The concept of Real World data
For regulators and reimbursement

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Areas to cover

Terminology and “Real World Data”
NICE and use of evidence of data
Future plans – statement of intent
Involvement in research
Data and NICE Connect
Terminology

Disagreement about the term ‘Real World Data’!

NICE has therefore opted to simply refer to ‘Data’ - any source of quantitative or qualitative data that is suitable for use in NICE’s work programmes, when examined using a range of analytic techniques.

Data includes:

• electronic health record data
• data collected outside of trials, eg registries
• any other relevant data.

It does not refer to published research findings and summary statistics.
### NICE and current use of data

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<th>Evaluating technologies</th>
<th>Developing guidelines</th>
<th>Assessing impact</th>
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<td>- Cancer Drugs Fund</td>
<td>- Routine use of published reports based on electronic health records</td>
<td>- Digital therapies and IAPT</td>
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<td>- Commissioning through Evaluation</td>
<td>- Occasional bespoke analysis of broader data, usually linked to economic analysis</td>
<td>- Resource impact assessment</td>
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<td>- Industry submissions</td>
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<td>- Innovation Scorecard estimates</td>
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<td>- Datasets to answer specific questions, eg for economics</td>
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<td>- Guidance uptake</td>
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<td>- Registry data for interventional procedures</td>
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Statement of intent for data analytics

Sets out our future ambition, covering:

- What kind of evidence NICE currently uses to develop guidance
- What broader types of data are available
- When and why broader types of data should be considered
- Practical considerations associated with data analytics

Technical detail on methodological considerations are not included in the statement and will be developed at a later stage.

Currently reviewing consultation comments.
Key challenges for NICE

Access to data, including data governance
(Poor) data quality
Capability and capacity to frame the questions and to carry out any analysis
Recognising a high quality analysis
Understanding when to use data exploitation – when is it an acceptable (or better) alternative to traditional research
Evidence and digital technologies

Challenges in setting out an evaluation framework

NICE led work on an Evidence for Effectiveness Framework

Levels of evidence required determined by risk assessment.
Best practice is a high quality RCT
Stratified evaluation of other technologies?

“High risk interventions”
Minimum requirement is RCT evidence

- **Clear evidence of benefit** – unrestricted approval
  - Eg NICE technology appraisals

- **Evidence is promising but not conclusive** – restricted approval with ongoing data collection for a defined period
  - EG CDF, digital IAPT, CtE
Illustration of current evidence gaps
Reasons for non-selection of medical technologies

- Lack of evidence
- Insufficient or uncertain benefit to the NHS
- Insufficient or uncertain benefit to patients
- Uncertain or no cost benefit
- Not novel
- Not clear how technology would be used...
- Design appropriate only to a small...
- Wrong comparator
- Usability or technology design issue
- Evidence does not translate to UK setting
- Insufficient demand

80 technologies notified to MTEP 2010-2014
Key questions

How do we define ‘high and low risk’?
Who funds the data collection?
What are the appropriate data collection requirements for lower risk products?
Do we need a separate framework for non-digital technologies?
How to link with other regulators?
When does environmental impact matter?
Ongoing research initiatives

**EU funded collaborations**
- Harmony (big data in haematology)
- EHDEN (federated data network)
- GetReal Initiative (think tank of leading opinion leaders in the RWE space)
- IMPACT HTA (tools and methods for evidence synthesis)
- HTx (‘next generation’ HTA)
Establishing a self-sustainable not-for-profit entity in 2020
Providing leadership in the use of RWE in pharma R&D and healthcare decision making
Providing education and tools

Highlight activity:
NICE co-leads a ‘Think Tank’ comprising KOLs in RWE. First F2F meeting 13 September
**Federation**
Creation of an EU-wide architecture for federated analyses of real world data

**Harmonisation**
Harmonise more than 100 million anonymised health records to the OMOP common data model

**Community**
Establish a self-sustaining open science collaboration in Europe, supporting academia, industry, regulators, payers, government, NGOs and others

**Outcomes**
Enabling outcomes-driven healthcare at a European level

**Education**
The establishment of an EHDEN Academy, webinars and face-to-face training sessions to train all stakeholders
Key considerations in NICE’s work:

- Is the common data model useful for HTA and guideline development?
- How can it be developed to better support regulation and subsequent guidance development for pharmaceuticals?
- Can it support outcomes-based payment systems?

How are we doing this?

- Developing use cases in different disease areas reflecting key challenges in evidence generation for HTA, e.g. extrapolation in oncology trials
- Comparing estimates in standardised data comparable to original data, e.g. primary care data in UK [CPRD]
- Working with ICHOM to support mapping of outcomes to a common data model
The HTx vision is based around the concept of learning healthcare systems

‘Ultimately this is a step change in HTA moving away from single assessment approach to providing ‘a menu of reimbursement decision options’ that could be recommended by HTA agencies to payers’

**Highlight activity:**

*NICE* is working with University of Manchester to develop a PhD project that will contribute to this work.
The HARMONY approach

Development of a clinical Big Data platform that:

- Enables collecting, sharing and harmonizing of data from high-quality multidisciplinary sources
- Enables assess and integration of large amounts of omics data by developing tools to analyse these complex data sets
- Facilitates drug development pipelines and accelerate the “bench-to-bedside” process in drug development
- Incorporates meaningful and harmonized clinical endpoints and outcome measures in haematological malignancies

Highlight activities:

NICE has a key focus on education and ‘demystifying’ the processes and needs of European payer and HTA organisations

With European partners provides view on harmonised clinical outcomes in haematological malignancies, including core outcome sets including HRQoL, and PROMs
In summary: NICE’s future ambition for use of data and analytics

Develop and update guidance more rapidly than we can achieve currently

Provide answers to questions that we cannot answer using our traditional approaches

• Extrapolation beyond clinical trials – predictive effectiveness
• Validation of intermediate outcomes

Measure the effectiveness of interventions in real-world settings;

Improve our tracking of guidance implementation, uptake and impact, and use of this information to inform the need to update.

NICE
Future vision for NICE guidance

Accessible, living, integrated guidance

To achieve this we will:

• Combine all our advice and guidance into an integrated product on the NICE website that follows the patient journey

• Rapidly sequence new drugs and technologies, so practitioners and commissioners can identify them and adopt more quickly

• **Keep recommendations up to date, so care is always based on the best available evidence and data**

• Integrate recommendations into IT systems, so it will be easier for practitioners to adhere to the evidence.