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European Federation of Pharmaceutical Industries and Associations

Secondary Use of Health Data & Responsible Transparency

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Nordic CT Conference

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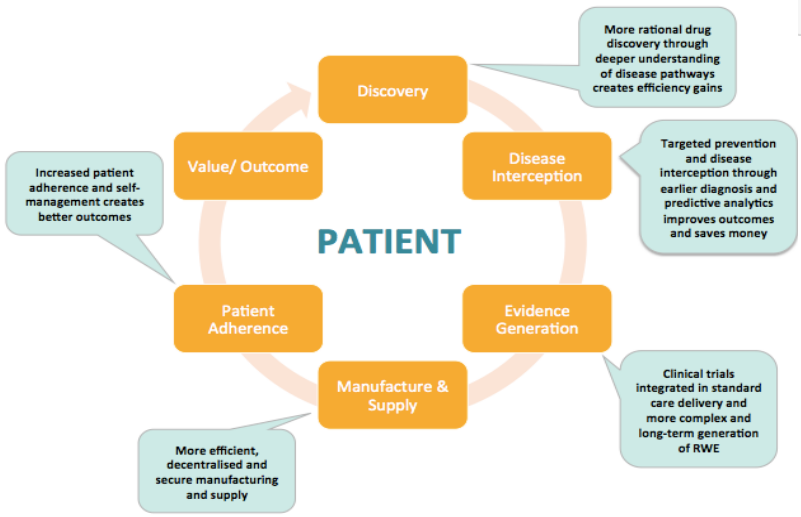
REALISE THE POTENTIAL OF HEALTHCARE DATA & ANALYTICS

Centred on the patient and across the life cycle of a medicines



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SECONDARY USE

What is Secondary Use?

- "All processing operations related to a specific clinical trial protocol during its whole lifecycle, from the starting of the trial to deletion at the end of the archiving period including data in marketing authorisation, shall be understood as primary use of clinical trial data"
- "This definition appears to exclude specific research activities that formed part of the planned further development of the medicine at the time that the trial was initiated but which go beyond the immediate trial. Researchers will need to reflect on how to ensure that relevant data is preserved during the longer-term development of the medicine."
- Also excluded is the wider area of data other than clinical trial data

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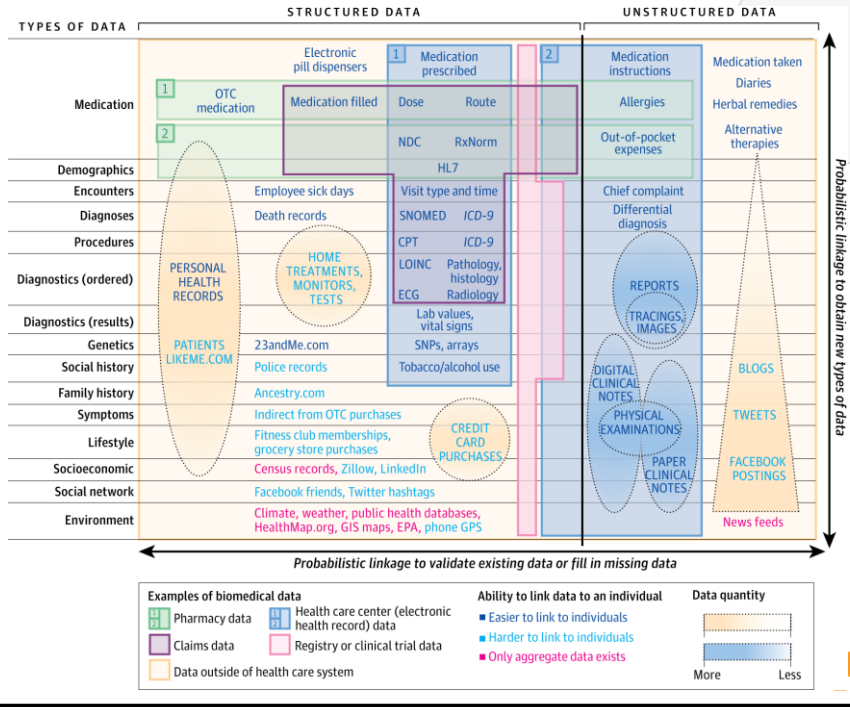

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At the discovery and drug development stage, real-world data are used:

- To identify diseases or indications of a significant burden to a wider population.
- To better understand a disease, e.g. the impacts of a disease on the wider health and well-being of patients, risk factors associated with a disease or disease progression.
- To understand the prevalence of a disease or condition.
- To provide new insights into disease associations or comorbidities and therefore to target new populations and indications for future research.
- To develop targeted and personalised therapies and drugs.
- To develop new analytical methods.

At the clinical research stage, real-world data are used:

- To inform clinical trial design, e.g. to improve the study population selection for clinical trials, to predict the number of potential patients or to assess the efficacy of a new drug.
- To create new approaches to patient stratification.
- In feasibility studies.
- Alongside or instead of control groups for trials to reduce the need to enrol patients as controls.

At the marketing authorisation and market access stage, health data are used:

- For medicine authorisation and regulatory purposes.
- To support market access discussions, e.g. to conduct health technology assessments (HTA), identify how competitive drugs are used on the market and to support pricing discussions.
- To conduct cost-effectiveness analyses.

At the post-authorisation stage, health data are used:

- To support pharmacovigilance, i.e. to identify safety issues and adverse reactions.
- For pharmacoepidemiology, i.e. to understand treatment effects across patient populations, to identify patient groups resistant to drugs, as well as to get insights into patient adherence.
- To add to the medical evidence base and inform changes in practice guidelines.
- To support effectiveness comparisons between new drugs and existing drugs.
- To inform drug repurposing, i.e. the identification of diseases and conditions that could be treated with an existing drug.

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SECONDARY USE

Risks & Benefits

"Identified potential positive impacts include: improved treatments for patients; accelerated research and drug development, and therefore also accelerated treatment access; better understanding of and improvement of treatment efficacy; improved pharmacoepidemiology and pharmacovigilance."

negative impacts of reuse of health data ..could occur if … companies broke personal data laws or if they used the data for purposes other than the original purposes. This could lead to less trust and restricted access to health data. Negative impacts of reuse of health data could also occur if…. companies conducted poor-quality analyses.

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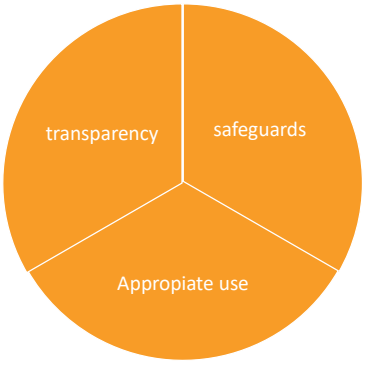
RESPONSIBLE TRANSPARENCY

Accountability Framework



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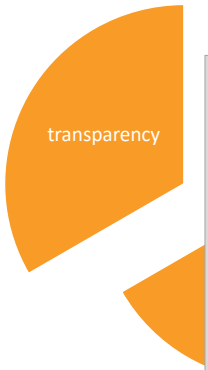
RESPONSIBLE TRANSPARENCY

Accountability Framework



- Industry commitment to support data-sharing for research purposes
  - Data Protection
  - Incentives for innovation
  - Regulatory decision-making
- Engagement with authorities in relation to institutional transparency policies
- Role of data stewards/ trusted third parties in providing transparency regarding data uses
- Internal company controls
- Implementation of GDPR
  - Subject rights
  - Legal basis
  - Data minimisation
- Public understanding

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Core recommendations

The development of timely, efficient and sustainable frameworks for data sharing and access is required

Further support mechanisms are needed to promote a data sharing culture

(Supported by subgroup recommendations # 3, 5, 9, 13, 18. 33, 18, 33, 37b, 40)

- Strongly recommend the establishment of distributed data networks to facilitate data sharing of sensitive healthcare data – **Ownership: EMA/HMA**
- Develop guidance for robust data governance and data anonymisation to deliver systems which secures patient trust - **Ownership - Common**
- Establish disease-specific minimum data elements to enable harmonisation of data across for e.g. national disease registries – **Ownership: EMA/HMA**
- Promote mandatory sharing of the analysis arising from data sharing activities e.g. by publication or open sharing via data access platforms – **Ownership: Common**
- Promote the sharing of qualified models– **Ownership: EMA/HMA**
- Support the development of policy initiatives to drive a data sharing culture which is mutually beneficial for all stakeholders. - **Ownership – Common**
- Proactively drive and/or support data sharing platforms and initiatives– **Ownership: EMA/HMA**
- Require the submission of data management plans at the start of all data generation exercises – **Ownership: EMA/HMA**
- Establish accountability for users– **Ownership: Common**
- Development of common principles for data anonymisation to facilitate data sharing – **Ownership: Common**

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ISPOR
Improving healthcare decisions

Improving Transparency in Non-Interventional Research for Hypothesis Testing—WHY, WHAT, and HOW: Considerations from The Real-World Evidence Transparency Initiative

Transparency should encompass all aspects of research, from initial RWD sourcing and curation, through study protocol development and analysis, to reporting of results.

As the potential use of RWE to support decision-making for market authorization, reimbursement, and clinical guideline development grows, the need to trust that evidence grows correspondingly.
Improving the culture of transparency can help shine light on study practices so that these end- users of the results are able to make a better determination about study quality for themselves.

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RESPONSIBLE TRANSPARENCY

Public Understanding

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SECONDARY USE & RESPONSIBLE TRANSPARENCY

Conclusions

- The opportunities available to improve patient outcomes from better use of RWD are compelling
- Realising the potential is a « team effort » requiring political leadership and significant investment
- Transparency is an important element, but not the only element in maintaining public confidence
- RWE transparency raises new issues to those we saw in relation to Clinical Trial Transparency
- The industry remains committed to responsible data-sharing and is ready to explore how this should work in the RWE space

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Thank you for your attention – any questions?






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