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SCIENCE MEDICINES HEALTH

Use of Big Data to Support Regulatory Decision Making

EMA-EuropaBio Annual Bilateral meeting

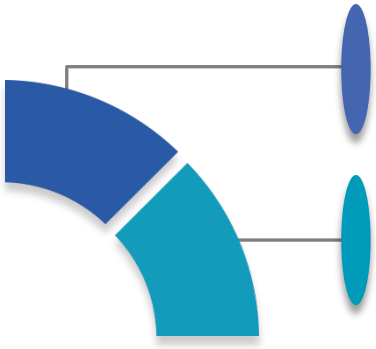
Dr Alison Cave,
Principal Scientific Administrator,
Pharmacovigilance and Epidemiology Department

Objectives

1

Regulatory Challenges

An increasing number of medicines with genomic mechanism and/or genomic biomarkers. Smaller, focused RCTs which increases the importance of post-marketing monitoring.



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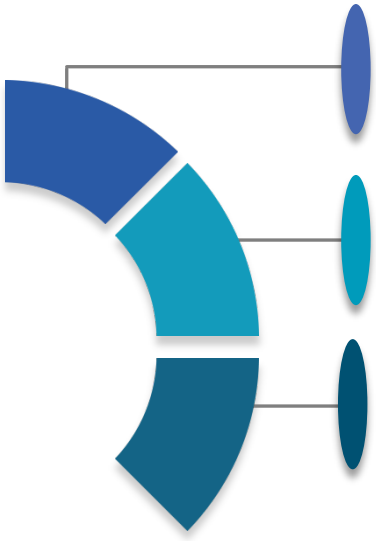

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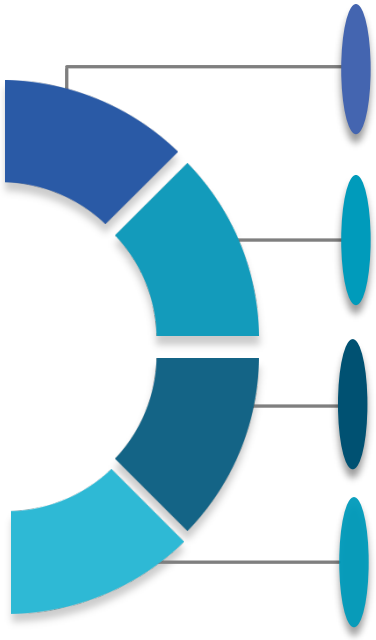

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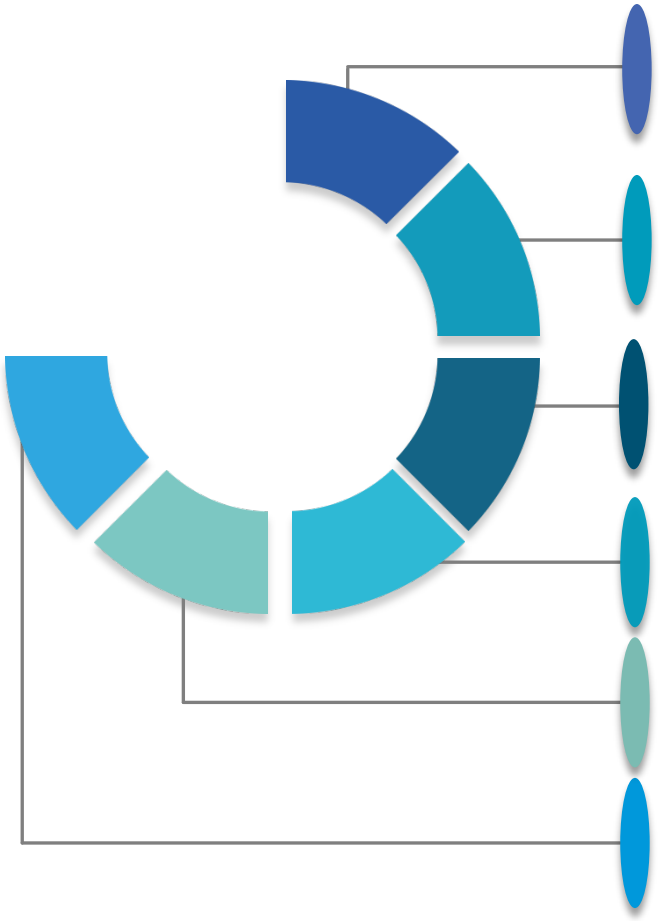

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Innovation driving personalised prescribing creates regulatory challenges.

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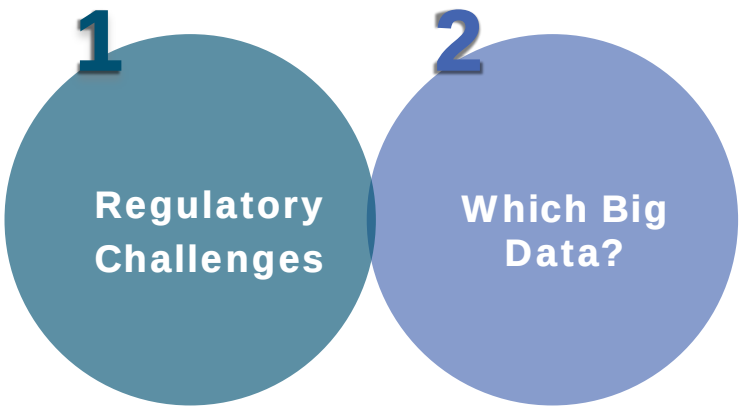
The high internal validity of clinical trials at the expense of external validity demands new approaches to gather complementary evidence

Additional data sources are needed to appropriately monitor risk/benefit in high risk groups often excluded from clinical trials

Increasing interest in the development of combination therapies to treat complex diseases creates regulatory challenges

Objectives



Which Big Data?

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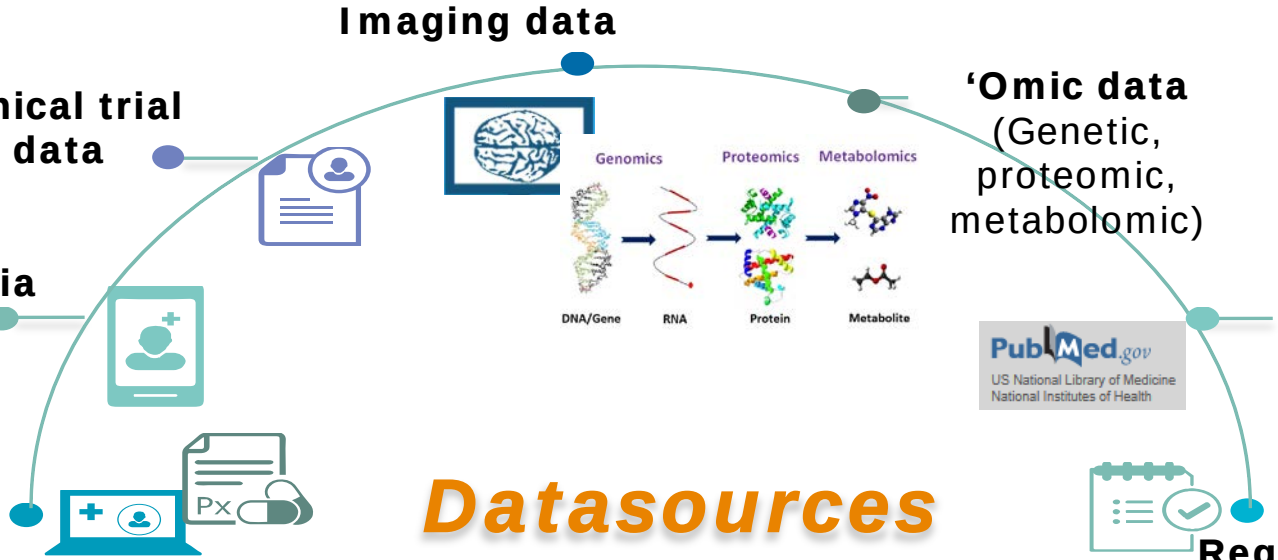

Objectives


1
Regulatory Challenges
2
Which Big Data?
3
What are the Challenges

Challenges

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90%

Of the world's data has been created in the past 2 years.

24 months

Frequency at which electronic healthcare data doubles

75%+

Percentage of patients expected to use digital health services in the future

Big Data Challenges

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Rate of accumulation

- 100,000 RCTs
- 424 million articles in 5600 journals
- >12 TB personal health data/lifetime

Inaccessible, siloed data

- 80% of data unfindable
- Lack of interoperability
- Governance and privacy concerns

Unstructured and heterogeneous

- 80% unstructured
- Valuable detail in unstructured text - Images, MRIs, X rays etc
- Multiple formats and provenance

Uncertain quality

- Variable standards
- Lack of validation – causality vs coincidence
- Managing bias and confounding

Regulatory Challenges

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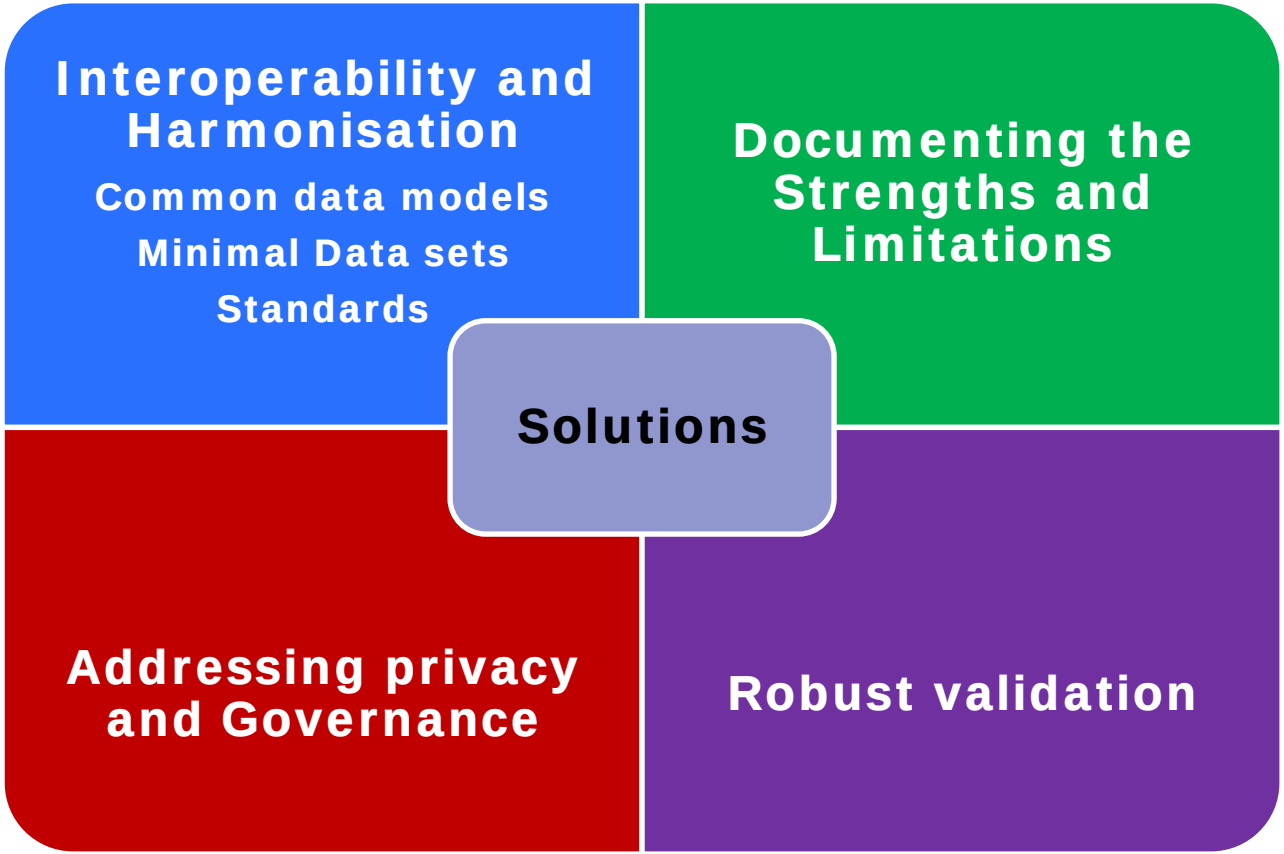
Structured data (RCT) generated in accordance with strict guidelines and known provenance

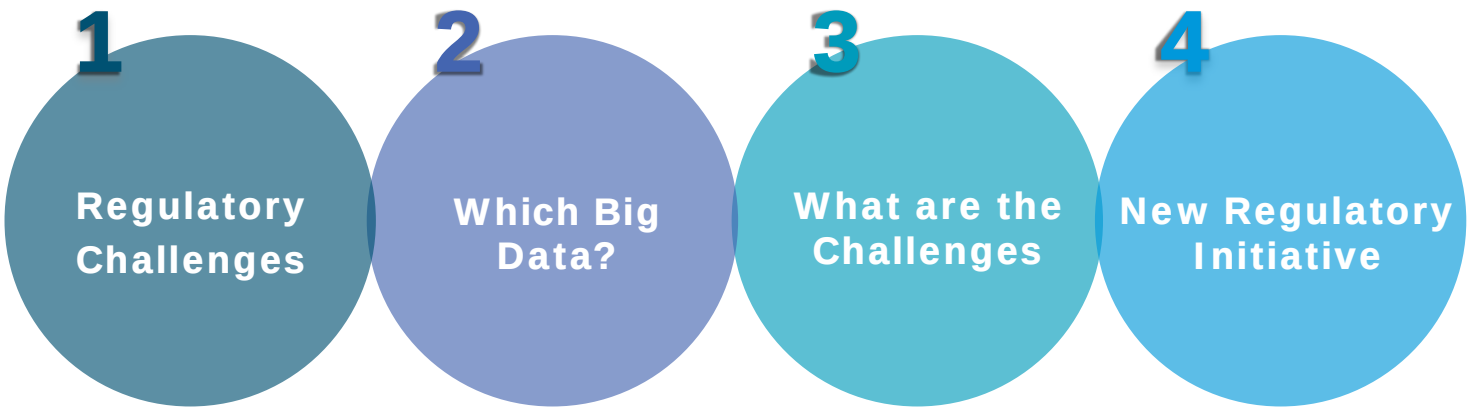
High certainty

Unstructured, unvalidated data of unknown provenance

more uncertainty


Interoperability and Harmonisation
Common data models
Minimal Data sets
Standards
Documenting the Strengths and Limitations
Solutions
Addressing privacy and Governance
Robust validation

Objectives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New Regulatory Initiative

HMA Heads of Medicines Agencies

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Mandate HMA / EMA Joint Task Force Big Data

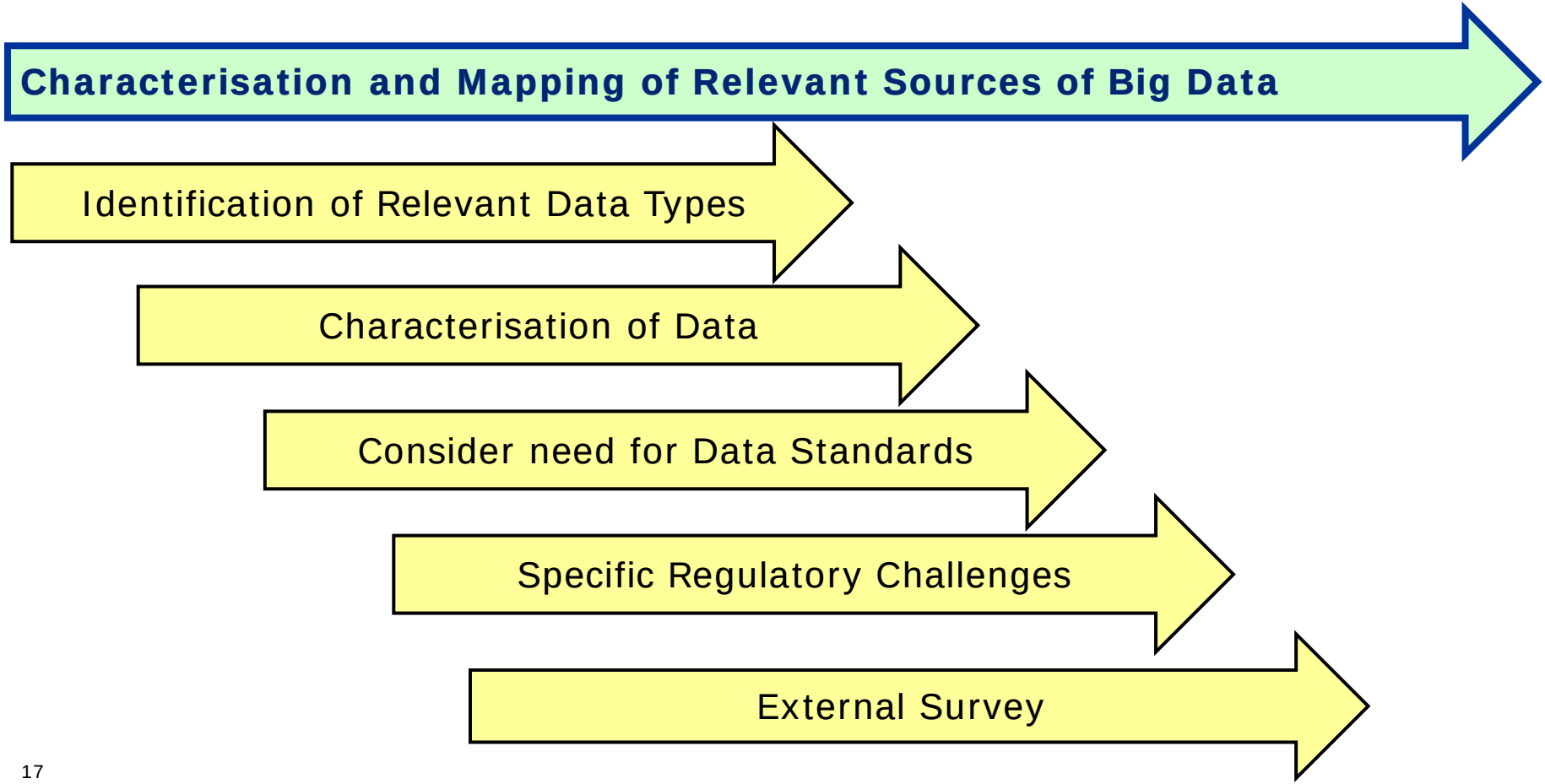
Priority: Reinforce the scientific and regulatory capacity and capability of the network, Innovation and access to new medicines, Optimisation of the regulatory operations

Chair: Thomas Senderovitz, DK

Co Chair: Alison Cave, EMA

Members: DE, DK, ES, FI, HU, IE, NL, NO, RO, UK

HMA / EMA Big Data Taskforce



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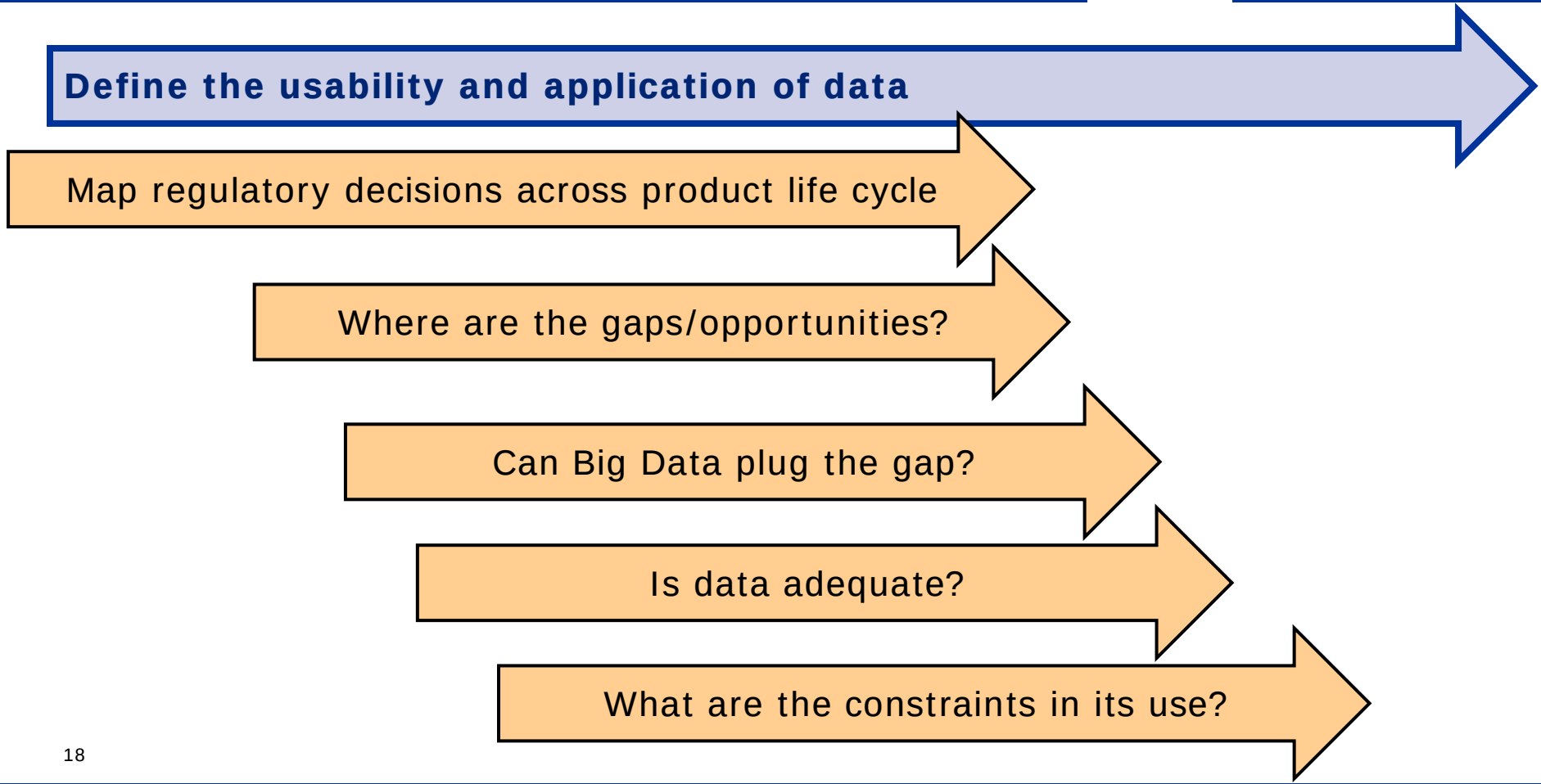

HMA / EMA Big Data Taskforce

Describe the current status, future needs and challenges

- What is the current landscape in NCAs
- What datasets are currently used?
- What is the expertise in data analysis?
- What are the anticipated future needs?

Generation of a set of recommendations and a roadmap

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Conclusions

Conclusions

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- Vast amounts of healthcare data are continually being generated, offering huge opportunities but making it impossible to keep pace with all the information.
- Harnessing of the potential of big data by researchers and regulators is hindered by the fact that it is often unstructured, noisy and inaccessible.
- Deciding which data to collect starts by asking the right questions about the benefits sought and problems faced.
- Access to data is a significant hurdle especially for observational data. Mechanisms to integrate the data to generate meaningful knowledge is needed.
- Validation that associations are causal is key for data to support regulatory decision making.

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Thank you

European Medicines Agency
30 Churchill Place London
E14 5EU

www.ema.europa.eu
info@ema.europa.eu

Workplan

- **Characterisaton of Relevant Sources of Big Data and Defining Format**

  Genomics
  Observational Data
  Social Media / M health
  Spontaneous ADRs
  Other 'omics
  Clinical Trial data
  IT / infrastructure

- **Identify areas of usability and application of datasets**

- **Describe the current status, future needs and challenges**

- **Generate a list of recommendations and Big Data road map**