REFORMER THOUGHTS

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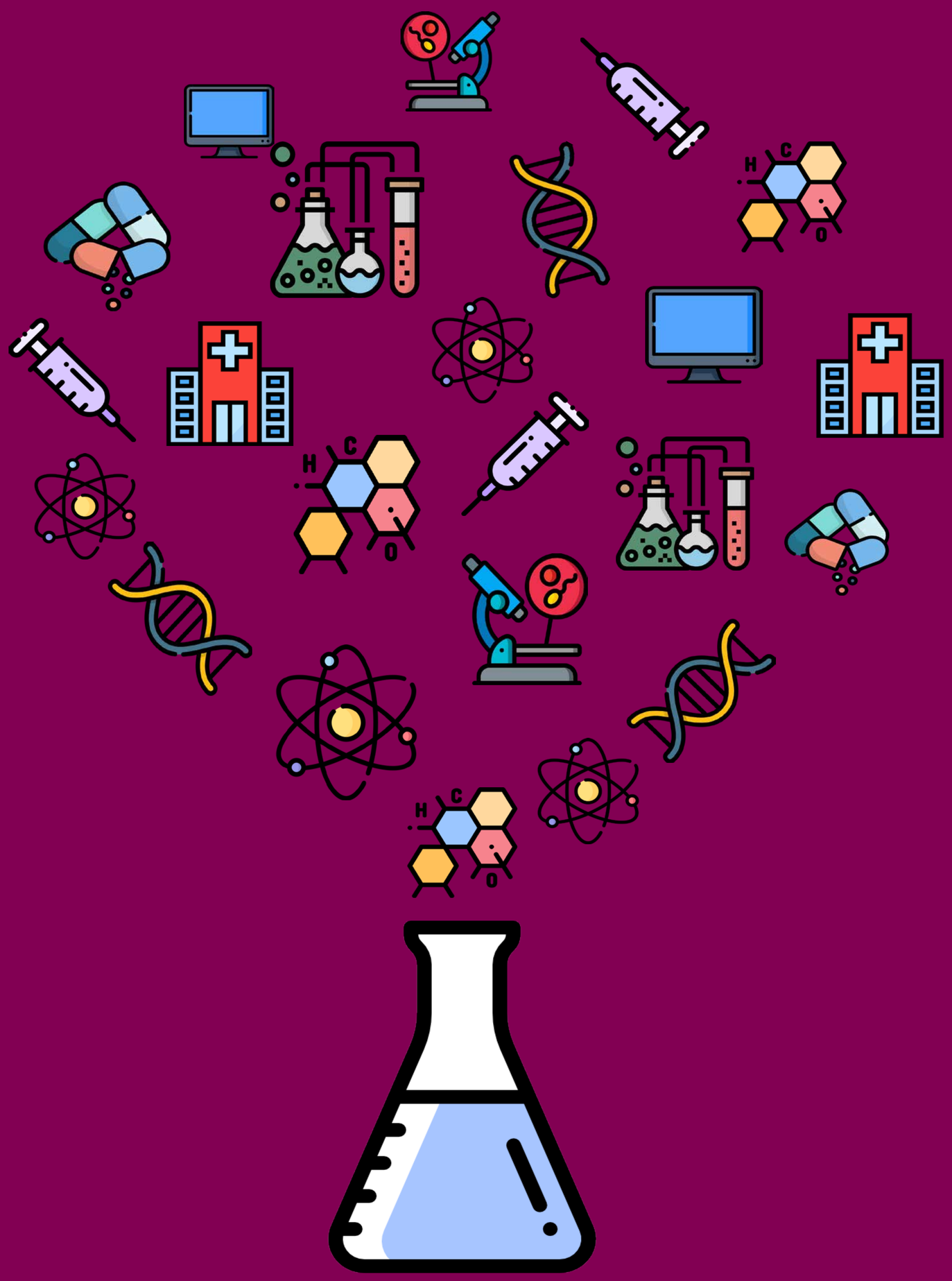

Driving innovation and long-term growth in the UK's life sciences sector

MSD
INVENTING FOR LIFE

About *Reformer Thoughts*

Reformer Thoughts brings together the opinions of leading experts from academia, business and government; frontline practitioners and public service users, to provide readers with valuable insight into the challenges shaping the policy debate. The series aims to give a platform to innovative ideas and facilitate an open and informed conversation about how we can improve public services.

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About *Reform*

Reform is established as the leading Westminster think tank for public service reform. We are dedicated to achieving better and smarter public services. Our mission is to set out ideas that will improve public services for all and deliver value for money.

We work on core sectors such as health and social care, education, home affairs and justice, and work and pensions. Our work also covers issues that cut across these sectors, including public service design and delivery and digital public services.

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Contents

Introduction

Re-imagining the future of the UK's life sciences industry

The UK is a recognised world-leader in life sciences. Supported by a rich ecosystem of world-class universities, charities, tech start-ups, and global healthcare companies, the UK life sciences sector is hailed as a 'jewel in the crown' and vital to boosting the country's productivity and growth. To retain and increase its leadership in this sector, the UK must continue to build on its reputation but also adapt to the ever-changing global life sciences market.

The Life Sciences Industrial Strategy, published in 2017, set out a bold vision to position the UK as a global hub for life sciences. The Life Sciences Sector Deals and Grand Challenges offer further practical action to help realise this vision, including commitments to increase R&D expenditure from 1.7% to 2.4% of GDP by 2027, develop high-quality skills and jobs, and radically boost investment in medical and clinical infrastructure.

Three years on, considerable progress has been made. Flagship initiatives such as the Accelerated Access Collaborative are now in place to make it easier for patients to access innovative medicines, and to support their adoption within the NHS. Progress is ongoing on genomics research with the recently announced National Genomic Healthcare Strategy setting out fresh commitments to support patients living with rare diseases through faster diagnostics and personalised care. New pricing and reimbursement models are being developed to incentivise investment in new drugs by ensuring that pharmaceutical companies receive a fair return on their investments. A stream of commercial investments in medical infrastructure have been made, including MSD's state-of-the-art Discovery Centre in London. Regional life sciences 'clusters' have been created to increase productivity and spread technological innovation across the country.

The life sciences sector has also become a critical part of the UK's economy, employing a quarter of a million people and generating over £70 billion in revenue every year.

Looking forward, the UK faces the important challenge of remaining competitive in the fast-evolving global life sciences market. Advances in artificial intelligence and machine learning are transforming the sector's approach to drug development and discovery, making it more efficient and cost-effective. The increased adoption of data-driven technologies, coupled with the ability to harness the power of rich genomic, molecular and behavioural data, are generating opportunities for better understanding disease and delivering personalised drugs and treatments. The UK's withdrawal from the European Union is set to reshape the current regulatory landscape for drugs and medical devices, as well as the industry's approach to acquiring talent and investing in R&D.

This *Reformer Thoughts* series brings together experts from Government, the research community and the life sciences industry to discuss the steps the UK must take to prepare for the future and remain competitive in the global life sciences market.

Claudia Martinez

Research Manager, *Reform*

"The UK faces the important challenge of remaining competitive in the fast-evolving global life sciences market"

Sustaining long-term investment in R&D

The Medical Research Council (MRC) funds research with the goal of improving human health. Innovative discoveries can have considerable economic impact – indeed the charity LifeArc, formed to translate the work of MRC research scientists, recently monetised over $1 billion in royalties from the cancer immunotherapy drug Keytruda, developed by Nobel Laureate Sir Greg Winter. Greg appreciated the importance of commercialising his discoveries early on and has described overhearing a seed investor say of him "OK, let's see how the boffin trots!"

What are the opportunities and challenges for fostering an integrated and collaborative R&D ecosystem in the life sciences? The foundation for success must be to fund outstanding research, while keeping an open mind about its potential applications.

For example, few would have predicted that research on the CRISPR/Cas genes of the bacterial immune system would provide a transformative approach to correcting genes in human diseases. The greatest threat to funding the best research is a lack of money. When the number of good grant applications far outweighs the money available, there is a tendency for science panels to fund safe science over risky science. So we need to fund the best research and have enough money to take risks within the portfolio.

It goes without saying that a vibrant R&D system requires investment in people. We have seen a heartening increase in the number of women entering biomedical research in the past 20 years, although more must be done to retain them from mid-career stages. But we need to go further, to be inclusive of all ethnic, cultural and socio-economic backgrounds, and to welcome talent from around the world. We sometimes forget the benefits of strong R&D to the local economy, creating demands for housing and leisure facilities as well as jobs. In Dundee, as just one example, a high proportion of the workforce contributes to the University in one way or another, and the community's pride in local R&D is evident.

But funding the best science and scientists is not sufficient to foster innovation. Too often scientists are so focused on their own research that they miss opportunities to exploit the findings in the clinic and business. I believe that the best way to overcome this hurdle is to encourage an entrepreneurial/translational mindset early in a research career. Fortunately many current PhD schemes provide flexible funds for PhD students to explore other sectors.

For example, one of my own students has just returned from a six-month secondment at M Ventures in Amsterdam. This experience has not only transformed her attitude to research but has also had a strong impact on her fellow students, who have been keen to hear about her experience and inspired to try out secondments for themselves. Another key intervention is to create more flexible opportunities for the clinical workforce to participate in research, for example via the new MRC Clinical Academic Research Partnerships scheme, since realising patient benefits depends on an early appreciation of the practicalities of implementation.

And finally, R&D takes time. When we read about breakthroughs in the press we don't always realise the years of effort and frequent failures that have laid the foundations for those successes. This is highlighted in a recent independent evaluation of 10 years of two of the MRC's translational funding schemes. The future is bright for R&D in the life sciences, provided that we don't forget about the foundations for success.

Professor Fiona Watt

Executive Chair, Medical Research Council

"Funding the best science and scientists is not sufficient to foster innovation"

Realising the promise of precision medicine

The delivery of healthcare is based on making the best decision possible with the available information. Traditionally, this plays out as: patient presents with symptoms, symptoms are most commonly associated with condition X, therapy Y is usually most effective and hence the first course of treatment. This makes healthcare a case of playing the odds.

Precision medicine uses new technologies to improve those odds and give us a more detailed look at the individual case. The intent is to make it easier to get the right drug to the right patient and avoid costly or harmful effects of a more generalist approach. The promise of precision medicine is improved healthcare outcomes for the patient and a more efficient model of delivery for the healthcare provider.

The UK has an incredibly rich history in developing the technologies that drove the human genome project and the move toward precision medicine. For a small island nation, we have an exceptional network of research institutes, innovative tech start-ups, big pharma, forward thinking regulators, a joined-up healthcare system, and a supportive government. You will not find that anywhere else in the world – and that is me speaking as a Canadian.

At Genomics England we sit at the nexus point between healthcare and research. As we help clinicians make diagnoses, we link genomic data with real world and clinical data to enable actionable research. Some of these researcher-led investigations directly unearth fresh diagnoses in our dataset. As these are returned to clinicians, it creates a symbiotic relationship between healthcare delivery and research.

That relationship creates opportunities: the richness of linked datasets opens up much more powerful research questions, makes it possible to quickly find patients for clinical trials, and it could support a step-change in industry's approach to developing precision medicine from drug discovery through to post-marketing.

We are working with industry to better understand and realise those pathways. In doing so it is helping to define what precision medicine really is and the benefit it can deliver to public health.

Understanding disease at a molecular level gives valuable insights that can inform preventative measures, as well as more powerful treatment programmes. Being able to better define cohorts and track their progression is exceptionally useful. It can help to improve the efficacy and safety of a drug, and our understanding of its potential impact. This is important in ensuring that patients have access to the best possible treatments at a fair price.

Working with industry is a vital part of our mission. We also recognise our responsibility to help lay the groundwork to enable precision medicine responsibly. This means we advise on relevant policy, work with regulators, and engage the public. Importantly, this is also a global endeavour. We collaborate with other genomic initiatives around the world and are active within the Pistoia Alliance and the Global Alliance for Genomics and Health.

Professor Joanne Hackett

Chief Commercial Officer, Genomics England

"Understanding disease at a molecular level gives valuable insights that can inform preventative measures"

There is a tremendous amount of work, flow of ideas, and genuine innovation across this sector. In an effort to not 'over hype' to the public, unfortunately, much of this goes unnoticed. Much of the forward movement is taking place in industry, which is often poorly understood.

This is a shame, as there is reason for pride and excitement in the leading role the UK is playing in advancing precision medicine.

Realising precision medicine starts with research. Understanding humans and their diseases at a molecular level is the critical step in everything else that follows: the diagnostics and therapies, and generating the evidence base that regulators and health technology assessors need to make them available.

Real-world evidence: from rhetoric to reality

Real-world evidence (RWE) refers to evidence generated outside the world of clinical trials. It includes a wealth of information generated through on-the-ground clinical practice, including analysing data stored in electronic patient records, disease registries, prescribing and payment information. Effective and more extensive use of these new sources of data holds great potential to support better decision-making in healthcare.

At NICE, our ambition is to increase the use of RWE and focus on opportunities to broaden the types of data available to us. RWE can help us gain insight into the potential use of new technologies in the NHS, using large, high-quality patient databases to provide better context to understand the results of clinical trials. It enables monitoring of the adoption and impact of our guidance by analysing general practice, prescribing and hospital databases. Also, RWE is critical for updating our guidelines by reviewing emerging evidence from health databases.

The managed access approach is a good example of the opportunities and challenges RWE presents. Managed access is a time-limited agreement between a company, NICE, NHS England and NHS Improvement, patient groups and clinicians. It enables patients to have earlier access to promising technologies which show potential to satisfy criteria for routine commissioning but have significant clinical uncertainties. Further systematic real-world data collection can help address these uncertainties, provide an indication of how patients would benefit, and reduce the risk of investing in a technology that could be ineffective.

In 2018, the managed access approach enabled the UK to pioneer a new cancer treatment, as it became one of the first countries in Europe to treat patients using CAR T-cell therapies. This treatment involves taking some of the patient's own white blood cells and re-engineering them in a laboratory so they can recognise and attack cancer cells before being infused back into the patient. The rapid introduction of this technology was possible due to a managed access agreement, including additional RWE generation within the Cancer Drugs Fund.

Over 40,000 patients have benefited from this treatment due to the access agreement, while further data is collected to address the clinical uncertainties that were identified during the appraisal. Five schemes which took place under the 'Commissioning through Evaluation' programme and two drugs in the Cancer Drugs Fund have been routinely commissioned following a period of managed access which we facilitated.

This is not to say that RWE does not present challenges. There are significant logistical issues associated with sourcing data, such as accessing existing sources or developing bespoke data collections. Both take time to set up and can have an impact on clinical workloads. Additionally, challenges exist with how RWE is used and whether it reduces uncertainty by confirming previous results or increases it by providing contradictory information.

NICE is building capacity, skills and infrastructure and exploring opportunities to incorporate data science, analytics and visualisation techniques into our processes and methods. This will enable us to work with greater insight about factors that are impacting across the health system and to respond with more tailored solutions.

Use of RWE is not only our reality but, through our managed access programme, is now becoming the health system's reality.

Brad Groves

Associate Director, Managed Access, National Institute for Health and Care Excellence (NICE)

"RWE is critical for updating our guidelines by reviewing emerging evidence from health databases"

Accelerating the pace of change in life sciences

Policymakers around the world are grappling with the health trilemma: how to adopt the latest advanced therapies, grow a domestic life science industry, and avoid bankrupting their health systems.

The NHS has been proactive at seeking to bring advanced cell and gene therapies onto the market, often with eye-watering price tags. Naturally, patient groups are excited about these classes of drugs, but they pose a huge challenge for the NHS's already stretched budgets - a challenge that will only grow as our population ages.

As well as the patient benefits of bringing new therapies into the NHS, there are wider economic benefits in the UK being seen as a country that accelerates effective therapies into frontline care. We have an enviable record in discovering new drugs, enjoy world-leading research universities, and a strong tech sector. But the NHS has always bargained robustly on pricing, drug adoption is slow by international standards, and we've lacked the patient capital needed to grow our own biotech firms to scale.

On a 30-year view, healthtech and life sciences must be leading UK industries, and this requires all parts of government – including the NHS – to see themselves as partners, not just customers. This is what the Life Science Industrial Strategy aims to do but there is much further to go.

So how can we reconcile these competing imperatives: the desire to bring new cures into the NHS without allowing the drugs budget to spiral out of control, and at the same time providing the financial incentives needed to encourage bio and healthtech companies to grow here? Here are four areas of action the government should pursue.

First, there is an urgent need for the NHS to see itself as an R&D organisation. As the UK seeks to grow the portion of the economy devoted to R&D from 1.7% to 2.4% then 3.0%, the NHS should be the prime location for this growth. That means carving more clinician time out for research, putting Chief Innovation Officers into every trust, and rapidly increasing the budget for the National Institute for Health Research which funds such bench-to-bedside science.

Second, we need to use the UK's exit from the European Union to create a radically more dynamic regulatory system. The UK has benefited from having the best agencies (National Institute for Care Excellence and Medicines and Healthcare products Regulatory Agency) within the European Medicines Agency (EMA) family - giving us access to a population of 500 million people. Depending on trade talks, this is an advantage we will most likely lose. At the same time, the EMA is slow moving and cautious – freed from its strictures we can move much more quickly.

That means creating new paths to licensing for digital health products, including self-learning algorithms; integrating the work of our two regulators into a single pathway; and properly funding the promised Innovative Drugs Fund so that all potentially transformative medicines (and not just drugs) can be swiftly accessed by, and evaluated within, the NHS.

Third, we need to fill the funding gap in the drug R&D value chain. The UK has a habit of doubling down on its strengths and ignoring its weaknesses, such as downstream incentives for scale-up and adoption.

Lord James O'Shaughnessy

Conservative peer, former Parliamentary Under Secretary of State, Department of Health & Social Care

"There is an urgent need for the NHS to see itself as an R&D organisation"

We could solve this structural weakness by using funds repatriated from the European Investment Bank to seed a series of new bio and healthtech investment funds in the UK.

We should also better incentivise the use of approved innovative therapies by NHS clinicians, and give patients who have run out of treatment options ethical access to more experimental therapies.

Finally, we need to leverage the strength of our health data assets. As a single-payer, cradle-to-grave health system, the UK has, in theory, the ability to compile the best health and care dataset in the world. This can bring tremendous benefits: to the quality of direct care, to the operational efficiency of the NHS, and to research. The value of this data is estimated at up to £10 billion per annum, and we are already seeing major innovations taking place in the UK. The Novartis-NHS-Oxford consortium led by Professor Martin Landray, for example, is utterly disrupting the standard approach to clinical trials and reducing by 90 per cent the cost of bringing a cardio-vascular drug to market.

Turning these one-offs into a true system of innovation requires billions of investment into the digital infrastructure of the NHS and a new cadre of clinical data scientists. Most of all it will need the deep involvement of the public in setting the vision and parameters of this strategy.
We finally have a government with a large enough majority to make the bold moves needed to solve the UK's health trilemma. It's in everyone's interests, but most of all patients, that they do so.


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Piloting and collaborating on new approaches to improve patients' health

As a company whose mission is to discover, develop, and provide innovative products and services that save and improve lives around the world, we work with health systems and partners globally to ensure the delivery of our life changing medicines and vaccines to the patients and populations that need them most.

Since partnering with UK scientists almost 75 years ago on the world's first double-blind, placebo-controlled clinical trial, we have been and remain, committed to the UK's leading medical research ecosystem.

The quality of the research and the collaborative opportunities in the UK have led us to recently confirm our commitment by making a significant investment to establish a new state-of-the-art scientific discovery centre in London. Our initial focus is on one of the most significant challenges for modern society: diseases of ageing, which includes Alzheimer's and Parkinson's Diseases. To pursue these targets, we are recruiting a team of 150 scientists, who will work alongside scientists at the Francis Crick Institute, as we build this research partnership and establish our new London premises.

Modern day research is fundamentally a collaborative and international endeavour, and we are aligned with the wider UK research community in calling for the Government's commitments on R&D intensity, skills, networks and funding to be delivered in full, so the UK can continue to be a world leading environment for life sciences. Alongside this, the NHS must be a champion for high-quality research, able to deliver top-tier high quality care and access to new, innovative medicines for UK patients – so the real-life, positive impact of leading life sciences research will be felt by the citizens of the UK.

MSD has and continues to work with the NHS to pilot new projects and seek to find joint solutions to some of the most pressing health challenges. Our work in Hepatitis C, Anti-Microbial Resistance (AMR) and oncology are examples we're particularly proud of.

As a delivery partner in the Hep C elimination strategy announced by NHS England in January 2018, MSD provides so much more than just our product. We provide point of care testing, informatics tools to identify patients, and through the Hep C Trust we're committed to providing peer-to-peer patient support. In AMR, MSD is one of the few global companies who remain committed to researching and developing new antibiotics and partnering to tackle the global challenge.

As part of our commitment, we're proud to be playing a leading role in the UK's pioneering work to support a sustainable reimbursement model for new antibiotics alongside proactive antimicrobial stewardship. In oncology, we were the first to work with the NHS and Government to trial a new approach that supported patients accessing our medicines earlier, whilst also delivering value for the NHS.

As we fully support the UK Government moving forward in implementing the Life Sciences Industrial Strategy and Sector Deals, we are equally focused on partnering to deliver the UK's world leading healthcare ambitions in challenging disease areas.

Success in these areas, together with continued investment in the NHS and improvements in data infrastructure, will be critical to maintaining the UK's place on the global map.

David Peacock

Managing Director
UK & Ireland, MSD

"Modern day research is fundamentally a collaborative and international endeavour"

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Towards a health data research revolution

The complex world of health data can sometimes leave clinicians and researchers frustrated and stuck in elaborate and overlapping processes for data access, which ultimately lead to delays in new medical discoveries and innovations that could save people's lives. It is clear that health data must be managed carefully, within robust governance standards to retain public trust and to have the best possible impact. But how can this be managed while also realising the enormous patient and public benefits?

Health Data Research UK are changing the landscape by uniting the UK's health data to enable discoveries that improve people's lives. By making health data available to researchers and innovators, responsibly and ethically, we believe we can better understand diseases and find new ways to prevent, treat and cure them.

If you need inspiration for why the use of large-scale health data can lead to ground breaking discoveries, the recently published suite of 23 research studies, by the Pan-Cancer project, demonstrates why we are still only at the start of the health data research revolution. In this project, over 1300 scientists from 37 countries created an unprecedented map of cancer genomes, to better understand tumour development, causation, progression and classification. This will lead to new approaches to improve the early detection and treatment of cancer, and ultimately save lives. Why was this project possible? Because the genomics community adopted a standards-based approach to data-sharing many years ago, enabled by robust ethical and collaborative standards, supported by organisations such as the Global Alliance for Genomics and Health.

But this study still "only" used data from 2600 patients and volunteers (generating 800 terabytes of genomic data!) who consented to have their data used in the research. Trustworthy use of 'routine data' generated in the day-to-day delivery of care is vital and as crucial as a clinical trial or other traditional research recruitment approaches. Routine data increase the scale and efficiency of research and extends the impact of a study well beyond the time that research data can be feasibly and accurately collected. Most importantly, it can positively impact groups within society who are the least likely to participate in a research study, but who could be the most likely to benefit from treatments.

It is, however, always important to remember that with big data comes big responsibility. Staying at the forefront of science and technology requires developments in safeguards and security. Health Data Research UK, the Academy of Medical Sciences and the Collaboration for the Advancement of Sustainable Medical Innovation (CASMI) jointly ran an event in January to inform how we realise patient and NHS benefits from health and care data to derive appropriate value for all.

At Health Data Research UK we continually aspire to achieve a transparent and open approach and are keen to share research outputs with colleagues and members of the public through our podcasts, blogs, monthly newsletters and more. We also have an ever-growing list of Open Access publications and case studies which are available to read. This demonstrates how our research outputs will be used to affect real-world change across the UK and is part of our efforts to do all we can in this space to make discoveries which improve people's lives.

Dr Rhoswyn Walker

Chief Science Strategy Officer,
Health Data Research UK

"With big data comes big responsibility. Staying at the forefront of science and technology requires developments in safeguards and security"

Conclusion

Delivering the vision for a thriving life sciences economy

The life sciences industry is being disrupted by new technologies, business models and regulatory structures. To remain competitive the UK must build on its unique strengths, particularly its world-leading expertise in genomics and access to 'cradle to grave' health data. Yet, long-term success will depend on the ability to scale-up innovation and forge high-value partnerships between Government, industry and academia.

The UK has a long history of pioneering cutting-edge innovations in medicine and biology – from developing the world's first vaccine to kick-starting a national genomics industry. The Life Science Industrial Strategy and Sector Deals have helped accelerate R&D innovation and position the life sciences industry as a major contributor to the country's economic growth.

The contributors to this *Reformer Thoughts* make a compelling case for the future of the life sciences sector. Yet, they also caution that the UK must not 'rest on its laurels' when pursuing growth in this sector.

Unlocking R&D investment at scale will be crucial to future success. Yet, as highlighted by Professor Watt and Lord O'Shaughnessy, for investment to be effective, it must be matched with action to deliver the right skills and capability for R&D and innovation to translate into on-the-ground practice and deliver true benefit to patients. The Government must also become bolder in its approach to funding R&D, striking-up a balance between investment in higher-risk discovery projects and 'proven' research.

A lack of good quality and well-structured data continues to be a barrier for development and drug discovery. A continued effort must be made to harness the wealth of healthcare data held within the health system. Progress is already underway with organisations like Health Research Data UK making high-quality health data available to researchers and innovators to better understand diseases and identify ways to prevent and treat them, and with the use of 'real-world' data becoming integral to how the NHS evaluates the safety and performance of treatments and drugs. Wide-scale adoption will require heavy investment to improve the NHS's digital infrastructure and upskill staff with the right capabilities and talent. It will also need an agile and fit-for-purpose regulatory system. The UK's departure from the European Union, coupled with the rapid pace of technological innovation and the advent of novel treatments, will provide the impetus for realigning current regulation so that promising treatments and drugs reach the market sooner.

Yet, these efforts will be meaningless unless underpinned by genuine collaboration between what the Secretary of Health & Social Care has referred to as the "holy trinity" of life sciences: private enterprise, academic research, and Government. This will require all actors participating in the UK's health economy to transition away from seeing themselves as 'customers' and start thinking of themselves as partners. This shift is starting to happen, with MSD's collaboration with the Francis Crick Institute exemplifying how high-value partnerships are bringing forward ground-breaking research and investment to solve society's most pressing health challenges.

The UK is well-placed to deliver on the life sciences' vision for an innovation-led health economy. Building on current success, fostering sector partnerships and getting the right environment for R&D and innovation will be key enablers to achieve this transformation.

Claudia Martinez

Research Manager, *Reform*

"These efforts will be meaningless unless underpinned by genuine collaboration between private enterprise, academic research and Government"

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