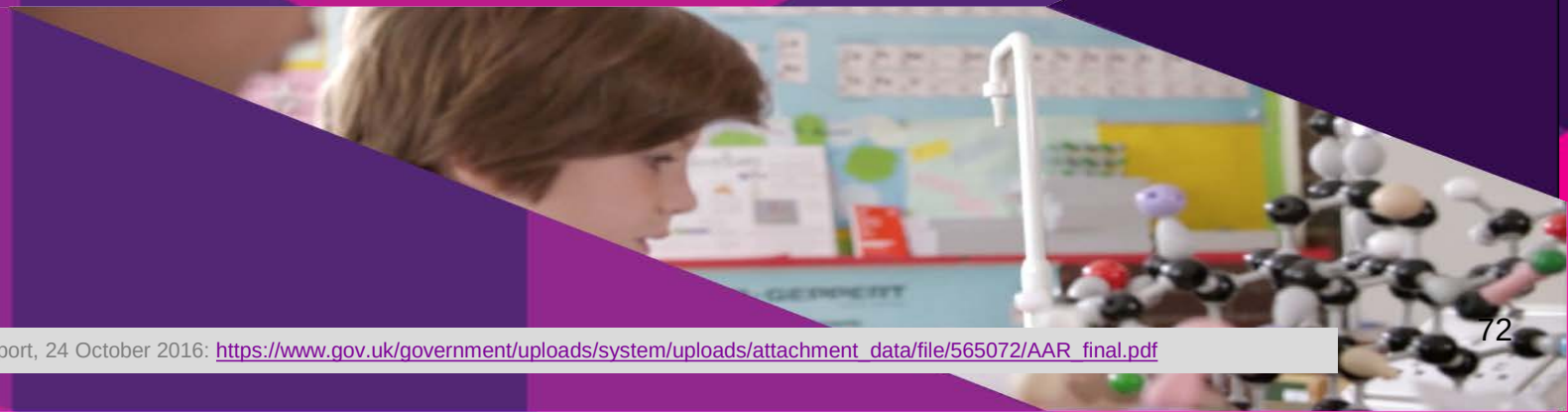
The future is exciting with challenges that can be overcome[1]



1. Accelerated Access Review Final Report, 24 October 2016: https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/565072/AAR_final.pdf

Despite great progress, there are challenges we must address across Europe

Innovation is challenging

- Industry is tackling more complex disease areas
- Longer, more complex clinical trials
- Higher regulatory hurdles
- Increased cost of R&D

Investment in innovation increasingly risky

- Government payers encouraging off label use of therapies to save money
- Flourishing parallel trade
- Fiscal austerity measures
- Unknown IP environment

Challenges exist impeding patient access

- Complex HTA processes delaying patient access
- Clinical guidelines and restrictive cost-effectiveness requirements limiting access to best care
- Contracting and tendering limiting therapeutic options

EFPIA, From innovation to outcomes; medicines costs in context 20/04/16, with language change from ABPI Page 52.

Healthcare systems in Europe face significant challenges in expanding access to healthcare while managing finite resources

From innovation to outcomes; medicines costs in context, EFPIA. Page 37. 20/4/16

Several challenges to overcome for health care systems to focus on patient outcomes

1	Lack of public and political awareness	▪ Low awareness of outcomes inequality, some of which are resulting from practice variation
2	Suboptimal outcome measurement & transparency	▪ Lack of a standard definitions, measurement and transparency of outcomes data
3	Few proofs of concepts	▪ Lack of comprehensive examples demonstrating what can be achieved through an outcomes-focused system
4	Health systems structural barriers	▪ Structural barriers in healthcare systems to move towards outcome-focused system

EFPIA. Healthier future external slide set, Shaping sustainable, outcomes-driven healthcare in Europe, Page 8.

It is important that we get the future R&D and regulatory pathways right in the UK

Science and technologies, as well as new sources of information, offer a wealth of opportunities to optimise R&D. It is essential that these advances are also reflected in regulatory and clinical practice to ensure that this potential is realised – and that patients can obtain much needed prevention and treatments much faster.

- The AAR sets out a bold new vision of better, cheaper and faster adoption of innovation through:

1. **Establishing streamlined mechanisms for prioritising emerging technologies and identifying strategically important innovations**
2. **Working with innovators to accelerate approvals speed up adoption and evaluate technologies efficiently using new data sources; and**
3. **Aligning national organisations to transform the NHS's ability to adopt the right innovations rapidly**

The Accelerated Access Review is an important foundation for building a Life Sciences Industrial Strategy and opens the door to greater collaboration between innovators, patients and the NHS to make the UK a world leader in researching, developing and using new treatments and technologies. The report recommends: the following around R&D & regulatory pathways.

- NICE should review its health technology assessment processes and methods to ensure they are fit for purpose to assess new types of emerging products and enable access to the products the NHS needs.
- NICE should develop a flexible health technology assessment pathway that can be tailored to a product's value proposition.
- NICE should refocus its work to place more emphasis on medical technologies, diagnostics and precision medicine tools, and a funding requirement should apply or those products that improve efficiency
- There should be a single set of clear national and local commissioning arrangements to get medical technologies, diagnostics, pharmaceuticals and digital products to patients

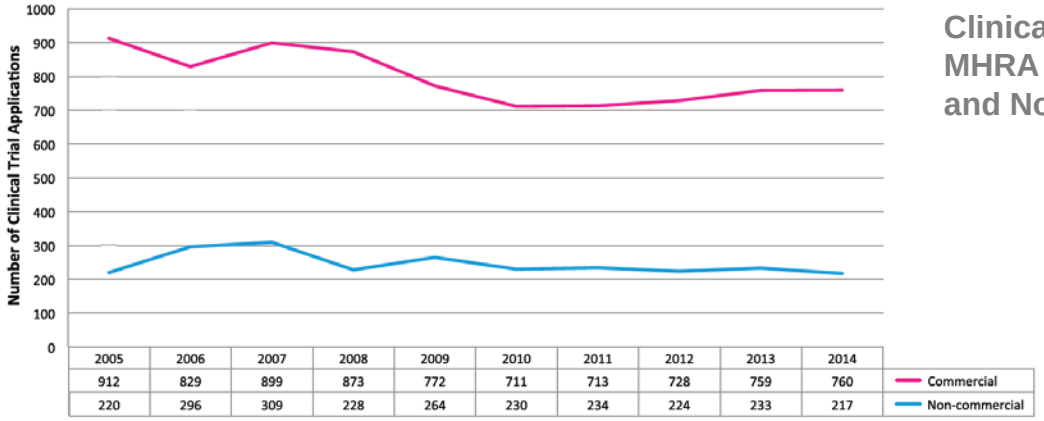
The Accelerated Access Review: Page 41 https://www.gov.uk/government/uploads/system/uploads/attachment_data/file/564145/AAR_final_A.pdf

We want to retain the UK as a base for clinical trials

The UK is a base for excellence in clinical trials – more than 618,000 people participated in clinical research in England in 2014

The Life Science Competitiveness Indicators, which reviewed the relative shares of patients recruited to global studies across all trial phases, revealed that the **UK has seen a decline its share of patients over time (2008 to 2012)**

According to the MHRA, the **number of applications for clinical trials in the UK has declined** since 2005 but amounted to 760 applications received in 2014. The UK National Institute for Health Research (NIHR) calculated that **more than 618,000 people participated in clinical research in the NHS in England in 2014,** with 35,000 participants recruited to studies sponsored by the pharmaceutical industry (an increase of 35% over the previous year)3. To put this in global context, in 2013, pharmaceutical companies sponsored 6,199 trials across the US involving 1.1 million participants[1].



Clinical Trial Applications Received by MHRA 2005-2014 All phases; Commercial and Non-Commercial

ABPI R&D Sourcebook 2015 . Page 26. http://www.abpi.org.uk/our-work/library/industry/Documents/ABPI_RD_Sourcebook.pdf
SOURCE: Adapted from MHRA Clinical Trials for medicines: authorisation assessment performance; https://www.gov.uk/government/publications/clinical-trials-for-medicines-authorisation-assessment-performance last accessed August 2015
NOTES: MHRA last updated the data in July 2015. The data sets out the number of applications assessed by MHRA split out by phase and commercial and non-commercial sponsors.

We want to retain the UK as a global destination for manufacturing

Opportunities

- Patients becoming partners in their own healthcare. packaging, design and content have to facilitate interactions and provision of information. Compliance benefits.
- Impact of “digital” on factory design and operation; using big data/ informatics - process control, connected supply chain
- How regulatory requirements keep pace with and adapt to recognise new technologies

Solutions

- Impact of Intelligent Products (and how we supply)
  devices and packaging coupled with ‘wearables’ and digitised medicine
- Manufacturing technologies and effective supply chains for new product types
  (such as those needed for ATMPs)
- Continued shift towards personalised medicines
  require ever more flexible and adaptable supply chains

Challenges[1]

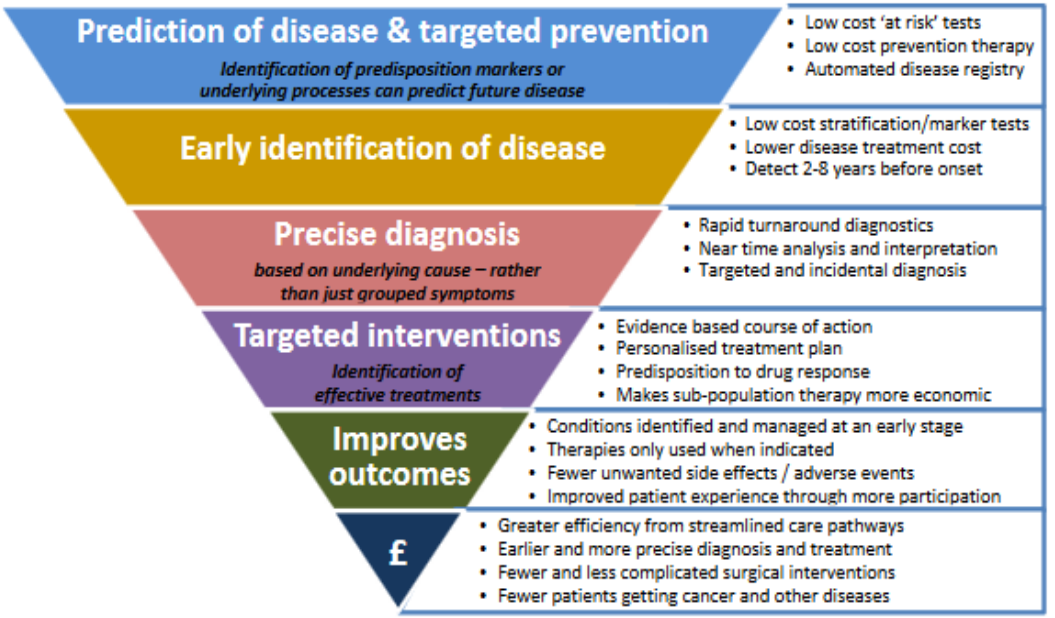
- Availability of skilled people to sustain, correct and deliver future technologies
- Availability of funding
  (government and private)
- Incomplete infrastructure of national facilities and capabilities to support product and technology development
- Attractiveness of UK tax structure

1. McCubbin, I. (Chair of MMIP) ‘Foreword from Co-Chairs of ATMT – Retaining and attracting advanced therapies manufacture in the UK MMIP Manufacturing Taskforce Report on Advanced Therapies.’ http://www.abpi.org.uk/our-work/mmip/Documents/Advanced-Therapies-Manufacturing-Taskforce-report.pdf

What is personalised medicine?

Personalised medicine is about moving away from a ‘one size fits all’ approach to the treatment and care of patients with a particular condition, to using diagnostics, genomics, data analytics and other emergent technologies to identify the underlying cause of disease.[1]

Figure 1: Personalised Medicine – improving outcomes

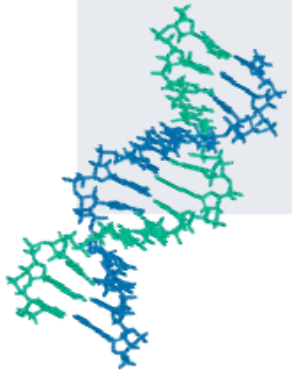


1. NHS England. Personalised Medicines Board Paper. https://www.england.nhs.uk/wp-content/uploads/2015/09/item5-board-29-09-15.pdf

Scientific advances fuel personalised medicines

1950s

Watson and Crick discover the structure of the **DNA double-helix**



1960s

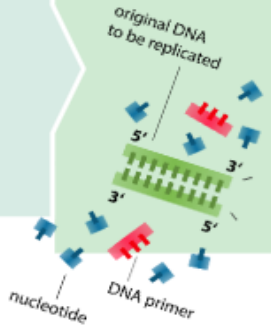
Researchers crack the **genetic code**

1970s

- First **DNA sequencing** technology developed
- Researchers discover first enzyme linked to individual variation in response to dosing

1980s

Polymerase chain reaction (PCR) first developed, allowing for **fast amplifi-cation of DNA** sequences



1990s

- Human genome project launched
- FDA approves **first personalized medicine** with a companion diagnostic, for the treatment of HER2 positive breast cancer

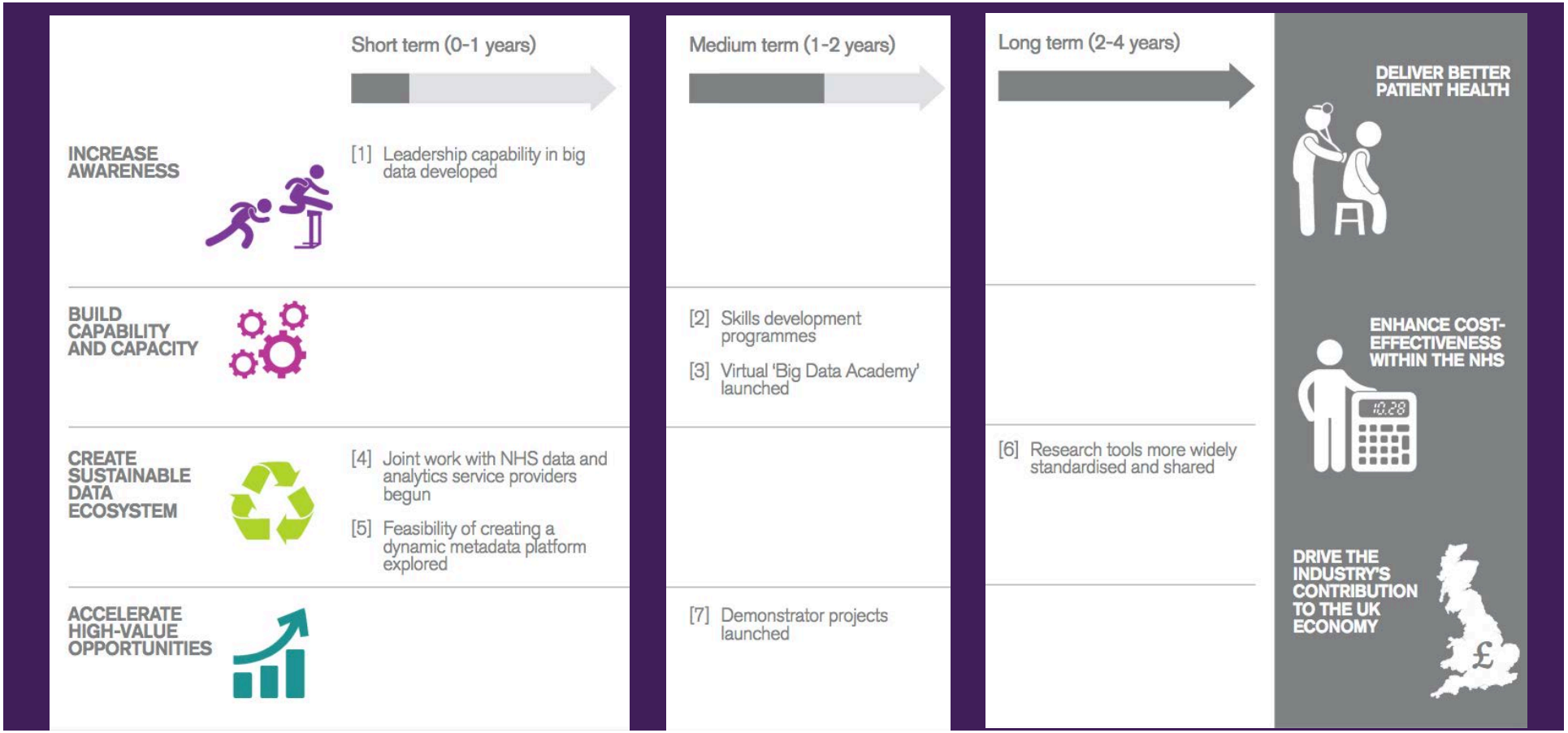
2000s to Present

- Human Genome Project completed
- First **targeted therapies** for lung cancer, leukemia, melanoma, cystic fibrosis, HIV, and many other diseases
- **42% of the industry's pipeline** has the potential to be personalized medicines

- L Pray, "Discovery of DNA structure and function: Watson and Crick," Nature Education, 2008; 1(1):100; Office of National Institutes of Health History, "Exhibit: Deciphering the Genetic Code: Marshall Nirenburg," http://history.nih.gov/exhibits/nirenberg/index.htm; Scitable by Nature Education, "The Order of Nucleotides in a Gene Is Revealed by DNA Sequencing," http://www.nature.com/scitable/topicpage/the-order-of-nucleotides-in-a-gene-6525806;
- PJ Murphy, "The Development of Drug Metabolism Research as Expressed in the Publications of ASPET: Part 3, 1984–2008," Drug MetabDispos, 2008 Oct;36(10):1977-82; National Center for Biotechnology Information, "Polymerase Chain Reaction," http://www.ncbi.nlm.nih.gov/probe/docs/techpcr/; NIH,
- Fact Sheet: Human Genome Project,"http://report.nih.gov/NIHfactsheets/Pdfs/HumanGenomeProject(NHGRI).pdf , Oct 2010; U.S. FDA, "Paving the Way for Personalized Medicine," Oct 2013; Tufts Center for the Study of Drug Development, "Impact Report," Volume 17, No.3, May/June 2015.

The role of data now and in the future is becoming more important

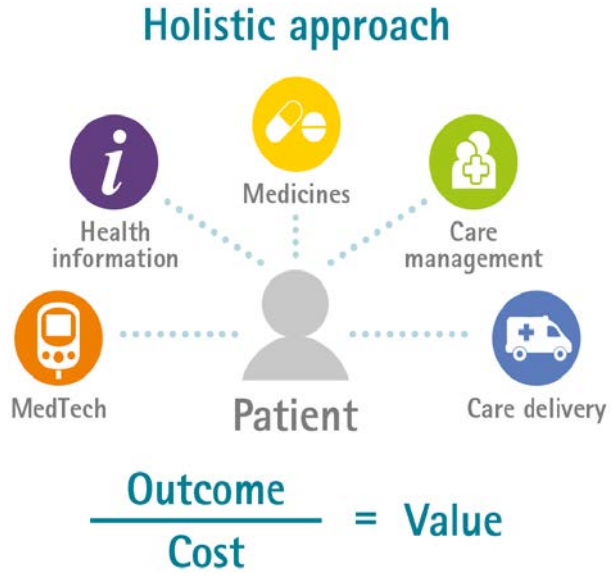
Big data is a common theme underpinning many of the proposed solutions to the challenges facing the NHS, the life sciences research community and the pharmaceutical industry. Big data technologies make it easier to work with large datasets, link different datasets, detect patterns in real time, predict outcomes, undertake dynamic risk scoring and test hypotheses.



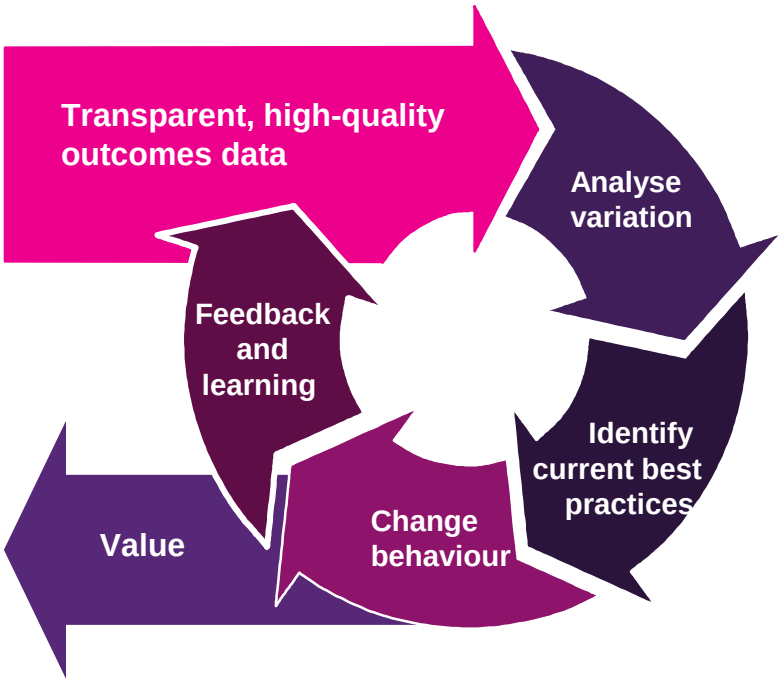
ABPI Big Data Roadmap 2013. Page 13. http://www.abpi.org.uk/our-work/library/industry/Documents/ABPI%20big%20data%20road%20map.pdf

Industry is keen to engage in the debate and to partner with government to deliver outcomes driven sustainable healthcare systems

The objective of outcomes-focused healthcare systems is to deliver **better patient outcomes** at the **same or lower cost...**



Relying on **quality outcome data** as starting point to improve care cycle



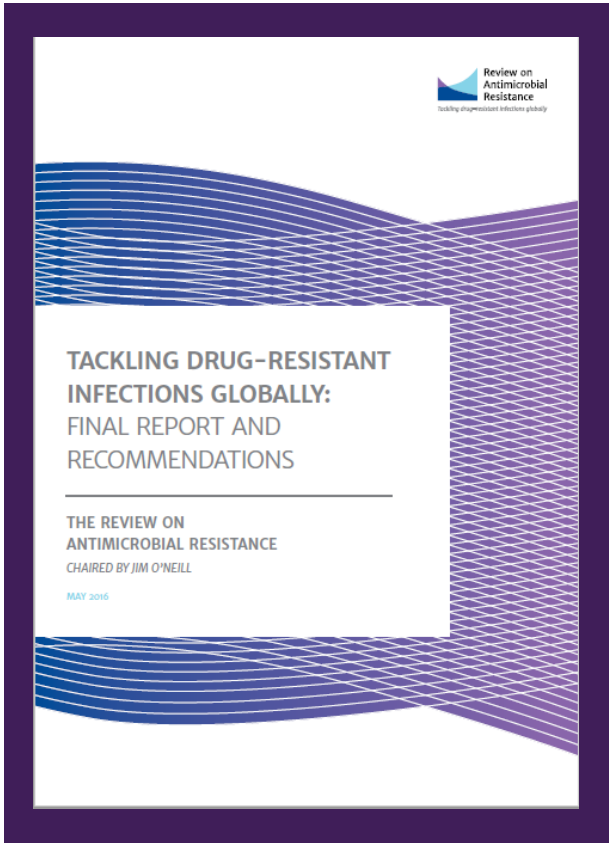
Benefits of a focus on outcomes: improved patient outcomes, reduced variation, reduced medical cost, continuous improvement

EFPIA, From innovation to outcomes ;medicines costs in context 20/04/16,

The pharmaceutical industry is working hard to tackle Antimicrobial Resistance

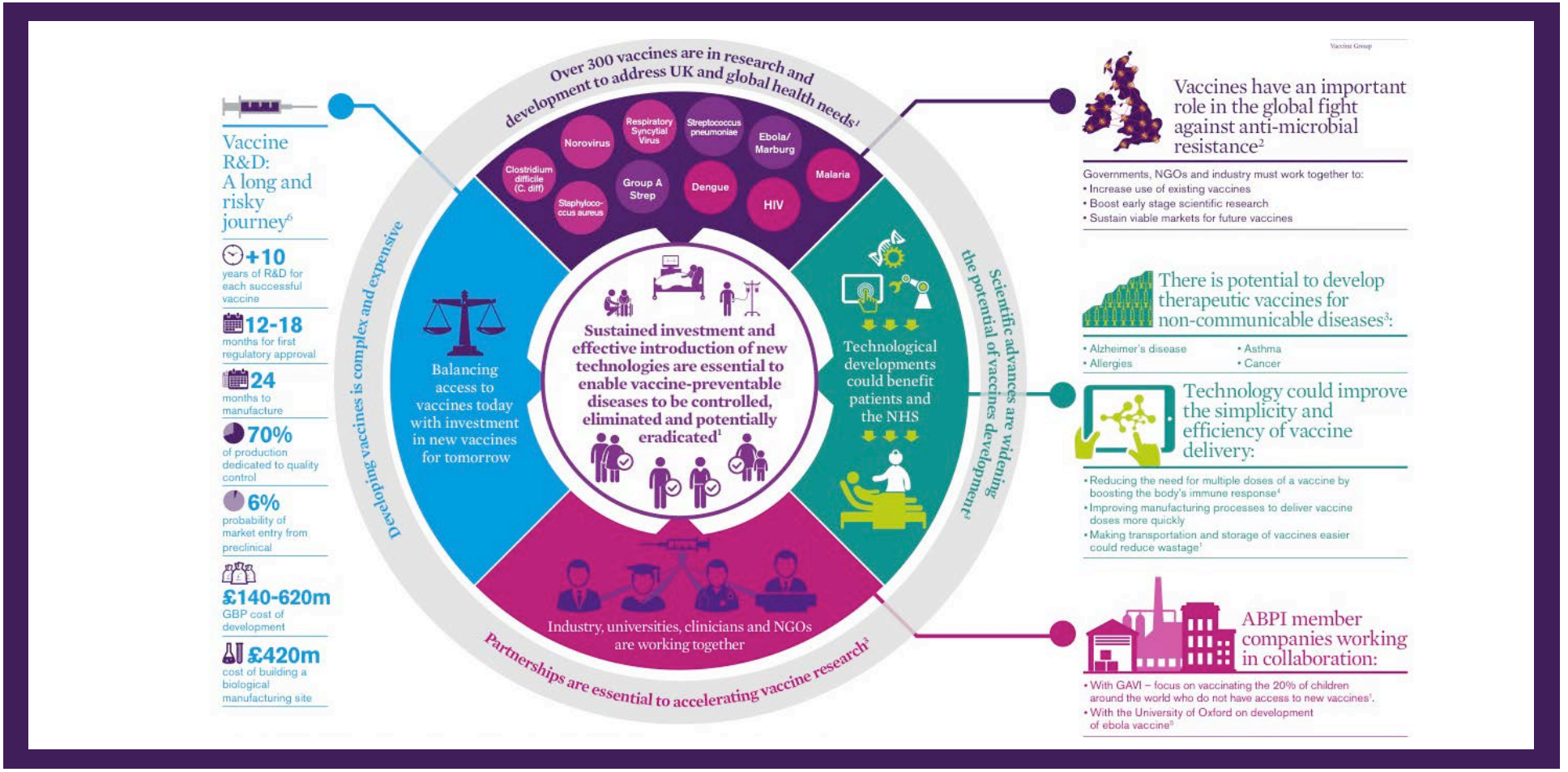
Review on Antimicrobial Resistance

- The May 2016 announcement from the UK Government on Antimicrobial Resistance was an important step forward in the collective action that must now begin to address drug resistance and the rise of the superbug.
- The pharmaceutical industry is committed to playing its part, but keeping antibiotics effective is everybody's responsibility, and detecting, preventing and controlling resistance requires a strategic, coordinated, and sustained global and local response.
- National government action and funding is a crucial component and we stand ready to work alongside policymakers, as well as the NHS, patients, healthcare providers, academia and the agricultural community in this fight.



Tackling drug-resistant infections globally. Final report and recommendations. https://amr-review.org/sites/default/files/160518_Final%20paper_with%20cover.pdf

Industry is committed to the future development of vaccines



Industry is committed to the future development of vaccines - referencing

1. IFPMA, Innovation for a Healthier World http://www.ifpma.org/wp-content/uploads/2016/01/IFPMA-ComplexJourney-FINAL-Digital.pdf
2. Review on antimicrobial resistance. 'Vaccines and Alternative Approaches: Reducing our dependence on antimicrobials' 2016. https://amr-review.org/sites/default/files/Vaccines%20and%20alternatives_v4_LR.pdf
3. Medical Research Council, Review of Vaccine Research 2014 https://www.mrc.ac.uk/documents/doc/mrc-review-of-vaccines-research-2014/
4. https://www.science.org.au/6-what-does-future-hold-vaccination
5. World Health Organisation, Ebola vaccines, therapies and diagnostics Q&A 6/10/15. http://www.who.int/medicines/emp_ebola_q_as/en/
6. IFPMA Maintaining the Vaccines Innovation Edge infographic. http://www.ifpma.org/resource-centre/maintaining-the-vaccines-innovation-edge/

Medicines are part of the solution and more can be done together with Government

Improve efficiency

Look at all healthcare costs, reduce administrative costs and waste, and improve efficiency.

Pay for value

Support evidence-based care and empowered patients and providers, backed by sound research and strong quality measures.

Find solutions

Avoid blanket policies that chill investment, and collaborate to find new approaches.

CONTINUE DEVELOPING INNOVATIVE THERAPIES, PROMOTE MEDICATION ADHERANCE, MAINTAIN EFFORTS TO SUPPORT BROAD PATIENT ACCESS

EFPIA, From innovation to outcomes,; medicines costs in context. Page 2. http://www.efpia.eu/uploads/Modules/Documents/efpia-annual-report-2015.pdf

Sources

Slide Title	Source
The future is exciting with challenges that can be overcome, slide 72	Accelerated Access Review Final Report.
Despite great progress, there are challenges we must address across Europe, slide 73	EFPIA, From innovation to outcomes, medicines costs in context. Page 52.
Healthcare systems in Europe face significant challenges in expanding access to healthcare while managing finite resources, slide 74	EFPIA, From innovation to outcomes, medicines costs in context. Page 37.
Several challenges to overcome for health care systems to focus on patient outcomes, slide 75	EFPIA. Healthier future external slide set, Shaping sustainable, outcomes-driven healthcare in Europe. Page 8.
It is important that we get the future R&D and regulatory pathways right, slide 76	The Accelerated Access Review: Page 41.
We want to retain the UK as a base for clinical trials, slide 77	ABPI R&D Sourcebook 2015 . Page 26.
We want to retain the UK as a global destination for manufacturing, slide 78	McCubbin, I. (Chair of MMIP) 'Foreword from Co-Chairs of ATMT'
What is personalised medicine?, slide 79	NHS England. Personalised Medicines Board Paper.
Scientific advances fuel personalised medicines, slide 80	L Pray, "Discovery of DNA structure and function: Watson and Crick," Nature Education, 2008; 1(1):100;
Scientific advances fuel personalised medicines, slide 80	PJ Murphy, "The Development of Drug Metabolism Research as Expressed in the Publications of ASPET: Part 3, 1984–2008," Drug MetabDispos, 2008 Oct;36(10):1977-82; National Center for Biotechnology Information, "Polymerase Chain Reaction.''
Scientific advances fuel personalised medicines, slide 80	Fact Sheet: Human Genome Project "Paving the Way for Personalized Medicine," Oct 2013; Tufts Center for the Study of Drug Development, "Impact Report," Volume 17, No.3, May/June 2015

Sources

Slide Title	Source
The role of data now and in the future is becoming more important, slide 81	ABPI Big Data Roadmap 2013. Page 13.
Industry is keen to engage in the debate and to partner with government to deliver outcomes driven sustainable healthcare systems, slide 82	EFPIA, From innovation to outcomes, medicines costs in context
The pharmaceutical industry is working hard to tackle Antimicrobial Resistance, slide 83	Tackling drug-resistant infections globally. Final report and recommendations.
Industry is committed to the future development of vaccines, slide 84	IFPMA, Innovation for a Healthier World, Page 15.
Industry is committed to the future development of vaccines, slide 84	Review on antimicrobial resistance. 'Vaccines and Alternative Approaches: Reducing our dependence on antimicrobials'. Page 3.
Industry is committed to the future development of vaccines, slide 84	Medical Research Council, Review of Vaccine Research 2014. Page 8.
Industry is committed to the future development of vaccines, slide 84	Australian Academy of Science. What does the future hold for vaccination?
Industry is committed to the future development of vaccines, slide 84	World Health Organisation, Ebola vaccines, therapies and diagnostics
Industry is committed to the future development of vaccines, slide 84	PMA Maintaining the Vaccines Innovation Edge infographic.
Medicines are part of the solution and more can be done together with Government, slide 86	EFPIA, From innovation to outcomes, medicines costs in context. Page 2.