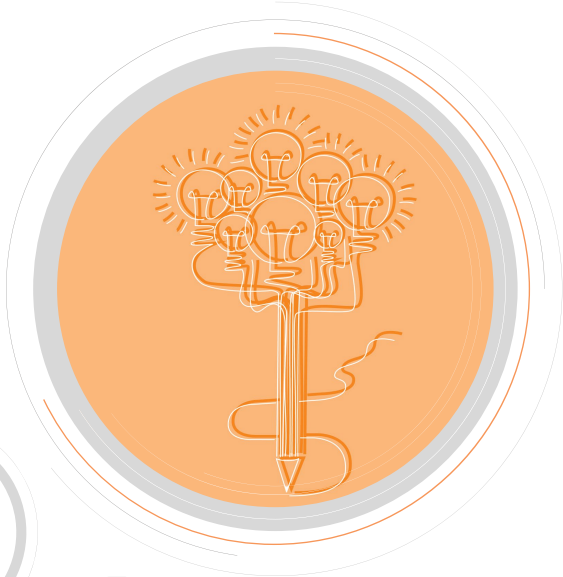
EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH



EMA Regulatory Science to 2025

Overview of the outcome of the Publication Consultation

Human Stakeholders Workshop

Presented by Tony Humphreys on 18 November 2019
Head of Scientific Committees Regulatory Science Strategy Division

An agency of the European Union

Why now?

- To monitor and sign-post emerging and future trends in science and technology
- To identify key priorities where new or enhanced engagement is essential to the continued success of the Agency's mission
- To prioritise use of resources and external collaborations to strategically advance regulatory science
- To shape and influence the vision for the EU Medicines Agencies Network (EMRN) Strategy 2020–25

Vision

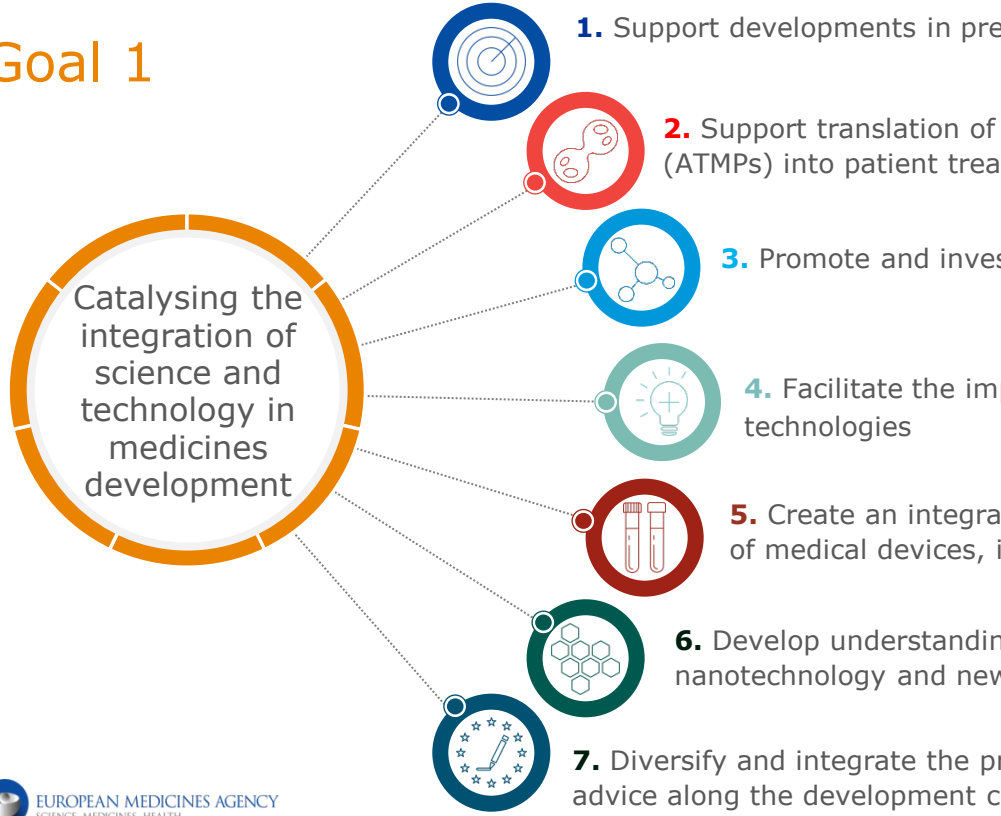
> To underpin its mission of protecting human health, **EMA must catalyse and enable regulatory science and innovation to be translated into patient access to medicines in evolving healthcare systems.**

EMA Regulatory Science to 2025

EUROPEAN MEDICINES AGENCY

Five goals for human medicines regulation

Goal 1



1. Support developments in precision medicine, biomarkers and 'omics'

2. Support translation of advanced therapy medicinal products (ATMPs) into patient treatments

3. Promote and invest in the PRIority Medicines scheme (PRIME)

4. Facilitate the implementation of novel manufacturing technologies

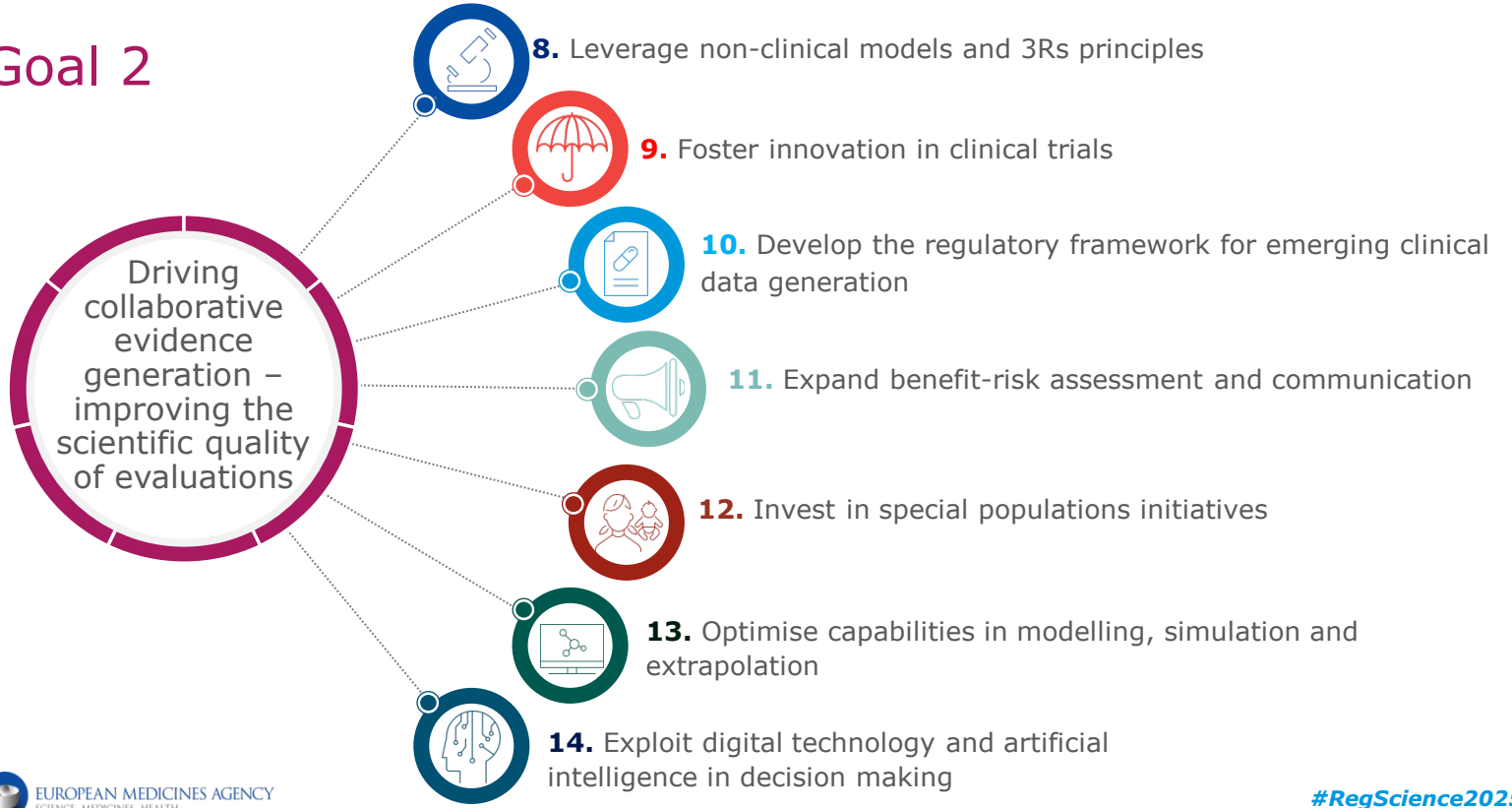
5. Create an integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products

6. Develop understanding of, and regulatory response to, nanotechnology and new materials in pharmaceuticals

7. Diversify and integrate the provision of regulatory advice along the development continuum

Five goals for human medicines regulation

Goal 2



Five goals for human medicines regulation

Goal 3

15. Contribute to HTA's preparedness and downstream decision making for innovative medicines

16. Bridge from evaluation to access through collaboration with payers

17. Reinforce patient relevance in evidence generation

18. Promote use of high-quality real-world data (RWD) in decision making

19. Develop network competence and specialist collaborations to engage with big data

20. Deliver improved product information in electronic format (ePI)

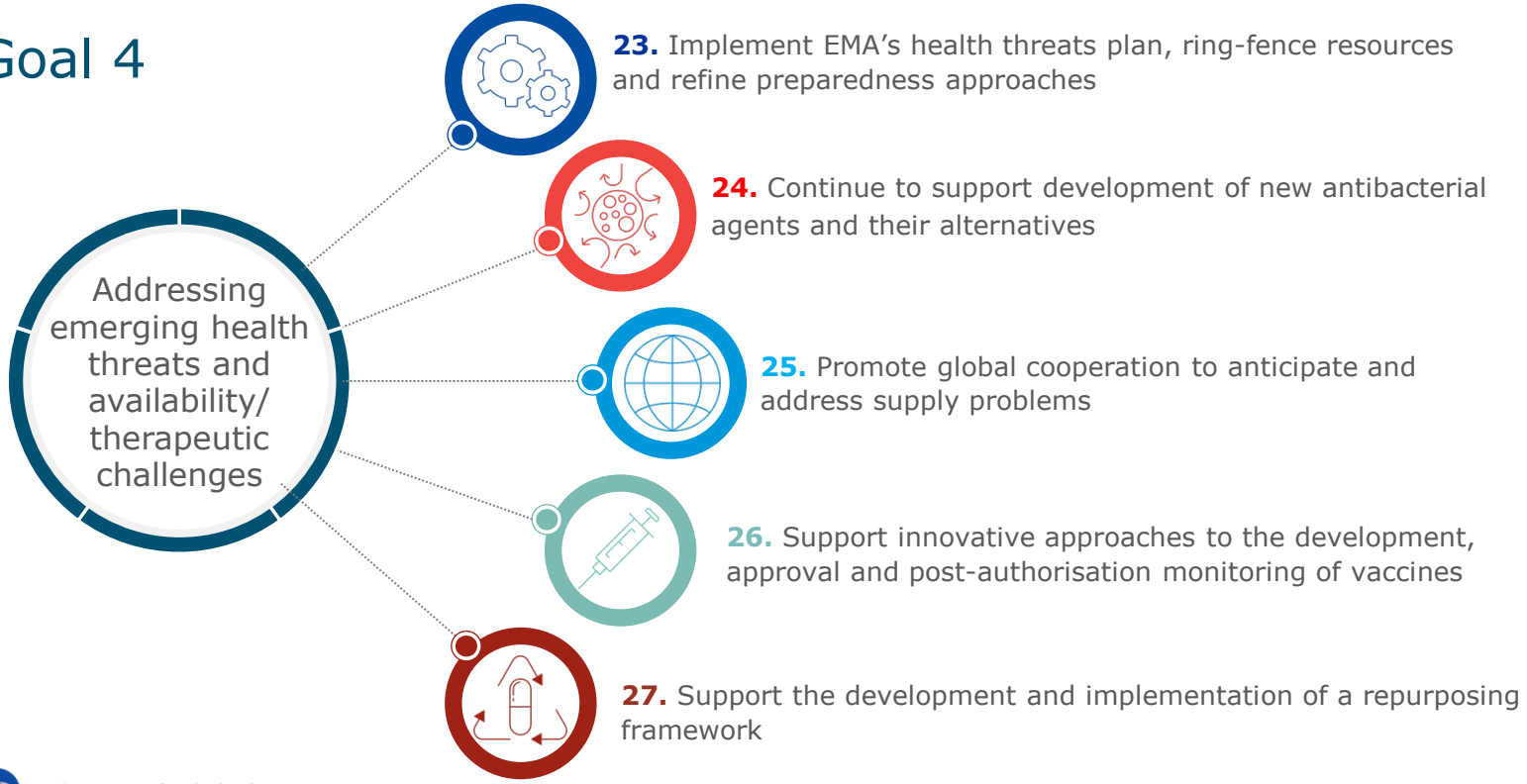
21. Promote the availability and support uptake of biosimilars in healthcare systems

22. Further develop external engagement and communications to promote trust and confidence in the EU regulatory system

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Five goals for human medicines regulation

Goal 4



Five goals for human medicines regulation

Goal 5

28. Develop network-led partnerships with academia to undertake fundamental research in strategic areas of regulatory science

29. Leverage collaborations between academia and network scientists to address rapidly emerging regulatory science research questions

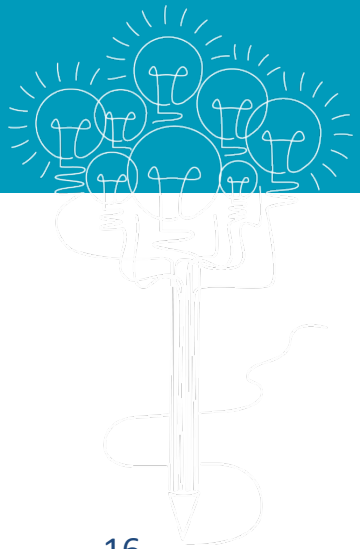
30. Identify and enable access to the best expertise across Europe and internationally

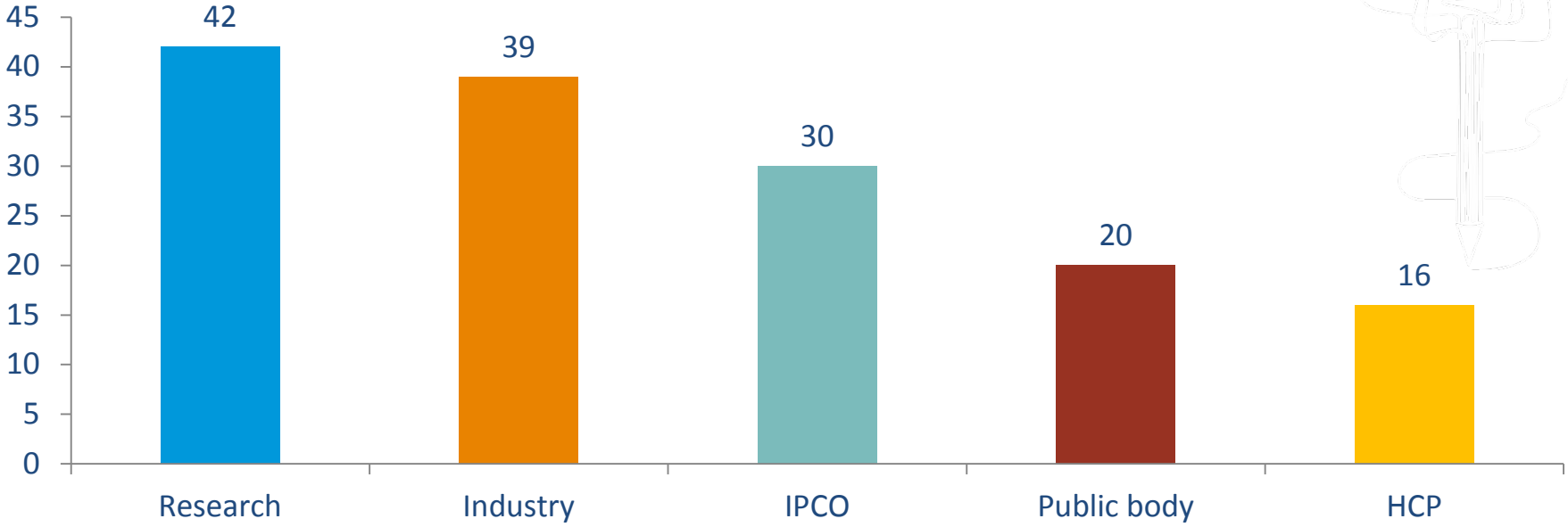
31. Disseminate and exchange knowledge, expertise and innovation across the network and to its stakeholders

Cluster composition

Cluster 1 (IPCO+)	Cluster 2 (HCP)	Cluster 3 (Research)	Cluster 4 (Public body)	Cluster 5 (Industry)
• Individual member of the public • Patient or Consumer Organisation • Advocacy Group	• Healthcare professional organisation • Healthcare professional	• Other scientific organisation • European research infrastructure • Academic researcher • Learned society	• EU Regulatory partner / EU Institution • Health technology assessment body • Payer	• Pharmaceutical industry(trade association, individual company, SME)

Overall public consultation responses per stakeholder cluster





Qualitative analysis of stakeholders‘ views on the Regulatory Science Strategy

DATA SOURCE:

- Responses to the open-ended questions (Q3,5,6,7) on:
  - Overall views
  - Core recommendations and their underlying actions
  - Missing elements

OBJECTIVES:

- Summarising these responses
- Identifying missing elements
- **Extracting *concrete actions* for further internal and external discussion**

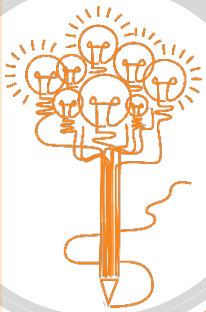
METHOD FOR ONGOING ANALYSIS:

- Framework analysis consisting of 5 iterative steps
- Investigator triangulation via independent analysis and comparison by a team of researchers

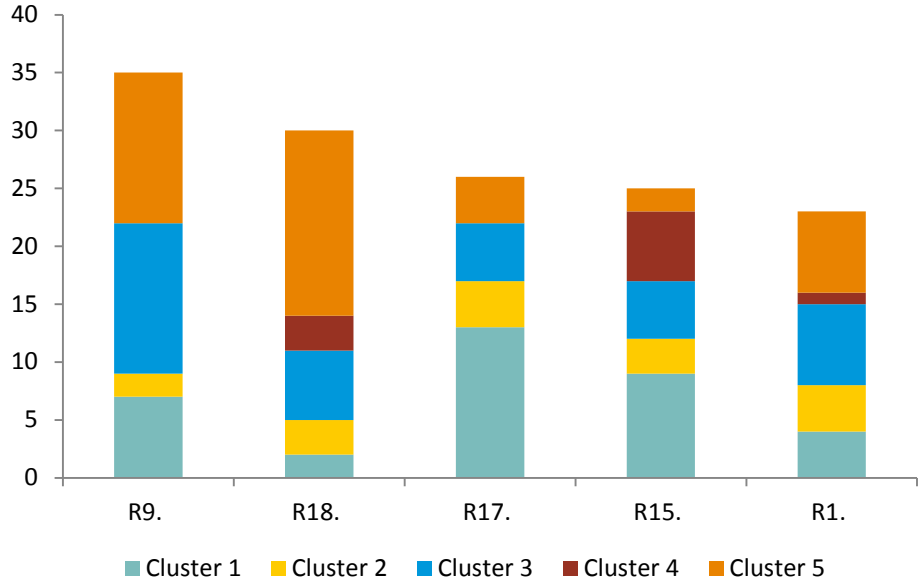
Lacey A. and Luff D., Qualitative Research Analysis. The NIHR RDS for the East Midlands/Yorkshire & the Humber, 2007.

Question 5

"Please identify the top three core recommendations (in order of importance) that you believe will deliver the most significant change in the regulatory system over the next five years and why."



Overall aggregate ranking of core recommendations – Top 5



9. Foster innovation in clinical trials

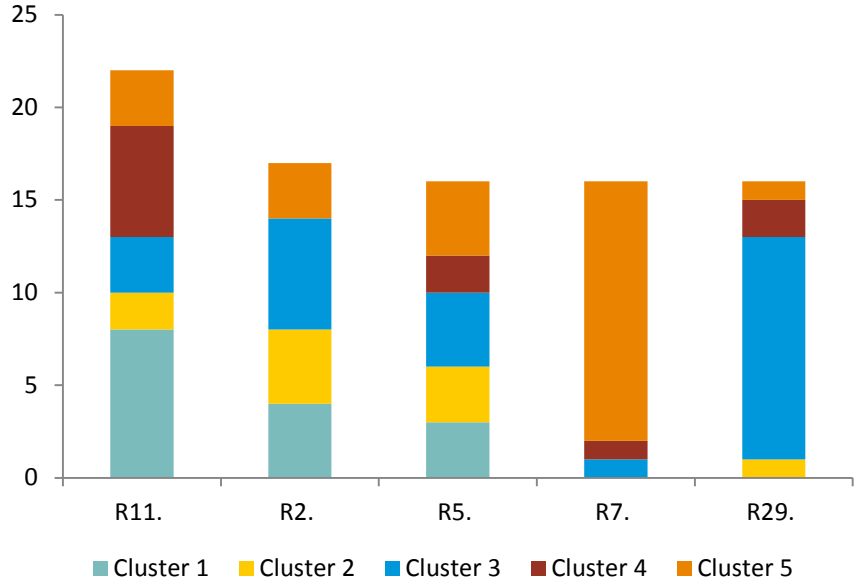
18. Promote use of high-quality real-world data (RWD) in decision making

17. Reinforce patient relevance in evidence generation

15. Contribute to HTA's preparedness and downstream decision making for innovative medicines

1. Support developments in precision medicine, biomarkers and 'omics

Overall aggregate ranking of core recommendations – Top 6-10



11. Expand benefit-risk assessment and communication

2. Support translation of advanced therapy medicinal products (ATMPs) into patient treatments

5. Create an integrated evaluation pathway for the assessment of medical devices, in vitro diagnostics and borderline products

7. Diversify and integrate the provision of regulatory advice along the development continuum

29. Leverage collaborations between academia and network scientists to address rapidly emerging regulatory science research questions

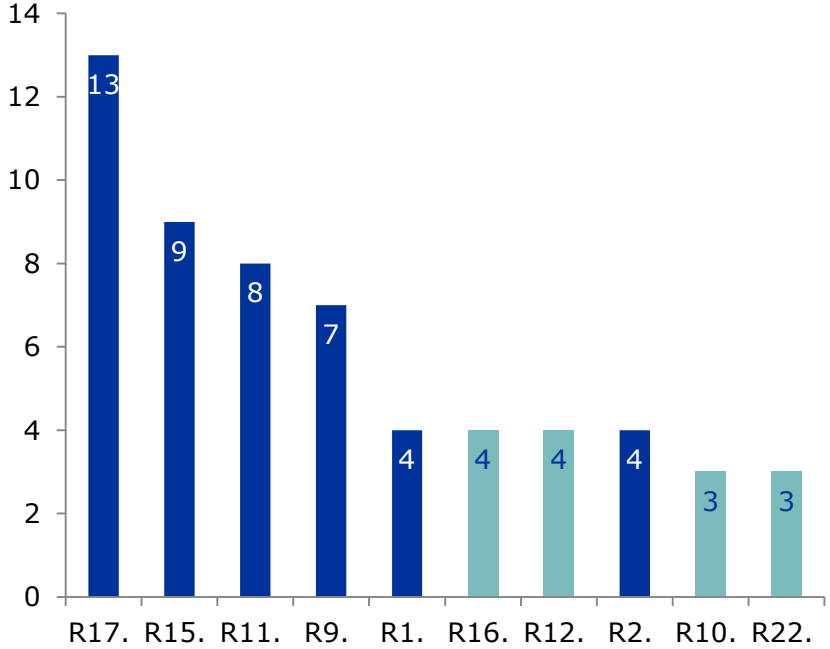
Cluster 1: Individual member of the public/patient or consumer organisation and advocacy groups

Comprehensive & relevant. Welcomed intent to take broader role both pre- and post- approval. Address pharmacovigilance overtly

- More ambition for systematic and meaningful patient engagement
- Critically reflect on early access schemes with increased uncertainty
- Increased stringency evidence requirements for pre-post approval studies
- Ensure clinical relevance of approved medicines
- Enhance use of patient relevant and reported outcomes in Benefit/Risk decision-making
- Need for increased patient involvement to make better use of pharmacovigilance and RWD
- Improve communication transparency and debate about regulatory decision-making
- Open science – make publicly available more documents (clinical trials, scientific advice, meetings, decision-making)

Cluster 1: Individual member of the public/patient or Consumer Organisation and advocacy groups

Ranking of top 3 core recommendations



10. Develop the regulatory framework for emerging clinical data generation

12. Invest in special populations initiatives

16. Bridge from evaluation to access through collaboration with payers

22. Further develop external engagement and communications to promote trust and confidence in the EU regulatory system

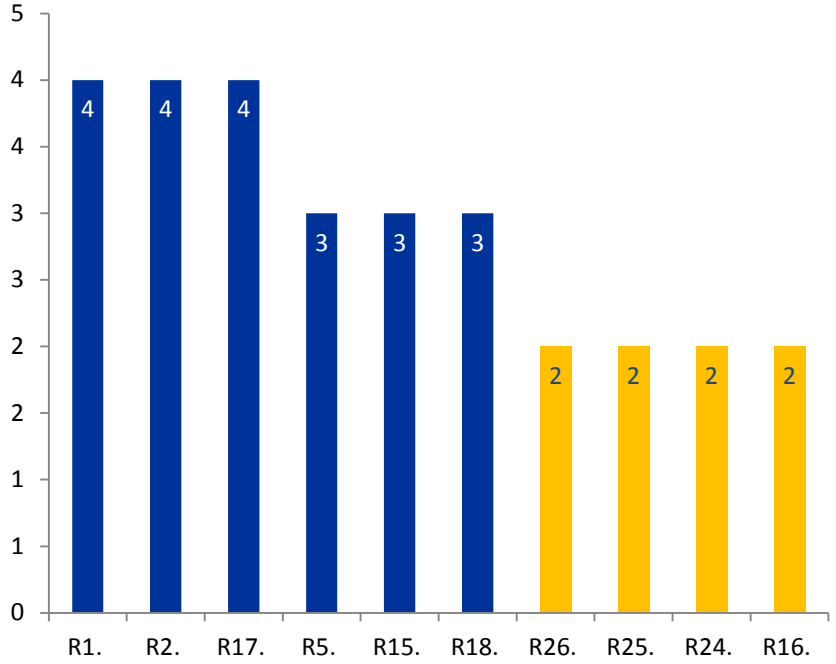
Cluster 2: Healthcare professionals + organisations

Comprehensive & ambitious. Seek collaboration with EU institutions to increase feasibility. Address pharmacovigilance overtly.

- RWD: need to establish and validate methods and requirements
- Need to critically think how to integrate digital biomarkers
- Need to critically review orphan legislation and incentives
- Need to make visible areas of unmet medical need
- Address medicines supply and shortages
- Balance between patient access and regulatory rigour
- Transparency and pharmacovigilance ensures balance
- Strategic priorities steer positive outcome for patients & help healthcare systems assimilate disruptive innovation

Cluster 2: Healthcare professionals + organisations

Ranking of top 3 core recommendations



16. Bridge from evaluation to access through collaboration with payers

24. Continue to support development of new antibacterial agents and their alternatives

25. Promote global cooperation to anticipate and address supply problems

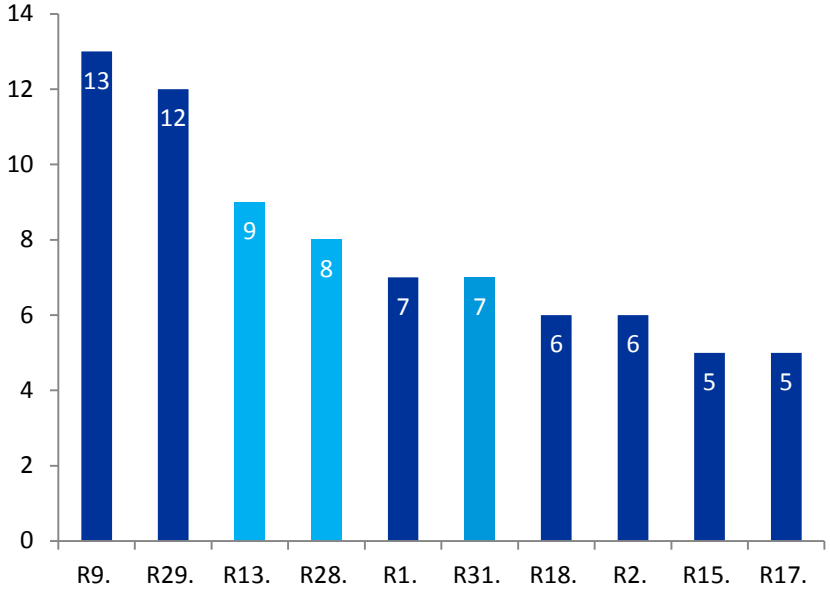
26. Support innovative approaches to the development, approval and post-authorisation monitoring of vaccines

Cluster 3: Researchers

Relevant and comprehensive. Ambitious in nature

- Facilitate greater collaboration and communication with stakeholders
  - To improve evidence generation
  - To close gap from approval to access
  - To encourage data sharing across EU network
- Ensure funding bodies are aware of RSS goals – regulatory science research questions to align funding priorities
- Align internationally to improve efficiency during development
- Show leadership in enhancing patient engagement
- Develop a set of 'patient important outcomes' to be measured in all trials
- Bolster efforts on 3Rs through qualification of novel methods and communicating acceptability
- Ensure regulatory preparedness for nanomedicines and borderline products

Ranking of top 3 core recommendations



13. Optimise capabilities in modelling, simulation and extrapolation

29. Leverage collaborations between academia and network scientists to address rapidly emerging regulatory science research questions

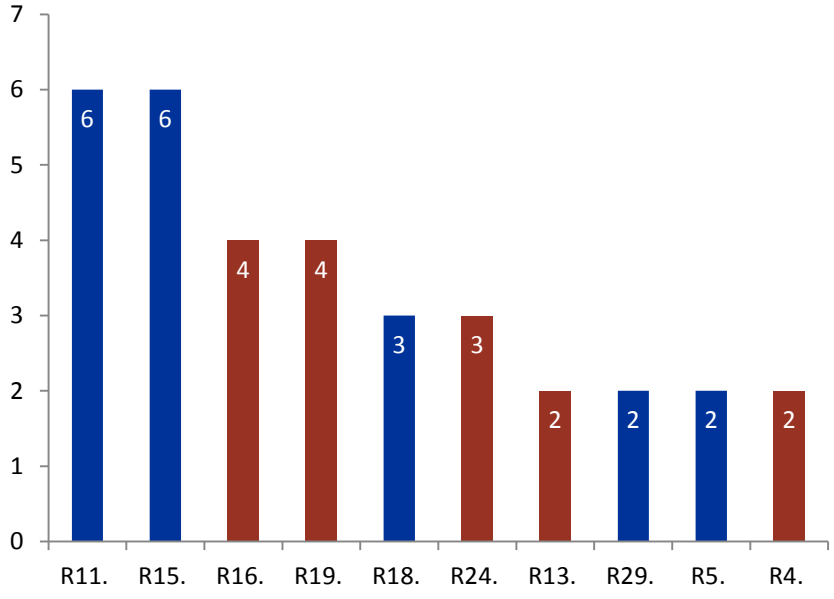
31. Disseminate and exchange knowledge, expertise and innovation across the network and to its stakeholders

Cluster 4: Public body

Comprehensive and a good basis to develop Network Strategy. Positive to proposed collaboration and exchange of information with EU and national regulators

- The importance of Big Data, RWD, data management and the need for data sharing and interoperability and international good practice
- Describe how patient experience would be translated into patient science
- Digital transformation of regulatory processes needs to be prioritised and resourced within telematics strategy
- Timely access linked to disease severity and lack of treatments
- Increase focus on pharmacovigilance, open science, environmental sustainability
- Calls for further alignment on scientific advice – pre-approval evidence generation and patient relevant endpoints
- Re-focus strategies on public health and not on innovation
- Reinforce quality of evidence stringency requirements for clinical data
- Critical review of adaptation of current regulatory approaches cMA, ODD, AA etc.

Ranking of top 3 core recommendations



4. Facilitate the implementation of novel manufacturing technologies

13. Optimise capabilities in modelling, simulation and extrapolation

16. Bridge from evaluation to access through collaboration with payers

19. Develop network competence and specialist collaborations to engage with big data

24. Continue to support development of new antibacterial agents and their alternatives

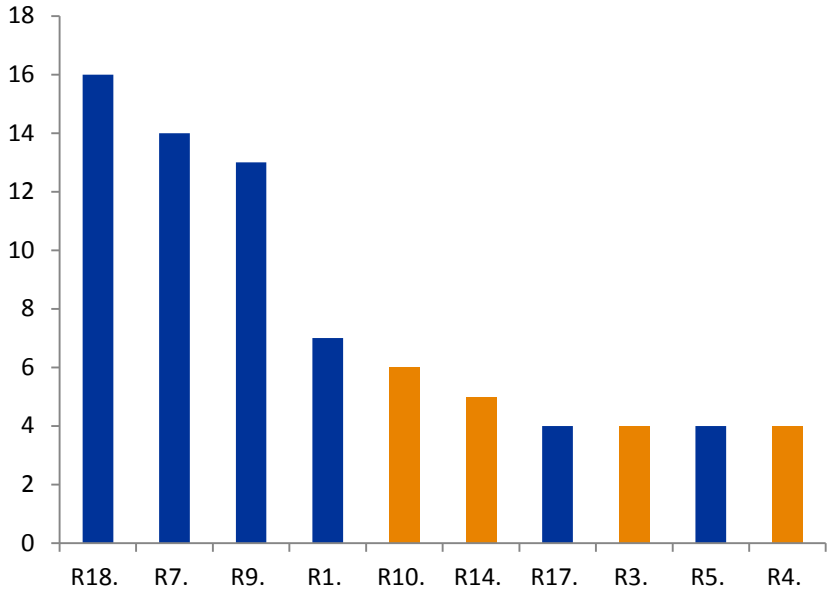
Cluster 5: Industry

Comprehensive – welcomed focus on innovation, consultative process and forward looking activities involving collaboration between stakeholders

- Importance of efforts to reduce complexity of EU system, reach global alignment and advance patient-centred access
- Alignment of regulatory science and frameworks with FDA/ICH etc. e.g. RWD
- Alignment of CT innovation, patients, NCAs, HTAs, and health systems
- Support increased regulatory science knowledge exchange – Cloud-based systems
- Importance to improve flexibility and harmonisation of scientific advice including stakeholders
- Imperative best use of new technology and data to support new healthcare paradigms
- Optimise emerging/new mfg approaches and develop new regulatory frameworks (complex generic, biosimilars, ATMP/ follow on biologics)
- Leveraging R&I – IT partners, expertise ehealth, AI, wearables etc.

Cluster 5: Industry

Ranking of top 3 core recommendations



3. Promote and invest in the PRIME scheme

4. Facilitate the implementation of novel manufacturing technologies

10. Develop the regulatory framework for emerging clinical data generation

14. Exploit digital technology and artificial intelligence in decision making

How it translated to the organisation of the workshop

1. Support developments in precision medicine, biomarkers and 'omics
2. Support translation of advanced therapy medicinal products (ATMPs) into patient treatments

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Catalyse and enable regulatory science and innovation to be translated into patient access to medicines in evolving healthcare systems

Catalysing the integration of Sci &Tech in medicines development

Session 2:
ATMPs & precision medicine

Driving collaborative evidence generation

Session 3A:
Developing scientific advice/assessment pathways

Session 4:
Clinical trials, digital therapeutics and modelling & simulation

Advancing patient-centred access to medicines in partnership with healthcare systems

Session 3B:
Optimising evidence for decision making and communication

Session 5C:
Reinforcing patient relevance in evidence generation

Enabling and leveraging R & I in regulatory science

Session 5D:
Network-led partnerships with academia

Addressing emerging health threats

Session 7:
Emerging health threats,
AMR and vaccines

Any questions?

Further information

RegulatoryScience2025@ema.europa.eu

Temporary visiting address Spark building • Orlyplein 24 • 1043 DP Amsterdam • The Netherlands
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Send us a question via www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000

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