NICE National Institute for Health and Care Excellence

The concept of Real World data

For regulators and reimbursement

Professor Gillian Leng CBE

Deputy Chief Executive, NICE

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

Evaluating technologies

- Cancer Drugs Fund
- Commissioning through Evaluation
- Industry submissions
- Datasets to answer specific questions, eg for economics
- Registry data for interventional procedures

Developing guidelines

- Routine use of published reports based on electronic health records
- Occasional bespoke analysis of broader data, usually linked to economic analysis

Assessing impact

- Digital therapies and IAPT
- Resource impact assessment
- Innovation Scorecard estimates
- Guidance uptake

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

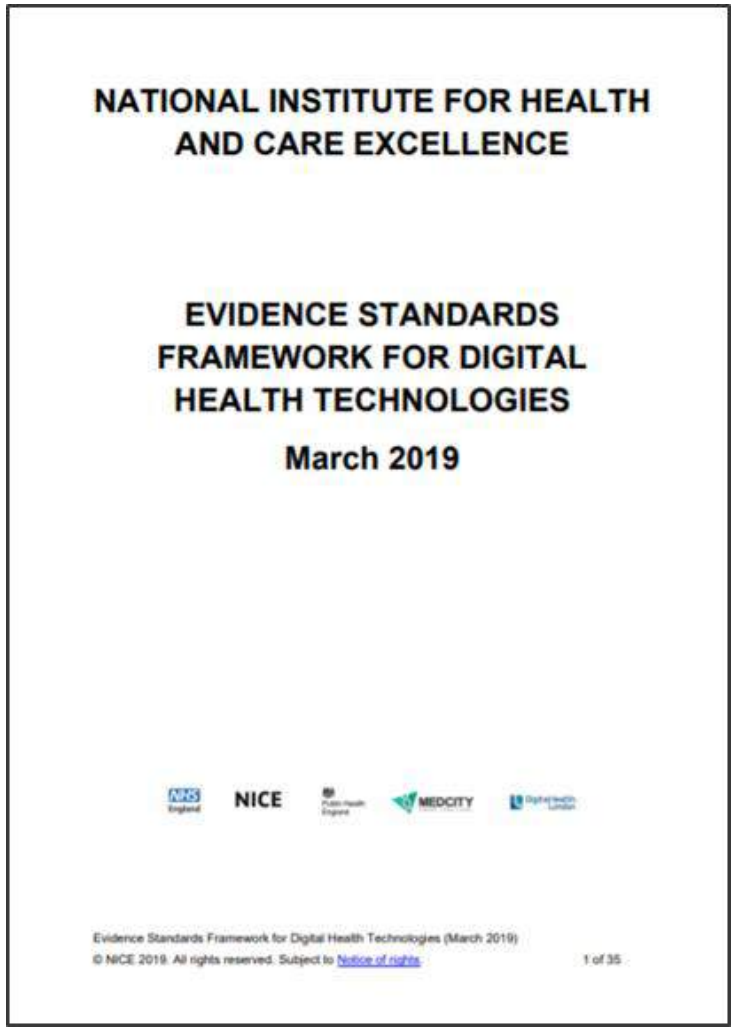
Understanding when to use data exploitation – when is it an acceptable (or better) alternative to traditional research

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.


NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

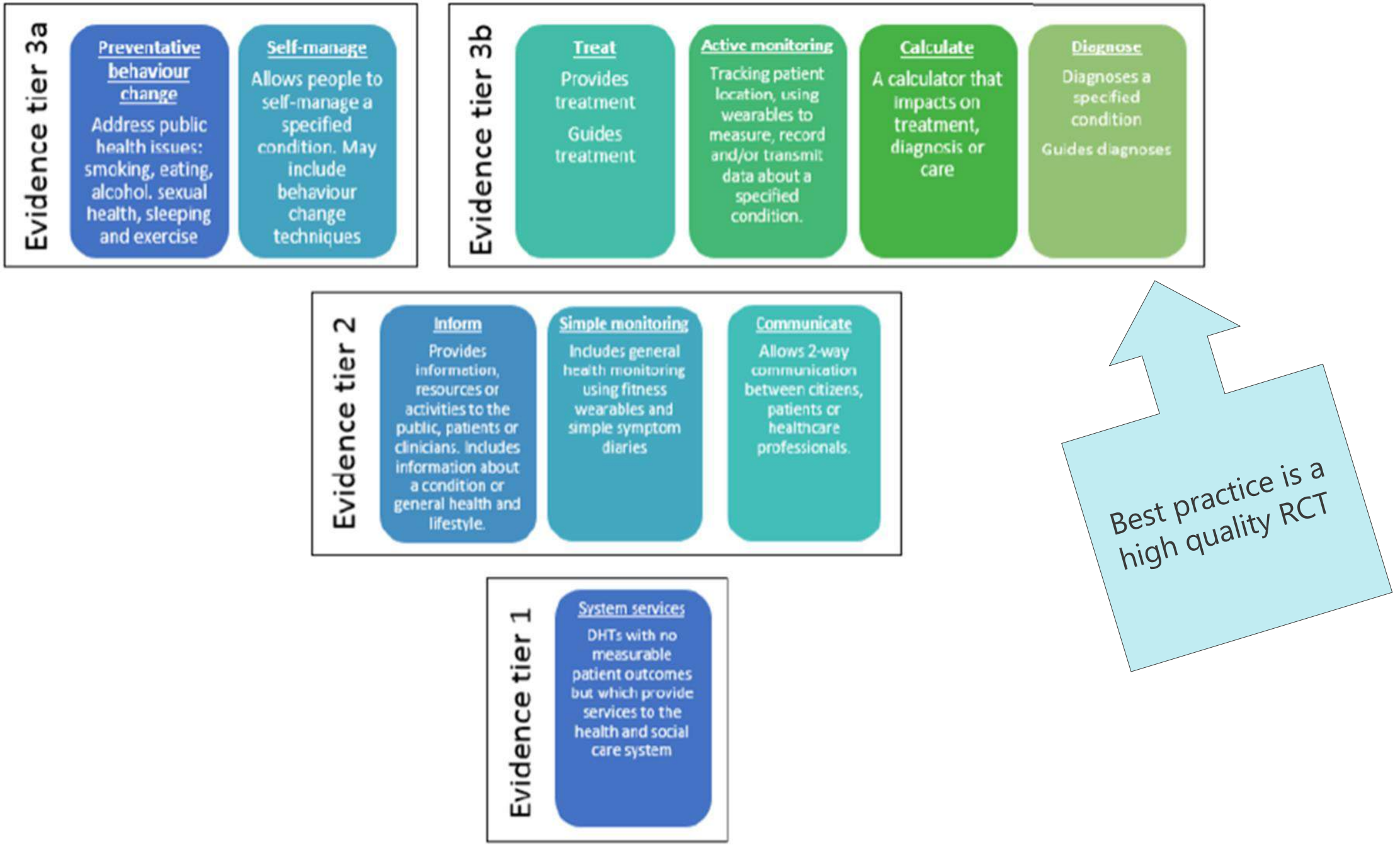
EVIDENCE STANDARDS FRAMEWORK FOR DIGITAL HEALTH TECHNOLOGIES

March 2019

NHS England NICE Public Health England MEDCITY Digital Health London

Evidence Standards Framework for Digital Health Technologies (March 2019)

1 of 35


Evidence tier 3a
Preventative behaviour change
Address public health issues: smoking, eating, alcohol, sexual health, sleeping and exercise
Self-manage
Allows people to self-manage a specified condition. May include behaviour change techniques
Evidence tier 3b
Treat
Provides treatment
Guides treatment
Active monitoring
Tracking patient location, using wearables to measure, record and/or transmit data about a specified condition.
Calculate
A calculator that impacts on treatment, diagnosis or care
Diagnose
Diagnoses a specified condition
Guides diagnoses
Evidence tier 2
Inform
Provides information, resources or activities to the public, patients or clinicians. Includes information about a condition or general health and lifestyle.
Simple monitoring
Includes general health monitoring using fitness wearables and simple symptom diaries
Communicate
Allows 2-way communication between citizens, patients or healthcare professionals.
Evidence tier 1
System services
DHTs with no measurable patient outcomes but which provide services to the health and social care system
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

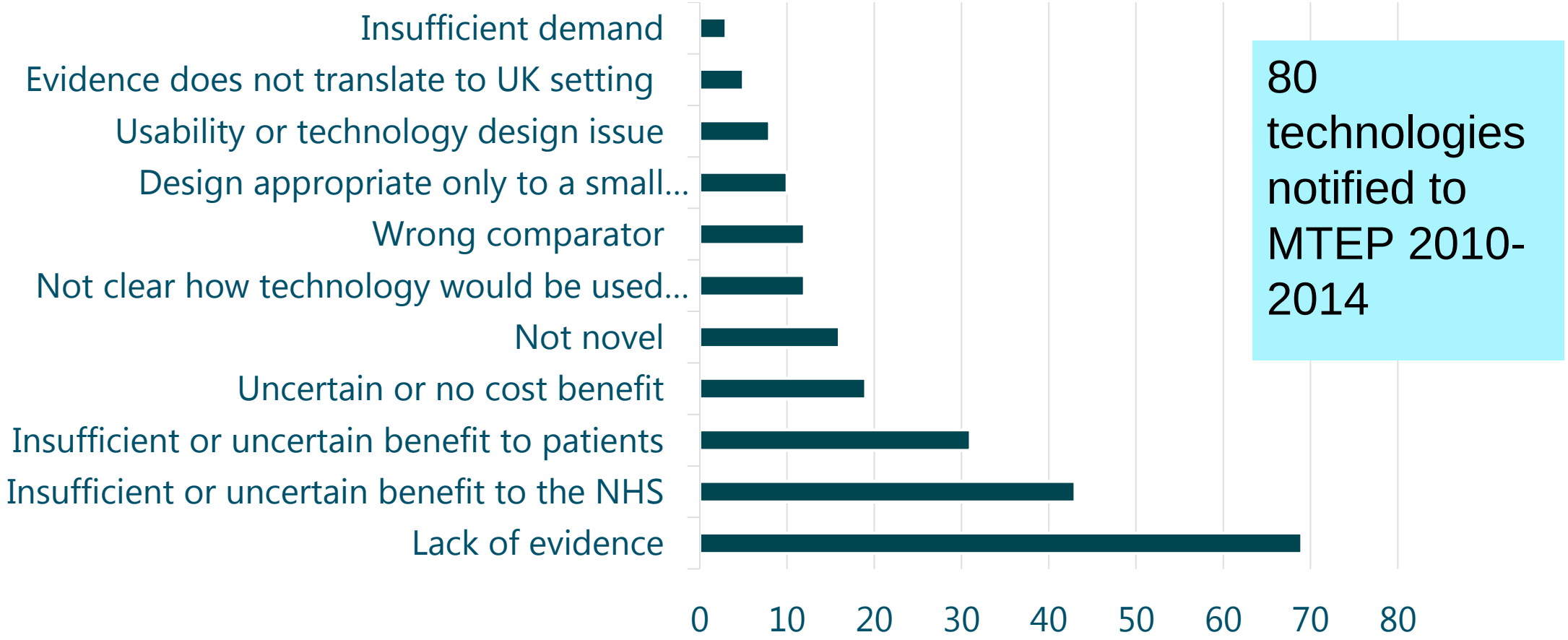
Eg NICE technology appraisals

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



NICE

Key questions

How do we define ‘high and low risk’?

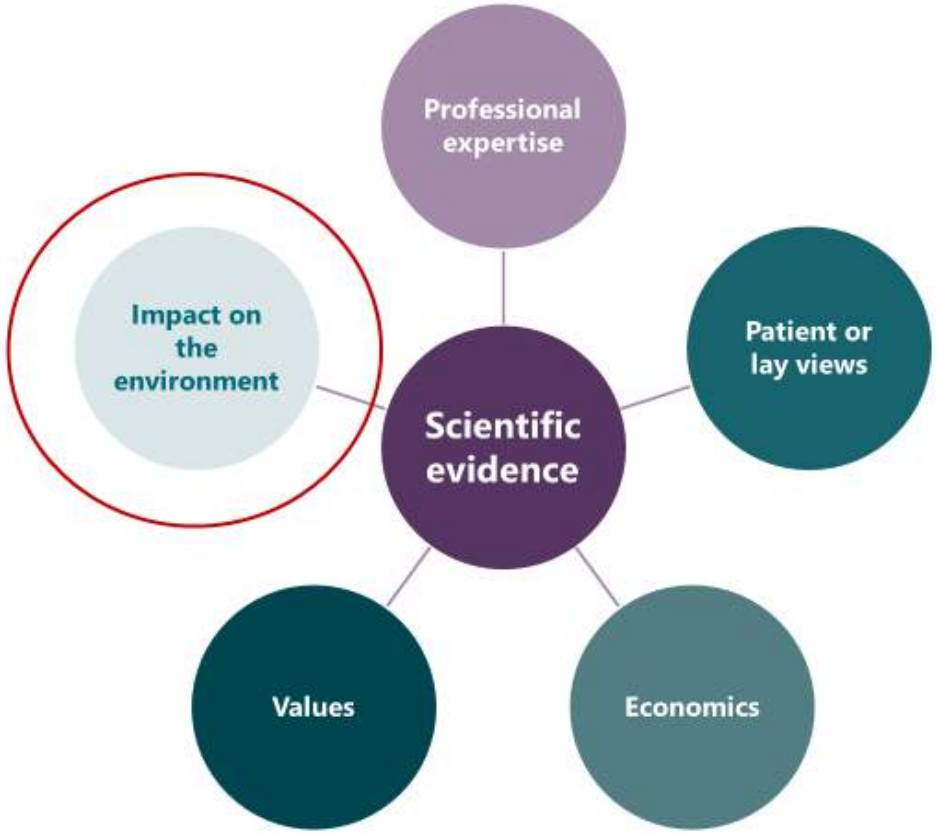
Who funds the data collection?

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)

GetReal Initiative

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

EHDEN
EUROPEAN HEALTH DATA & EVIDENCE NETWORK

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:

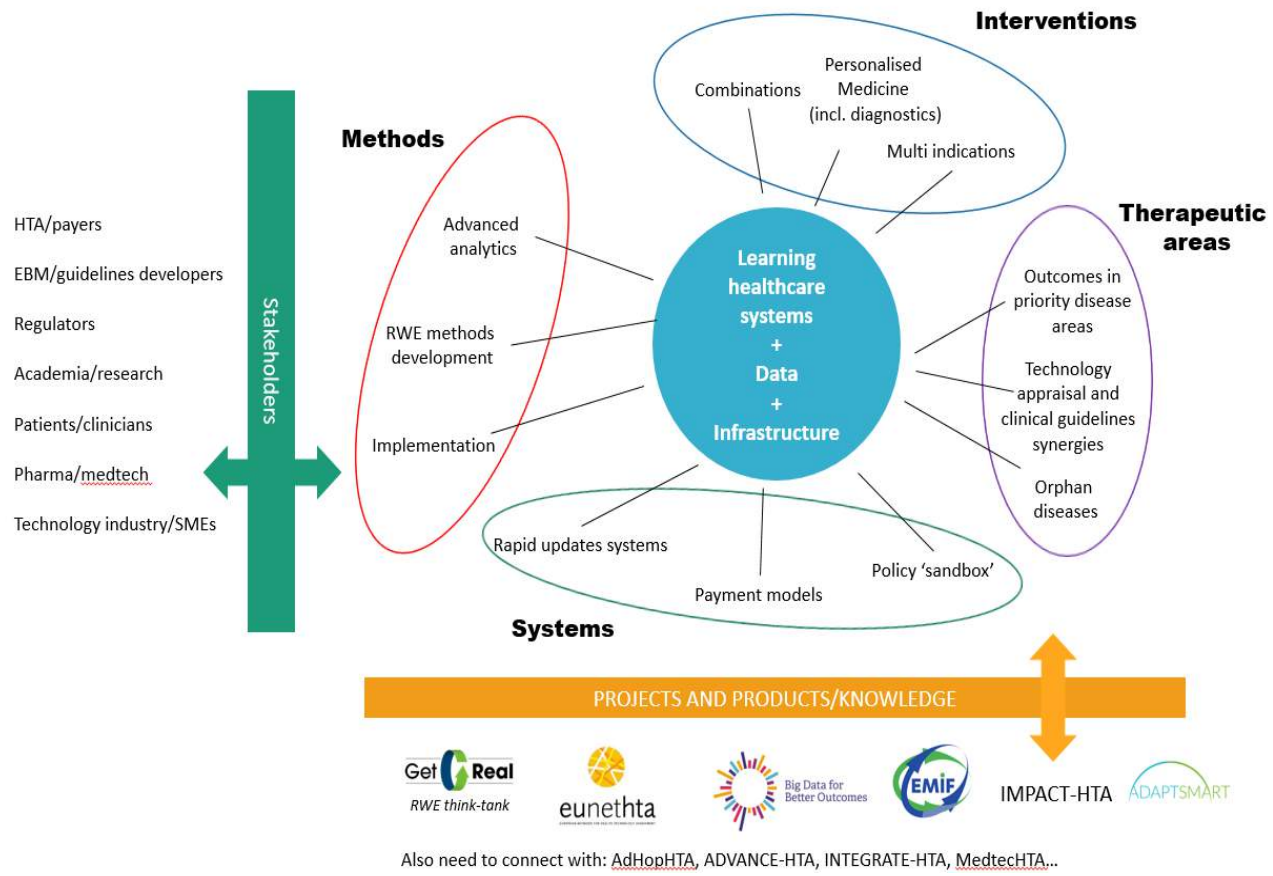
- Is the common data model useful for HTA and guideline development?
- How can it be developed to better support regulation and subsequent guidance development for pharmaceuticals?
- Can it support outcomes-based payment systems?

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

Next Generation HealthTechnology Assessment

The HTx vision is based around the concept of learning healthcare systems

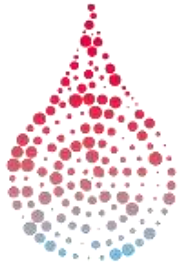


'Ultimately this is a step change in HTA moving away from single assessment approach to providing *'a menu of reimbursement decision options'* that could be recommended by HTA agencies to payers'

Highlight activity:

*NICE is w*orking with University of Manchester to develop a PhD project that will contribute to this work

NICE



HARMONY

Healthcare Alliance for Resourceful Medicines Offensive against Neoplasms in Hematology

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

NICE

In summary: NICE's future ambition for use of data and analytics

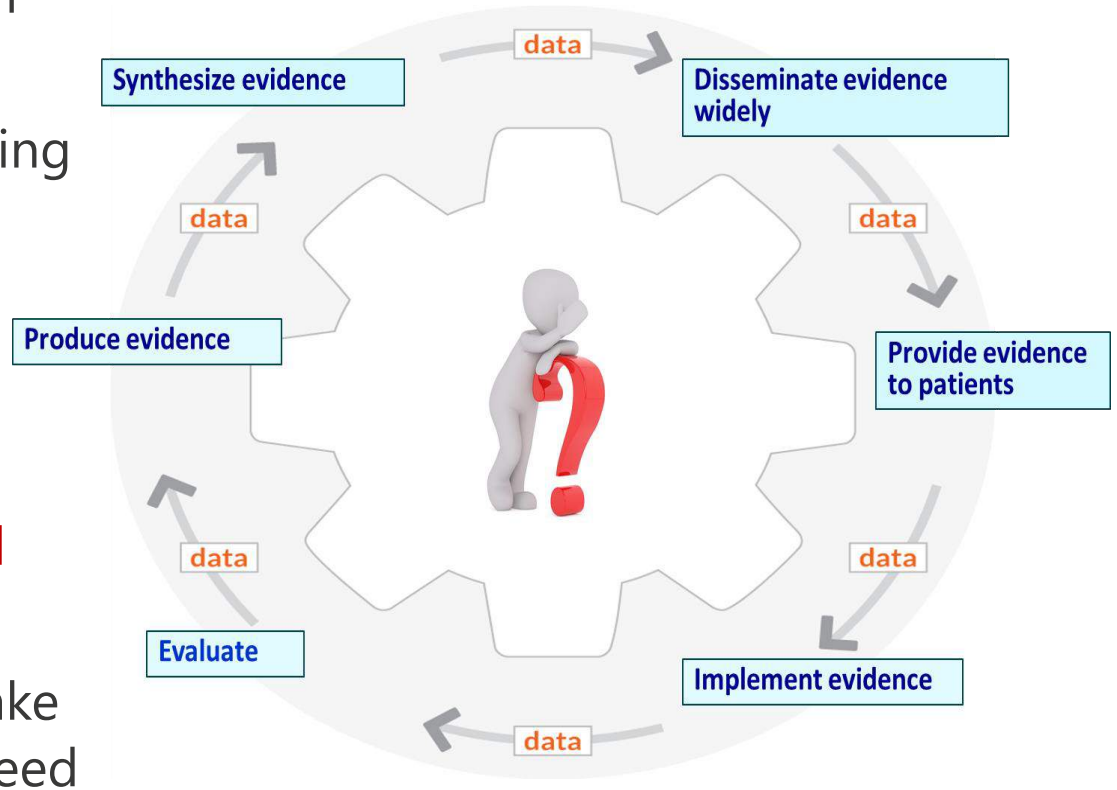
Develop and update guidance more rapidly than we can achieve currently

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.