EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Regulatory perspectives on use of clinical data in the regulatory review process and scientific advice by regulatory authorities

EFSPI Statistical Leaders meeting, 30 June 2021

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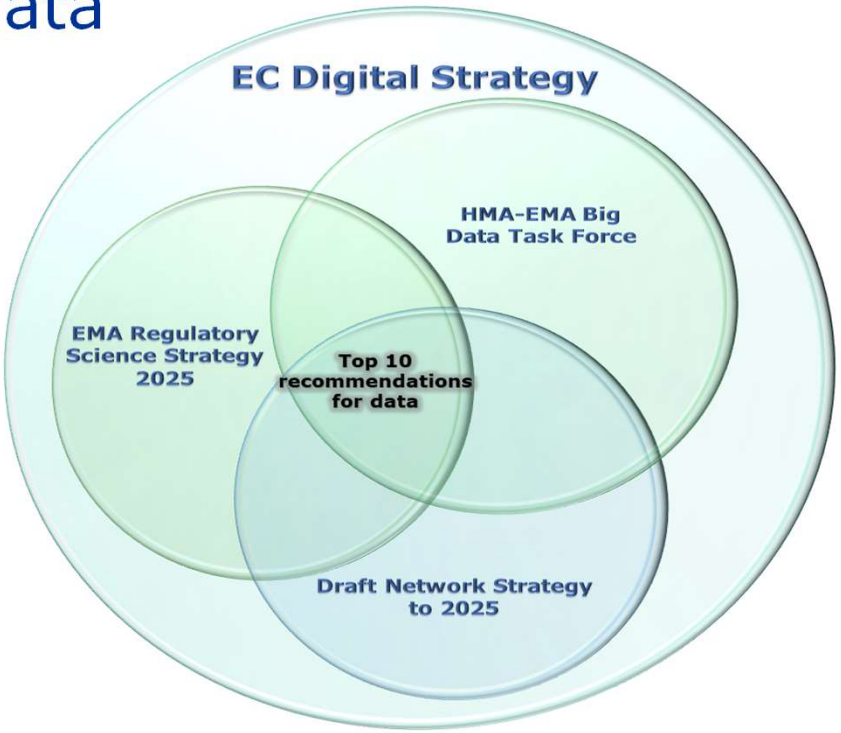
An agency of the European Union

Disclaimer

The views expressed in this presentation are my personal views and may not be understood or quoted as being made on behalf of or reflecting the position of the European Medicines Agency or one of its committees or working parties.

The timing is now… for raw data

- Commission digital strategy: **"EU health data space"** (EHDS)
- Joint HMA EMA Big Data Task Force **Top-ten data recommendations**
- **EMA Regulatory Science Strategy to 2025**
- **EU Network Strategy to 2025** includes data and digital pillar
- EC **Pharma Strategy** and **Health Union**



Vision: innovate to turn data into decisions on medicines that create a healthier world

Planning a world
..... beyond COVID-19

March 2020 COVID-19 pandemic

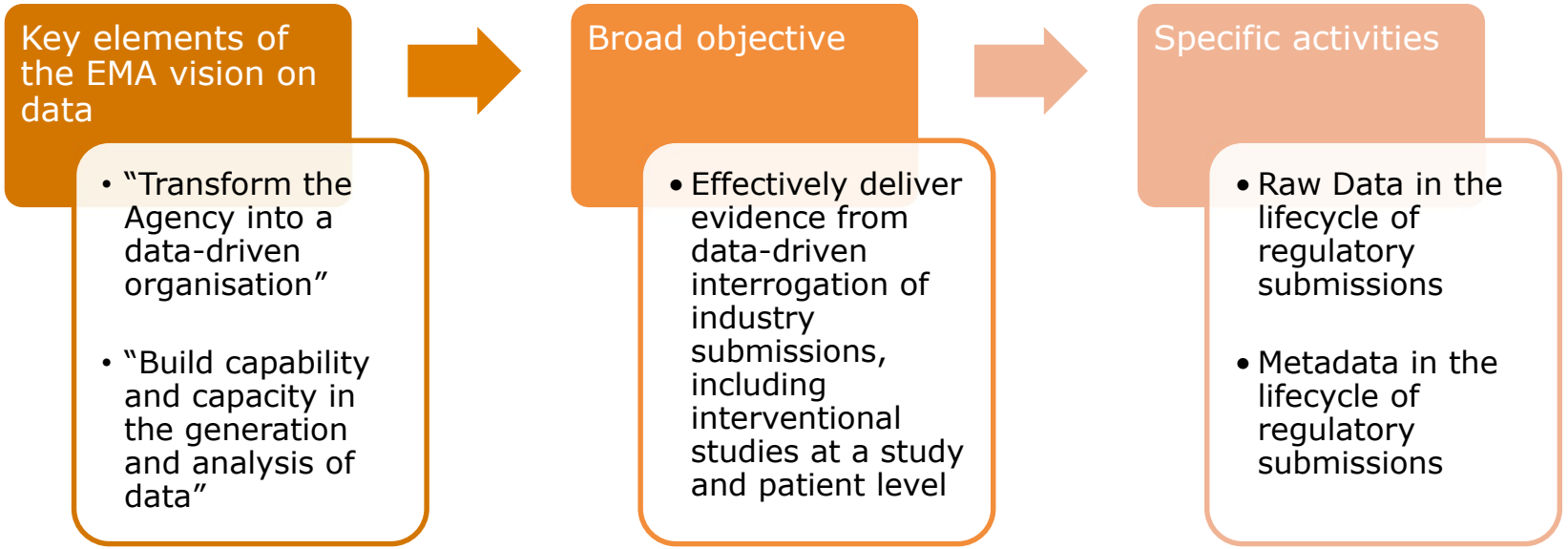
EMA Priorities:

- Maintain core business
- Rapid and robust opinions on COVID-19 vaccines and therapeutics
- Prepare for post COVID-19 transformation… including data-driven regulation

Big Data Task Force Priority recommendations

1	Deliver a sustainable platform to access and analyse healthcare data from across the EU Data Analysis and Real World Interrogation Network: **DARWIN EU**
2	Establish an EU framework for data quality and representativeness
3	Enable **data discoverability**
4	Develop EU Network skills in Big Data
5	Strengthen EU Network **processes** for Big Data submissions
6	Build EU Network capability to analyse Big Data (technology / analytics)
7	Modernise the delivery of expert advice
8	Ensure data are managed and analysed within a secure and ethical governance framework
9	Engage with international initiatives on Big Data
10	Establish an EU Big Data 'stakeholder implementation forum'
11	Veterinary recommendations

Transform recommendations into actions



Raw Data Project Objectives

Support better decision-making throughout the product lifecycle of medicines with valid and reliable evidence from clinical trials

- Establish the requirements for technical infrastructure, data standards and tools needed for use of IPD, based on resource and IT utilisation assessment, and including a technical development path adequate to the foreseen needs
- Put in place procedures and safeguards to process raw data, including **clinical**, **quality** and **non-clinical data**, in accordance with data protection legislation
  - Establish an advisory group on lifecycle regulatory submissions raw data in order to examine in detail network capacity and capability issues;
  - Perform a proof of concept **pilot**;
  - Foster stakeholders' engagement through a communication plan

Approach to address Project Objectives

Learning

- Learn from past regulatory experience with raw data;
- Be opportunistic when assessment requires submission of raw data;
- Plan a series of proof-of-concept pilots.

Understanding

- Identify clinical, quality and non-clinical use cases across the lifecycle of submissions;
- Interview Rapporteurs and assessors about practical use of raw data;
- Prioritise use cases based on business needs of the Agency.

Communication

- Big Data Steering Group and CHMP;
- Working Parties and cross-NCA advisory group;
- Stakeholders communication plan.

Points for discussion today (1)

- Establish value and benefits of access to raw data for regulators and applicants
  - General ideas about saving time in assessment and regulatory interaction, reduce number of questions, identify questions impacted by regulatory access to raw data
  - Can we define metrics that measure impact on process and quality?
  - Higher expected benefit in understanding or robustness of dossier?
- Proof-of-concept pilots
  - Are there ideal medicine candidates for pilots or should we be opportunistic?
  - How can industry contribute to the learnings?
  - Testing various options for the operational model in the regulatory network

Points for discussion today (2)

- Define process for raw data submission that are implementable
  - Align to the extent possible to FDA and PMDA requests? (e.g. data standards)
  - What can be improved from current raw data submission elsewhere?
- Interaction with stakeholders during the project
  - What are the key stakeholder groups to target from your perspective?
  - What could EFSPI's and statisticians' role be in the stakeholder interaction?
- Longer term…
  - Also non-clinical and quality raw data: when is the right timing?
  - Clinical trial metadata can be standardised (ICH M11), potential synergy?

Any questions?

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