Excellence in the UK Research Ecosystem
At its heart, IQVIA is a company driven by advancing human health. We do this by conducting clinical trials of new medicines across the globe, but also by using breakthroughs in insights, technology and human intelligence to help medical and scientific advances benefit patients.

The UK has historically been one of the best places in the world to conduct clinical research, and IQVIA believes the country can retain this position in a post-Brexit world. IQVIA now has 4 of its prestigious Prime Sites, out of 38 across the world located in the UK, conducting cutting edge clinical trials. The life sciences sector is unquestionably one of the UK’s most important industries with some world-leading strengths, including its university sector; the untapped potential of NHS datasets; strength in early stage and translational research and a leading edge in emerging fields such as genomics.

Whilst the outcome of the 2016 Referendum and subsequent UK Government plans to leave the European Union has created uncertainty about the UK market, it has also been a catalyst to change, bringing the key stakeholders across government, academia and the commercial sector together to focus on strategies to ensure that the UK will not only retain, but grow its world leading position in the life sciences field. There will no doubt be challenges ahead, but by focusing on what the UK does well and by exploring steps that can be taken to make the UK even stronger on the global stage, there is reason to have every confidence that the UK will continue to go from strength to strength as a destination location for life sciences investment. In the post Brexit era, the UK will continue to lead the world in life sciences research through enablement of health data analytics and partnership with globally leading science organisations such as IQVIA. Health Data Research UK (HDR-UK) Health Data Research Hubs will transform the capacity of the UK to curate and analyse the UK’s unique health data ecosystem to enable cutting-edge research for health discoveries and to give patients across the UK faster access to pioneering treatments. Health Data Research Hubs for Cancer (DATA-CAN), the national cancer health data research hub is one of seven health data research hubs announced by HDR-UK in September 2019. IQVIA has collaborated with 5 NHS organisations in DATA-CAN to provide its Oncology Data Network (ODN) a scalable, 48-hour latency non-identified set of cancer clinical phenotype and outcomes data secured- while maintaining strict controls around data privacy and consent from primary clinical systems.

IQVIA is confident about the future of the UK and looks forward to continuing to play its part in this flourishing and unique health ecosystem.
The National Institute for Health Research (NIHR) Clinical Research Network (CRN) provides infrastructure to support Clinical Trials across the NHS in England. A key part of our work is to liaise and collaborate with the leading global life sciences companies to realise the full potential of our research environment.

For many years the UK NHS has provided the test bed for healthcare innovation and research through its globally leading universities. In recent years the NIHR has fostered relationships between academics and life science companies leading to a vibrant clinical trials environment. The 2018/19 NIHR annual statistics show that more than 870,250 participants took part in clinical studies supported by the NIHR CRN in 2018/19, a 20 per cent increase since 2017/18. A huge expansion in the science around cancer with genomics, proteomics, phenomics, and new immunotherapies studies coming through is reflected in a record number of new cancer studies added to the CRN portfolio (408), and a record number of ongoing studies open for recruitment (1167).

Making sure that we provide the very best care for our children is one of the most important things we can do as doctors, nurses and healthcare professionals - and children’s health research CRN speciality saw an 84 per cent increase in the number of participants since 2017/18.

The NHS health data ecosystem is internationally the richest resource for the data revolution which will drive faster diagnosis and personalised treatments for patients. Combining the information from individual health care records with genomic data from the 100k Genomic England cohort is the start of a new age of medical research. Much of this data will be anonymised, but the NIHR is committed to keeping the public fully informed on how it uses data to improve health. Our commitment is to embrace these partnerships whilst keeping the public fully informed.
Executive Summary

This report explores the strengths of the UK as a location for clinical research and makes recommendations as to how the UK can stay ahead of the curve in a fast moving and innovative environment.

Section 1 explores the current attractiveness of the UK as a location for healthcare research. The UK has long been recognised for its strength in early stage clinical research, with world-class universities and a history of cross-border collaborations involving industry and academia. Alongside this, the UK’s rich health data ecosystem, with unique longitudinal datasets, overlaid with the latest advances in genomic testing. There is also growing strength in real world evidence, with the UK leading the way in collecting and using this data for approval and reimbursement of new medicines. On top of this, there is a history of leading academic institutions and supportive governments, willing to work in partnership with industry and researchers to create a supportive environment in which life sciences can flourish.

Section 2 sets out emerging technological areas in which the UK must invest in order to stay ahead of the curve. These include investment in the curation and linkage of datasets, so they can be used for clinical research and real world evidence generation. It also requires investment in and support for emerging technologies, such as genomics and artificial intelligence (AI), which will become increasingly important in a data-driven world. Finally, the Government must continue to take steps to improve the clinical trials environment, making it easier for patients and healthcare professionals to engage in research.

Section 3 includes a range of recommendations as to immediate steps the UK Government could take to ensure it remains a world-leader in the life sciences sector:
Recommendations

1 Ensure that the UK becomes the regulatory and licensing ‘Kitemark’ country in the world, via the Medicines and Healthcare products Regulatory Agency (MHRA) being the first choice regulatory authority for global pharma, biotech and medtech. This would move the UK from being a ‘participatory’ country to being a landmark registration country, running full studies and not a small element of them as is the case today. The ambitious plans of the MHRA to accelerate licensing and start-up in a post-Brexit world must be supported by collaboration between commercial research organisations, industry and government. The MHRA should mainstream Real World Evidence (RWE) in its approvals.

2 Centralise Ethics Approval. A professionally funded full-time organisation that is specifically employed to review commercial research in a timely manner and with appropriate expertise in next wave research – genomics, virtual trials, synthetic studies, adaptive design etc.

3 Promote innovation in clinical research capitalising on the unique research offering in the UK: NHS data, integrated health systems, Genomics England and the UK Biobank. The Genomics England - IQVIA collaboration facilitates genomically-enabled clinical trials, putting the UK ahead of the rest of the world. Potential trial patients can be identified from Genomics England datasets, and for trials with a UK investigator site there is the option of having a whole genome sequence performed on these patients in parallel to the clinical trial.

4 Mainstream clinical trials with GPs and Consultants as a routine option for NHS Patients and encourage both NHS Consultants and GP practices to contribute to primary care research datasets. Digitise GP recruitment of patients in studies to address the limited consultation time with each patient by accelerating the adopting of digital innovations such as IQVIA-EMIS ‘One Click’ studies and introduce electronic pre-screening to improve the ‘pick-up’ rate. Educate the general public about the benefits of clinical trial participation through a patient-public engagement programme.

5 Introduce the ‘Right to Write’ to allow doctors to search clinical records and directly contact patients who might be suitable for a clinical trial relevant to their condition (with ethical approval).

6 Improve the UK record for uptake of innovative medicines by ensuring health economic and societal values are mainstreamed in clinical research protocols and encourage the MHRA to be the conduit between clinical trial and Health Technology Assessment (HTA) approval by an MHRA programme that positively discriminates research protocols that include health economic parameters.

7 Create a national policy for the use of de-identified patient data for research – this should be driven by ‘opt-out’ in the same way is the case for Organ Donation in Wales.

8 Globally promote the Health Data Research UK (HDR-UK) health data research hubs to facilitate health data-enabled research develop a more pragmatic approach to linking datasets across care settings.

9 Create an environment to support AI solutions for large population screenings of undiagnosed disease.

10 Align to EU Clinical Trial Regulation (CTR) and Clinical Trial Portal in event of a no-deal exit from the EU.
1. The Current UK Research Environment

The UK has all the components to make it the best place in the world to conduct healthcare research. It has a history of innovation, world-leading universities and research institutions and a flourishing life sciences industry. It also has the healthcare data of 65 million people in the world’s largest nationalised healthcare system.²

Importantly, the UK also has a history of supportive governments. Over the last few decades all governments have understood that not only does the sector employ 482,000 people and contribute £30bn to GDP,³ but it also makes a wider contribution, in terms of improved population healthcare and a flourishing university sector.

Despite the challenges and uncertainties of Brexit, there are some fundamental strengths of the UK as a destination and location for global life sciences investment:

Healthcare Data

The UK has some of the richest health care and research data assets in the world. The universal health system provides a whole population perspective and the NHS number provides a unique patient ID. There is national coverage of hospital inpatient administrative data from the Hospital Episode Statistics (HES), and a well-developed ecosystem of registries. In primary care there are large scale primary care electronic medical records for research as well as community prescribing and dispensing data. Britain is also leading the world with unique data assets such as those from Genomics England. Some assets are well organised, such as the Clinical Practice Research Database (CPRD) and UK Biobank, although currently only a fraction of NHS and research data is currently accessible at a national level and being used for research and innovation.

The Government has also invested in Local Health and Care Record Exemplars, which aim to ‘raise the bar’ in how the NHS and its partners share information safely and securely to deliver better care. This includes funding of up to £7.5m per exemplar, with matched funding coming from the locality.⁴ The aim is primarily to improve individual care, but local communities will also be able to use this information to support local health and care planning and management, as well as to better understand the health and care needs of their local population.

National Health Data Research Hub for Cancer (DATA-CAN)

DATA-CAN is a new National Cancer Health Data Research Hub, designed to transform the accessibility of high-quality cancer data across the UK. This collaboration is built on rigorous patient privacy and data protection governance, with the goal of improving clinical outcomes for the 500,000 people in the UK diagnosed with cancer every year. Improving outcomes will directly impact half of the UK population who will face a cancer diagnosis at some point in their lifetime and could potentially contribute to saving 30,000 lives a year.

DATA-CAN will link clinical and genomic health data, while supporting universities, NHS hospitals and companies wishing to engage with this health data cancer research hub. This initiative is intended to accelerate oncologic diagnoses and the discovery of new personalised medicines for patients with cancer. The collaboration will also help empower the NHS to benefit from existing data sets to improve care for cancer patients and drive patient enrolment in clinical trials and real world evidence studies.

DATA-CAN will transform the collection, accessibility and analysis of health data to advance the discovery of new diagnostics and personalised medicines for cancer, whilst protecting patient privacy and keeping data safe. Initial data assets encompass disease-specific datasets (for colorectal cancer and paediatric malignancy); linked datasets from primary, secondary, and tertiary care; and IQVIA’s Oncology Data Network, providing real-time clinical analytics and clinical trial matching services. All have been selected as mature programmes with existing ethics approval in place, allowing immediate access.

DATA-CAN aims to support the use of data to deliver benefits to the NHS, its patients and healthcare professionals, improve the UK’s cancer health outcomes, enhance innovation and attract new global investment into UK life sciences. HDR UK’s Health Data Research Hub programme is part of the Government’s Life Sciences Industrial Strategy to make the UK a global leader in health data science.

Data security will be central to the collaboration’s operations and patients will be centrally involved in decisions about how their information might be used. Data will be made available for appropriate use by approved researchers and innovators, and the collaboration will support them by providing expert advice from specialists who understand cancer and health data.
Historically the country has a strong research bedrock. The UK is home to four of the world’s top six universities for research and study of clinical, pre-clinical and health topics (Cambridge, Imperial College London, Oxford and UCL). The life science sector also has a broad geographical base across the country, with biotech clusters and partnerships throughout the UK, including MedCity in the South East, Northern Health Science Alliance (NHSA) in the north of England and Industrial Biotech Innovation Centre (IBioIC) in Scotland, ensuring that every part of the country benefits from the investment and high skilled jobs that the life science sector brings.

**Strong Legal Framework and Employment Practices**

There is also a sophisticated regulatory and intellectual property (IP) protection system, and a commitment to retain regulatory alignment, even from outside of the Single Market and Customs Union. Irrespective of the outcome of Brexit, the UK continues to offer advantages over other countries in Europe, including the language, the ease of recruiting a workforce and excellent transport connections. However, fundamental to the UK’s success is the negotiation of a trade deal between the EU and the UK, which will have a major impact on the strength of the wider economy.

**Collaboration**

The country has a history of collaboration, with the UK as a top five collaboration partner for each of the other 27 EU member states, and a contribution of almost 20 per cent of the total research work carried out within EU health programmes between 2007 and 2016. There has also been extensive participation in the Innovative Medicines Initiative (IMI) – over 90 per cent of IMI projects have involved at least one UK institution. The Government has committed that the UK will continue to be involved in major scientific work in Europe and across the world, including confirmation that it intends to seek an ambitious science and innovation agreement with the EU that will support and promote science and innovation across Europe and beyond. This collaboration is particularly important in some fields - such as precision medicine and rare disease - as it provides access to large and diverse population groups for medical research and clinical trials.

**The UK is one of the world leaders in Real World Evidence in both demand and supply**

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### DATA-CAN will have three areas of primary focus:

1. Enable and expand UK-wide cancer dataset access for research
2. Provide high-quality real world data to support health and care transformation
3. Use real world data to support patient enrolment in clinical trials, improve clinical trial design and reduce delays in clinical trial start-up times

### The DATA-CAN collaboration of NHS bodies, and research organisations includes:

- UCLPartners
- Queens University, Belfast, representing Northern Ireland and Wales
- University of Leeds and Leeds Teaching Hospitals, representing Yorkshire and Humber
- Genomics England
- IQVIA

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**Research Environment**

Enable and expand UK-wide cancer dataset access for research

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The UK is one of the world leaders in Real World Evidence in both demand and supply
Real World Evidence (RWE)

The UK is a global leader in the adoption of RWE into regulatory and health technology assessment (HTA) decision-making. It has influenced global processes through its methodological innovation in assessing new and increasingly complex medicines and devices. Our unique free at the point of care, single-payer healthcare system with many existing healthcare databases and disease registries; and the strong links between the pharmaceutical industry and academia, enables access to the required skills for the collection, analysis and use of real world data.

National Institute for Health Research (NIHR) and Health Data Research UK (HDR UK)

The NIHR and HDR organisations also offer significant advantages for the UK. In 2017/18, 8,359,904 people participated in research supported by the NIHR Clinical Research Network and the Government provided £1bn in funding. For example, the collaboration and additional resource provided by NIHR, enabled researchers on the internationally acclaimed Salford Lung Study to observe ‘real patients’ in a ‘real-setting’. It demonstrates a major advance in the way the UK undertakes clinical trials.

HDR UK is also a positive development in making the UK an excellent place to conduct research. It aims to unite the UK’s health data to make discoveries that improve life by bringing together 22 research institutes from across the country. It has set out the ambition that ‘every health and care interaction and research endeavour will be enhanced by access to large scale data and advanced analytics.’

By uniting health data assets across the UK and making data available to researchers and innovators the HDR aims to better understand disease and discover ways to prevent, treat and cure illness.

Life Sciences Industrial Strategy

The Government has demonstrated its commitment to the life sciences sector with the publication of the Life Sciences Industrial Strategy (LSIS) and two Sector Deals, as well as the appointment of a committed champion, Professor Sir John Bell, Chairman of the Life Sciences Industrial Strategy Board. This has resulted in funding and infrastructure commitments with the aspiration to ‘put the UK in a world-leading position to take advantage of the health technology trends of the next 20 years.’

The LSIS sets out a long-term vision, based on large research infrastructure projects and ‘moonshot programmes’ with the ambition to create two to three entirely new industries within the field over the next decade. There is also continued investment in new initiatives, including £146m for five major projects in the field of advanced therapies, advanced medicines, vaccine development and manufacturing, as well as a further £14m for eleven medical technology research centres to promote collaboration between the NHS and industry. The Government is committed to the Accelerated Access Collaborative (AAC) as the vehicle to implement the recommendations of the Accelerated Access Review (AAR). The AAR aims to develop innovative commissioning routes including steps towards a streamlined approvals system and a £86m investment to support small and medium sized businesses.

The LSIS and Sector Deals are evidence of the Government’s commitment to the sector, outlining investment in the Health Advanced Research Programme (HARP), manufacturing, business scale-up and NHS collaborations. Of note in the first Sector Deal is the aspiration to ‘strengthen the UK’s position as a global centre for clinical research and innovation’. The Sector Deal recognised that:

‘NHS data is a precious resource ... and there remains a significant opportunity to create greatly improved data infrastructure around the UK that has the potential, in the first instance, to improve the quality of care provided to NHS patients, and to support better planning and delivery, allowing NHS managers to run their services more effectively. The benefits of this infrastructure for research activities are also clear, allowing for the development of algorithms to transform clinical services and evaluation of new, innovative medical products in a more systematic way. The size of the UK population, combined with a long-established cradle-to-grave healthcare system, means that the scale of patient data potentially available is unique’.

The Sector Deal recognises the importance of data flowing in a ‘legal, secure and appropriate way ... and wherever possible, for purposes other than direct care, anonymised data would be used’. The first Sector Deal also committed to the establishment of Digital Innovation Hubs.
The Government has recognised the importance of the development of skills in areas where there are gaps, including data scientists, informatics and AI; and a commitment to the training of these individuals, either via apprenticeships or other means. The Government has already invested in skills in the AI sector, with a nationwide programme of industry funded AI Masters courses, coupled with work-based placements. This investment will create up to 200 new AI Masters places at UK universities, and 1,000 PhDs at dedicated UK Research and Innovation AI Centres for Doctoral Training. There will also be \$,UHVHDUFK\$VROYH\$ORZ\$VUHD\$WGLQ\$RO\$DER\$UD\$WLRQ\$ZLWK\$The Alan Turing Institute to both attract and retain the best research talent from around the world.

Announced in September 2019, the UK Government committed £37.5m to develop regional interoperable Health Data Research Hubs which will support the use of data for research purposes within the legal framework and meet the strict parameters for sharing data and the security standards set out by the National Data Guardian. They create controlled environments for real world clinical studies, the application of novel clinical trial methodology, and the comprehensive evaluation of new innovations so that patients can benefit from scientific breakthroughs much faster.

The Health Data Research Hubs programme, part of UK Research and Innovation’s Industrial Strategy Challenge Fund (ISCF) Data to Early Diagnosis and Precision Medicine Challenge is being led by HDR UK, the National Institute for Health Data Science. This programme acts as an accelerator to enable the UK to be at the leading-edge of the growth of the global big data analytics in the healthcare market and will ensure that benefits which are developed through increased use of data are returned to the NHS.

The new hubs will help connect regional health and care data with biomedical data in secure environments. This will pave the way for NHS, academic researchers and industry innovators to harness scientific knowledge and emerging technologies to develop new drugs and devices and improve health services. The Health Data Research Hubs will securely and safely connect data across regions of 3-5 million people and create an accessible layer of data from GP practices, hospitals, social and community care providers, alongside genetic and biomedical information and other datasets for research and innovation.

Combined with the unique research expertise across UK universities and industry, this initiative offers an unprecedented opportunity to use data to improve the long-term health of the public. It has the potential to create new jobs in the UK’s life sciences economy, drive medical innovation and ensure that NHS patients benefit from new treatments first.

This Government commitment to the Life Sciences Sector goes beyond the UK Government, with the Scottish Government also taking steps to attract life sciences investors. Scotland is already a leading global hub for life sciences, employing over 37,000 people across some 700 organisations. Companies in this sector contribute in excess of £4.2bn turnover and about £2bn gross to the Scottish economy. Since 2010, company turnover has increased by 29 per cent, gross value rose by 24 per cent and total employment in companies by 13 per cent.\textsuperscript{13}

NHS Scotland treats a population of around 5m, via a unified healthcare system, and to some of the best integrated data in the world. This data includes:

- The UK’s national prescription/dispensing and hospital imaging datasets.
- Primary care data connections being developed through the national SPIRE programme (Scottish Primary Care Information Resource).
- Multiple disease-related registries.
- A network of tissue bank repositories.

Through NHS Research Scotland, the health service provides a single access point for industry, dedicated >
clinical research facilities and globally competitive approval and business start-up times. It works collaboratively across all health boards to provide a comprehensive and complete service. This national approach results in fast, efficient and reliable support to deliver high-quality clinical trials in Scotland.

NHS Research Scotland also plays a key role in supporting the delivery of high-quality clinical research in genetics, and managing participant recruitment to meet deadlines and targets, both for genetics studies led from Scotland, and studies led from other nations in which Scottish sites are participating.

Innovators within NHS Scotland are also encouraged to work with industry. Key physicians are to be found in NHS Scotland and in the medical departments of universities, adding quality to key clinical opinion leaders.

### Public Engagement and Attitudes to Use of Data

Alongside building the infrastructure, steps must be taken to increase public support for and understanding of research. Currently two thirds of British people say they are willing to allow the NHS to use their healthcare data for medical research.\(^{14}\) However, there remains a lack of public understanding of how patient data is used and there is an appetite from both patients and healthcare providers for greater education. A recent survey suggested that there is a recognition that the use of data (including by commercial partners) offers both the potential for large benefit. Forty-nine per cent of UK adults surveyed would not be comfortable for their medical data to be used to develop algorithms that could improve healthcare, but 40 per cent were comfortable with this, even after it was explained that data security could not be guaranteed.\(^ {15}\) Maintaining and building this number is vital if we are to have a true cross section of British society’s data to work with, particularly when it comes to rare diseases.

However, issues such as care.data damage public support for the use of healthcare data in research and must not be repeated. The Code of Conduct for Data Driven Health and Care Technology\(^ {16}\) is an important first step, but all stakeholders need to be engaged with making sure this is a transparent, easily understood and beneficial experience for patients.

The Chief Medical Officer, Professor Dame Sally Davies called for a ‘social contract’ for medical practice and research in the UK. This would require the NHS to set out a mutually acceptable statement of what expectations patients can reasonably have of the uses of their data. It would also require the setting out of reassurances around data governance, and the protections that will be put in place to ensure that patients are not harmed through the uses of their data and that access to the tools developed through such uses will be equitable.\(^ {17}\)

There is also an important role for National Data Guardians (NDGs) who are responsible for advising and challenging the health and care system to ensure that citizens’ confidential information is safeguarded securely and used properly.

To date, opt out rates for the use of personal healthcare data in research have been low, giving a large dataset from which
Despite this strong history, excellent infrastructure and Government commitment, there are some weaknesses surrounding the UK as a place to conduct healthcare research.

Data sources are fragmented, and not all hospital records are electronic. Despite some initial progress on interoperability, open standards and data access, implementation has been slow, and data sources are often siloed and rarely linked.

Patient access to innovative treatments also remains a challenge in the UK, and despite being the third highest country in the EU for introducing medicines, the uptake of those innovations is the lowest in Europe with only eighteen British patients accessing a new treatment within the first year for every one hundred Germany patients. As a result, standard of care often falls behind comparable European countries, making it harder to place trials in the UK.

In the Life Sciences Industrial Strategy (LSIS) the Government set an ambitious target:

“The UK should be in the top quartile of comparator countries, both for the speed of adoption and the overall uptake of innovative, cost-effective products, to the benefit of all UK patients by the end of 2023.”

Alongside this, the Government and NHSE have committed to an upper quartile target for the five highest health gain categories during the first half of the scheme. NICE has also committed to appraising all new active substances as quickly as possible, and new active substances will be exempt from payments for the first three years of the new Value Pricing and Access Scheme (VPAS). Achieving these goals would benefit not only UK patients, but also make the UK a more attractive location for research investment.

Central government research funding is also low when compared with other countries. The commitment to increase UK spending to 2.4 per cent of gross domestic product by 2027 is relatively modest, particularly in comparison to the 3 per cent EU-wide target set in 2000. It is markedly lower than the 2015 R&D level in Israel, South Korea, Japan, Sweden, Austria, Denmark, Finland, Germany, Belgium and the United States which range from 2.4 to 4.2 per cent.

There have been challenges in conducting some elements of research in the UK. The siloed nature of NHS organisations seemed to have worked against innovation, from trial bureaucracy to speed of uptake. Progress is being made, and the Health Research Authority (HRA) should be commended on the progress it has made in reducing trial approval times.
However, multicentre trials remain a challenge in the NHS, particularly as every Trust must review and agree the local protocol and ethics. Finding eligible patients also remains a highly manual process, and the lack of a French-style national molecular testing facility can make national recruitment to trials utilising biomarkers a challenge.

Clinical Trial Regulation

In a post-Brexit world, it is vital that the UK remains aligned with EU Clinical Trial Regulation. In 2017, there were 823 applications for commercial clinical trials of medicines in UK and it is in the interests of patients, the NHS and the wider economy that this continues. The EU’s new Clinical Trials Regulation (CTR) is expected to be implemented in 2020 and would therefore apply to the UK under the terms of the time limited implementation period. The Clinical Trials Regulation allows for a streamlined application process, harmonised assessment procedure, single portal for all EU clinical trials and simplified reporting procedures, including for multi-Member State trials. The UK was involved in developing the new regulation that has been widely welcomed by Europe’s research sector, including academia, medical research charities and industry.

However, if there are delays in the implementation of the CTR - so that it happens after 2020 - it could have an impact on the UK’s ability to participate, particularly in the shared central IT portal and the single assessment model, both of which would require a negotiated UK/EU agreement regarding UK involvement following the end of the implementation period.

In addition, it is vital that restrictions to trade do not impact on clinical trials. Both the UK and EU must ensure that trade barriers do not impact the availability or movement of investigational medicinal products (IMPs), clinical trial supplies and medicines after the UK leaves the EU. Currently 70 per cent of all IMPs in ongoing EU trials are quality released from the UK. It is important to make sure that patients who are participating in trials – both in the UK and within the EU - at the time of Brexit are able to continue to access their trial medicines.
The UK is already a world leader in healthcare research (see Section 1), but it is important the UK continues to evolve and develop, particularly in the emerging fields of genomics, artificial intelligence and machine learning. Doing so will ensure the UK remains an attractive location for investment, which benefits not only the UK economy, but more importantly, patients.

**Data can Provide a Public Good**

The use of de-identifiable patient data in clinical research has several benefits for patients, for the NHS and for the wider UK economy.

**Benefits to the patient:** Greater use of data interrogation and AI algorithms should allow more patients to be identified as likely to benefit from a clinical trial. By enabling patients to access trials they are likely to have better health outcomes, and the data generated may help contribute to future healthcare advances. Patients on trials are sometimes asked to manage some of their own data input, through apps and other devices, and evidence suggests that patients who are engaged in their care have better outcomes. In addition, more widespread use of data can contribute to the development of guidelines and diagnostic algorithms, as well as safety and effectiveness for patient sub-types, which in turn will benefit individual patients.

**Benefits to the NHS:** Good data evaluation can be used to assess the effectiveness of decisions, drive evidence-based outcomes improvements and service transformation, with the ultimate aim of designing cost effective services, which in turn could lead to financial savings. The NHS also benefits from patients being on clinical trials; as the medicine is received free during the trial period, and clinicians are given early familiarisation with the latest treatment options.

**Benefits to the UK:** The Life Sciences sector is already a significant contributor to the UK economy, generating £64bn of turnover and employing more than 233,000 scientists and staff. By becoming a world leader in healthcare data science, particularly real world evidence, the UK will attract investment in clinical research, enhancing its reputation as a great place to conduct healthcare research. In addition, promoting the research environment will benefit the wider economy, as research creates jobs, which in turn generate taxes.

**Different Types of Data: RWE v RWD v RWI**

It is increasingly clear that Real World Data (RWD) will become a key element of healthcare decision making. In some cases, this is mandatory for UK patients to access new medicines, such as with the Cancer Drugs Fund (CDF), which requires manufacturers to collect two years of RWD.

However, there remains some confusion about the definition of RWD and its sub-types. For clarity and ease of communication, it is important this terminology is used correctly.

**Real World Data (RWD):** RWD is all patient level data not collected in conventional randomised controlled trials. It is normally collected through electronic medical records, disease registries, chart reviews etc.

**Real World Insights (RWI):** RWI refers to using RWD to generate insights about what is happening in real-life healthcare. It must involve the appropriate scientific and/or commercial analytics to be credible. It is not only de-identified, but statistically anonymous.

**Real World Evidence (RWE):** RWE takes it one step further. It is the subset of real world insights developed with the intention to support a claim or belief to produce evidence for multiple stakeholders, including regulators, payers, providers and patients. Learnings from this can be published.

**De-identified Data:** Patient level data with identifiers removed so that patients cannot be easily identified. De-identification has many levels to it, with the deepest under GDPR being ‘WP-29 anonymous’.
One of the best ways to achieve speed diagnosis and to engaging patients accurately is to combine existing real world data sources in order to enable more sophisticated research than is possible with a single discrete source.

Infographic: Types of Real World Data (RWD)

Real World Data can be used throughout the care pathway:

Research and Development: RWD can be used in R&D, particularly around clinical trials. It can support the identification of patients for research, as well as target discovery and validation. Increasingly HTA bodies, such as NICE, are requesting RWD as part of the approval process, such as the UK Cancer Drugs Fund requirement to collect two years of RWD as a prerequisite for approval and payment. Overall, this data is financed by those who are studying it, normally the manufacturer or academic organisation overseeing the trial. It tends to be purchased on a ‘fee for service’ rather than a ‘fee for data’ basis.

Care System Redesign: RWD can also be used to support care system redesign by facilitating data driven evaluation of care systems to drive evidence-based outcomes improvements and service transformation. Primarily used to improve patient care by reducing costs and improving care pathway efficiency or to assess commissioning decisions, the data collection and analysis is normally funded from discretionary hospital or payer budgets, although there are increasing examples where biopharmaceutical partners contribute to the budget.

Direct Patient Care: RWD can also be used to directly support patient care, primarily by monitoring individual patient care, normally via ‘care system apps’ which are either purchased by the end user or commissioned by the health system and given to the user. Such RWE collection can assist in prevention, early detection, diagnosis and treatment.

RWE has the potential to offer significant benefits, but it is a rapidly evolving arena, which some stakeholders are struggling to fully understand and adopt. In 2017 only 12 per cent of initial submissions to NICE that included RWE received a negative recommendation compared with 24 per cent for those without.27 In the UK, the share of NICE submissions that included RWE has steadily increased from 9 per cent in 2015 to 22 per cent in 2016 and 37 per cent in 2017.28 Amongst healthcare stakeholders, 48 per cent believe that RWE is highly likely or very highly likely to improve the development of clinical guidelines.29
Achieving the LSIS Goal of the UK being in the upper quartile of the OECD average for uptake of innovative medicines by 2023 using RWE

For payers and regulators, RWE allows them to understand clinical value when there is limited traditional clinical trial evidence available, either because the product is on an accelerated or early approval track in an area of high unmet need, or there is an inherently small population, or because it is in single arm trials. Alongside Phase III clinical data, it allows regulators and payers to access a broader range of data points. For healthcare professionals RWE can support clinical decision-making by allowing for the segmentation of patient populations, using basic patient characteristics, clinical markers, biomarkers or genomic markers. RWE has an invaluable role in reducing uncertainty and guiding clinical decision-making.

Rare Diseases

Using data to identify and target patients is particularly beneficial for patients with rare diseases, or rare variations of common diseases, where clinicians do not see many cases, and where time to diagnosis can be long. On average, the length of time from symptom onset to an accurate diagnosis is around 4.8 years for a rare disease, and patients see more than seven 7.3 physicians before a diagnosis is made.31 It is estimated that there are 30 million people in Europe who have a rare disease, of which 50 per cent are children,32 and currently only five per cent have an approved drug treatment.33 In the UK it is seven per cent of the population, or 1 in 17 people.34

Despite this, companies are heavily investing in research for rare conditions, and in recent years the number of medicines approved for orphan conditions has outstripped that of non-orphan conditions.

In the US and EU more orphan drugs are being approved than non-orphan drugs35

<table>
<thead>
<tr>
<th>Year</th>
<th>FDA</th>
<th>European Medicines Agency</th>
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<tbody>
<tr>
<td>2014</td>
<td>40</td>
<td>58% Non-Orphan, 42% Orphan</td>
</tr>
<tr>
<td>2015</td>
<td>39</td>
<td>46% Non-Orphan, 54% Orphan</td>
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<td>27</td>
<td>41% Non-Orphan, 59% Orphan</td>
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<td>46% Non-Orphan, 54% Orphan</td>
</tr>
<tr>
<td>2018</td>
<td>42</td>
<td>48% Non-Orphan, 52% Orphan</td>
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FDA & EMA novel drug approvals are now predominantly Orphan Medicines

Source: European Medicine Agency, FDA
One of the challenges of orphan medicines is evaluating their effectiveness and cost-effectiveness, as there is often less data than with traditional new chemical entities. Payer expectations of what constitutes ‘unique’ is also changing, and the bar for health technology assessment (HTA) approval is being raised in many countries. This has had the impact of fewer medicines being recommended outright, and more being given a ‘conditional’ or ‘restricted’ approval.

**Case Study: Finding Rare Patients**

Idiopathic cardiovascular disease is a rare condition. The symptoms are non-specific and there is poor awareness of the disease outside of specialist centres. Patients have very high levels of activity pre-diagnosis: on average a patient has 25 hospital events in the three years prior to diagnosis. It can therefore be challenging to identify patients early and they are often diagnosed too late in the course of a disease.

IQVIA utilised the Hospital Episode Statistics database to build a data-driven AI algorithm to enable better detection of these hard-to-find patients. This utilised nearly 700 individual prioritised clinical codes to define patients’ medical history from EMR data. The AI algorithm was applied to the frequency, timings and number of unique events defined by these codes to predict patients earlier in the disease course. As a result of the success of the algorithm, a follow-on pilot has been established to explore the deployment of a clinical support tool at clinical sites.

This case study demonstrates it is possible to use AI analysis of medical history data to reveal undiagnosed patients and detect patients earlier in the course of a disease.

**Case Study: Birmingham Rare Disease Centre**

IQVIA is committed to delivering paediatric and rare disease studies. The aspiration is to direct more patients to fewer, high performing sites while working closely with investigators. Understanding these diseases however requires a non-traditional and creative approach integrating different stakeholders to build robust and effective solutions. Since 2014 this approach has delivered 364 Phase I-IV studies, with more than 175,000 patients in 101 countries.

An exemplar of this way of working is the Birmingham Rare Disease Centre where the IQVIA team is embedded in the study team as a value-added resource. They work alongside the study team and investigating clinicians and provide expert input at all parts of the process, from protocol writing to database lock. Not only does this enhance the skills of the onsite team, it also ensures iterative improvements to improve the experience and outcomes for the patients, their carers and the clinicians involved.
Patient Registries

Patient Registries are one of the best sources of data on the existing patient pools, particularly for rare diseases. The UK has a strong history of registry data, with cancer registries dating to the 1950s, and tertiary referral centres and disease specific registries held at a range of sites. Patient Registries in rare diseases are evolving – but there are a very limited number of international registries that exist due to limited funding. In a recent study covering the European Union, 747 rare disease registries were counted, of which 69 per cent were national, 10 per cent regional, 8 per cent pan-European, and 13 per cent were global. This needs to change; as rare diseases have very few patients and local or individual country efforts to recruit patients for trials are slow and can fail because of the very small numbers of patients in the pool. The pool can be reduced further by the inclusion/exclusion criteria for trials or other barriers.

Rare disease registries are founded with a range of aims, including better understanding of the natural history and outcomes of the disease (by identifying patients and following them over time), connecting patients, healthcare professionals and caregivers, and supporting research on the genetic, molecular and physiological basis of the disease. Lastly, very importantly for orphan medicines companies, Patient Registries establish a patient basis for evaluating new medicines. An example of the breadth of data that a well-established patient registry can provide is that of the Cystic Fibrosis Foundation Registry Program in the US. This registry was initiated in the 1960s and currently has active records of 29,000 patients, or 84 per cent of all CF patients in the US. In the UK, the equivalent registry for Cystic Fibrosis (CF) holds the data of more than 99 per cent of all CF patients in the UK.

Patient Registries have huge potential to improve the development of therapies for rare diseases, and this progress will be enabled by new technologies which make it easier to aggregate the clinically rich type of de-identifiable patient data that is required to drive more effective clinical trial design, and also the development of better ways to identify patients for treatment.

Case Study: Advanced Analytics in Clinical Trial Recruitment

For clinical trials in rare diseases, patient recruitment is often very difficult. The combination of small numbers of geographically dispersed patients, few - if any - dedicated treatment facilities, and limited expert healthcare professionals means identification of centres and clinical trial candidates is challenging. Better use of healthcare system data, via advanced analytics, can speed up and improve the accuracy of site selection and patient recruitment.

To overcome these barriers, IQVIA leveraged existing data sources in a novel approach to boost patient enrolment in a trial for a rare form of blindness. The challenge was finding US specialists who were currently treating patients, a minority of all the specialists who could potentially be involved. Using physician registry data, specialists who were particularly engaged with the therapy area, could be identified, but this did not provide insight into their recent patient activity.

To find the physicians with both the specialism and current patients, IQVIA used prescription and claims data to identify those who had seen at least one patient within the last twelve months, limiting the database to just ten per cent of physicians. Through this data-driven referral support, 19 per cent of all contacted physicians agreed to refer patients to the investigator site, with 29 patients referred. Ultimately, this produced 19 patients enrolled to the study, equating to 41 per cent of the total patients enrolled.

This US based study would currently not be possible in the UK due to the inability to search the data sources effectively and efficiently.
Improving Clinical Trials

Clinical trials are a costly element of medicine research; however, recruitment of sites and enrolment of patients can be very inefficient. Researchers estimate that 48 per cent of all clinical trial sites miss their enrolment targets and 11 per cent of investigators never enrol a single patient. Non-enrolling investigators and under-performing sites are a primary driver of increased trial cost and timelines.

On average, 80 per cent of clinical trials are delayed, mostly due to recruitment issues. Trials are a global endeavour, and the UK needs to be competitive. When sites do not enrol patients according to plan, protocol amendments often follow, which cost on average $500,000 USD and add two months to the timeline, which can add up to millions of dollars in delayed revenue and absolute losses of opportunity in sub-optimal sequence to market and positioning. This has a detrimental impact on patients, for whom access to new innovative medicines is delayed. Improving the efficiency of trials, and targeting the right centres and patients is therefore essential for both speeding development times and reducing costs and ensuring swift patient access to new medicines. Ways of doing this include working with a Prime Site, which are proven clinical research networks, as well as using data interrogation and artificial intelligence to target centres and identify patients.

Engaged Clinicians, Engaged Patients

Better engagement with healthcare professionals and patients boosts trial recruitment. In Europe and the US, physicians refer less than 0.2 per cent of their patients onto clinical trials, and nurses an even lower percentage. The most common reasons given for low patient recruitment were lack of information about trials, time and no follow up from the investigative site staff, with nearly 30 per cent of physicians and 45 per cent of nurses reporting that they never received initial or follow-up contact from investigative staff following a referral, and a higher proportion never receiving their patient’s clinical results.

This substantial missed opportunity could be addressed with better tracking throughout the clinical process and better engagement with the right healthcare professionals. It can also be supported with initiatives to engage patients. The trend toward more engaged patients, enabled by social media, also makes highly targeted direct-to-patient recruitment possible, speeding recruitment with more committed patients. Alongside the ability for researchers to use medical records and clinicians’ ‘Right to Write’ this could have a transformational impact on clinical trial recruitment.

There is also a challenge in retaining patient commitment to a trial. Patient, carer and patient group involvement can help design a trial that is clinically relevant to patients, ensuring that the study is feasible as well as fulfilling regulatory requirements. This means understanding how to avoid the trial having too great an impact on school, work and family life, by, for example, minimising visits and interventions and reducing travel for patients. All of this while continuing to collect the optimal level of data. Digital technologies enabling remote data capture will become increasingly useful.

Patients and patient groups can also be good partners to recruit participants. Websites, mobile apps and social media can also help patients find the studies and sites and see how they can be involved. Patient groups can also help to educate study sites and investigators about the disease – for example, on the patient pathway and the most effective way to communicate with patients on the possibility of joining trials, and to continue engagement throughout the trial.

In the EU, since 2016, 24 European Reference Networks (ERNs) have been established, each around a group of similar disorders. The 24 ERNs involve over 900 specialist units in over 300 hospitals across 26 countries.

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Case Study: Better Clinical Trial Site Selection in Idiopathic Pulmonary Fibrosis

Good data understanding and access to datasets allow data to be used to improve research and development.

Idiopathic Pulmonary Fibrosis is a rare condition in which the lungs become scarred and breathing becomes increasingly difficult. Identifying suitable sites for a clinical trial is challenging, due to the rare nature of the illness. By using data-driven insights and data interrogation it was possible to map the high concentrations of patients, and target centres nearby. As a result of this tailored approach there was a 20 per cent improvement in site engagement with the study, rising from 47.6 per cent using traditional methods of site identification to 57.1 per cent with this data driven approach.

As a result, more patients with this rare condition, many from deprived areas, were able to access this study, benefiting their own health and potentially providing insight into treatment options.
companies bringing orphan medicines to the European markets needs to understand and engage with these international organisations. They also need to understand how they might evolve. The UK has been historically involved in 23 out of 24 of the ERNs and led six of them. As a preparation for Brexit, the European Commission asked UK hospitals leading ERNs to step down, and future involvement of the UK is currently uncertain. The UK’s participation will continue to evolve as the Brexit process is finalised.

This is not only relevant for Phase I-III trials but could be particularly beneficial in the context of real world evidence trials, where patient engagement, technology and cross-country collaboration are particularly beneficial in recruiting and retaining patients.

**Case Study: We’ve Got Your Back**

One way of engaging patients in research is through an Enriched Observational Study, which combines Primary Data Collection (in this case patient reported outcomes) with a secondary dataset, (in this case EMR). The National Institute for Health Research (NIHR) is undertaking a study on back pain management, called “We’ve Got Your Back” which aimed to assess the quality of life over time of patients suffering from back pain. Patient recruitment and data collection was done via an innovative app (OneCLICK), prescribed by GPs.

Patient characteristics, medical history and treatment was taken directly from the EMR dataset, and combined with patient reported outcomes on humanistic burden and loss of work productivity reported directly by patients via an app.

**Genomics as a Unique Differentiator**

Alongside traditional clinical datasets, such as primary care or hospital inpatient data, the UK is building a reputation as one of the best places in the world to explore emerging technologies such as genomics. It is the first country in the world to sequence 100,000 genomes and has an aspiration to reach 5 million.

Genomics has the potential to transform patient outcomes. By mapping a person’s DNA and comparing it to a database of DNA maps it is possible to identify the genes that causes disease facilitating future diagnoses and enabling the design of more targeted treatments. Genomics is a rapidly evolving branch of science. In 2003 scientists sequenced the first human genome, and in December 2018 Genomics England announced they had achieved their ambition of mapping 100,000 genomes in just four years, with a new goal of five million genomes mapped in the next five years. Technology is moving so quickly that while it once took thirteen years and £2billion to sequence a genome, it now takes two days and costs £1000.46

This is a revolution in medical science. Doctors have always tried to personalise treatment, but they have had to match symptoms with available treatments, in the full knowledge the patient is likely to experience side effects. Genomics will allow us to predict who is likely to develop a disease and how an individual will respond to treatments.

**Genomics England**

Genomics England was announced by former Health Secretary, the Rt. Hon Jeremy Hunt MP during the 65th anniversary of the NHS celebrations in July 2013. Its goal was to deliver the 100,000 Genome Project, which aimed to sequence 100,000 whole genomes from NHS patients with rare diseases and common cancers. Genomics England is the central coordinator of whole genome sequencing in the UK. It oversees sample and clinical data collection at Genomic Medicine Centres, storage, extraction, sequencing, interpretation and reporting.

**Objectives of Genomics England:**

- To bring benefit to patients.
- To create an ethical and transparent programme based on consent.
- To enable new scientific discovery and medical insights.
- To kick-start the development of a UK genomics industry.46

In December 2018 Genomics England announced it had achieved its objective of sequencing 100,000 genomes.

In October 2018 the new Secretary of State for Health and Social Care the Rt. Hon Matt Hancock MP announced an expanded role for Genomics England with new goals for UK genomics: >
• Expansion of the 100,000 Genomes Project to see 1 million whole genomes sequenced by the NHS and UK Biobank in five years.

• From 2019, the NHS will offer whole genome analysis for all seriously ill children with a suspected genetic disorder, including those with cancer. The NHS will also offer the same for all adults suffering from certain rare diseases or hard to treat cancers.

• Sequence five million genomes in the UK, within an unprecedented five-year period.

According to the second LSIS Sector Deal the Government commitment has already generated significant interest and potential new investment to the UK with several companies and charitable organisations expressing an interest in partnering on these projects. 47

In addition, Genomics England has announced it will undertake detailed development work on a new service to enable genomic volunteers to pay for a personalised report on their unique genetic makeup. This anonymised data will then be made available to researchers and scientists.

Genomics England is funded by the NIHR and NHS England. The Wellcome Trust, Cancer Research UK and the Medical Research Council have also funded research and infrastructure for the 100,000 Genomes Project.

The UK has a world leading genomics dataset from Genomics England, Genomics Scotland and UK Biobank which can be used to stratify patients, improve drug development, identify biomarkers and enable researcher access to samples. Together, this will result in better healthcare outcomes for patients through:

**Precision Trials:** A molecule for the treatment of pancreatic cancer has been granted US FDA Orphan Drug Designation and is currently being trialed in patients as a result of Genomics England’s provision of genomic sequencing and longitudinal follow up for UK study patients. This has not only speeded up the molecule’s progression through testing, but reduced trial costs by between 30 and 50 per cent due to better targeting of patients.

**Patient Diagnosis:** In very rare conditions, it can be hard to identify patients. Genomic data can help clinical researchers and the NHS identify patients in order that the health service can offer them treatment and gather further data for use in research.

**Collaboration:** Collaboration with Genomics England also enables a safe environment for research, with secure data documentation, tools analysis and robust workflow and collaboration tools to allow integrated working from multiple sites and locations.

**Genomics England and IQVIA Collaboration**

In response to the need for robust, future proofed systems to manage genomic data, link it to clinical data and make it available for research, Genomics England and IQVIA have recently announced a collaboration to advance healthcare through the appropriate use of clinical-genomic data. By using IQVIA data management tools combined with Genomics England datasets, the ambition is to enable faster and more efficient research, which in turn should lead to more treatment options for patients. This collaboration has three strands:

**Feasibility Research:** This enables partners (primarily pharmaceutical and biotech companies, but also research organisations and not-for-profits) to access and interrogate Genomics England’s de-identified clinical and genomic datasets using IQVIA’s E360™ Platform. This will allow them to quickly assess the usability of the data for research projects and run leading-edge analytics on patient and disease traits.

**Analytical Consultancy Services:** When a specific research question requires further interrogation and analysis of the data, IQVIA, with support from Genomics England, will carry out analysis for customers for specific subsets of Genomics England’s clinical and genomics datasets. This could include:

• Burden of illness
• Treatment pathways studies
• Comparative safety/efficacy studies
• Drug discovery
• Drug Target Identification
It is responsible for 15 per cent of cases of childhood end-stage renal failure – with no treatment currently available to prevent it\(^4\). Using *HQRPLFV(QJODQG·VGDWDVHW$OH[LRQKDVLGHQWLÀHG undisgnosed patients, recruited as part of the 100,000 Genomes Project’s rare disease programme, who carry WKHJHQHGHOHWLRQFDXVLQJWKHGLVHDVH7KHVHÀQGLQJV have been shared with Genomics England and fed back WRWKHSDWLHQWV·1+6FOLQLFDOWHDPV7KLVLVDQH[FLWLQJÀUVW step in identifying the cause of the illness, which aims to lead to further research and treatment.

CLN2 disease is a very rare inherited disorder caused by mutations in the TPP1 gene and one of a group of OLIHOLPLWLQJFRQGLWLRQVFDOOHG%DWWHQ'LVHDVH7KHÀUVW symptoms strike after the age of 2, with symptoms typically emerging in children between the ages of 2 and 4. CLN2 is one of a group of disorders collectively known as Batten Disease and can lead to seizures, muscle twitches, vision loss, intellectual disability and behavioural problems.\(^4\) Around 30 to 50 children live with the condition in the UK, and life expectancy is around 10 years. Currently, there is no cure or life-extending treatment for CLN2.

Biopharmaceutical company, BioMarin, is another Discovery Forum member focused on rare disease SDWLHQWV,WKDVLGHQWLÀHGRQHSDWLHQWUHFUXLWHGLQWRWKH 100,000 Genomes Project for a condition unrelated to CLN2, but who carries two pathogenic mutations of the TPP1 gene. Moving forward, BioMarin intends to engage the UK’s National Institute for Health and Care Excellence (NICE) and NHS England on the use of its cerliponase alfa treatment in the NHS. Ultimately, it is hoped that work from BioMarin and others, supported by evidence from the Genomics England database, will bring clinicians more options in the treatment of CLN2.

**Case Study: Genomics England Discovery Forum**

As members of the Genomics England Discovery Forum companies have access to selected, de-identified whole genomes and clinical data from the Genomics England dataset. In return, all discoveries must be shared with Genomics England’s science team as well as all the other Forum members, guaranteeing the quickest possible developments for patients. As a result, two forum members - Alexion and BioMarin - have identified previously undiagnosed patients with severe conditions.

Nephronophthisis (NPHP) is a childhood genetic disorder affecting primarily the kidneys. It is rare (around 1 in 60,000 births) and very serious, usually resulting in kidney failure by the age of 15. It is responsible for 15 per cent of cases of childhood end-stage renal failure – with no treatment currently available to prevent it.\(^4\) Using Genomics England’s dataset, Alexion has identified 10 undiagnosed patients, recruited as part of the 100,000 Genomes Project’s rare disease programme, who carry the gene deletion causing the disease. These findings have been shared with Genomics England and fed back to the patients’ NHS clinical teams. This is an exciting first step in identifying the cause of the illness, which aims to lead to further research and treatment.

CLN2 disease is a very rare inherited disorder caused by mutations in the TPP1 gene and one of a group of life limiting conditions called Batten Disease. The first symptoms strike after the age of 2, with symptoms typically emerging in children between the ages of 2 and 4. CLN2 is one of a group of disorders collectively known as Batten Disease and can lead to seizures, muscle twitches, vision loss, intellectual disability and behavioural problems.\(^4\) Around 30 to 50 children live with the condition in the UK, and life expectancy is around 10 years. Currently, there is no cure or life-extending treatment for CLN2.\(^5\)

Biopharmaceutical company, BioMarin, is another Discovery Forum member focused on rare disease patients. It has identified one patient recruited into the 100,000 Genomes Project for a condition unrelated to CLN2, but who carries two pathogenic mutations of the TPP1 gene. Moving forward, BioMarin intends to engage the UK’s National Institute for Health and Care Excellence (NICE) and NHS England on the use of its cerliponase alfa treatment in the NHS. Ultimately, it is hoped that work from BioMarin and others, supported by evidence from the Genomics England database, will bring clinicians more options in the treatment of CLN2.

**AI and Machine Learning**

UK healthcare datasets are world leading. Without a way to manipulate and learn from them however their value is limited. Combining them to provide RWE provides good insight. But consideration must also be made to how artificial intelligence and machine learning can be used to rapidly search and interpret datasets.
What is meant by AI and Machine Learning?

In its broadest terms, AI is using ‘smart’ algorithms to carry out tasks, such as object and audio recognition, learning and problem solving. Machine Learning is a subset of AI and is the use of statistical methods for predictive analysis. It is extracting information from data to determine patterns and predict future outcomes. It is focused on prediction at the observational level as opposed to the group level.

There are already some data-driven innovations in this field, including apps and clinical decision support tools using intelligent algorithms. AI and Machine Learning have the potential to improve diagnosis, treatment, system efficacy and patient outcomes across the health system.

As with RWE, AI and machine learning can be used throughout the care pathway:

**Direct Patient Care:** AI can be used for diagnosis and clinical decision support by the NHS.

**Care System Redesign:** AI can also support care system redesign by optimising management of healthcare services, for example minimising readmissions and ‘do not attends’.

**Research and Development:** AI can be used in knowledge discovery, such as phenotype identification, or in identifying suitable patients for trials.

Using AI and machine learning is not without its challenges and its detractors, and concerns have been raised about how some ‘intelligent algorithms’ will be used. For this reason, NHS England and the Department of Health and Social Care developed the Code of Conduct for Data Driven Health and Care Technology which sets out ten principles for safe, ethical and effective data-driven health and care technologies.

This world leading Code of Conduct provides a clear and transparent framework in which companies can operate and should enable the sector to flourish and the UK to lead the world in this rapidly evolving field. The code will provide:

- Clarification of what is expected from suppliers of data-driven technologies, and what the government can do to support innovators, such as trusted approval systems and coherent pathways for suppliers to enter the market.
- The basis for ongoing engagement and conversation on how we should use new technology to provide better and more sustainable services in partnership with academia, industry, NHS, patients, clinicians and the wider public.
- The basis for stakeholders to enter into commercial terms in which the benefits of the partnerships between technology companies and health and care providers are shared fairly.

**Case Study: Machine Learning in Prospective Patient Identification**

Better use of existing healthcare system data - combined with advanced analytics - is a powerful tool when an Orphan Medicine has been approved and market access granted.

The next challenge for companies is to generate the optimum level of uptake. To do this, there is again the two-fold challenge of finding physicians and finding patients.

For an ultra-rare neuromuscular disorder in the US, characterised by under-diagnosis due to slow disease progression and complex presentation, there were very few known patients according to claims data. In addition, standard prescription data sources were not useful in identifying the physicians who might treat patients. IQVIA used the data that was available on existing patients to teach a machine learning algorithm to identify potential at-risk patients. When this algorithm was run on the wider set of all claims data, which contained about 1,500 pre-identified disease sufferers, it identified about 4,700 patients who were highly likely to be undiagnosed sufferers of the same condition. It was then possible to link these at-risk patients to physicians to provide an alert to review those patients as potential sufferers of the disorder as well as an ongoing service, providing new potential patient alerts as they emerged from new claims data.
Case Study: Hospital Optimising Benchmarking Solution

Hospital Optimising Benchmarking (HOB) is a financial benchmarking solution in UK NHS hospitals. There are currently over thirty hospitals in the HOB, with the aspiration to expand to more than fifty within a year.

Benchmarking is used by hospitals to compare their financial and operational activity performance with peers across the country to identify and share clinical and financial best practice with other likeminded NHS organisations. HOB can improve efficiency and reduce costs.

HOB uses IQVIA standard solutions, such as data transfer and active directory framework to provide analytic dashboards in areas including medicines, procedures, costs and theatre usage. For example, in Maidstone and Tunbridge Wells NHS Trust Ophthalmology Department the following changes have been made, resulting in efficiencies:

- Introducing 8:30am start
- Reviewed space utilisation
- Increased junior doctors per clinic
- Transferred out patient activity from low-volume sites
- Moved Saturday cataract lists to weekdays
- Increased throughput
- Reduced overheads by more than 10 per cent
- Increased productivity
- Minimised fixed costs
- Reduced waiting list initiative payments

Health Optimisation Benchmarking can also be used to reduce medicine costs and realise savings. The variation in clinical prescribing for the same or similar treatments or conditions accounts for millions of pounds of potential savings opportunities across the NHS each year.

Whilst currently only used for financial benchmarking, it would be feasible to extend the system to the identification of patients for study recruitment.

Big Data

It is already possible to combine local datasets from primary (CCGs) and secondary (NHS trusts) care to obtain a sub-national view of healthcare services, demand and outcomes. Excellent coding data also allows analysis of the costs of the care pathway, including the costs of sub-optimal care. This information can be used to highlight and address inefficiencies. It is also possible to combine this information with other sources, such as demographic data to forecast capacity and demand to enable effective system planning. However, doing this at speed and scale on a routine basis is not yet standard practice.
Case Study: Cancer Vanguard Partnership

Real world insights from existing clinical datasets can be used to design and refine care systems that better meet patients’ needs. Under the Cancer Vanguard Partnership, the NHS, IQVIA and pharmaceutical company Merck identified variation in drug usage, treatment costs, pathway variations and outcomes in metastatic colorectal cancer as well as the impact this had on patient experience during a nine-month project.

By collecting relevant data, it has helped share an evidence based and patient-centric approach to service redesign and medicines resource optimisation. One novel aspect of the study involved asking patients to record their symptoms, such as mobility and energy levels – using innovative digital technology. Patients highlighted a decrease in quality of life within the first two months, driven by increases in severity of anxiety and depression.

In total the project collected 111,000 data points over a 35-week project. The Vanguard discovered there were 13,000 patient pathways in the treatment of colorectal care, with a seven-fold difference in the proportional use of biological therapies versus chemotherapy. This demonstrated a significant deviation from the NICE recommendation on the use of biological therapies.

The project team developed a knowledge discovery platform which highlighted the patient experience and the variation in care and supports multi-disciplinary teams in redesigning existing pathways to replicate best practice and to better adhere to the NICE guidance.

Case Study: Using Data to Investigate the Long-Term Impact of Influenza

Working in partnership with Sanofi Pasteur, IQVIA used national HES data to investigate the long-term hospital impact in influenza cohorts. To calculate this, it was necessary to understand the full hospital pathway for the more than 44,000 patients diagnosed with influenza. A comparator cohort of non-influenza patients was also created.

Across all influenza groups, total patient journey costs increased by approximately 50 per cent when patients were infected with influenza. Both the influenza and control groups were split by comorbidity profiles into diabetes, cardiovascular and respiratory cohorts. A statistically significant difference was observed across all three risk groups, with an average cost increase of £8,260 per influenza versus control group patient.

Over the last five years, influenza infection increased the burden of care in terms of bed days, readmission rates and total care events per patient. In total, over the last five years these 44,000 patients have been associated with over £510m in tariff spend, and over 58 per cent of this spend was associated with those with Chronic Heart Disease.

Using this information, it may be possible to re-engineer pathways, or target patients who are at high risk of influenza and have other comorbidities for the influenza vaccine. It also allows hospitals to forecast likely bed requirements and readmission rates for patients with influenza, helping with winter capacity planning.
In Section 1 we outlined the current attractiveness of the UK as a location for healthcare research and in Section 2 we set out emerging technological areas in which the UK must invest in order to stay ahead of the curve.

In this Section we make recommendations as to immediate steps the UK Government could take to ensure the UK remains a world-leader in life sciences.

1. Promote clinical trials as a routine option in care. It is vital that patients, irrespective of their location and medical history, know that clinical trials are a possible care option. Steps have been taken by the NHS and by independent organisations such as PatientsLikeMe to encourage patients to ask about trials, but all clinicians should be encouraged to talk to their patients about trials. This is particularly true for primary care where less than one percent of practices are active in commercial research compared to 99 per cent of Hospital Trusts.

2. Introduce the ‘Right to Write’. Currently in England, doctors are not permitted to proactively contact a patient who is not under their direct care. By introducing the so called ‘Right to Write’ provision, it would allow doctors to search clinical records and directly contact patients who might be suitable for an available trial. This would significantly increase the pool of patients included in research. This could be challenging and may require primary legislation, but the bigger barrier maybe overcoming GP reluctance to share the patient data which they ‘own’. There is also currently no appetite from the NHS to make this change.

3. Facilitate local health data research hubs. There are several developing initiatives in the UK to integrate common data areas, including HDR UK’s seven Health Data Research Hubs, Local Health and Care Record Exemplar programmes and Prime Sites. The aspiration must be for an integrated, seamless, data enabled research environment combining primary and secondary care datasets to enable feasibility for patient screening. Ultimately, the goal should be to support continuous patient research through data capture at point of event (A&E, Primary, Secondary, etc.,) to ensure patient safety, research integrity, and reduced patient burden. For this to happen there will need to be education of front-line healthcare professionals as well as data custodians that ownership and management of this activity would sit within the health service, erasing the misconception of loss of patient control. It is also vital that the patient actively consents to participation in research and subsequent use of their data – ethically approved digital screening could enable that choice.

4. Encourage and incentivise all GP practices to contribute to primary care research datasets. Given the role of GPs as the first point of contact with the health system, there is currently relatively low representation of the UK population within the research datasets, less than ten per cent. As a result, it can be difficult to move beyond the national level with sufficient statistical power to understand variations in regional health environments. There can also be a lower data quality in important diseases and data fields due to the time constraints on GP practices for the administration of data. GPs are now being incentivised via the Quality and Outcomes Framework (QOF), which has increased the reporting of information for the key diseases identified in such datasets with a clear benefit for the validity of research. The UK Government has announced funding to further develop CPRD; enabling secondary care data flows from hospitals to support specific real world clinical and patient-consented studies in combination with CPRD primary care data. This is likely to achieve even greater efficiencies in delivering real world clinical trials across the health care spectrum. The aspiration should be to establish a near-census level of primary care data to reflect the comprehensive and national nature of Hospital Episode Statistics in the hospital setting. Expanding the principles, incentives and outputs of the QOF framework could drive the value of primary care EMR in healthcare management and research as well as encourage data suppliers to provide more open licensing of the dataset for both academic and commercially funded research.

5. Introduce a national molecular pathology service to identify patients for trials. Most new cancer innovation requires a molecular test to identify patients who will benefit as well as knowledge of their previous therapy. Access to such molecular testing is a postcode lottery, with even cost-saving innovation like KRAS testing not reaching most patients. This is reflected in other areas of cancer care, and in regional mortality variation. Currently, in the UK there is no centralised uniform molecular testing facility making recruitment to trails utilising biomarkers a challenge. In contrast, systems
like the one established in France have uniform national testing explicitly designed to facilitate late stage trials whereby patients have access to national pathology test results within 24 hours via Institut National du Cancer (INCa).

6. Link oncology clinical trials with the NCRAS real world dataset. Improving cancer outcomes remains a high priority for the Government and the NHS. Advances in genomics and biomarkers makes clinical decision making increasingly complex. Increasingly segmented patient populations in cancer, and accelerated access pathways for cancer drugs mean that there is a need for more evidence to support use of innovative treatments. IQVIA UK has developed a strong collaboration with the National Cancer Registry and Analysis Service (NCRAS). This has enabled an understanding of UK cancer patient treatments at a census level and help advance research into new therapies. NCRAS currently operates on an ‘opt-out’ model of participation, ensuring high levels of representation and information-rich data at a national scale. Cancer research would benefit greatly from a combined view of both in-depth clinical information from oncology clinical trials and long-term longitudinal follow up of cancer patients in NCRAS. This would benefit patients, reduce inefficiencies in the management of cancer, help identify patients that would benefit from innovative therapies and assess their subsequent care journey. It would also make the UK a more attractive place to conduct trials than in countries where long term secondary monitoring is not possible. This also follows parallel principles with the Salford Lung Study, linking pragmatic clinical trials information with long term community EMR data. This could be done via a unique patient identifier to ‘follow’ the ongoing routine care in NCRAS of any patient who has participated in a clinical trial. The challenge lies in linkage, and in incorporating the right opt-out mechanism for patients during the informed consent process which takes place at clinical trial enrolment.

7. Implement Generation Genome. Implement in full the recommendations of Dame Sally Davies’ Report Generation Genome. There has been significant progress, including a national network of genomics services and making data accessible – but this valuable report should be revisited, and its recommendations reviewed.

8. Improve the connection between clinical care and R&D. Too often, research and development and patient care teams work in silos, particularly when it comes to new innovations such as AI and machine learning. Innovators in this field often come from sectors that are not necessarily familiar with medical ethics and research regulation and who may use datasets and processing methods that sit outside existing NHS safeguards. It is vital to enhance the connection between clinical care and R&D teams, so that R&D is considered a routine part of clinical care and the two are seamlessly integrated.

9. Create an environment able to support AI solutions for large population screening of undiagnosed disease. In many care settings, lack of accurate and timely diagnosis is a substantial challenge, particularly in oncology and rare diseases. In some cases, this is hindered by poor awareness of the disease in more general care settings or clinical manifestation that can either be non-specific, atypical or masked by co-morbidities. This significantly impacts patients whereby an appropriate care plan is either completely absent or the diagnosis is established too late in the disease stage for treatment to be effective. Screening for undiagnosed diseases powered by AI could accelerate time to diagnosis by identifying patients with high risk of undiagnosed diseases through application of AI to existing large-scale RWD. By identifying patients in earlier stages of disease progression it would provide greater opportunity for good treatment outcomes. This would benefit patients by providing a targeted referral to the relevant specialist to expedite diagnosis and treatment with an ambition to reduce healthcare utilisation when a patient is symptomatic but undiagnosed. Moreover, specialists receiving referrals are more likely to receive high risk, relevant patients whose care can be managed in their practice. This would obviously have significant data requirements, as well as require engaged clinicians, willing to assist with scaling across the population.

10. Implement the Clinical Trial Regulation (CTR). In a post-Brexit world, it is vital that the UK remains aligned with the EU CTR. The EU’s new CTR is expected to be implemented during 2020 and would therefore apply to the UK under the terms of the time-limited implementation period. The CTR allows for a streamlined application process, harmonised assessment procedure, single portal for all EU clinical trials and simplified
reporting procedures, including for multi-Member State trials. However, if there are delays in the implementation of the CTR - so that it happens after 2020 - it could have an impact on the UK’s ability to participate, particularly in the shared central IT portal and the single assessment model, both of which would require a negotiated UK/EU agreement regarding UK involvement following the end of the implementation period. Without this, the risk is that the UK could be excluded from participating in some clinical trials without additional bureaucracy, which will damage the UK’s attractiveness as a location for investment and could result in fewer UK patients being able to access trials and therefore innovative medicines.

11. Ensure MHRA alliance with the EMA. UK patients must not be at a disadvantage when it comes to accessing new medicines post-Brexit. The MHRA must align itself with EMA decisions in order that UK patients do not have slower access to new medicines. In addition, the MHRA could look to position itself ahead of the EMA and become a reference regulator to accelerate approval and give early direction to pharmaceutical companies.

12. Consult on and introduce clinical research performance metrics. Currently there are no standardised performance metrics to measure the success of large multicenter randomised trials. Individual manufacturers and Clinical Research Organisations (CROs) use their own measures to assess the performance of a study, but there are no comparable metrics globally. A standardised set of clear and accessible summaries of site performance could facilitate the timely identification and resolution of potential problems, as well as assessing the comparable performance of sites and countries. In order to address this issue, the UK Government should consult on and introduce clinical research performance metrics aligned with global standards to improve clinical trial success rate and predictability.

13. Develop a more pragmatic approach to linking datasets across care settings. Care of patients is becoming more complex. In order to deliver on long term sustainability objectives, the NHS needs to fully understand current treatment paradigms, care inequities, variation, and inefficiencies, and adopt an intelligent, data-insight-driven approach to redesign and optimise existing systems. Currently, vast amounts of clinically impactful data is collected across the NHS. However, transparency and connectivity between settings represents a significant barrier in identifying whole system inefficiencies and quantifying the real world impact of variation, which ultimately leads to inequities of care and poorer patient outcomes in all settings of care. NHS datasets all feature NHS numbers as a common factor, which can facilitate a linkage across the diversity of data. Currently this is linked in an ad-hoc manner by NHS Digital, resulting in significant time periods and delays in requesting data, governance and using relevant date periods. For example, there is a longitudinal linked dataset produced from NHS Business Services Authority on reimbursed pharmacy prescribing data compared with HES. This information is being developed as part of the Medication Safety - Indicators Specification reports featured in ePACT2, a primary care prescribing dataset. Granting access to these types of existing datasets would quickly enable exploration of the true impact of polypharmacy, medication adherence, best practices and subnational variation; all aligned to core NHS focuses of improving sustainability.

14. Establish a framework of standards for data analysis using RWD-equivalent to Good Clinical Practice for research. Compared to European peers, the UK is behind the curve on key ‘analogue to digital’ clinical data, such as molecular information (vs. France and Belgium) and routine clinical outcomes (vs. Belgium, Netherlands and Germany), partially as a result of NHS fragmentation. Additionally, the UK is behind Germany in information science privacy norms and whereas Germany will be driving to sophisticated privacy norms suitable for precision medicine, the UK is still lagging behind. Likewise, physical protection privacy norms vary across the NHS estate, and between user groups, in contravention to GDPR. The goal must be to define pragmatic outcomes for cancer and other key disease to be rolled out into routine practice as part of national care quality and the drive to value based care, and away from activity based key performance indicators, and ensure these outcomes are interoperable with key international research partners.

15. Greater public transparency from National Data Guardians (NDG) on which research programmes adhere to the highest standards of data regulation. Currently, the UK NDG provides guidance to companies in private as to whether their health data schemes are compliant.
However, this information cannot be shared with NHS Trusts, who as a result are uncertain about which innovations and partnerships are most likely to maximise patient benefit while maintaining data use standards. The Office for National Statistics will not allow commercial access to death information, which is essential for real world research and understanding mortality rates across disease areas and geographies. Finally, different NHS bodies have different approaches on the technical definition of anonymity in health data making research data linkage in mid-common disease more challenging in the UK than continental countries. For this to be overcome the following progress needs to be made:

- NDG should emulate the French National Commission on Informatics and Liberty (CNIL) and provide public transparency on which health schemes are GDPR compliant;
- ONS should allow commercial research access to death information, as the most important outcome for medical research;
- HDR-UK should define new standards for research;
- Development of some archetypical deals so that industry ‘knows where it stands’.
Caldicott Principles were developed in 1997 following a review of how patient information was handled across the NHS. The Review Panel was chaired by Dame Fiona Caldicott and it set out six Principles that organisations should follow to ensure that information that can identify a patient is protected and only used when it is appropriate to do so. Since then, when deciding whether they needed to use information that would identify an individual, an organisation should use the Principles as a test. The Principles were extended to adult social care records in 2000.

The Code of Conduct for Data Driven Health and Care Technology sets out the behaviours expected from those developing, deploying and using data-driven technologies, whilst tackling some of the emerging ethical challenges around using this data.

Digital Innovation Hubs aim to bring together the NHS, academia, the public and industry to establish a robust health data research infrastructure, with the ambition to improve the clinical care of individual patients and health of the population, as well as enabling the UK to become a world leader in health data-led fields including genomics, ‘real world trials’ and diagnostics.

Health Data Research is the national institute for health data science, responsible for uniting health data to enhance research.

Local Health and Care Record Exemplars aim to create an information sharing environment between local healthcare providers.

‘Moonshot’ programmes derives from the Apollo 11 spaceflight project, which landed the first human on the moon in 1969. Such projects or programmes are intended to be ambitious, exploratory and ground-breaking without any expectation of near-term profitability or benefit and also, perhaps, without a full investigation of potential risks and benefits.

National Data Guardians advise and challenge the health and care system to help ensure that citizens’ confidential information is safeguarded securely and used properly.

Non-identified patient level data has been subject to a process that removes the association between a set of identifiable data and the patient so that there is no reasonable basis to believe that the information can be used to identify an individual.

Prime Sites connect Clinical Research Organisations with IQVIA to support the delivery of medical innovation and research with the overall goal of driving better health outcomes, access and cost. Prime Sites offer ‘best-in-class’ performance, joint governance, wide therapeutic capability and a collaborative approach to running clinical trials.

Real World Data (RWD) is all patient level data not collected in conventional randomised controlled trials. It is normally collected through electronic medical records, disease registries, chart reviews etc.

Real World Evidence (RWE) is Real World Insights developed with the intention to support a claim or belief to a produce evidence for multiple stakeholders, including regulators, payers, providers and patients. Learnings from this can be published.

Real World Insights (RWI) is using RWD to generate insights about what is happening in real-life healthcare. It must involve the appropriate scientific and/or commercial analytics to be credible. It is not only deidentified, but statistically anonymous.
About Public Policy Projects

Public Policy Projects (PPP) has a 20-year history of delivering policy advisory in the health, care, life sciences and local government sectors. PPP, chaired by the Rt. Hon Stephen Dorrell, offers practical policy analysis and development. PPP has hosted speakers including Rt Hon Matt Hancock MP, Rt Hon Jeremy Hunt MP, Andrew Gwynne MP, Simon Stevens, Lord Carter, Professor Dame Sally Davies and many other senior thought leaders. The network consists of senior leaders across the health, care, life sciences and local government sectors. PPP also advises on policy development internationally. The parent company of PPP is Dorson West Ltd.

About IQVIA

IQVIA is a leading global provider of advanced analytics, technology solutions and research services to the life sciences industry dedicated to delivering unique and actionable insights. In the UK, IQVIA serve pharmaceutical and biotech companies and most of the NHS Trusts to drive innovations and improve patient outcomes, all whilst protecting patient privacy. Formed through the merger of IMS Health and Quintiles, IQVIA is the UK’s 4th largest employer supporting life sciences industry, with a team of 4,000 spread across the UK, including 270 real world data scientists, and is the largest life sciences employer in Scotland with research facilities and over 900 employees. Key UK Locations include London, Reading, Edinburgh and Brighton. IQVIA conducts over 20% of all commercial clinical trials in the NHS with 5,500 patients enrolled in active trials, as well as four ‘Prime Sites’ covering a population of around 20 million people in Scotland, UCL Partners London, the South West Peninsular and Northern England.

Included in the 2018 Life Science Sector Deal and as part of IQVIA’s commitment to UK Life Sciences, the Northern Prime Site is formed through a collaboration with the National Institute for Health Research (NIHR) and NHS hospitals across the region including the Christie NHS Foundation Trust, Manchester, St James University Hospital, Leeds and Sheffield University Hospitals. Through this Prime Site, IQVIA will be able to place more interventional and observational clinical trials across all therapeutic areas and clinical indications, providing the 8 million citizens of the Manchester and Yorkshire regions with enhanced access to clinical research opportunities and data insights that will improve diagnostic, treatment outcomes and human health.

The Sector Deal also included IQVIA’s collaboration with Genomics England to enable faster and more efficient drug research, more robust evidence to support treatment value, and greater access to personalised medicines. To do so, IQVIA and Genomics England are co-developing a platform that connects clinical and de-identified genomics data to accelerate treatment advancements for NHS patients, a critical step in advancing genomic research.

About National Institute for Health and Research

The National Institute for Health Research (NIHR) is the nation’s largest funder of health and care research. It was established in 2006 to improve the health and wealth of the nation through research and is funded by the Department of Health and Social Care. The NIHR:

- Attracts, trains and supports the best researchers to tackle the complex health and care challenges of the future
- Invests in world-class infrastructure and a skilled delivery workforce to translate discoveries into improved treatments and services
- Partners with other public funders, charities and industry to maximise the value of research to patients and the economy
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