EUROPEAN MEDICINES AGENCY

EMA recommendations from the Regulatory Science Strategy to 2025

Implications for patients and healthcare professionals

PCWP/HCPWP joint meeting

Presented by Tony Humphreys on 3 March 2020
Head of Regulatory Science and Innovation Task Force

An agency of the European Union

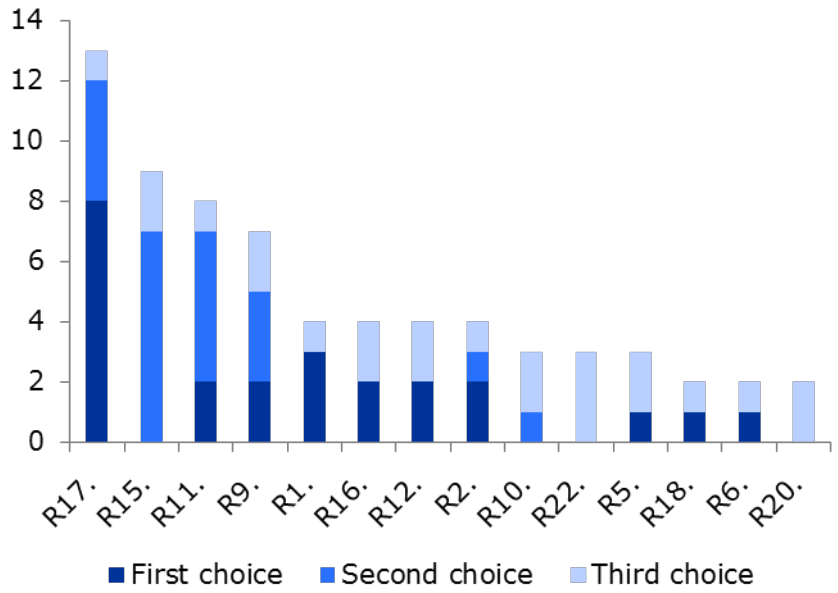
Human strategic goals

#RegScience2025

Since stakeholders' workshops

Patients

Aggregate ranking of core recommendations for Cluster 1 – Top 5



17. Reinforce patient relevance in evidence generation*

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

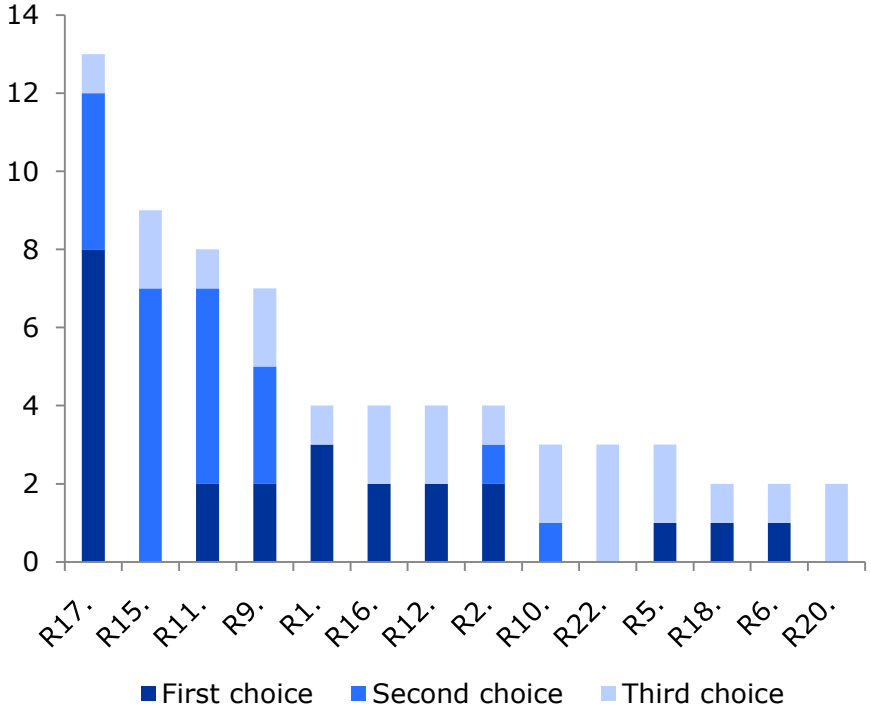
11. Expand benefit-risk assessment and communication*

9. Foster innovation in clinical trials*

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

* Discussed during 2019 Stakeholders' workshop

Aggregate ranking of core recommendations for Cluster 1 – Top 6-10



16. Bridge from evaluation to access through collaboration with payers*

12. Invest in special populations initiatives

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

10. Develop the regulatory framework for emerging clinical data generation*

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

* Discussed during 2019 Stakeholders' workshop

Reinforce patient relevance in evidence generation

Pre-consultation

- Enhance patient involvement in EMA scientific committees
- Coordinate the Agency's approach to patient reported outcomes (PROs). Update relevant clinical guidelines to include reference to PROs addressing study objectives, design and analysis
- While validating PROs, address patients' needs and leverage patients' expertise
- Co-develop with HTAs a core health-related quality-of-life PRO to implement in trials and to bridge the gap with comparative effectiveness assessment
- Explore additional methodologies to gather and use patient data from the wider patient community during benefit-risk evaluation.

Post-consultation

- Revise the existing patient engagement methodology and review and update EMA's existing 'Framework for interaction with patients and patient organisations' to reflect EMA's evolving approach to patient data and enhanced patient involvement in EMA scientific committees;
- Explore and deploy additional methodologies to collect and use patient data for benefit-risk assessment;
- Update existing, and develop new EMA guidelines on patient data collection;
- Coordinate the approach to patient reported outcomes (PROs);
- Promote use of core health-related quality-of-life PROs.

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

Pre-consultation

- Ensure the evidence needed by HTAs and payers is incorporated early in drug development plans
- Enable information exchange with HTAs to support bridging from benefit-risk to relative effectiveness assessment
- Discuss with HTAs guidance and methodologies for evidence generation and review
- Contribute to the identification of priorities for HTAs
- Monitor the impact of decision-maker engagement through reviews of product-specific experience.

Post-consultation

- Ensure the evidence needed by HTAs and payers is incorporated early in drug development plans, including requirements for post-licensing evidence generation;
- Enable information exchange with HTAs to support bridging from benefit-risk to relative effectiveness assessment;
- Discuss with HTAs guidance and methodologies for evidence generation and review;
- Collaborate with HTAs on the identification of priorities;
- Monitor the impact of decision-maker engagement through reviews of product-specific experience;
- Further develop the structured interaction between EMA and HTA bodies, respecting the respective remits.

Human core recommendation

Expand benefit-risk assessment and communication

Pre-consultation

- Expand the benefit-risk assessment by incorporating patient preferences
- Develop the capability to analyse Individual Patient Data to support decision-making
- Promote systematic application of structured benefit/risk methodology and quality assurance systems across the network
- Improve communication with HTAs and payers regarding therapeutic context, comparison vs. placebo/active-control, patient perspective
- Enhance structured benefit/risk assessment to improve communication to the public
- Incorporate academic research into evidence-based benefit-risk communication.

Post-consultation

- Include patient preferences to inform the benefit-risk assessment:
  - ✓ Develop guidance building on recent developments (e.g., IMI PREFER) of appropriate methods for patient preference study design, conduct, analysis, and presentation for regulatory purposes, ensuring high quality methodology and independence;
  - ✓ Provide guidance on the roles of patient preferences in the different therapeutic contexts and regulatory decisions, i.e., how preferences can help regulators interpreting clinical trial outputs, how they can inform shared decision-making; how to handle heterogenous or conflicting preferences; how to communicate patient preferences in regulatory decisions;
- Promote systematic application of structured benefit-risk methodology and quality assurance methods across the network;
- Enhance structured assessment of benefits, harms, and uncertainties to improve communication to the public;
- Develop the capability for analysing individual patient data to support decision-making;
- Improve communication with HTAs and payers regarding therapeutic context, comparison vs. placebo/active-control, patient perspective.

Foster innovation in clinical trials

Pre-consultation

- Drive adoption of novel practices that facilitate clinical trial authorisation, GCP and HTA acceptance
- Critically assess the clinical value of new and emerging endpoints and their role in facilitating patients' access to new medicines
- Work with stakeholders to encourage collaborative clinical trials
- Collaborate with international partners in ongoing initiatives such as the Clinical Trial Transformation Initiative and ICH.

Post-consultation

- Establish a multi-stakeholder, neutral, platform, to enable new approaches to clinical studies and to position the EU as a preferred location for innovative clinical research;
- Drive development and adoption of novel practices that facilitate clinical trial authorisation, GCP and HTA acceptance at EU and international level;
- Work with stakeholders, the EU Medicines Regulatory Network and the European Commission to promote and facilitate the conduct of complex clinical trials and other innovative clinical trial designs;
- Promote increased information sharing on clinical trial design, conduct, results and best practices. Build on this information and the multi-stakeholder platforms to enable further education, training and sharing of best practice in order to accelerate innovative change;
- Critically assess the clinical value of new and emerging endpoints and their role in facilitating patients' access to new medicines;
- Promote the inclusion of neglected populations such as pregnant women, the elderly and those of diverse ethnicity in clinical trials.

Support developments in precision medicine, biomarkers and 'omics

Pre-consultation

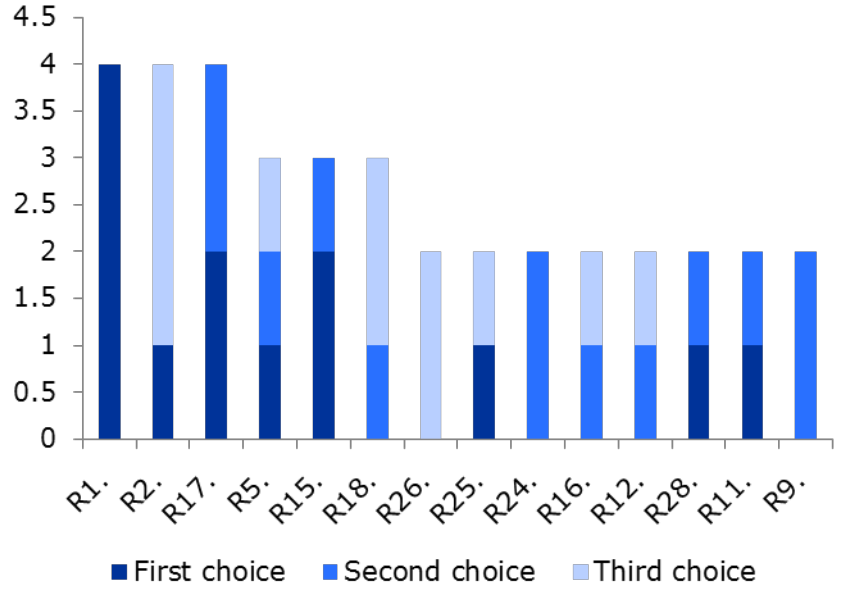
- Enhance early engagement with novel biomarker developers to facilitate regulatory qualification
- Address the impact of emerging 'omics' methods and their application across the development life cycle
- Evaluate, in collaboration with HTAs, payers and patients, the impact of treatment on clinical outcomes measured by biomarkers.

Post-consultation

- Enhance early engagement with novel biomarker developers to facilitate regulatory qualification;
  - ✓ Critically review the EMA's biomarker validation process, including duration and opportunities to discuss validation strategies in advance, in order to encourage greater uptake and use;
- Address the impact of emerging 'omics' methods and their application across the development life cycle;
- Evaluate, in collaboration with HTAs, payers and patients, the impact of treatment on clinical outcomes measured by biomarkers;
- Optimise the European research infrastructure for developing personalised medicine.

Healthcare professionals

Aggregate ranking of core recommendations for Cluster 2 Top 5



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

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

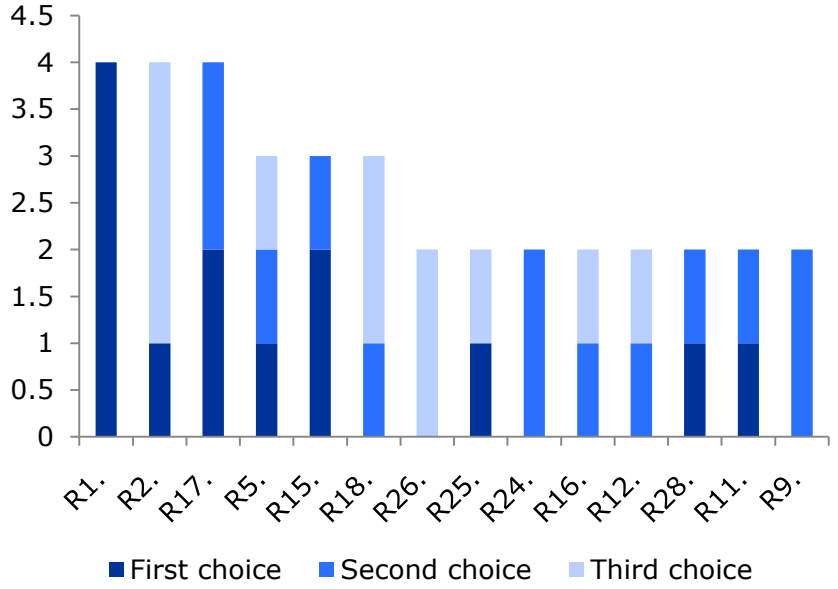
17. Reinforce patient relevance in evidence generation *

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

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

* Discussed during 2019 Stakeholders' workshop

Aggregate ranking of core recommendations for Cluster 2 Top 6-10



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

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

25. Promote global cooperation to anticipate and address supply challenges

24. Continue to support development of new antimicrobials and their alternatives

16. Bridge from evaluation to access through collaboration with Payers

* Discussed during 2019 Stakeholders' workshop

Human core recommendation

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

Pre-consultation

- Identify therapies that address unmet medical need
- Provide assistance with early planning, method development and clinical evaluation
- Support evidence generation, pertinent to downstream decision-makers
- Address the challenges of decentralised ATMP manufacturing and delivery locations
- Raise global awareness of ATMPs to maximise knowledge sharing, promote data collection.

Post-consultation

- Identify therapies that address unmet medical need;
- Provide assistance with early planning, method development and clinical evaluation;
- Address the challenges of decentralised ATMP manufacturing and delivery locations;
- Support evidence generation, pertinent to downstream decision-makers;
- Evaluate and improve interactions with European institutions (research, financial and environmental);
- Raise global awareness of ATMPs to maximise knowledge sharing, promote data collection;
- Engage with other international regulatory agencies to foster global convergence of requirements for ATMPs.

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

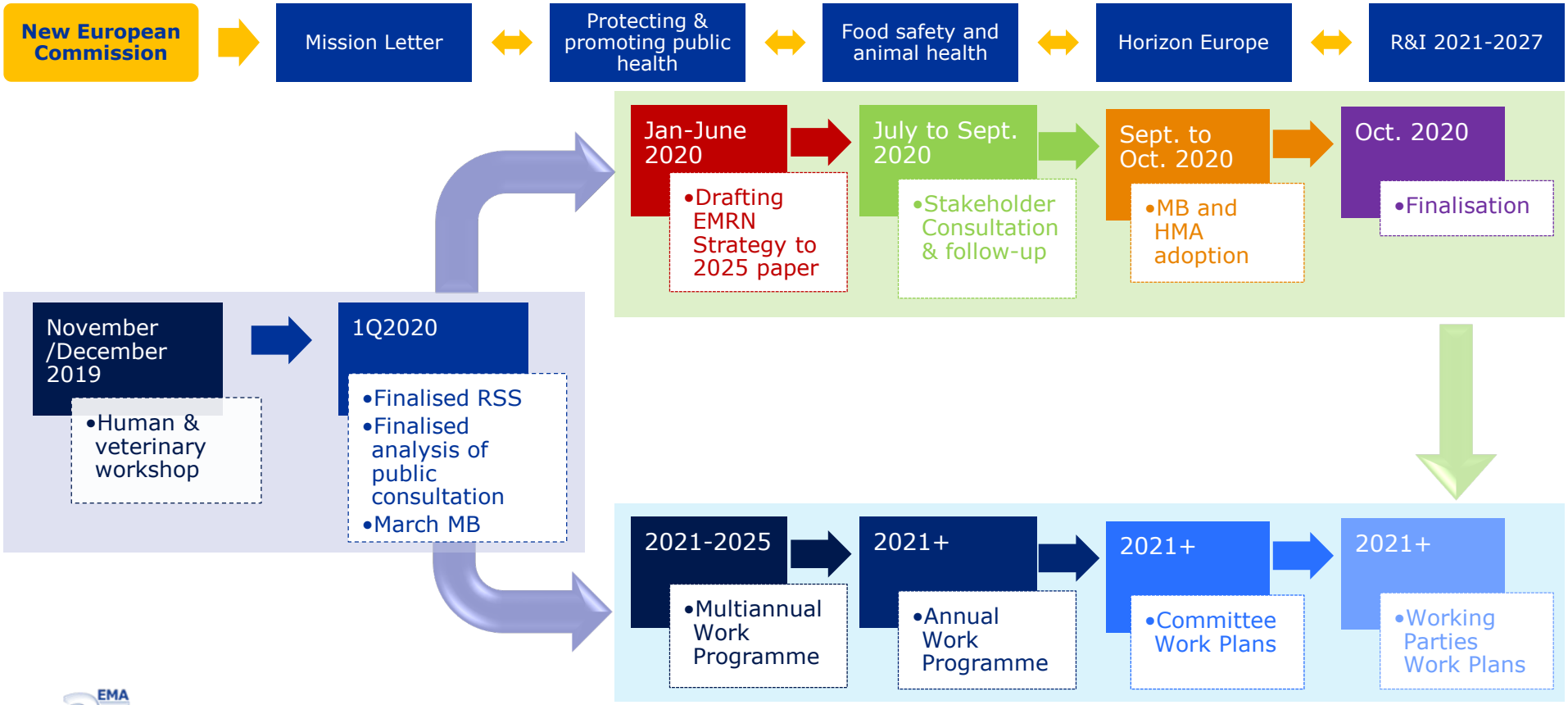
Pre-consultation

- Define how benefit-risk of borderline products is assessed and communicated;
- Enrich expertise at the interface between medicines, medical devices and borderline products;
- Facilitate the regulatory pathway between notified bodies and medicines' regulators;
- Gain insight in innovation on drug-device combination products via horizon scanning.

Post-consultation

- Facilitate the regulatory pathway between notified bodies and medicines' regulators:
  - ✓ Establish a process for multi-stakeholder scientific advice to support development of medicine-device combinations, qualification methodologies and the use of companion diagnostics;
  - ✓ Create a process to consult medical device authorities and/or NB (as applicable) for device-related aspects throughout the product lifecycle, including post-authorisation safety related events;
  - ✓ Adapt consultation processes to address emerging digital technologies and wearables;
- Build a network of expertise to regulate and provide support throughout the product lifecycle;
- Define how benefit-risk of borderline products is assessed and communicated;
- Gain insight in innovation on drug-device combination products via horizon scanning

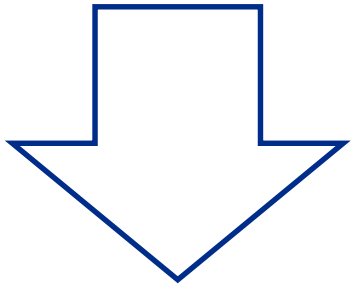
EMA Regulatory Science to 2025



Programme management

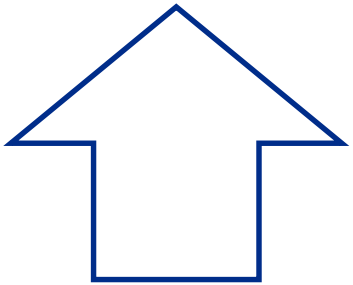
- 5 year implementation planning
  - Recognising interdependencies
  - Prioritisation of actions
  - Identifying enablers
- Resourcing to ensure success
  - Staffing
  - Expertise
  - Budget availability
  - Training

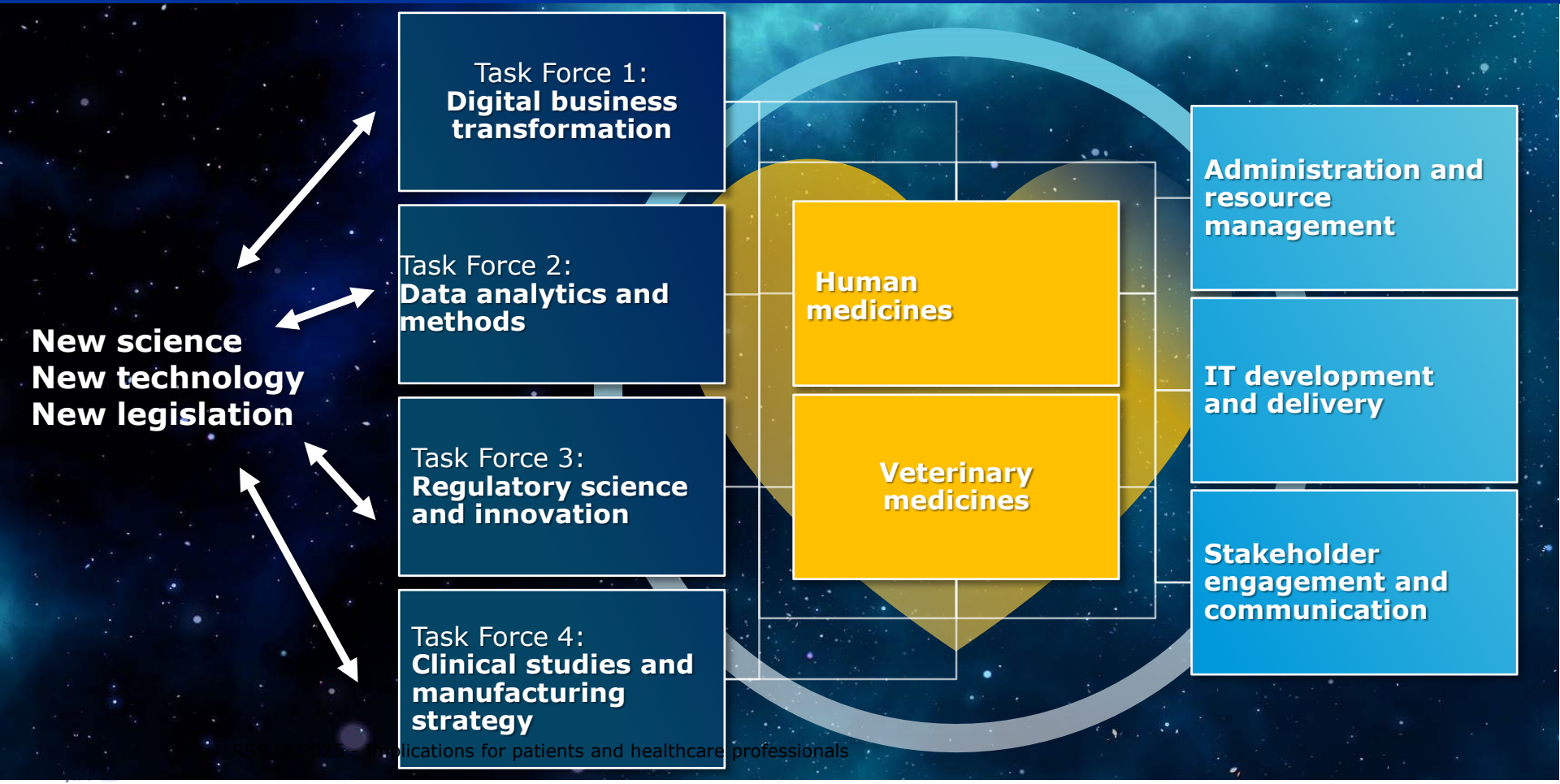
Need new approach



Demand to do more with restrictive budget pressures

Global Standards of Regulatory Excellence rising




New science
New technology
New legislation
Task Force 1:
Digital business transformation
Task Force 2:
Data analytics and methods
Task Force 3:
Regulatory science and innovation
Task Force 4:
Clinical studies and manufacturing strategy
Human medicines
Veterinary medicines
Administration and resource management
IT development and delivery
Stakeholder engagement and communication

Engagement

Done

- RSS consultative process
- EMRN leadership
- Outreach
- Stakeholders consultation exercise (patients, healthcare professionals, learned societies, academia, industry, HTA/payers etc.)

Yet to do

- **Need** for continued **stakeholders engagement** in delivering actionable areas
- Need **collaboration** to deliver:
  - **Patients and Healthcare professionals**
  - **EC/EP/NCAs**
  - **HTA/Payers & Medical Device Authorities**
- Need to **leverage** NCAs and International Regulators' **regulatory science** programmes

Any questions?

Further information

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