate medicine development advice across the EU regulatory ecosystem. The overall value of pan-EU scientific advice is undermined when contradictory opinions emerge during the development of a product. This can be through the different EMA Committees, but also, through the Member-State-led approach to decision-making for clinical trials. This national approach to clinical trials and the EU centralised approach to the provision of scientific advice also mean that there is no unified 'line of sight' on the progress of a product during its development from early clinical trials through to approval.

Providing enhanced advice options with greater flexibility in the delivery of this advice is needed to reflect the changing pace and process of innovation along the development continuum. This envisaged dynamic advice is also needed to adequately accommodate specialised input for specific types of products. In addition, EFPIA considers that this broadening and integration of regulatory advice should better bridge the advice and decision-making gap across the EU regulatory system (i.e., EMA, EMA's Committees, NCAs) and beyond (e.g., EUnetHTA, US FDA).

Defining shortages;
EU regulators should ensure a consistent and workable definition of shortages (covering both the supply and demand sides) and agree on standardised reporting requirements on clearly defined shortages based on patient needs and not on national demand giving priority to critical products with high potential impact. The information should be streamlined with an effective alert system as well as an alignment across the data provided from different sources. The information contained in the national data repositories set up in the context of the Falsified Medicines Directive (FMD) could be used to monitor net stocks levels at aggregate level.

Broader use of existing serialization data can enhance the area of inventory usage and stock levels to mitigate shortages through European Medicines Verification System (EMVS) if all supply chain actors collaborate and accept their FMD obligations (reference: EFPIA position paper 'Policy proposals to minimize medicines supply shortages in Europe – Lessons from COVID-19 crisis'; https://efpia.eu/media/15427/policy-proposals-to-minimise-medicine-supply-shortages-in-europe-march-2014.pdf). Patient relevant shortages, as opposed to manufacturing/supply shortages, will only be visible if patient level supply data becomes

available. EU-wide harmonised definitions for shortages at both the manufacturer and patient levels are needed, and a common reporting system should be used.

ROG activities;
A key challenge for the availability of medicines lies in the application of the EU variations legislation and the need to use the Regulation for minor administrative changes. EFPIA supports the work done by the Regulatory Optimisation Group (ROG) to simplify and automate regulatory processes and revise the variation procedure following internationally harmonised standards. EFPIA supports that the work of ROG be reinitiated and integrated into the EUNS 2025 plans.

Question 8: Are you undertaking concrete actions in this field that could support or complement EMA/HMA network activities?

- (●) Yes
- ( ) No

If yes, please elaborate which ones and provide details on how these could be considered.

EFPIA proposals for prioritised objectives;
EFPIA has presented proposals to the European Commission (EC) for a revision of the delegated variation regulation (1234/2008) to decrease unnecessary administration and adapt the regulation to scientific and technological developments. In order to effectively and efficiently evolve the EUNS 2025 to an action plan, EFPIA proposes the following as key, near-term priority objectives. EFPIA is undertaking practical and policy initiatives in several of these areas and looks forward to discussing its approaches with the EMRN as the EUNS 2025 is finalised and implementation begins:

- Goal 1/Objective 1: Identify the specific root causes of shortages and develop strategies to improve prevention and management of shortages. A better understanding of the specific causes for shortages of generics/off-patent products versus products still under patent protection is essential
- Goal 1/Objective 2 (partially – see comments above): Help to identify and suggest areas where changes to EU or national legislation could improve supply (such as (EFPIA suggests adding here “and receive respective information from other actors in the supply chain”) implementation of ePI…
- Goal 1/Objective 4: Improve coordination of information and actions, including implementation of best practices, both for EU regulatory authorities, stakeholders and international partners
- Goal 1/Objective 5 (see EFPIA proposed rewording above): Ensure availability of critical medicines in the EU/EEA by supporting increase of production capacity to meet demand
- Goal 2/Objective 1: Develop better scientific evidence which serves different decision makers along the decision chain (regulators, HTA bodies, payers)
- Goal 2/Objective 2: Better scientific evidence to support post-licensing follow-up of medicinal products
- Goal 2/Objective 3: Stimulate the life-cycle approach to evidence generation and the possibility to adjust decisions based on new evidence
- Goal 2/Objective 4: Clear and enhanced communication to patients, health care professionals as well as down-stream decision makers about the regulatory assessment including information gap inherent for medicinal products approved on the basis of limited scientific data and secondary endpoints (e.g. orphans)

Question 9: Are there any other ongoing or planned initiatives that should be considered for this proposed strategic theme area?

- (●) Yes

- ○ No

If yes, please provide details of the ongoing or planned initiatives.

EFPIA has called for High-Level Forum on Better Access to Healthcare Innovation;
The challenges linked to availability of medicines are complex and policy solutions require a holistic, comprehensive approach that reflects the multiple root causes, different stages in the production and supply, and different stakeholders involved. Furthermore, addressing the root causes when it comes to availability and access to medicines is a marathon, not a sprint. Action is needed on multiple fronts to achieve relevant and sustainable results.

EFPIA – together with other EU Health stakeholders – has called upon the EC to set up a High-Level Forum on Better Access to Healthcare Innovation involving Member States, the EC, and stakeholders to develop multi-stakeholder solutions to introducing new technologies into health systems and reduce the time patients in Europe wait for access to new treatments. This collaborative dialogue must be evidence-based, requiring an EU-led analysis of the root causes and drivers of access, supply and shortage issues.

We concur with the HMA that digital advances in the provision of labelling (particularly electronic patient information or ePI) could help mitigate the regulatory burden that contributes to challenges in the availability of medicines (EFPIA's ePI proposals are included under Theme 6). In addition, in view of potential vaccine supply constraints in some countries, electronic leaflets will facilitate the exchange of products between countries.

Fostering alignment of national implementation of compassionate use programmes is important but, in EFPIA's view may be better achieved via an IMI/IHI project; a PPP will help to involve multiple stakeholders and thus achieve a widely supported and sustainable approach.

Strategic Theme area 2: Data analytics, digital tools and digital transformation

Question 6: Do the objectives adequately address the challenges ahead?

- ◉ Yes
- ○ No

Comments on objectives of the strategic theme area:

Digital transformation opportunities are limitless;
The opportunities for digital approaches and data to transform the healthcare ecosystem are significant. These include primarily transformation of the regulatory network and its capacity to leverage technology and use new source data. It will be important that actions undertaken as part of the European Medicines Regulatory Network are complimentary with other initiatives at EU and national level. In particular, there should be strong alignment and synergies with the EC's Digital Strategy, aimed at shaping the EU's digital future including Artificial Intelligence (AI). The outlined goals and respective objectives cover many of the areas EFPIA agrees are required for digital transformation to occur. EFPIA believes that there must be:

- Appropriate governance and oversight of digital healthcare to encourage and enable secure sharing and use of high-quality health data within a European Health Data Space (EHDS);
- Enhanced digital literacy, competence and capacity building across the European Network;
- Greater use and reliance on real world data (RWD) uncovering insights from better data analytics and sharing information across the healthcare network;
- Adequate, clear and cohesive provisions to develop the use of AI; and
- Greater education of all stakeholders (patients, physicians, healthcare decision makers, etc.) and communications on the potential added value of digital transformation to society.

EFPIA is aligned with the HMA environmental analyses regarding the opportunities and challenges that digital data, broadly defined, brings to healthcare. EFPIA believes more emphasis is needed on the specific requirements for medicines regulation inherent in this emergent stage for data science and proposes specific strategic actions for each enabler. EFPIA supports the following positions:

Appropriate governance and oversight of digital healthcare;
These are needed to encourage and enable secure sharing and use of high-quality health data within a EHDS:

- EFPIA recognises the need for data standardisation in terms of collection, quality and management. Well defined standards for data quality can yield more consistent data sets and drive interoperability, leading to insights that address unmet medical needs, and the development of innovative care pathways and treatment paradigms. They set the foundation for advanced transformative technologies to derive meaningful insights from data at scale through AI and machine learning (ML). Further standards for high quality interoperable data accelerate research and innovation to improve patient care.
- EFPIA believes that accountability is at the core of data ethics and governance, providing clarity and distributing accountability fairly to ensure overall trust in AI and Big Data solutions. With appropriate governance, validation and internationally and globally recognised standards, we can ensure interoperability of the digital infrastructure, reliability of the technology and mitigate the risks of error, concerns about privacy, bias or inequality.
- Finally, international cooperation for high quality interoperable data standards and data sharing would enable more effective response to, and ideally prevention of, future global health issues such as pandemics. While the importance of data interoperability across data sources has long been acknowledged, the need for data sharing has arguably been underappreciated. Since there has been an unprecedented willingness to share data and ideas in the current COVID-19 pandemic, post-pandemic, regulators should facilitate processes for more formal data sharing agreements between and among stakeholders.

Greater use of RWD;
Greater use and reliance on RWD will uncover insights from better data analytics and sharing information across the healthcare network. EFPIA proposes pursuing RWE use cases / pilot programme to subsequently elaborate an EU RWE framework that sets out the principles for data use and standards, regulatory acceptance as well as best practices for application of analytical methods, in alignment with international efforts. EFPIA applauds the EMA and other international regulators (via ICMRA) for their collaboration on RWE considerations related to COVID-19. While we acknowledge the ongoing EMA-FDA interactions on RWE, we encourage the EMA to extend this to other international regulators, post-pandemic.

Question 7: Are there any other challenges that should be addressed by the EMA/HMA network in this area?

- (●) Yes
- ( ) No

If yes, please specify

Please remember to specify if a particular comment relates specifically to the human or veterinary part.

Transforming the use of data for decision-making and ultimately for the greater benefit of patients;
Addressing the objectives and their challenges will require significant investment and a collective willingness and commitment. The benefits of this investment are clear but should be elaborated on to ensure implementation is executable and fit-for-purpose. The breadth of goals described indicate a need for systematic collaboration across stakeholders beyond the EMRN and therefore leadership and collaboration may be required above and beyond the EMRN, i.e. globally. Implementation of the objectives will require a regulatory framework and guidance to provide each of the stakeholders with a common lexicon and set of expectations on how to transform the use of data for decision-making and ultimately for the greater benefit of patients. Open science platforms / "Commons" may be useful to consider, and early efforts to explore these approaches should be the focus of multi-stakeholder workshops. It is essential for Europe to stay in close collaboration with regulators globally to address advances in technologies and practice since data technologies, including AI, are/will be globally developed and disseminated, and other countries and regions may experience developments first.

Increasing the acceptability of RWE to support decision making throughout the product lifecycle;
EFPIA considers that the acceptability of RWE to support decision making throughout the product lifecycle needs to be enhanced. This can be accomplished by shared learning and pilot programmes resulting in guidance development, while addressing the various challenges associated with the use of RWE:

- There are concerns about data relevance, depth and quality necessitating related improvements as well as the infrastructure for interoperability of RWD sources such as that being utilised in the IMI EHDEN project. EFPIA welcomes the proposal to develop the DARWIN platform and recommends leveraging existing expertise with other similar platforms e.g. FDA's Sentinel. EFPIA also stresses the importance of enhancing data quality at the point of care since any such enhancements will provide value to all stakeholders (clinicians, patients, researchers, HTA bodies, etc.).
- Patients have privacy related concerns about how their data could be used beyond the originally intended purpose for which it was shared as well as the possibility of their data being shared with a third party, without permission. Thus, addressing data privacy concerns is needed for appropriate and sustainable access to and use of RWD.
- The ability to access data sources can also be challenging. For example, clarity regarding which stakeholder will have access to the proposed DARWIN platform would be welcome as well as a mechanism for sustainable funding. More generally, EFPIA encourages the EMA to recognise the importance of data sharing and facilitate a process for more formal data sharing agreements between and among stakeholders.
- There is a need for greater familiarity with and acceptance of study designs (e.g., registry-based randomized clinical trials) and modern causal inference methods (such as score methodology and methods for managing missing data or informative censoring) which necessitates development of best practices.

EFPIA offers the following proposals to the EUNS 2025;

- The EUNS 2025 should explicitly consider Software as a Medical Device (SAMD), particularly as this is used in combination with treatments including medicines. Moreover, the stakeholders likely to develop SAMD (as well as the wider work around AI and ML) may be new to medicines regulation and/or regulatory authorities engaged in assessing the SAMD (including AI and ML) and/or may have little experience with the software. Therefore, specific consideration should be given regarding outreach and training.
- Greater attention should be paid to how Big Data powered by AI/ML will be used by regulators in their decision making, or by others in their own activities (e.g. beyond the Public Assessment Report). This would include other regulatory agencies and downstream decision makers (e.g. HTA Bodies); as well as patients, healthcare professionals, academics and the general public. EFPIA recommends that Regulators' deployment of AI for dossier review should be phased based on the maturity of the technology and competencies, and initially address simple situations. Furthermore, when regulators introduce dossier review by AI, there should be transparency for the applicant to understand the output of the AI process; and how this is validated or reviewed.

Question 8: Are you undertaking concrete actions in this field that could support or complement EMA/HMA network activities?

- (●) Yes
- ( ) No

If yes, please elaborate which ones and provide details on how these could be considered.

EFPIA continues its active engagement in advancing digital health;
EFPIA is actively working on digital technologies and data with a view to transform healthcare and has the experience and expertise of medicines and device development and use. As an organisation focused on patients and health outcomes improvements, EFPIA has been actively engaging in digital health. Below is a list of some relevant EFPIA actions:

- Actively participating in Innovative Medicines Initiative (IMI) PPP consortia, e.g. Mobilise-D; Trials@Home; GetReal; Big Data 4 Better Outcomes; EHDEN; Electronic Health Records 4 Clinical Research;
- Working with the eHealth Stakeholders Group;
- Contributing to the 'Data Saves Lives' campaign to enhance trust and encourage sharing of health data for a positive benefit to health;
- Contributing to the EC's consultations on digital including those on the EHDS and the appropriate framework for the use of AI;
- Interacting actively with the HMA-EMA Big Data Taskforce;
- Working with EMA and other regulators on the future use and reliance on RWE/Registries, and has contributed to consultations, e.g.on the registries discussion paper, HMA-EMA Big data report, GDPR issues associated with secondary use of data; of note EFPIA has proposed Registries as a topic for a future ICH topic, and is currently considering revisiting its proposal to gain ICH acceptance.
- Joint publication with EMA and EUnetHTA in the British Journal of Clinical Pharmacology of an article on 'Regulatory and health technology assessment advice on postlicensing and postlaunch evidence generation is a foundation for lifecycle data collection for medicines' (Moseley et al, March 2020).

Develop use of AI;
EFPIA supports adequate, clear and coherent provisions to develop the use of AI:

- EFPIA seeks adequate, clear and coherent provisions (regulations and/or guidelines) regarding AI. At present, different types of regulation apply and are not aligned. We recognize that AI solutions are challenging for medicines regulation, as noted in the Strategy and its rejection of "black box" processes. Sectors that are pioneering in AI are focused on the outcome, not the black box process of algorithmic adaptation. The EUNS 2025 should consider how it would be possible to "open the black box" through a forensic review in a time- and cost-efficient manner, engaging the AI R&D community in this challenge. As in other sectors, there is also the need to identify areas where AI can be used safely without too much risk for patients or society, probably as a first step in a stepwise approach. We advocate for appropriate access to high-quality data in order to assure high quality AI solutions and to safeguard the EU's competitiveness in the international AI landscape; easy yet compliant access to high quality data will be indispensable. Transparency on methods, data, limitations and benefits should be non-negotiable for all actors in an environment that trusts AI powered solutions to impact people's health and wellbeing.
- Because data science, AI and digital healthcare are still emerging technologies, we suggest that adaptive/dynamic regulation be the preferred approach taken, as described in the Strategy. Achieving this is by no means simple and, at its core, should include more frequent and regular dialogue with stakeholders to evaluate progress and revise planning. An optimised, integrated process for scientific advice delivery is

required, ensuring alignment with HTA bodies (see EFPIA comments on Theme 1).

Capacity, capabilities, and resourcing may prove rate-limiting;
Expertise, and not technology, may prove the most significant rate-limiter, with funding coming a close second.

- The ERMN should first assess the current capacity and capability for biostatistics and data analytics. The proposal for clusters of expertise is a practical means to build capacity quickly. EFPIA would advise using these clusters then to establish a community of biostatistics and data analytics/AI experts across regulators, medicine developers and academia.
- EFPIA supports continuous education of all stakeholders (patients, physicians, healthcare decision makers, etc.) and communications, on the potential added value of digital transformation to society.
- While all of the objectives identified are important, it is likely that there are some fundamental building blocks to digital transformation that have to be initiated first, such as Goal 4, that will establish the correct governance and Goal 1, which will enhance the availability of relevant data. Subsequently, EFPIA believes the focus should shift to developing capacity and capability and ensuring that the regulatory and access systems adapt to cope with this new flow of data aimed at improving EU citizen's health.

Question 9: Are there any other ongoing or planned initiatives that should be considered for this proposed strategic theme area?

- (●) Yes
- ( ) No

If yes, please provide details of the ongoing or planned initiatives.

A more collaborative governance model should be part of future telematics strategy;
Industry's vision of a more collaborative governance model is to be part of the committee drafting the future Telematics Strategy, but also of a cross-Telematics Steering Committee consisting of EMA, HMA, NCAs and medicine developer representatives from the relevant trade associations, and beyond the current consultation on a per-project basis. Industry also sees the need to balance the strategic direction with near-term "wins" through tactical efforts (such as opportunities to connect and leverage existing data source). The Steering Committee should include experts with different backgrounds and competencies (business and IT experts) and discuss overarching topics such as priorities, timelines and milestones for project delivery, dependencies between projects, funding model, governance, expected outcomes and value realisation and the creation of buy-in from all stakeholders. Furthermore, we propose stronger emphasis on:

- Encouraging trust and secure data sharing within the context of GDPR;
- Supporting appropriate governance of the EHDS;
- Supporting the establishment of a framework for appropriate use of AI/ML;
- Supporting efforts to establish and adopt European EHR format;
- Promoting and supporting initiatives raising adoption of data standards and improving interoperability e.g. common outcomes definitions and measurement guidelines;
- Engaging with organisations and associations to align on digital health;
- Engaging with EC and platforms such as eHealth Network;
- Engaging with the clinical/medical and patients' community on data collection;
- Engaging with existing national clinical research networks and the wider research community to advocate for access to RWD;
- Engaging with existing national clinical research networks and the wider research community to promote access to RWD in addition to traditional clinical trial research.
- Engaging with supply chain stakeholders to explore the possibility for data collected through (EMVS) to be accessible for other purposes (research, pharmacovigilance, adhesion services, etc.); and

- Bringing the topic to the International level, i.e. through ICH and/or ICMRA.

In terms of next step needed actions and sequencing, in the short term (2020-2021), EFPIA recommends:
- Establishing an EHDS with appropriate Governance;
- Focusing on encouraging data sharing under a more collaborative governance model.

Over the medium to long-term EFPIA recommends for EMA to:
- Develop guidance on SAMD, AI, digital endpoints, rolling/dynamic review, the use of RWE to support lifecycle decision making, etc.:
- Institute a leaner, quicker advice process that can be used for priority products during the peri-approval phase, where appropriate;
- Develop best practices on analytical approaches used to generate RWE from RWD;
- Implement societal digital literacy program;
- Support the development of harmonised, international (i.e. ICH) guideline on the use of RWE (including registries) to support lifecycle regulatory decision making.

The future of digital health is now;
Success will ultimately have to be measured by enhanced access to innovative therapies that improve health outcomes for European citizens. Digital technologies have the potential to facilitate medicine development by improved target identification of new therapies. These technologies may also result in greater use of available and new health-related data as well as adoption of new endpoints associated with the use of wearable devices or smartphone applications that provide: (1) insights that are not yet harnessed, and enhanced connection of information regarding how a product is used; and (2) ongoing feedback and adjustment about how best to utilize digital technologies with the most appropriate/targeted patients. These create efficiencies for the overall healthcare system, ultimately leading to a learning, more agile and adaptable healthcare system that is better connected within countries and across the European Union. These advances could place the EU in a leading position at a global level.

Strategic Theme area 3: Innovation

Question 6: Do the objectives adequately address the challenges ahead?

- (●) Yes
- ( ) No

Comments on objectives of the strategic theme area:

EUNS 2025 Innovation theme vitally important;
Overall, EFPIA rates the Strategic Theme of Innovation as “Very Important”, although it considers that the totality of draft EUNS 2025 goals and objectives are not sufficient to fully support the needed advances. Welcoming that one of the core themes is “Innovation”, EFPIA contends that the EUNS 2025 should further demonstrate how the EMRN will advance its global competitiveness to truly support the capabilities, policies, and plans needed to foster the next wave of innovative medicines for the benefit of patients in Europe. As promising as the RSS 2025 was in charting a navigable course for regulatory science to ‘regulate new types of medicines’ for patients with unmet medical need, the EUNS 2025 seems, in some important areas, to be less connected to this strategic imperative. While the draft EUNS 2025 prominently includes this separate theme on Innovation, EFPIA considers that its description does not go far enough (and may not be fast enough) to advance the EU’s global competitiveness. Additionally, it is unclear if and how the EUNS 2025 Innovation Theme’s goals and objectives, and those described across the full strategic document, will fully deliver in spite of the fundamental and mounting challenges that medicine innovation faces.

The EMRN 5-year strategy comes at a critical juncture for the EU regulatory system and its collaborators. The next few years will be characterised by a changing landscape for innovation - shaped by the arrival of new technologies and new sources of evidence – which must be matched by expanding capabilities on the part of European and global regulatory communities. The reality of new technologies (e.g., advanced therapy medicinal products (ATMPs), digital therapeutics) and new modalities for drug discovery and development (e. g., AI, advanced analytics, in silico studies and coordination with medicinal devices) will only increase over the next five years. Effectively identifying, treating and preventing diseases and their complications will require medicine developers and regulators to optimise the use of novel evidence in decision-making, leading to stronger collaboration with all stakeholders including the patient community, health care professionals, HTA bodies and academia. EFPIA very much hopes that the learnings gained from regulatory agilities and flexibilities introduced to combat COVID-19 will find their place in future EMRN plans.

EMRN collaboration with other stakeholders including industry;
In EFPIA’s view, the EMRN is optimally placed to progress Europe’s role in the globally competitive regulatory ecosystem to help foster future innovative medicines for patients with unmet medical needs. Within the EUNS 2025 Innovation Theme, there are many objectives that overlap with the final recommendations already outlined in the EMA’s RSS 2025. From EUNS 2025, it is at times unclear how these will be reconciled, e.g. what can the EUNS focus on that will complement the EMA’s planned actions. EFPIA believes that the EMRN is uniquely placed to drive innovation in domains such as clinical trials operations and medical devices (some EU NCAs have both medicines and medical devices within their remit).

In EFPIA’s view, the draft EUNS 2025 does not go far enough in addressing these essential and rapidly evolving innovation enablers. EFPIA’s comments on the draft EUNS 2025 will highlight potential areas for increased connectivity with its own strategic regulatory science initiative (i.e. RRI). Not surprisingly, the RRI conclusions - particularly relevant in light of the current COVID-19 crisis – are to: encourage the use of new types of clinical trials; allow greater use of data from real world use; allow ongoing dialogue and discussion about a treatment throughout development; and simplify how medicines and other healthcare products are regulated. Furthermore, while the final RSS 2025 incorporated a tiering of its recommendations based on stakeholder input, it is unclear if and how the final EUNS 2025 will prioritise and order implementation across the many included draft objectives. As such, EFPIA offers its suggestions for prioritising the Innovation Theme’s objectives within the following responses to the EMRN’s questions with specific proposed actions.

Question 7: Are there any other challenges that should be addressed by the EMA/HMA network in this area?

- (●) Yes
- ( ) No

If yes, please specify

Please remember to specify if a particular comment relates specifically to the human or veterinary part.

Fostering innovation in clinical trials;
EFPIA is fully supportive of the EMRN's inclusion of the objective to 'foster innovation in clinical trials'. As such, there are important challenges that EFPIA wishes to amplify. Integrated therapeutic solutions, i.e. pharmaceuticals and medical devices (including digital tools) and in vitro diagnostics, including companion diagnostics and a combination of these tools, require unique support for clinical trials. There is strong correlation needed between the scientific advice coordinated by EMA on clinical research and national clinical trial authorisations and ethics reviews. As such, it is increasingly important for medicine developers to gain alignment and coordination of the different contributors e.g. CTFG, national agencies' clinical trial units and ethics committees, SAWP, PDCO and/or CHMP. Indeed, the focus is currently on complex clinical trials, e.g. platform trails, adaptive trial designs, remote decentralised trials, umbrella or basket trials, and on remote inspections.

Additionally, innovative trial designs require adequate support by the right statistical expertise and computational capacity in both industry and regulatory agencies. For example, many designs with adaptive elements require extensive simulations, and the number of simulations needed is associated with high computational intensity. It is critical that EMA ensures sufficient statistical and computational capacity to review protocols of innovative trials and provide advice that is consistent with current best practices regarding e.g. Bayesian designs, Registry-based CTs or Modeling and Simulation to evaluate trial operating characteristics, and other novel approaches.

The MS are the backbone of clinical research in Europe. While the EMA (with its RSS 2025) can help to drive scientific advances in CTs, it is the EMRN (and the MS NCAs) that should ensure all nationally-placed stakeholders (ethics, healthcare professionals, academics, HTA bodies) are all actively engaged with developments into the novel approaches taken to conduct clinical research. Therefore, 'Foster innovation in CTs' should be elevated from an objective to a theme or goal with its own specific objectives.

Integrating regulatory advice along the development continuum;
Consistent with its response on this topic in Theme 1, EFPIA considers that the regulatory science approach to optimally integrate the provision of regulatory advice along the development continuum could likewise be further strengthened within the EUNS 2025. Engagement between regulators, medicine developers, and other stakeholders in early advice is an essential component to ensuring acceptance of increasingly complex data generation. While incorporating elements of integrated and iterative advice (for example, Goal 2 /Objective 8: Develop further the collaboration of various groups involved with scientific advice and/or regulatory guidance; Goal 4/Objective 3: Increase collaboration with Medical Device Authorities (should be reworded to add "and notified bodies"); and Goal 4/Objective 4: Identify and enable access to the best expertise across Europe and internationally), the EUNS 2025 should describe in detail the connections between these objectives as well the role of regulatory dialogue and advice. How will these currently separate objectives be connected? How will implementation of these objectives result in greater advice agility and efficiency to reflect the changing pace and process of innovation?

EFPIA supports opportunities for EU-wide advice in order to achieve more iterative, agile and aligned advice whilst maintaining the high-quality scientific discussion currently available. It is important that the scheme is adequately resourced by the participating agencies, to ensure that the process is streamlined and efficient and does not jeopardise development timelines. Additionally, EFPIA strongly supports the creation of a more

integrated advice and evaluation pathway for medicine-medical device combination products, but also for medicines that are developed and used in combination with companion diagnostics. In sum (and as noted in comments on Theme 1), there is a need to better link and integrate medicine development advice across the EU regulatory ecosystem. Providing enhanced advice options with greater flexibility in the advice delivery is needed to reflect the changing pace and process of innovation along the development continuum.

Advancing use of real-world data (RWD) and evidence (RWE);
In terms of prioritising RWD/RWE, EFPIA has offered detailed comments and proposed actions in its response to the EUNS 2025 Theme 2 on digital health. Importantly, there is an essential link of RWD/RWE to innovative CT design as well as to integrated advice. Therefore, EFPIA proposes that the EUNS 2025 incorporate the necessary links between these three regulatory science concepts.

Question 8: Are you undertaking concrete actions in this field that could support or complement EMA/HMA network activities?

- (●) Yes
- ( ) No

If yes, please elaborate which ones and provide details on how these could be considered.

Maintaining important distinctiveness of regulatory processes and decisions;
In overall Goal 2 (and connected to Theme 2 on Digital Health), EUNS 2025 proposes to Foster collaborative evidence generation - improving the scientific quality of evaluations and ensuring generation of evidence useful to all actors in the lifecycle of medicines, including HTAs, and pricing and reimbursement authorities. Consistent with EFPIA's comments on RSS 2025, EFPIA believes that regulatory processes and regulatory determinations should remain distinct from decision making for different purposes (pricing, terms of access). The opportunity to determine the value of a medicine in healthcare follows a regulatory decision, and this opportunity is appropriately based in the specific context in which healthcare is delivered. Payers are a fundamental decision-maker with regard to access to medicines, and there are benefits related to engaging with payers earlier, to gain insight into their perspectives on unmet medical needs and priorities. Early engagement may help to prepare payers for potential impacts from breakthrough innovation. Of course, it is at the discretion of each individual company – rather than of regulatory officials - to engage with payers in light of their portfolio and planning, to pursue this engagement at the most suitable time.

Prioritising the Innovation Theme's objectives;
As stated in its response to the draft RSS 2025, EFPIA would welcome the opportunity to share more detailed proposals for advancing improvements and strategic initiatives across the Innovation Theme. In order to effectively progress to an action plan, EFPIA offers the following list of near-term priority objectives. EFPIA is undertaking practical and policy initiatives in several of these areas and looks forward to discussing its approaches with the EMRN as the EUNS 2025 is finalised and implementation begins for the following proposed priorities:

- Goal 1/Objective 1: Support developments in precision medicine, biomarkers and 'omics and translation of advanced therapy medicinal products (ATMPs) into patient treatments
- Goal/Objective5: Promote and invest in the PRIME scheme
- Goal 2/Objective 1: Foster innovation in clinical trials
- Goal 2/Objective 8: Develop further the collaboration of various groups involved with scientific advice and/or regulatory guidance
- Goal 4/Objective 3: Increase collaboration with Medical Device Authorities (EFPIA suggests adding

here “and with Notified Bodies”)

EFPIA considers that the following EUNS 2025 innovation-related objectives should be given the next highest level of priority across the draft proposals:

- Goal 1/Objective 6: Facilitate the implementation of novel manufacturing technologies
- Goal 1/Objective 8: Improve expertise to accommodate rapid evolution of the regulatory system
- Goal 4/Objective 4: Identify and enable access to the best expertise across Europe and internationally

EFPIA’s proposed actions to foster innovation in clinical trials;
EFPIA proposes that this objective is elevated to an EUNS 2025 theme or goal. Consistent with the noted priorities, EFPIA continues to develop proposals to best implement its RRI with some goals quite similar to those included under the EUNS 2025 Innovation Theme. In order to ‘Foster innovation in clinical trials’ EFPIA proposes that EMRN, particularly under EMA’s leadership, undertake efforts to:

- Develop a new strategic initiative to broaden the use and acceptability of innovative clinical trial designs based on experiences so far and with the support of all relevant stakeholders and experts;
- Coordinate cooperation opportunities (e.g., multi-stakeholder workshops, demonstration projects) and pilot schemes to discuss case studies with developers reflecting range of complex study designs and share learnings);
- Enhance discussion opportunities with NCAs ahead of CT application submissions (under the CT regulation)
- Develop further the CT Information System to best accommodate innovative and complex CTs (e.g. resolution of the issue relating to submission of multiple CT modifications);
- Support increased education of ethics committees who may not understand complex designs (can be an issue during review of CT application);
- Facilitate better alignment between EU regulators in the CT pathway; and
- Advance global coordination on the topic.

EFPIA’s proposed actions to advance iterative advice;
To further advance scientific advice and regulatory guidance, EFPIA suggests that the EUNS 2025:

- Lead the redesign of a more flexible and integrated R&D product support mechanism;
- Enhance the coordination of advice across EMA Committees, NCAs and other pertinent stakeholders;
- Ensure wider stakeholder involvement; and
- Consider special perspectives (e.g., paediatrics, drug-device combination products) within the advice continuum.

Question 9: Are there any other ongoing or planned initiatives that should be considered for this proposed strategic theme area?

- (●) Yes
- ( ) No

If yes, please provide details of the ongoing or planned initiatives.

As mentioned in this EUNS 2025 response, EFPIA stands ready to collaborate with the EMRN to ensure effective implementation of its final plan. As such, EFPIA supports the inclusion of greater clarity for how the EUNS 2025 will manage network partnerships with all stakeholders, including medicine developers to ensure the most complete and patient-centered approach possible with the aim of facilitating new medicines innovation. Engaging in regulatory science collaborations internationally is also critical for the EMRN as demonstrated in the unprecedented global fight against the COVID-19 pandemic. EFPIA looks forward to contributing to these solutions (e.g. IMI EU PEARL project on platform trial).

Advancing precision medicine;
EFPIA agrees with the EUNS 2025 Goal 1/Objective 1 to 'Support developments in precision medicine, biomarkers and 'omics and translation of advanced therapy medicinal products (ATMPs) into patient treatments', which is also well aligned with RSS 2025. Scientific and technological advances such as gene and personalised therapies, smart health applications, medical technologies, including AI, are transforming the medical landscape. However, the EU regulatory system needs to embrace this concept further, e.g. individualised medicines require adaptation in how CTs are conducted. Enhanced collaboration (including for CTs) across the broader network of decision makers including NCAs for GMO and Tissues & Cells is important to ensure a coherent and streamlined approach for development of ATMPs. EFPIA considers that the development of solutions to ease regulatory burden and streamline filing procedures to enable use of platform technologies and other manufacturing-enabling technologies, is needed for ATMP production. Also, complex medicines delivering healthcare solutions with possibly different converging technologies will require a flexible regulatory system that is integrated with medical device regulation. With these gaps considered, the EU is lagging behind other global regulatory agencies in the development of supportive guidelines and frameworks. Additionally, the biomarker qualification framework should be improved and, hopefully, harmonised with the US FDA.

Promoting and investing in the PRIME scheme;
Through PRIME, the EMA offers earlier and enhanced interactions on scientific data and development plans. In order for PRIME to achieve its promise, an earlier window of engagement should be implemented for all developers based on compelling non-clinical data in a relevant model and first-in-man studies indicating adequate exposure for the desired effect and tolerability. For PRIME to foster the early development of innovative medicines in Europe and to accelerate patient access to medicines for unmet need, the scheme will require effective cooperation between theEMA and the different working parties, NCAs, HTA bodies and the EC, also in connection with European and national innovation schemes. The value of PRIME would need to be translated at the National level through a coordination or transfer of scientific positions and assessments to bridge the EMA advice to any national activities (e.g. SA to CTA, GMO, ATU, etc.). This is even more important for complex innovations (e.g. ATMP, innovative combination products) involving different national authorities. Such an iterative coordination dialogue would facilitate the convergence of evidence requirements through the EMRN. Resources and capabilities are needed to foster the scheme. Finally, and consistent with RSS 2025, the expansion of the PRIME scheme to new indications of registered products will be critical.

While the strategy appears to acknowledge the need to address alternate approaches in the clinical arena that have the potential to accelerate development programmes, there is no clear mention of the impact of such clinical acceleration on the CMC programme and ultimately the submission content. The strategy should address the need for a shift in such cases to science- and risk-based CMC submission content which facilitates acceleration, and would be useful for Adaptive Pathways products.

Strategic Theme area 4: Antimicrobial resistance and other emerging health threats

Question 6: Do the objectives adequately address the challenges ahead?

- ○ Yes
- ◉ No

Comments on objectives of the strategic theme area:

AMR is a well-established major public health threat;
Despite the fact that AMR is known as a well-established major public health threat, slow progress has been made in addressing this rather predictable and preventable health threat. COVID-19 has demonstrated the public health and economic impact when countries are not fully prepared for a health emergency and do not have the medicines, vaccines, and diagnostics needed. There needs to be renewed focus on addressing AMR to respond to this known threat. Several stated EUNS 2025 objectives would not necessarily fall under the remit or responsibility of the regulatory network. Therefore, EFPIA strongly supports increased multi-stakeholder cooperation and dialogue at EU level and believes that the EC should have a central role in facilitating a debate among all relevant actors, including Member State governments, to define a comprehensive approach to combat AMR. EFPIA believes that the EU and MSs have a critical role to help avert this looming public health crisis by:

- Creating an environment where stakeholders can collaborate to address the challenge at national and EU level;
- Closely collaborating with all stakeholders (including medicine and vaccine developers) to ensure the development and implementation of national AMR Action Plans that include a holistic approach to monitoring and surveillance, and by encouraging the use of non-traditional approaches and prevention options including vaccines;
- Putting in place market-based solutions to stimulate the development of new antibiotics and vaccines at the EU level (e.g., transferable exclusivity) and encouraging MSs to enact necessary reimbursement and HTA reform;
- Adapting the regulatory framework to enable efficient pathways for drug development in this area;
- Addressing environmental concerns related to AMR from all sources (e.g., manufacturing, sanitation, runoff, waste-water treatment); and
- Leveraging international attention to the issue to deliver on urgent actions.

Challenges with current economic model and need for incentives;
The current market for antibiotics does not provide an appropriate return to innovators and investors to take on the necessary risk and uncertainty that comes with the development of new antibiotics and vaccines. The unique challenges and dynamics of the antibiotics market require unique market-based measures to establish an economic environment that will sustainably incentivise new and ongoing antibiotic R&D. A package of incentives needs to be put in place to address the challenges across the antibiotic product lifecycle and meaningfully impact private investment in antimicrobial R&D. These incentives – push and pull – should be designed to stimulate R&D across the full R&D lifecycle, from discovery through development and commercialisation. As part of this package of incentives, EFPIA believes that there is an urgent need for novel pull incentives implemented at the EU-level, combined with reimbursement and HTA reform at the Member State-level that would create supportive market conditions for development of innovative products targeting unmet medical needs. In contrast, not-for-profit development models have not delivered the level of innovation needed in other therapeutic areas. Further, not-for-profit developers would still be subject to the same scientific, regulatory, and economic challenges facing all developers. Shifting to a not-for-profit model

would require significantly higher public investment than the proposed set of incentives, since it would require the public sector to shoulder all costs and risk of research, development, and commercialisation; market-based incentive models leverage public funding to secure significantly greater private investment. The developer takes on the risks and costs of failures, since the public only pays for products that reach the market.

It will be essential that regulators engage with stakeholders and other national authorities in supporting the appropriate valuation of novel antimicrobials to complement the EU-level pull incentives, as well as ensuring national reimbursement systems that enable responsible use of novel antimicrobial products. HTA bodies play a critical role in addressing AMR. HTA and reimbursement reforms are needed to better reflect the full value of antibiotics and enable their appropriate access. The unique development challenges of antibiotics are poorly understood by many stakeholders. A key focus for future activities needs to address the link between regulatory evidence and access discussions (HTA, payer) with a focus on supporting market viability for these products. The EMRN is ideally placed to strengthen dialogue and engagement with HTA bodies including opportunities for joint scientific advice with HTA bodies and publication of articles to explain evidentiary standards and the basis of assessment for antibiotics and how they differ from other therapeutics.

Question 7: Are there any other challenges that should be addressed by the EMA/HMA network in this area?

- (●) Yes
- ( ) No

If yes, please specify

Please remember to specify if a particular comment relates specifically to the human or veterinary part.

Capturing the breadth of the issue and ensuring necessary regulatory guidance;
Although the term AMR (antimicrobial resistance) is used, the focus in the EUNS 2025 is on antibacterials in line with the EMA approach. However, in the EC document, “A European One Health Action Plan against Antimicrobial Resistance (AMR)”, there is a broader scope since it also includes antifungals, antiparasitics, and antivirals. The role of vaccines should also be considered. The EMRN should ensure the breadth of this scope is explicitly captured within the strategy. In this respect, the focus of Goal 3 should not be restricted to veterinary medicines but should also include human medicines and vaccines.

Prioritisation is needed to efficiently incentivise R&D and direct research investment to areas of highest unmet medical need. Guidance on where to focus efforts to address unmet medical needs in antimicrobial research is required, and in this respect, EFPIA welcomes efforts such as the WHO priority pathogens list. EFPIA also believes that the EC should develop a European priority pathogens list, taking into account the WHO list, following a discussion with all relevant sector stakeholders, including the private sector. This list would help determine research priorities and stimulate R&D in Europe, if coupled to appropriate pull mechanisms, and ensure that the EU continues to contribute to globally relevant solutions.
- Reference to more detailed proposals: https://www.efpia.eu/media/219769/joint-ebe-efpia-and-ve-statement-on-amr-1.pdf.

Fostering the development of new antimicrobials;
Goal 5/Objective 1 calls for “Foster development of new antimicrobials including new antibacterials” and the Strategy states “Regulators…can guide developers on regulatory requirements, including platforms for dialogue such as PRIME and ITF”. EFPIA considers that there is opportunity for the EMRN to consider additional regulatory specific actions such as:

- Broadening the definition and application of “unmet need” with respect to the treatment of bacterial infections;
- Consider greater use of all existing tools in the current regulatory framework for antimicrobial products (including vaccines) that address serious or life-threatening infections and/or are of major interest for public health. These include many of the approaches being undertaken to address COVID-19 including expediting therapeutic innovation by accelerating development and regulatory assessment e.g. conditional approval (particularly relevant for less prevalent resistance – where Phase 3 data are more difficult to gather), use of adaptive pathways and approval under exceptional circumstances; and
- Additional guidance on PRIME eligibility for antibiotics, including exploring the criteria and timing of inclusion considering the way antibiotics are currently developed should also be considered. The development of prophylactic vaccines (playing a role in addressing antimicrobial resistance) should also benefit from regulatory support under PRIME.

Other emerging health threats;
COVID-19 highlights the critical role played by medicines and vaccines in addressing emerging health threats, but also the challenges linked to accelerated development of these products, in particular vaccines. Europe needs to put in place a more comprehensive, sustained commitment to pandemic preparedness, to tackle future outbreaks and other health security threats such as AMR. This can help the region be better prepared for future pandemics and ensure long-term resilience. Learnings from COVID-19 should drive a reflection for better EU health threat preparedness, for instance on a) specific regulatory pathways (e.g. for registration of platform technologies); b) reinforcement of surveillance structures (epidemiology, vaccine safety and effectiveness); and c) appropriate incentives for the development of vaccines.

Question 8: Are you undertaking concrete actions in this field that could support or complement EMA/HMA network activities?

- (●) Yes
- ( ) No

If yes, please elaborate which ones and provide details on how these could be considered.

EFPIA suggests the following as priority EUNS 2025 objectives for AMR;
In order to effectively and efficiently evolve the EUNS 2025 to an action plan, EFPIA offers the following list of near-term priority objectives. EFPIA is undertaking practical and policy initiatives in several of these areas and looks forward to discussing its approaches with the EMRN as the EUNS 2025 is finalised and implementation begins.

- Goal 3/Objective 1: Adapt existing guidelines and develop new guidance on antimicrobials (particularly taking into consideration the situation concerning limited markets in veterinary medicines) to implement new legal requirements and emerging science
- Goal 4/Objective 1: Define value of new antibacterial agents to inform new business models (EFPIA suggests rewording to “Provide input on the value of new antibacterial agents, which could inform new business models”; EFPIA considers that the establishment of value and business models is beyond the remit of regulators)
- Goal 4/Objective 2: Cooperation on new business models
- Goal 4/Objective 4: Contribute to global antibiotic innovation
- Goal 5/Objective 1: Foster development of new antimicrobials including new antibacterials
- Goal 5/Objective 2: Define regulatory pathways for phages and other innovative products
- Goal 5/Objective 4: Engage with relevant human and veterinary stakeholders to effectively discuss the issue
- Goal 5/Objective 5: Ensuring necessary guidance is in place for authorisation of veterinary and human alternatives to antimicrobials
- Goal 5/Objective 6: Foster development of new antimicrobials for human use
- Goal 6/Objective 1: Refine regulatory activities in inter-epidemics periods to increase preparedness
- Goal 6/Objective 2: Harmonise regulatory framework and approaches for investigation of medicinal products during emergencies

Question 9: Are there any other ongoing or planned initiatives that should be considered for this proposed strategic theme area?

- (●) Yes
- ( ) No

If yes, please provide details of the ongoing or planned initiatives.

Connecting industry's ongoing activities with EUNS 2025 for AMR;
EFPIA wishes to call attention to the commitments and associated actions of the AMR Industry Alliance, which are intended to address the following four areas. These are well aligned with the EUNS 2025:

- Invest in R&D to meet public health needs with new innovative diagnostics & treatments;
- Improve access to high-quality antibiotics and ensure new ones are available to all;
- Work to reduce the development of antimicrobial resistance; and
- Support measures to reduce environmental impact from production of antibiotics.

- An Executive Summary of progress to date is available:
https://www.amrindustryalliance.org/wp-content/uploads/2020/02/AMR-2020-Progress-Executive-Summary.pdf

In addition, EFPIA suggests prioritisation of the activities within the EUNS 2025 in alignment with the activities of the newly launched AMR Action Fund https://www.amractionfund.com/.This commitment of US$1 billion by over 20 large pharmaceutical companies will help sustain the current antimicrobial pipeline, which is close to collapse, and provide time for governments – including both the EU and MSs – to implement the necessary market-based reforms to create an environment where antimicrobial R&D can flourish for decades to come.

Finally, actions of the cross-industry initiative Eco-Pharmaco-Stewardship (EPS) should be considered, including a multi-stakeholder initiative on medicines disposal. One priority behind EPS activities is the compilation of best industry practices in the management of pharmaceutical manufacturing effluents so that drug producers can minimise the risks to the environment and the spread of AMR.
More information available from the links:
- EPS: https://www.efpia.eu/about-medicines/development-of-medicines/regulations-safety-supply/pharmaceuticals-in-the-environment-pie/)
- Medsdisposal: http://medsdisposal.eu/

Strategic Theme area 5: Supply chain challenges

Question 6: Do the objectives adequately address the challenges ahead?

- ○ Yes
- ◉ No

Comments on objectives of the strategic theme area:

EFPIA supports concrete actions to progress innovation in manufacturing and supply technologies;
Several of the objectives in this theme appear to be based on a perception that poor quality practices are at the core of supply chain challenges and hence more supervision and inspections are proposed. Before implementing such solutions, however, the root causes of the challenges need to be fully analysed and understood. In addition, challenges and solutions should be considered in global context, and not addressed in isolation at EU or Member State level. In addition, EFPIA would expect more concrete outcomes supporting innovation in manufacturing and supply technologies. If Europe wishes to place itself as a global leader for innovative medicines manufacturing and supply technologies, EMRN support is needed.

Supply chain management will have to adapt to reflect changing expectations;

The objective to ‘Analyse the possible implications of new manufacturing technologies’ seems to not be directed towards a clear outcome in support of innovation. Although not mentioned in the objectives, it is equally important to facilitate the development and optimisation of current manufacturing methods, for example, through support for science and risk-based control strategies and their lifecycle management. Likewise, these objectives do not clearly describe removal of barriers for new models such as continuous manufacturing, or adoption of digital technologies and approaches associated with Pharma 4.0 across the supply chain. The evolution of the “connected patient” via smart technology and monitoring systems, online prescriptions and pharmacy e-commerce, personalized medicines, etc. can enhance healthcare delivery and create new patient expectations for accelerated availability of medicines. The pharmaceutical supply chain will need to adapt to reflect these changing expectations, and a key goal for the EMRN should be to have the appropriate regulatory and data privacy in place to protect patients while avoiding the EU falling behind globally. EFPIA believes that the use of hospital exemptions for ATMPs for economic reasons could grow over the coming years, which could undermine regulatory oversight and protection of public health. This should be addressed by increasing transparency and convergence of requirements across Member States and limiting use to situations where there is no alternative treatment or clinical trial (see reference: https://www.ebe-biopharma.eu/wp-content/uploads/2017/10/2017-10-10-EBE-EFPIA-Position-Paper-on-HE-FINAL.pdf).

Promoting harmonised regulatory requirements;
The EU should continue to promote globally harmonised regulatory requirements, reliance on regulatory decisions and to support strengthening of local oversight by regulatory agencies, especially in third countries to ensure the quality of medicines imported into the EU. Inspection reliance can be leveraged through PIC/S. EFPIA recommends that GDP aspects of the supply chain be addressed with a focus on harmonising implementation of the GDP requirements across MSs.

Preventing falsified medicines from entering the supply chain;
EFPIA recognizes that it is important to prevent falsified medicines from entering the legitimate supply chain and suggests that the focus should be on fully implementing and enforcing the FMD, especially the mandatory use of the EMVS by all players, including dispensing in community pharmacies and hospitals.

Promoting supply chain resilience;
EFPIA believes that the sophisticated orchestration and flexibility of today’s global pharmaceutical manufacturing and supply network should be acknowledged. Supply chain resilience can best be promoted through regulatory initiatives, in the EU and harmonised globally, that facilitate business continuity planning and enable implementation of technologies and systems that strengthen operational resilience and robustness throughout a product’s lifecycle.
Supply chain resilience, in general, should remain a responsibility of the MAH. The greatest problem with shortages appears to be for older, off-patent products, and therefore providing detailed information on supply chain in MA dossiers at the time of MAA or product launch might not be helpful in assessing risk, leading instead to a disproportionate increase in administrative burden and reduction in manufacturing and supply chain agility.

Efforts to promote supply chain resilience should focus on the most critical products (e.g. medically necessary or with high risk for supply). Extending Mutual Recognition Agreements (MRAs) concepts, harmonised regulatory requirements, and implementation of reliance on inspections conducted by a PIC/S member can enable supply chain flexibility and resilience and assure the quality of medicines through risk-based GMDP regulatory inspections and QP (GMP) and RP (GDP) oversight.

Question 7: Are there any other challenges that should be addressed by the EMA/HMA network in this area?

- (●) Yes
- ( ) No

If yes, please specify

Please remember to specify if a particular comment relates specifically to the human or veterinary part.

Supply chain resilience will require supportive regulatory agility;
EFPIA believes that regulatory agility, successful implementation of the relevant ongoing IT projects, and an effective forum for regular and/or continuous scientific dialogue with key stakeholders on CMC-GMDP will be important measures, and therefore, should have even more focus in EUNS 2025. The EMRN strategy document notes the impact of the COVID-19 pandemic and EFPIA believes that the lessons learned from the pandemic are still emerging and need follow up. Supply chain resilience will require supportive regulatory agility, and the regulatory flexibility agreed during COVID -19 for supply of crucial medicines (as described in the EU Regulatory network Q and As for COVID-19) will provide a good basis for further progress in this area (see EFPIA SCWG and RSC Drug prevention shortages paper (in progress)).

Connecting to EU Telematics Strategy;
Transparency on manufacturing and supply chain requires the successful implementation of relevant ongoing IT projects (Art 57, SPOR, TOM, EMVS). Therefore, the EUNS 2025 needs to be closely linked to the EMRN's Telematics Strategy.

EFPIA recommendations for progressing the Supply Chain objectives;
EFPIA offers support and recommends that consultation processes/engagement with industry, as a key stakeholder for Theme 5, need to be greatly increased to ensure that the objectives of the EUNS 2025 can be achieved. EFPIA recommends that the EU regulatory system develop an effective forum for regular and /or continuous scientific dialogue with key stakeholders on CMC-GMDP manufacturing and supply topics.

EFPIA proposes to prioritise the following objectives further;

- Goal 1/Objective 2: Tackle falsified medicines; prevent presence of falsified medicines in the supply chain:

Full implementation and enforcement of FMD across all stakeholders should remain a priority where support of the EMRN is needed.

- Goal 2/Objective 3: Harmonise approach to regulatory/inspection procedures:

EFPIA recommends that the EMRN partners with industry to strengthen the capability of inspectors, especially in relation to new technologies. Enhancing the capability of inspectors and assessors and their understanding of new technologies is also important to avoid creating regulatory barriers to innovation. This can be further enhanced by harmonising the approach to regulatory inspection procedures across EU MSs and, where possible, fostering reliance on regulatory oversight in Third countries by local competent authorities and the understanding of EU GMP/GDP requirements.

EFPIA proposes that the scope of the following objectives should be reconsidered;

- Goal 2/Objective 2: Promote a more tailored supervision of API manufacturers through assessment and inspection of their API development and risk management practices in technology transfer:

With regard to a more tailored supervision of API manufacturers, while this objective may contribute to improvements in the quality and/or supply for some products, API manufacturing does not appear to be the major cause of supply disruptions or quality issues (references: EFPIA survey on location of APIs manufacturing/outsourcing and possible impact on supply disruptions (21 February 2020); ECIPE Key Trade

Data Points on the EU27 Pharmaceutical Supply Chain (9 July 2020)). EFPIA believes the existing framework assigning responsibilities to the QPs is well equipped to ensure adequate supervision and encourage the network to collaborate with industry with respect to development of training.

- Goal 3/Objective 1: Develop EU level data integrity guidance:

This objective risks creating disharmony with other expectations of leading regulatory authorities and could have the undesired consequence of discouraging innovation: as noted above, consultation with industry experts will be essential to avoid a negative outcome.

- Goal 4/Objective 3: Promote reliability of the source of starting materials:

Root causes of supply chain issues are manifold and need further investigation. Encouraging supply chain resilience is an important goal but any actions in this area should be undertaken after consultation with supply chain experts from industry to avoid measures being counterproductive. For example, EFPIA believes measures such as national stockpiling and national restriction of export deplete the overall EU supply (See Preparing for a second potential COVID-19 crisis: joint statement by Medicines for Europe and EFPIA with AT Kearney 22 June 2020, EFPIA June 2020 report 'The root cause of unavailability and delay to innovative medicines').

Question 8: Are you undertaking concrete actions in this field that could support or complement EMA/HMA network activities?

- (●) Yes
- ( ) No

If yes, please elaborate which ones and provide details on how these could be considered.

EFPIA has a clear vision for improvements in this area;
There has been an important ad hoc dialogue with all key stakeholders during the COVID -19 crisis under the leadership of the EMA and the EC with significant contributions by EFPIA and other industry associations. Important learnings on supply chain management, as listed above, have been derived from the joint efforts during the crisis. In terms of innovation in manufacturing, based on past stakeholder workshops, EFPIA has provided clear proposals to EMA in the form of a white paper: "White paper on manufacturing and upscaling submitted to EMA for discussion in the context of COVID-19". As noted above, EFPIA recommends that the consultation processes/engagement with industry, as a key stakeholder for Theme 5, be greatly increased to ensure that the objectives of the EUNS 2025 can be realised. EFPIA recommends that the EU Regulatory system should develop an effective forum for regular and/or continuous scientific dialogue with key stakeholders on CMC-GMDP manufacturing and supply topics.

Question 9: Are there any other ongoing or planned initiatives that should be considered for this proposed strategic theme area?

- (●) Yes
- ( ) No

If yes, please provide details of the ongoing or planned initiatives.

Developing long-term, high-level strategy on European manufacturing embracing innovation;
EFPIA proposes to develop a more long-term, high-level strategy on European manufacturing embracing the

innovations in manufacturing ahead of us. For reference see “MQEG Digitalisation in Manufacturing Position paper”. EFPIA also wishes to share resources on experience with GMP/GDP Inspections. For reference see “EFPIA Annual Inspection survey and related position papers” (incl. in collaboration with IFPMA). Short-term, it will be important to start the dialogue with EMRN on the CMC white paper focusing on manufacturing and quality aspects in the context of COVID-19 supply management and learnings thereof. Longer term, a more continuous dialogue with EMRN on CMC-GMP manufacturing/supply issues should be established.

Furthermore, EFPIA recommends that the EMRN adopts clear metrics or KPIs to enable progress against the goals/objectives to be monitored. The following are KPI proposals for consideration:

Availability and accessibility of medicines - Shortages;

- Implementation of harmonized EU definitions of Drug Shortage based on patient needs (EU Regulatory Agencies with EMA) in all MSs
- EU harmonized definition of critical supply medicines
- iSPOC in place and working to allow shortage mitigation across EU for critical medicines without undue duplication of national efforts (EU Regulatory Agencies with EMA)

Supply Chain Challenges;

- Full implementation and enforcement of FMD (EMVS) across all countries and stakeholders. Availability of patient level supply data to the MAH to steer supply chain end-to-end (supported by Regulatory Agencies but driven by Ministries)
- Harmonised GDP implementation across MSs
- Innovative manufacturing technologies registered in EU vs other regions (e.g. US, UK, Switzerland)
- Metric to demonstrate more efficient utilization of network inspection resources in relation to inspections in Third Countries – use of inspections by PIC/S members or bi-lateral/multilateral agreements

EFPIA supportive resources (*available upon request)

- MQEG Digitalisation in Manufacturing Position Paper*
- EFPIA Annual Inspection Survey and related position papers (incl. in collaboration with IFPMA)*
- MQEG Regulatory response to COVID-19 crisis: Proposals for Quality and GMDP aspects white paper*
- EFPIA’s response to the EC’s Pharmaceutical Industry Strategy Roadmap: https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/12421-Pharmaceutical-Strategy-Timely-patient-access-to-affordable-medicines/F535948
- EFPIA position paper: Policy proposals to minimize medicines supply shortages in Europe – Lessons from COVID-19 crisis; May 2020*
- Preparing for a second potential COVID-19 crisis: Joint statement by Medicines for Europe and EFPIA with AT Kearney (22 June 2020) https://www.efpia.eu/news-events/the-efpia-view/statements-press-releases/preparing-for-a-potential-second-covid-19-wave/
- EFPIA June 2020 report ‘The root cause of unavailability and delay to innovative medicines’: https://www.efpia.eu/media/554527/root-causes-unavailability-delay-cra-final-300620.pdf

Strategic Theme area 6: Sustainability of the Network and operational excellence

Question 6: Do the objectives adequately address the challenges ahead?

- ○ Yes
- ◉ No

Comments on objectives of the strategic theme area:

Strengthening the integration of EUNS 2025 and RSS 2025 to achieve operational excellence;
EFPIA concurs with most of the challenges identified in the EUNS 2025 document and wishes to offer strategic input relating to the goals and objectives needed to establish sustainability and operational excellence. EFPIA considers that a single EMRN strategy, integrating the final RSS as appropriate, is essential to deliver the scientific and regulatory capacities and capabilities needed for the cohesiveness of the European system. As such, EFPIA welcomes this EUNS 2025 objective. However, to achieve this interconnected strategic plan, in the final EUNS 2025, EFPIA suggests adding clarity on the roles of EMA and of EU NCAs in meeting the EUNS 2025 objectives and connecting to implementation of the RSS 2025.

In order to truly achieve operational excellence, several challenges will need to be sufficiently addressed in the EUNS 2025. Appreciating the scarcity of resources across the system and stakeholders, the EMRN should implement actions to leverage all possible synergies, ensure risk-based approach to regulating, and minimise regulatory redundancy. Indeed, optimisation of the current framework through efficiency of existing operations is a fundamental principle for the network to be effective. For example, initiatives discussed and agreed within the Regulatory Optimisation Group with regard to simplification of variations (including the use of PMS data to eliminate some administrative variation types) should be progressed.

Leveraging EU network expertise and capabilities;
With a view to promoting the best use of the (scientific) expertise within the EMRN, a more optimal organisation of the available expertise across the network should be considered. The approach should avoid duplication of work and facilitate enrichment of the expertise through more collaborative working, including enhanced outreach at national level for academic expertise and international expertise. To achieve this objective, a fully coordinated, aligned, and holistic approach, leveraging all available expertise (rather than on geographical representation) and organising resources mindfully within the network is needed. Just as the model for R&D is continually evolving, the corresponding regulatory model will continue to concurrently adapt considering expertise needed in the long-term.

Implementing ePI as a priority;
While the EUNS 2025 states that ePI “should be considered as a way to facilitate marketing of (newly authorised) medicines in all Member states”, there is not a specific objective prioritising the implementation of ePI. The RSS 2025 included a recommendation to “Deliver improved product information in electronic format (ePI)”, and EFPIA suggests similarly incorporating an objective within EUNS 2025 given NCAs’ role. There is important work already underway to progress the ePI agenda by the EMA and HMA, with opportunities to reach key milestones in the near-term and these efforts should be emphasised and integrated. As such, EFPIA supports appropriate funding for initiation of the ePI set-up project, the establishment of clear timelines and roadmap planning, and the introduction of a stakeholder platform to further define steps needed in the development of the ePI creation tool and common standards.

Achieving a sustainable financial model for the network;
EFPIA rated Goal 3 (Achieve a sustainable financial and governance model for the network) as “Very Important” as the current levels of resource and investment in the EU regulatory system seem insufficient to ensure competitiveness and allow rapid adoption of new science and technologies. A balance between EC funding for public health and funding from medicines developers for fee-for-service activities is needed to deliver a world-class regulatory system in the interest of patients. The EUNS 2025 highlights “the need to ensure an appropriate funding model for the Network going forward and support recruitment, retention and development of staff with the right competencies”. EFPIA believes that efficiency improvements instituted to

simplify the fee system would have a substantial positive resource effect on EMA and NCAs. EFPIA welcomes the ongoing evaluation of the current EMA fees system to ensure that a suitably resourced EU regulatory system can fully support the innovative medicines of today and tomorrow, and as such, has generated several ideas to further streamline and enhance fees approaches. EFPIA communicated some of its preliminary proposals in its response to the EC's 2019 Inception Impact Assessment and looks forward to further dialogue on its funding positions.

Question 7: Are you undertaking concrete actions in this field that could support or complement EMA/HMA network activities?

- (●) Yes
- ( ) No

If yes, please specify

Please remember to specify if a particular comment relates specifically to the human or veterinary part.

Developing an integrated EU Telematics Strategy;
An underlying factor when addressing the sustainability of the EMA and ERMN is the ever-increasing reliance on digital infrastructure and integrated IT systems – highly critical for a modern operation of the overall regulatory system. However, within the EUNS 2025, this foundational telematics element is somewhat diluted because references are spread across the different themes. Additionally, the EUNS 2025 refers to the EU Telematics Roadmap and also to the EU Telematics Priorities documents, neither of which are currently available. The recent past has shown that IT processes have commonly encountered significant delays (e.g., clinical trials database, SPOR system) and have become bottlenecks to regulatory innovation. Other aspects of the EU Telematics Strategy also need consideration such as workflows supporting review and approval processes. Therefore, a foundational cross-topic strategy should be incorporated.

The EUNS 2025 text for Theme 6 highlights that governance of IT as one of the key challenges; however, IT governance is not identified within the list of included objectives. As previously shared with the EMRN, EFPIA would welcome the opportunity to contribute in a formal Telematics governance role and to share its ideas on developing a comprehensive digital strategy.

Similarly, the challenge of IT funding has been identified, although the EUNS 2025 does not describe an objective (e.g. process streamlining, infrastructure investment) for seeking resolution. The resourcing and funding for current telematics projects (IDMP/SPOR, CTIS, eCTD v4, CESSP, ePI) must be considered to allow the more visionary projects in the EMRN strategy to be realised. A comprehensive EU Telematics Strategy must also be fully integrated into the EUNS 2025 and appropriately prioritised and resourced. EFPIA considers that one of the issues that needs to be reconciled is that at the EMA level, IT funding comes from the EU, whereas at the MS NCA level, the IT funding comes from the national governments. This situation can result in competing and/or inoperable systems between the EU level and the MS level (and between MS).

Question 8: Are you undertaking concrete actions in this field?

- (●) Yes
- ( ) No

If yes, please elaborate which ones and provide details on how these could be considered.

Progressing and institutionalising learnings from COVID-19 pandemic fight;
EFPIA fully supports the Goal 5 to Enable quick, consistent and adequate response to public and animal health challenges including the current COVID-19 pandemic. EFPIA believes that the application of learnings from the COVID-19 response should be considered and institutionalised across the regulatory system beyond just public health emergencies. Indeed, reviewing the learnings from COVID-19 is inherently linked to other objectives on capacity and capability building, work-sharing, and efficient use of the EMRN. Best work-sharing and reliance practices should be applied within the EU and also with trusted regulator partners globally. This can be supported through digital tools and avoidance of duplication in assessments.

EFPIA suggests the following as priority EUNS 2025 objectives;
In order to effectively and efficiently evolve the EUNS 2025 to an action plan, EFPIA offers the following list of near-term priority objectives. EFPIA is undertaking practical and policy initiatives in several of these areas and looks forward to discussing its approaches with the EMRN as the EUNS 2025 is finalised and implementation begins. EFPIA key priorities are:

- Goal 1/Objective 1: Integrate EMA's Regulatory Science Strategy to 2025 within the EMRN 2025 Strategy
- Goal 1/Objective 2: Ensure 'fit-for-purpose' scientific capability of the Network
- Goal 2/Objective 1: Optimise the current regulatory framework by ensuring efficiency of the existing regulatory operations
- Goal 3/Objective 2: Ensure best use of resources through promoting mutual reliance and work-sharing
- Goal 4/Objective 1: Establish an IT operating model and services, in support of the digital strategy and digital business transformation
- Goal 5/Objective 1: Review learnings from COVID19 and strengthen EU coordination and response to public health emergencies, including crisis communication

EFPIA next level priorities are:

- Goal 1/Objective 3: Ensure optimal organisation of the available expertise within the Network
- Goal 2/Objective 3: Continue already initiated IT process improvements to further professionalise securing, provisioning and running of technology services
- Goal 3/Objective 1: Contribute to the revision of the current fee regulation, and implement the final solution
- Goal 3/Objective 3: Continuously seek effectiveness and efficiency gains to maximise use of scarce resources

Question 9: Are there any other ongoing or planned initiatives that should be considered for this proposed strategic theme area?

- (●) Yes
- ( ) No

If yes, please provide details of the ongoing or planned initiatives.

Ongoing benefit-risk initiatives;
Goal 2/Objective 4 is intended to Expand benefit-risk assessment and communication for human and veterinary medicines. EFPIA concurs that informed regulatory decisions are best made on the basis of both benefit and risk with a good understanding of the patient perspective. Consistent with its comments on the similar RSS 2025 recommendation, EFPIA welcomes the incorporation of patient preferences and improved

communication with HTAs bodies. There are a number of initiatives ongoing within the EMRN on this topic, including participation in related IMI projects. EFPIA would encourage continuation of the current initiatives. In particular, the EMRN should advance science and methodology alignment on gathering patient preferences and the relevance of benefits and risks from the patient perspective.

Any other comments

Please feel free to provide any other additional comments not provided in the previous questions

EFPIA overarching comments applicable across the EUNS 2025 Themes: We would like to suggest assigning unique identifiers (e.g. 1.1.1) to each objective across the EUNS 2025 to ease its navigation.

Additionally, EFPIA concurs with many of Vaccines Europe's comments, and as such, EFPIA has avoided redundancy in its comments as much as possible.

Thank you very much for completing the survey. We value your opinion and encourage you to inform others who you know would be interested.

Useful links

EU Medicines Agencies Network Strategy (https://www.ema.europa.eu/en/about-us/how-we-work/european-medicines-regulatory-network/eu-medicines-agencies-network-strategy)

European Medicines Agencies Network Strategy to 2025 (https://www.ema.europa.eu/en/documents/other/european-medicines-agencies-network-strategy-2025-protecting-public-health-time-rapid-change_en.pdf)

Pharmaceutical Strategy for Europe (https://ec.europa.eu/info/law/better-regulation/have-your-say/initiatives/1242-Pharmaceutical-Strategy-Timely-patient-access-to-affordable-medicines/public-consultation)

Background Documents

european-medicines-agencies-network-strategy-2025-protecting-public-health-time-rapid-change_en.pdf

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