Maximising the potential of Real World Data for evidence generation & decision making

NICE Conference 2023

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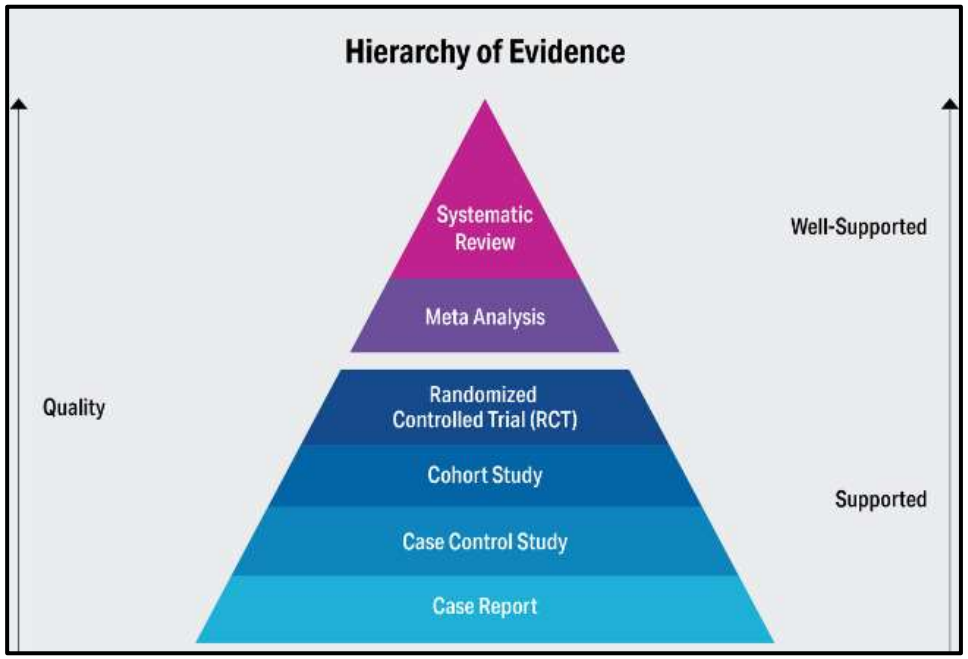
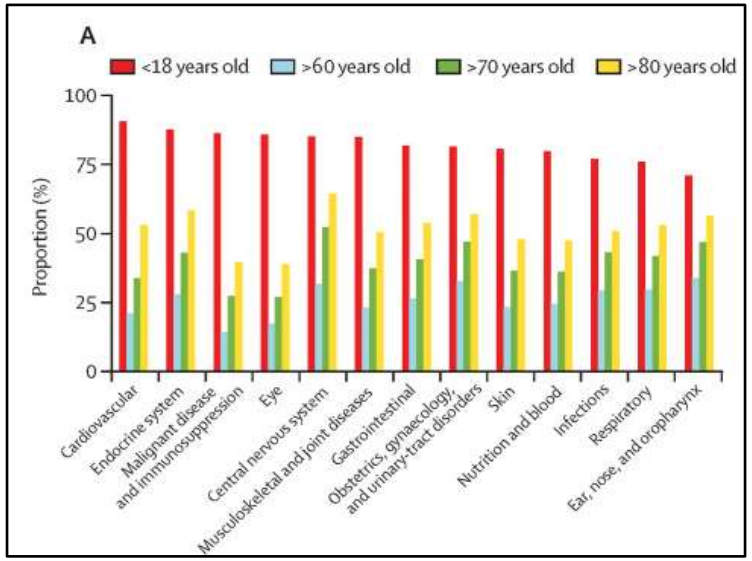
Who we are…

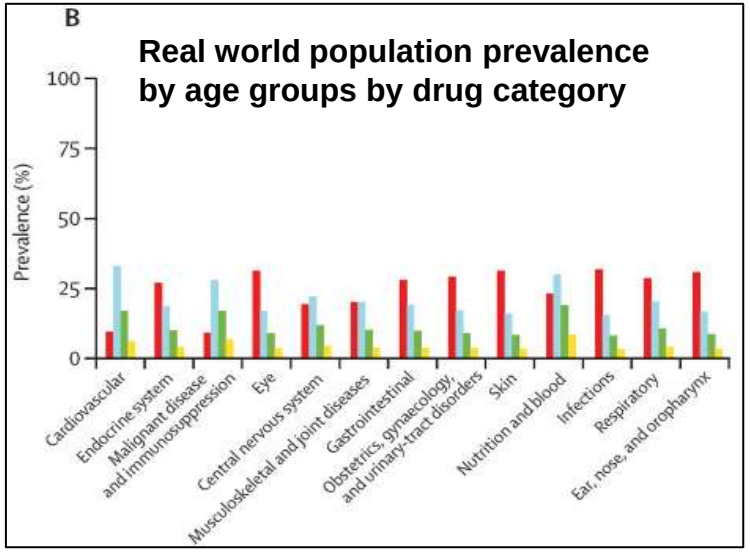
- **The Association of the British Pharmaceutical Industry (ABPI) exists to make the UK the best place in the world to research, develop and use new medicines. We represent companies of all sizes who invest in discovering the medicines of the future.**
- Our members supply cutting edge treatments that improve and save the lives of millions of people. We work in partnership with Governments and the NHS so patients can get new treatments faster and the NHS can plan how much it spends on medicines.
- Every day, we partner with organisations in the life sciences community and beyond to transform lives across the UK.

RCTs gold standard to evaluate effectiveness but not representative of real world patient populations



RCT exclusion proportion by age groups by drug category





Comparing clinical trial population representativeness to real-world populations: an external validity analysis encompassing 43 895 trials and 5 685 738 individuals across 989 unique drugs and 286 conditions in England
Yen Yi Tan *et al* The Lancet Health Longevity
VOLUME 3, ISSUE 10, E674-E689, OCTOBER 2022

Increasing diversity in clinical research

The ABPI is working with government, system partners, and patient organisations to improve diversity and inclusion in research:

- The ABPI's People-centred Research Hub, ABPI member case studies that promote industry best practices in diversity and inclusion, patient and public involvement, and research transparency.
- The ABPI's partnership with Genomics England to support their Diverse Data Initiative, aiming to increase the diversity of genomic datasets and resulting research


Medicines & Healthcare products Regulatory Agency

Consultation outcome

Government response to consultation on legislative proposals for clinical trials

Updated 21 March 2023

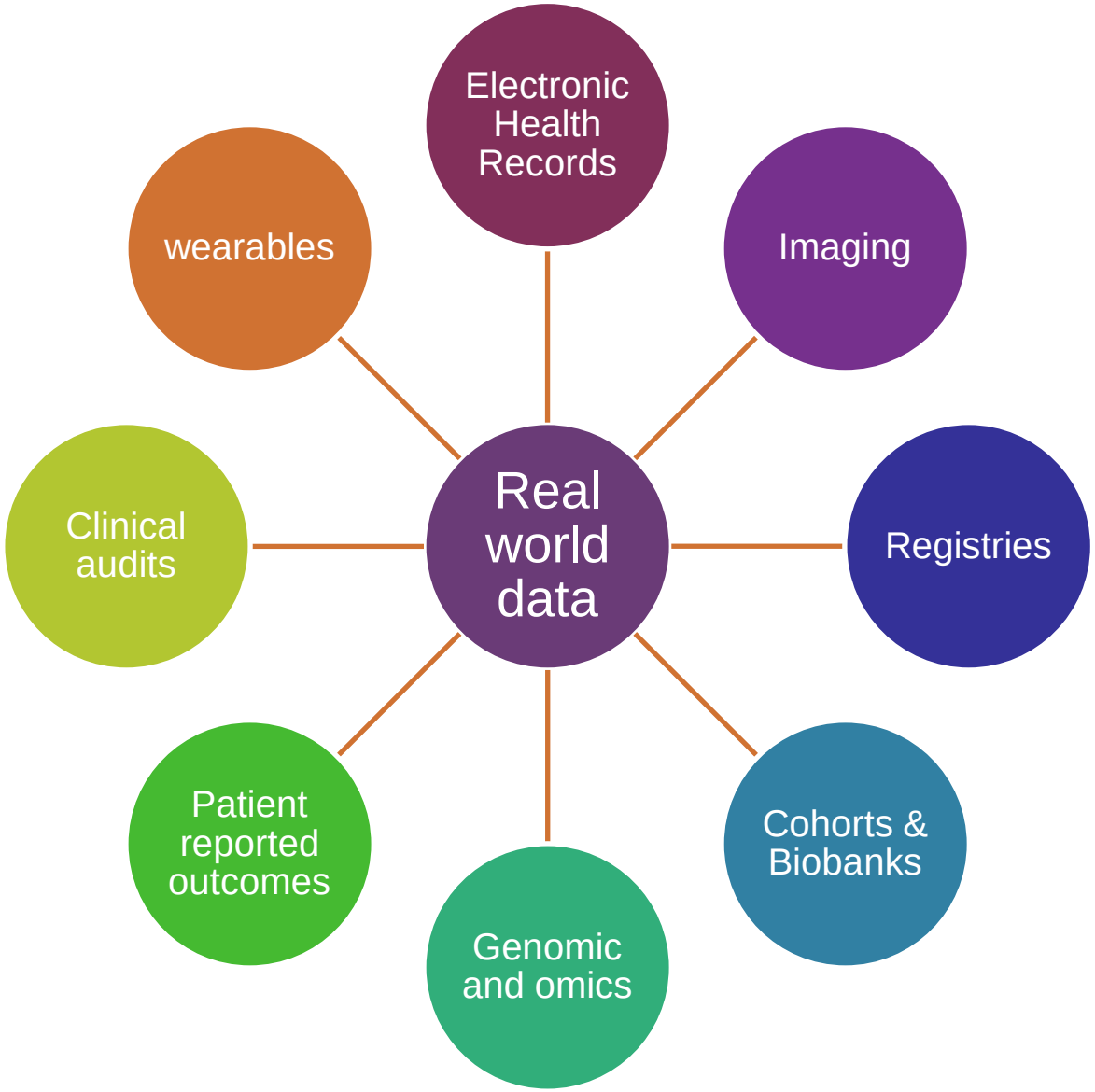
Executive Summary

The MHRA and the Department of Health in Northern Ireland consulted on a set of proposals to update, improve and strengthen the UK legislation that underpins the regulation of clinical trials. Having analysed over 2000 responses received, we will now take forward legislation to reform of the UK clinical trials regulatory framework that will:

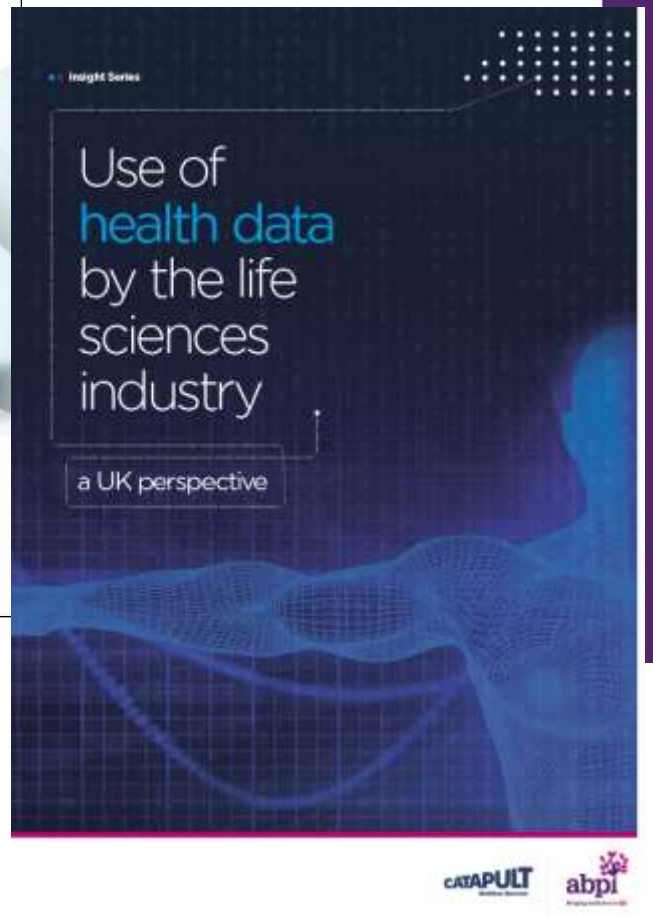
- **Ensure patients and their safety are at the focus of all clinical trials and bring the benefits of clinical trials to everyone**

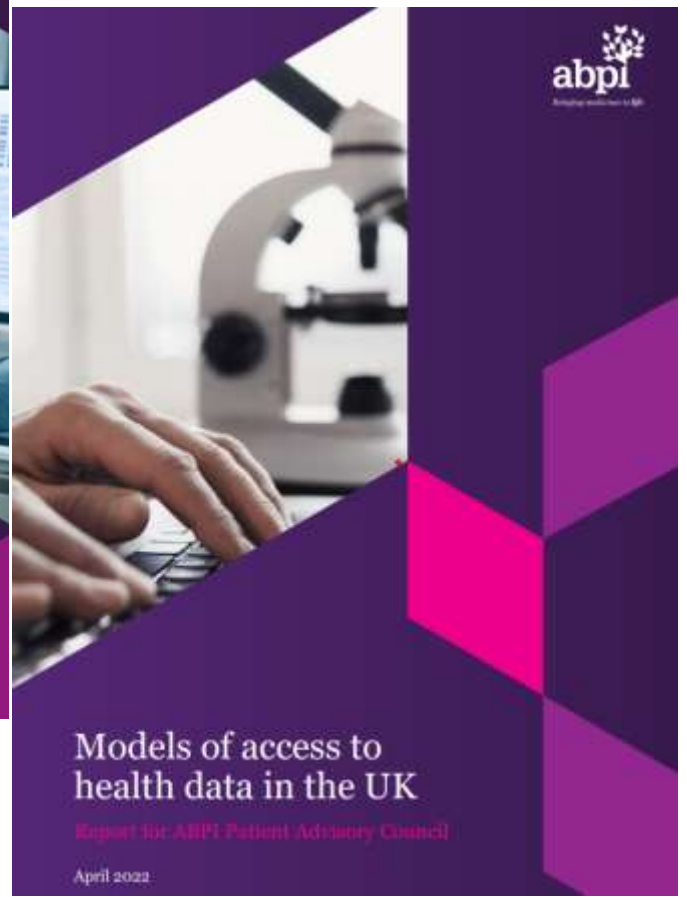
We are committed to ensuring new medicines are safe and effective for the whole population and to reducing health disparities. Clear guidance on diversity in trials will drive a vital shift in representation in, and access to, research, without imposing targets or arbitrary quotas. This will help to ensure that participants come from diverse backgrounds, so the findings of research reflect prevalence and clinical need across the population, and all can benefit from new treatments.

What is real world data?



ABPI long term commitment to improving health data environment to support industry research





RWD used extensively by the pharmaceutical industry

Market access assessment

- Disease natural history
- Care pathways
- Healthcare resource utilisation

Clinical trials

- Design & feasibility
- Recruitment & follow up
- External controls

Health Technology Assessment and reimbursement

- Clinical effectiveness
- Cost effectiveness
- Innovative payment models

Post authorisation safety

- Risk management
- Safety monitoring

Real world performance

- Adoption and uptake
- Off label use & new indications
- Evaluation of clinical guidance and health policies

Novel use of RWD to facilitate inclusion in clinical trials

Table 1. Demographic Characteristics of the Participant Population Enrolled in the NHS-Galleri Trial, by Invitation Route and Overall (n [%])

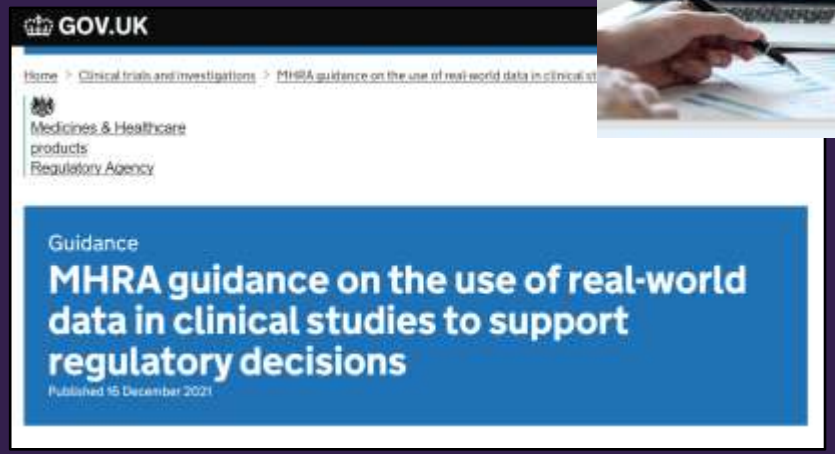
		Centralised Targeted Invitation N=139,617	Open Enrolment N=2358	GP PIC Sites N=965	NHS-Galleri trial N=142,940	Cancer Alliance Regions[a]	England[b]
Years of Age[c]	50–54	14,851 (10.6)	429 (18.2)	157 (16.3)	15,437 (10.8)	1,700,000 (21.4)	3,908,000 (21.0)
	55–59	21,864 (15.7)	470 (19.9)	131 (13.6)	22,465 (15.7)	1,678,000 (21.1)	3,806,000 (20.5)
	60–64	26,087 (18.7)	506 (21.5)	228 (23.6)	26,821 (18.8)	1,450,000 (18.3)	3,256,000 (17.5)
	65–69	30,142 (21.6)	434 (18.4)	239 (24.8)	30,815 (21.6)	1,247,000 (15.7)	2,767,000 (14.9)
	70–74	29,911 (21.4)	358 (15.2)	161 (16.7)	30,430 (21.3)	1,266,000 (16.0)	2,797,000 (15.1)
	75–77	16,426 (11.8)	157 (6.7)	48 (5.0)	16,631 (11.6)	599,000 (7.5)	1,323,000 (7.4)
Sex[c]	Female	69,876 (50.0)	1444 (61.2)	536 (55.5)	71,856 (50.3)	4,072,000 (51.3)	9,169,000 (51.4)
	Male	69,741 (50.0)	914 (38.8)	429 (44.5)	71,084 (49.7)	3,869,000 (48.7)	8,687,000 (48.7)
Ethnicity[d]	White	130,399 (93.4)	2055 (87.2)	730 (75.6)	133,184 (93.2)	8,737,000 (91.9)	19,206,000 (89.9)
	Asian	4509 (3.2)	163 (6.9)	54 (5.6)	4726 (3.3)	401,000 (4.2)	1,127,000 (5.3)
	Black	1856 (1.3)	76 (3.2)	125 (13.0)	2057 (1.4)	226,000 (2.4)	594,000 (2.8)
	Other	604 (0.4)	13 (0.6)	10 (1.0)	627 (0.4)	77,000 (0.8)	267,000 (1.3)
	Mixed	1467 (1.1)	43 (1.8)	35 (3.6)	1545 (1.1)	67,000 (0.7)	177,000 (0.8)
IMD Quintile[c,e]	1 - Most Deprived	31,766 (22.8)	365 (15.5)	222 (23.0)	32,353 (22.7)	1,694,000 (21.3)	2,870,000 (16.7)
	2	27,315 (19.6)	456 (19.3)	299 (31.0)	28,070 (19.7)	1,521,000 (19.2)	3,194,000 (18.6)
	3	29,340 (21.0)	529 (22.4)	257 (26.6)	30,126 (21.1)	1,620,000 (20.4)	3,560,000 (20.8)
	4	28,258 (20.2)	539 (22.9)	170 (17.6)	28,967 (20.3)	1,618,000 (20.4)	3,726,000 (21.9)
	5 - Least Deprived	22,479 (16.1)	461 (19.6)	14 (1.5)	22,954 (16.1)	1,488,000 (18.7)	3,805,000 (22.2)

[a]Age, Sex, and IMD, N=7,941,000; Ethnicity, N=9,508,000; [b]Age and Sex, N=17,857,000; Ethnicity, N=21,371,000; IMD, N=15,166,000; [c]Cancer Alliance and England data age range 50–77 years; [d]Cancer Alliance and England data age range ≥50 years; [e]IMD quintile was based on lower-layer super output areas (LSOAs) in England.
GP, General practitioner; IMD, Index of multiple deprivation; PIC, Participant identification centre.

- NHS Galleri trial is a large pragmatic RCT assessing clinical utility of blood-based cancer detection test in adults aged 50-70
- NHS DigiTrials used NHS data to invite eligible patients to participate
- Approach allowed for enrichment of more deprived & older age groups compared with other recruitment methods
- Use of RWD enabled recruitment of diverse participants with respect to age and socioeconomic deprivation

Swanton C, Bachtiar V, Brentnall AR, Mathews C, Lowenhoff I, Waller J, Bomb M, McPhail S, Pinches H, Smittenaar R, Hiom S, Neal RD, Sasieni P. NHS-Galleri Trial Enrolment Approaches and Participant Sociodemographic Characteristics. Poster presented at European Society for Medical Oncology Annual Meeting. October 20-24, 2023; Madrid, Spain

Medicines regulators increasingly interested in use of RWD to shape regulatory decisions

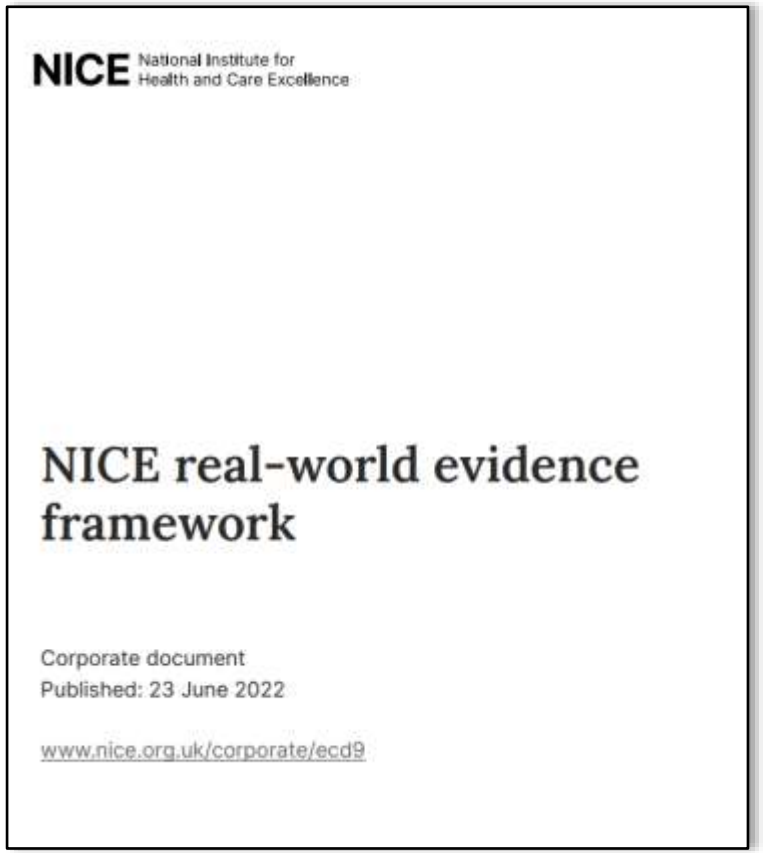


Accelerated patient access to innovative treatments increases reliance on evidence generation from real world data

- Pre-licensing: The **Early Access to Medicines Scheme** recognises the value of collecting RWD pre-authorisation and includes a supportive framework for the collection of RWD without the need for clinical trial approval

- Post licensing: The **Innovative Licensing and Access Pathway** includes a continuous benefit risk assessment integrating real world evidence tool, designed to fill evidence gaps within the right time frames to support accelerated access to medicines

ABPI welcomes NICE real-world evidence framework



- Agree with increased focus on use of RWE in NICE guidance
- ABPI had significant input into Framework development
- Valuable summary for how RWD can be used, challenges and guidance for planning, conduct and reporting RWD studies
- Companies invest considerable resources in RWD studies
- Committees must take proportionate and consistent approach to considering RWE biases and limitations, in the same way that biases and limitations are considered for RCT data
- Vital that Framework translates to greater acceptance of RWE in NICE decision making and guidance development, particularly to demonstrate treatment effect

Evaluating the impact of the NICE framework

Two key metrics to evaluate the success of the Framework could be:

RWE Submission Quality

Set a quality baseline by reviewing past submissions

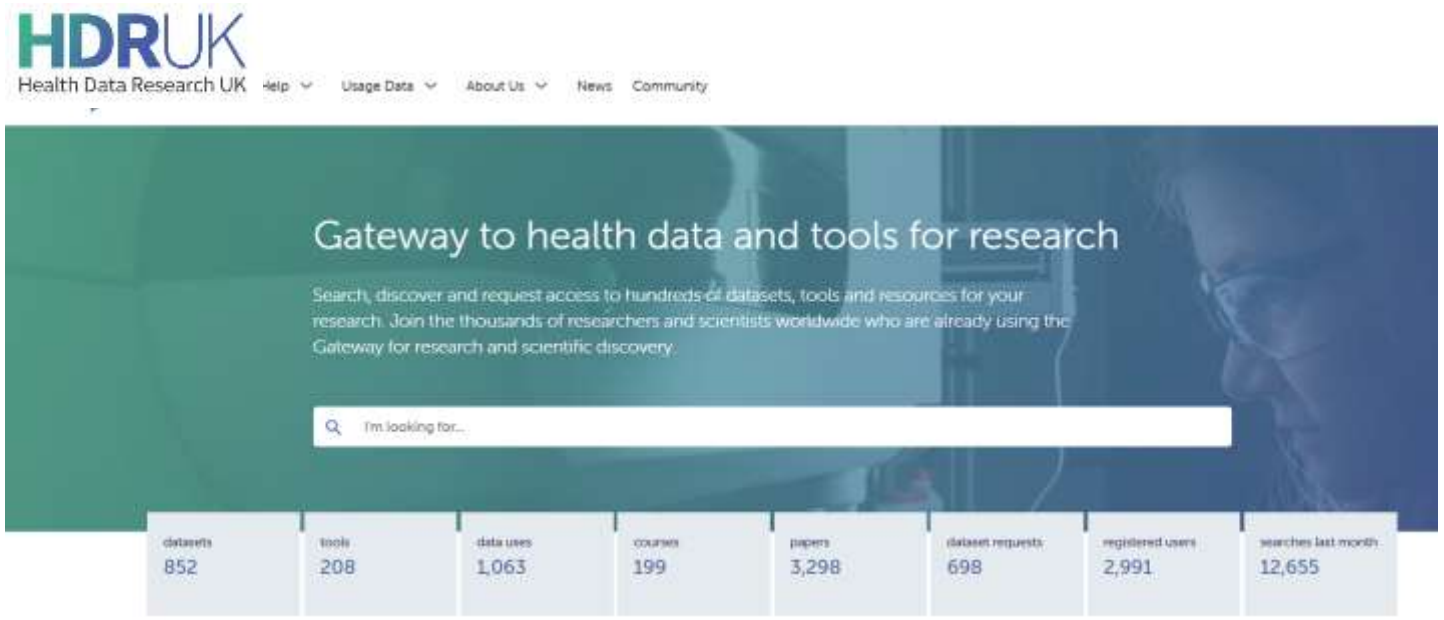
Assess against agreed criteria whether there has been an increase in good quality RWE submissions

Increase in acceptability of RWE

Record when committees have accepted RWE in their decision making especially acceptance of real world estimates of treatment effectiveness

Capture qualitative feedback from committee members and NICE staff to inform iterative updates of Framework and manuals

Recent advances in data provenance tools



- 856 datasets from 60+ custodians
- Metadata collection includes
  - Coverage
  - Format and standards
  - Provenance

Digital Object Identifiers (DOIs) for datasets

The CPRD database releases listed below include a digital object identifier (DOI). This means that specific database versions can be cited in publications.

This supports transparent, reproducible research and provides additional assurance of data provenance.

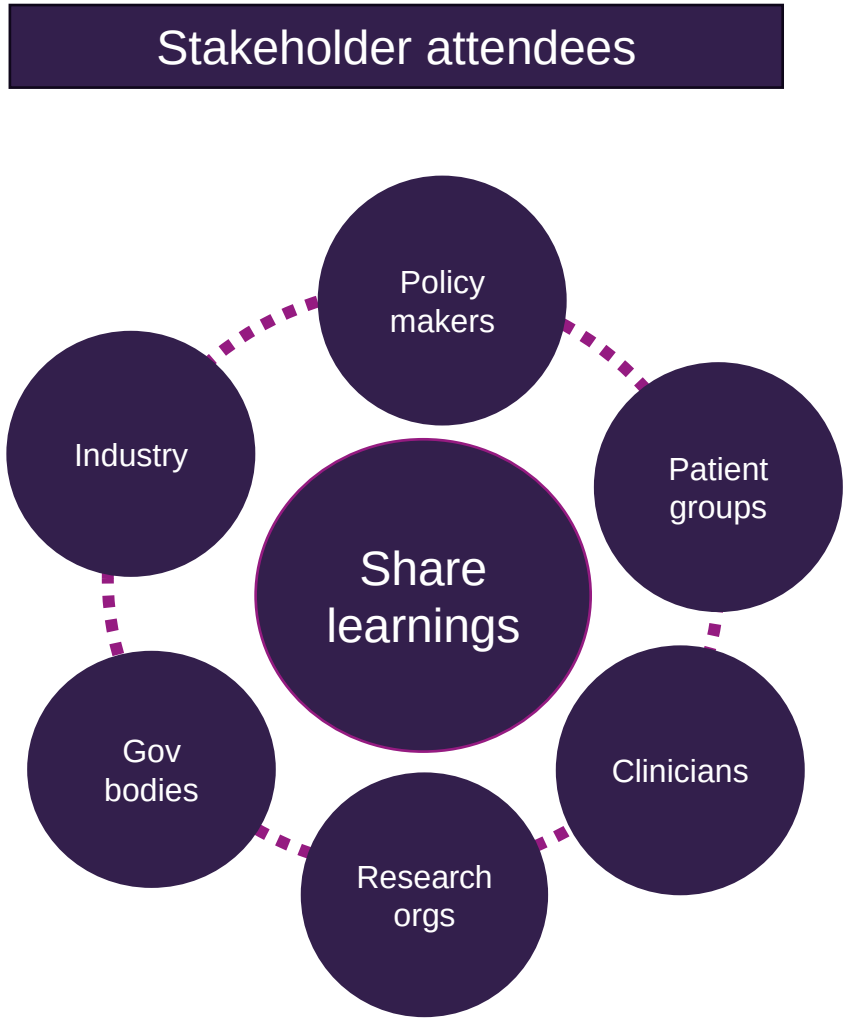
Please click on the title below for more detail and information about how to cite the data in a publication.

Information is also available from DataCite at DataCite Commons.

CPRD | UK data driving real-world evidence

Use of real world data to evaluate patient outcomes

- The ABPI convened a workshop in October 2023: **Using Real World Data to Evaluate Patient Outcomes**
- Attended by range of stakeholders from England, Wales and Scotland
- Discussions focused on **opportunities and challenges of using RWD to evaluate patient outcomes and clinical effectiveness** in the real world
- Aim - **share experiences and learnings** and identify areas where stakeholders could potentially **work together to gain a better understanding** of patient outcomes in response to medicines



Key insights from workshop discussions

Data Collection	Infrastructure & access	Opportunities
• Stakeholder engagement on what data to collect • Drive higher quality data capture by explaining rationale for collection to clinicians • Balance workforce burden with need for data collection	• Need infrastructure and data access policies to enable timely access to high quality data • Interoperability and linkage between different datasets is key • Widespread adoption of data standards is necessary	• Develop shared understanding of uses of RWD to evaluate patient outcomes to inform clinical and commissioning decision making • Potential for greater cost efficiencies through adoption of value based healthcare payment models • Reduce inequalities in access to clinical trials and medicines

Building public support for sharing health data is essential

Stakeholder collaboration is critical to success

Building public trust in industry's responsible use of health data

Principles for analysis and use of health data by ABPI members

abpi
Bringing medicines to life

About the ABPI

The ABPI exists to make the UK the best place in the world to research, develop and use new medicines and vaccines. We represent companies of all sizes which invest in discovering the medicines of the future.

Our members supply cutting edge treatments that improve and save the lives of millions of people. We work in partnership with Government and the NHS so patients can get new treatments faster and the NHS can plan how much it spends on medicines.

Every day, we partner with organisations in the life sciences community and beyond to transform lives across the UK.

What is Health Data?

The Data Protection Act 2018 defines 'data concerning health' as personal data relating to the physical or mental health of an individual, including the provision of health care services, which reveals information about their health status.

'NHS health data' is data produced by the NHS in the process of care delivery, including genomic data.

'Data custodians' are the organisations that have the legal right to grant access to health data.

About the principles

Using health and genomic data can accelerate understanding of disease, improve efficiency of healthcare services and support the discovery, development and evaluation of new medicines. UK health data has exceptional potential to advance these goals, as the NHS routinely collects and stores data on health services, treatments and outcomes across the country.

This document sets out principles that ABPI members will adhere to when analysing and using NHS health data. These will complement the governance processes established by the data custodians.

It is intended that these principles, and our members' commitment to them, will underpin our work to generate and maintain public trust in the research-based biopharmaceutical industry's use of health data for research purposes.

In developing these principles, the ABPI has sought feedback widely through a public consultation and several deep dive interviews with individuals from patient representative groups, researchers from academia, industry and charity sectors, government and the NHS.

These principles are intended to complement the Department for Health and Social Care's Five Guiding Principles as set out in the publication *"Creating the right framework to realise the benefits for patients and the NHS where data underpins innovation"*, as well as adhering to all other legal and regulatory requirements.

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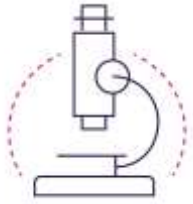
First published in 2022 & re-released as user friendly handbook in September 2023

Where should we focus our efforts next?



Collaborate with key UK stakeholders to build public understanding of the benefits of using health data in research

Influence implementation of data infrastructure and access policies in England to ensure these are fit for purpose to support the global pharmaceutical industry

Work with system partners to increase use of RWD to evaluate early access therapies and for innovative payment models

Partner with NICE to raise awareness of where RWE has been used to inform decisions

Thank you for listening

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