



A practical toolkit for identifying, selecting, and measuring outcomes

IMI DO>IT

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Big Data for
Better Outcomes

Improve health outcomes
and healthcare systems in
Europe by maximising the
potential of Big Data



Big Data for Better Outcomes (BD4BO) Mission



Mission: Improve health outcomes and healthcare systems in Europe by maximising the potential of Big Data



CO-ORDINATING PROJECTS:

DO->IT: Coordination & support action

Electronic Health Data and Evidence Network (EHDEN)

DISEASE-SPECIFIC PROJECTS:

ROADMAP: Alzheimer's disease

HARMONY: Haematologic malignancies

BigData@Heart: Cardiovascular diseases

PIONEER: Prostate cancer

More to come....

THEMES /
ENABLERS:

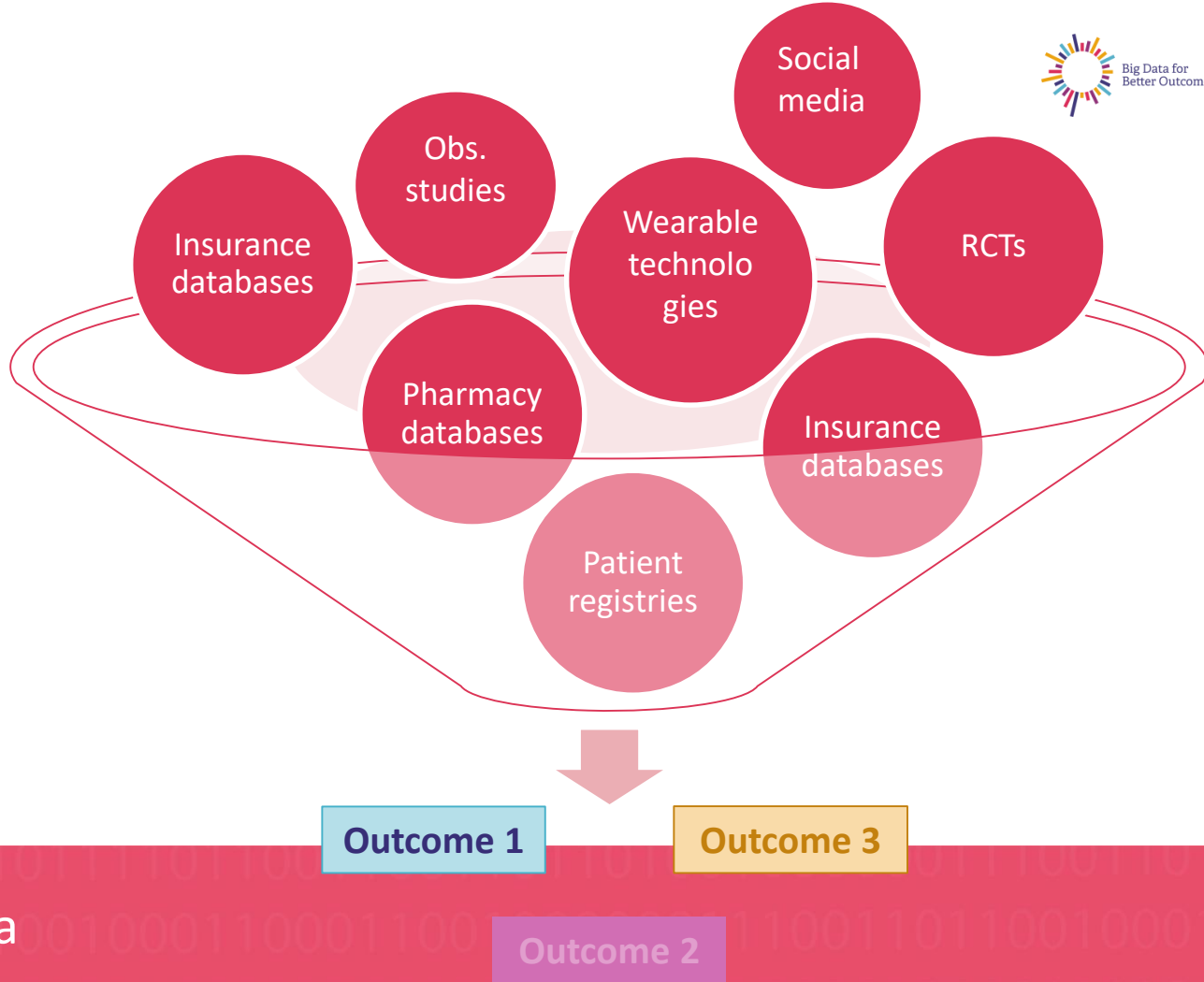
Design sets of standard outcomes and demonstrate value

Increase access to high quality outcomes data

Use data to improve value of HC delivery

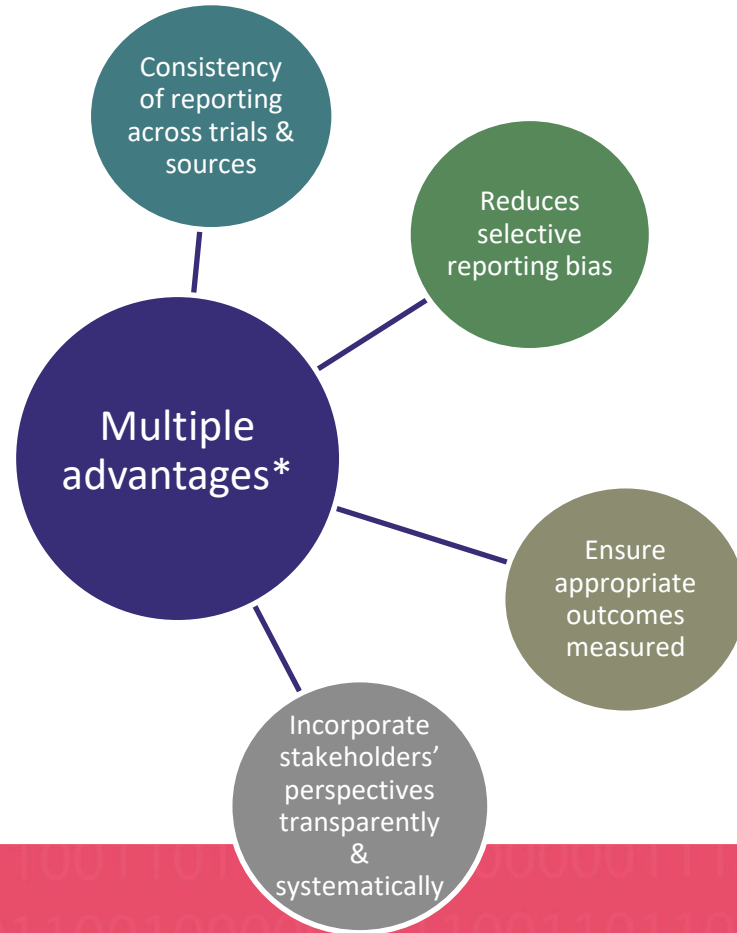
Increase patient engagement through digital solutions

Different sources
may collect
different
outcomes



Sources of big data

A core outcome set is the **minimum** outcomes that should be measured and reported



* Not comprehensive

Core outcome sets (COS)

A PRACTICAL TOOLKIT

FOR THE IDENTIFICATION, SELECTION AND
MEASUREMENT OF OUTCOMES INCLUDING IN
REAL-WORLD SETTINGS

IMI DO→IT DELIVERABLE 2.2



This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 116055. This Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA



- ❖ 6-stage approach
- ❖ flowcharts
- ❖ key questions
- ❖ simple clear language for those new to the area
- ❖ signpost to methodological options
 - ❖ advantages and disadvantages
- ❖ emphasis on RW settings
- ❖ checklists

<http://bd4bo.eu/index.php/toolkit>

Existing initiatives and resources



Harmonising Outcome Measures for Eczema
(HOME)



Green Park
Collaborative

What is NEW in the toolkit

- Use of case studies in BD4BO disease areas
- Focus on HTA, regulatory, payer perspectives
- COS for real-world settings. New pieces of work in appendices i.e. work on patient-reported outcome measures

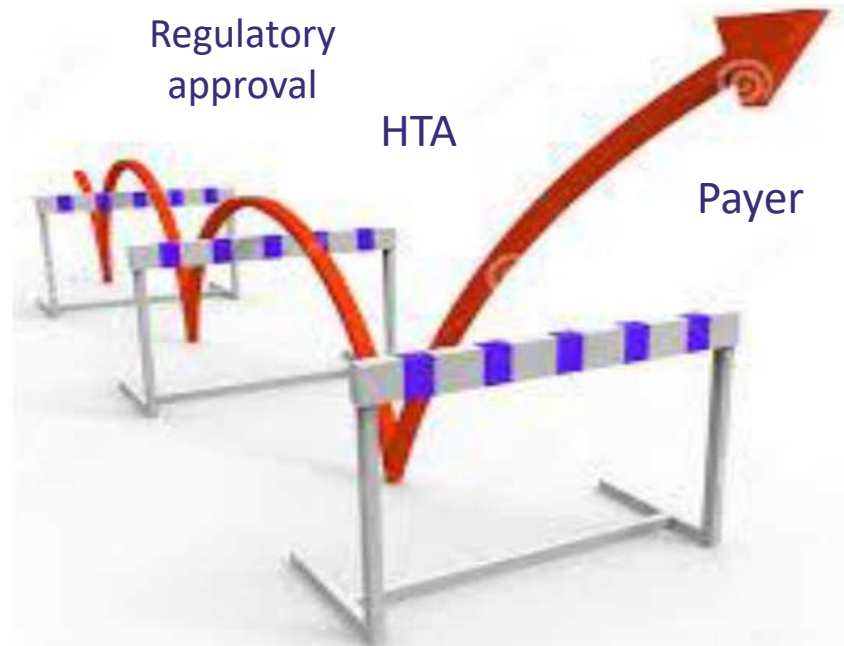
What is NEW?

Increasing uptake of COS

Need to understand current systems and landscape

- ❖ Who has an influence
 - ❖ Processes for outcome selection
 - ❖ Preferences and requirements
- ❖ Drivers for key decision makers with regard to outcomes
 - ❖ Make the COS relevant and applicable for their needs
- ❖ Toolkit: we investigated a variety of stakeholder perspectives and how to incorporate these into COS key decision makers

How to influence and increase COS uptake – a user perspective



- Toolkit assessed incorporating regulator, HTA and payer perspectives
- Access to novel products
- Factor in evidence generation and selection of outcomes from the start

Why include HTA, regulator, payer perspectives in COS

How do the policies and processes of different regulatory, HTA and payer organisations influence the preferences of outcomes to these stakeholders?

- ❖ Exploratory research:
 - ❖ Guidance documents and semi-structured telephone interviews
 - ❖ Approached 27 organisations; national, regional, umbrella
 - ❖ Availability of guidance on preferred outcomes
 - ❖ Outcome preferences
 - ❖ What factors influence the preferences
 - ❖ Do they vary and within stakeholder group
 - ❖ Level of detail required
 - ❖ Processes for selecting outcomes
 - ❖ Patient engagement
 - ❖ Clinical experts
 - ❖ Literature
 - ❖ COS?

Regulators

Outcome preferences, methods and processes tend not to vary between regulators (as they all follow EMA guidance)

- ❖ Marking authorisation and pharmacovigilance
- ❖ National, mutual recognition, decentralised and centralised procedures across the 50 national competent authorities
- ❖ Pre-specification of outcome preferences are outlined in detailed scientific guidance harmonised (ICH guidance) between EMA and EU member states
 - ❖ Generic as well as disease-specific
 - ❖ COS limited to a few specific diseases
- ❖ Focus on efficacy and safety
- ❖ **HTA and regulator preferences do not always align**
 - ❖ **Acceptability of surrogates, PROM-disease specific instruments preferred**

HTA organisations

- ❖ >59 HTA statutory agencies/bodies across EU (national + regional)
- ❖ High level of variability with regard to remit, process and methodology
- ❖ In general support decision making around reimbursement status/price negotiation processes- provide information on effectiveness of new treatments compared to available treatment options and resource use
- ❖ Clinical outcome preferences **do not** vary substantially between the majority of HTA agencies (longer term hard outcomes preferred)
- ❖ Quality of life measures are often integral and required by many HTAs- the measurement instrument acceptability varies by HTA and economic evaluation methodology used
- ❖ Method use can vary within HTA –this may be on a ‘case-by-case’ basis
- ❖ Majority of HTAs do not pre-specify outcomes – or see themselves as ‘selecting’ outcomes
 - ❖ Case by case approach
 - ❖ Very limited awareness of COS, no identified standardised use
 - ❖ Changing landscape-EUnetHTA core model

Payers

- ❖ Payer landscape is highly variable across Europe
 - ❖ Reflecting differing healthcare systems
 - ❖ Different processes for reimbursement
- ❖ Outcome preferences tend to align more with HTA preferences and depend on their the payers exact role- national/regional/local
 - ❖ Many contribute to HTA agency work - on Committees/boards
 - ❖ Many take & implement decision supported by HTA done by another body and consider budget impact
 - ❖ Some do HTA themselves

How to increase uptake and implementation of COS



❖ Increase awareness

- ❖ Benefits of using COS (consistency, comparability of data, stakeholder views on, relevant outcomes)
- ❖ Identify who within organisations to target- such as Early Scientific Advice, Scientific Advice interact with evidence generators at an early stage

❖ Incorporating Regulators and HTAs perspectives in COS development

- ❖ For Regulators/HTA to adopt a COS it needs to reflect their preferences/requirements
- ❖ Complicated to select which HTA due to case by case approach and variability in requirements
- ❖ Regulators are perhaps key influencer at present

Toolkit highlights a number of mechanisms

- ❖ How to get regulator and HTA perspectives for COS development
- ❖ How to get recognition and adoption of COS by regulators
 - ❖ EMA Qualification of Novel Methodologies
 - ❖ EMA Innovation Task Force a forum for early dialogue to support the development of innovative methodologies

How to influence and increase COS uptake – a user perspective



Thank you!

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 [@bd4bo](https://twitter.com/bd4bo)



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