

Science Capital innovative healthcare 2014

Stratified medicine – from design to delivery



***Dr Louise Leong PhD FSB
Director of R&D Policy***

Birmingham, 11 September 2014

Association of the British Pharmaceutical Industry



- **Innovative research-based biopharmaceutical companies, both large and small**
- **Supply 90% of all medicines used by the NHS**
- **Researching two-thirds of the medicines pipeline**
- **The statutory body for negotiating on the PPRS**

First, let's take a step back to look at the UK ecosystem ...

These are some of our members....



... including CROs and technology companies



QUOTIENT BIORESEARCH



tranScrip partners

Bringing medicines to *life*

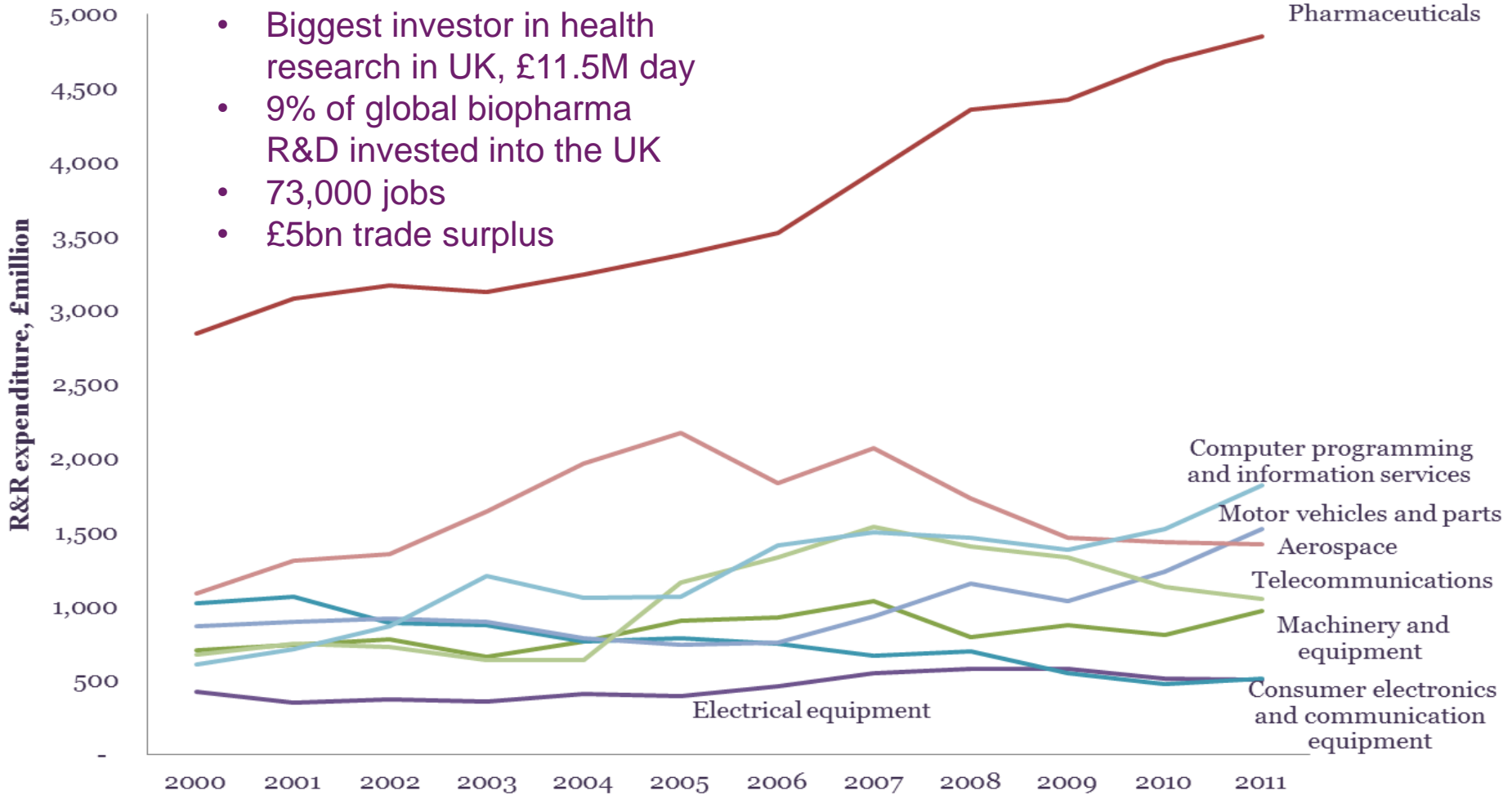


PRIME VIGILANCE
YOUR PV PROVIDER



SUCAMPO
PHARMACEUTICALS, INC.

No other industrial sector invests more in R&D



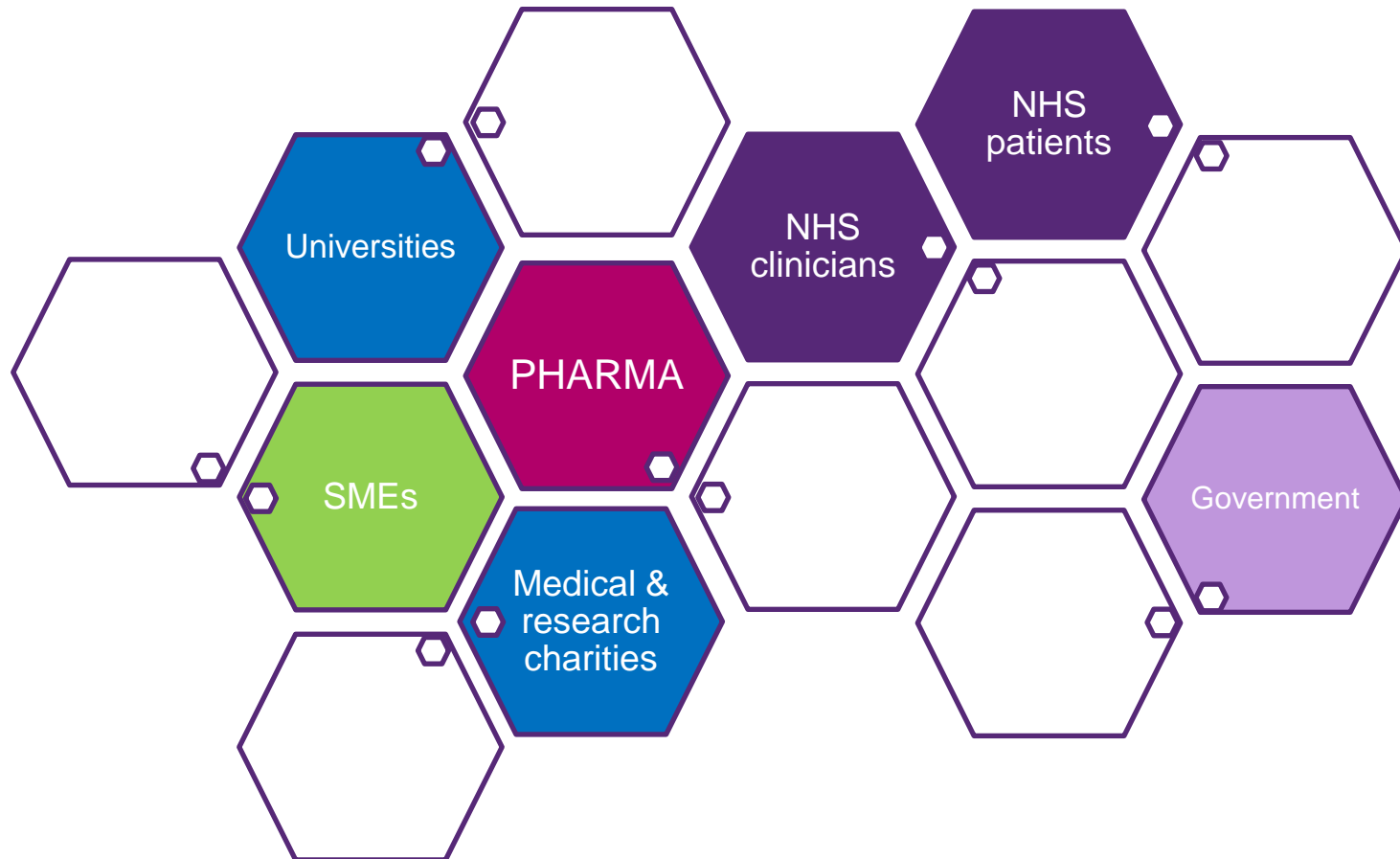
Source: ONS Business Enterprise R & D 2011

Bringing medicines to *life*

Evolving industry - the R&D ecosystem



Multiple players and interdependencies



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What are relevant UK strengths?



- Science base and talent
- Global pharma presence
- Well funded infrastructure for experimental medicine
- National Health Service, potentially – “every patient a research patient”
- Commitments to capacity and capabilities in big data – Farr, CPRD, etc
- Strong third sector, e.g. research charities
- Robust ethics and governance
- Public dialogue and engagement
- Enterprise, financial markets
- First launch market (historical)

Are we leveraging our strengths?

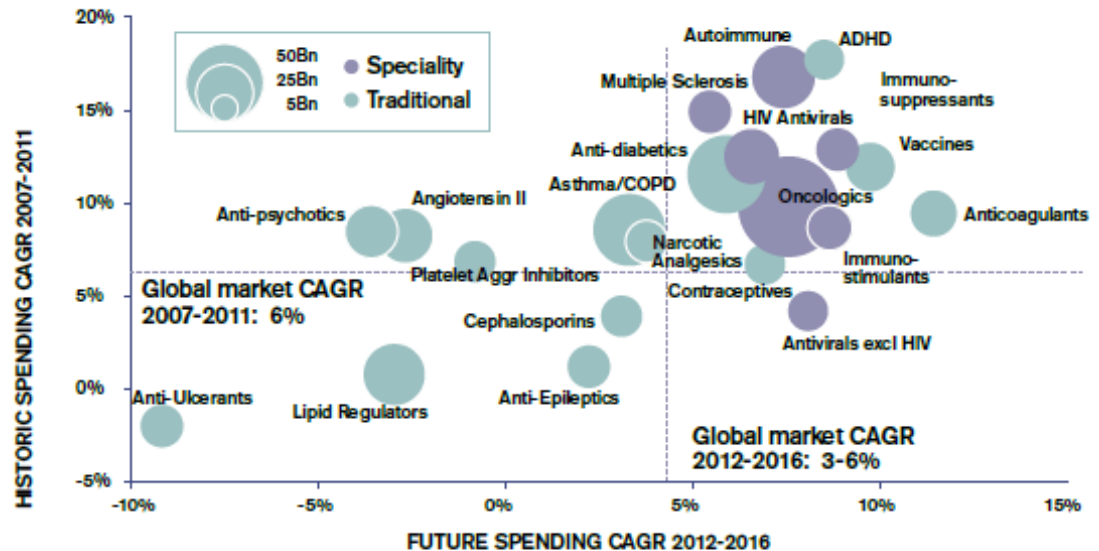
Why is stratified medicine a priority?



- Value for patients
- Enable more efficient R&D, more effective medicines
- Require industry & stakeholders to sustain a strong focus to effect complex system changes
- Require partnership approach, with concerted effort

Stratified medicines are with us:

- Increasing part of candidate development pipeline
- Increasing launches both post-hoc and prospective



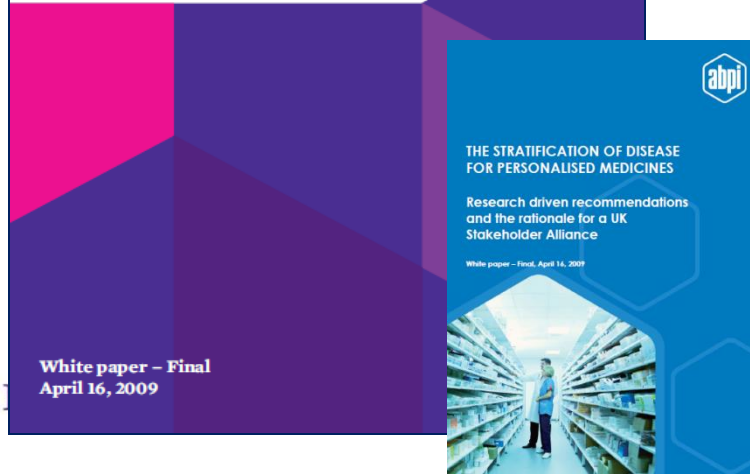
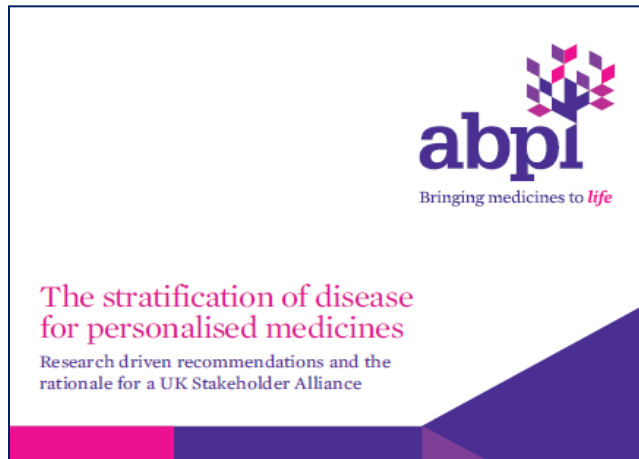
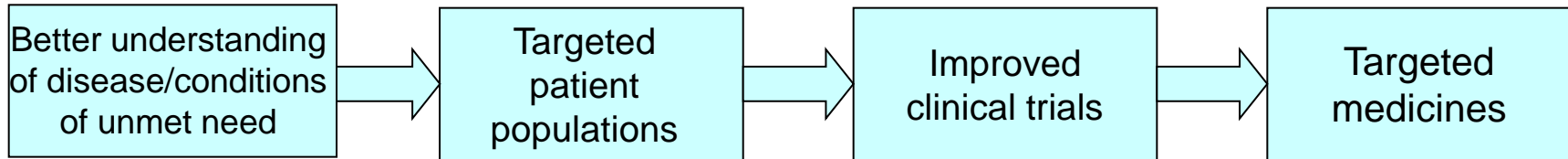
What does success look like?



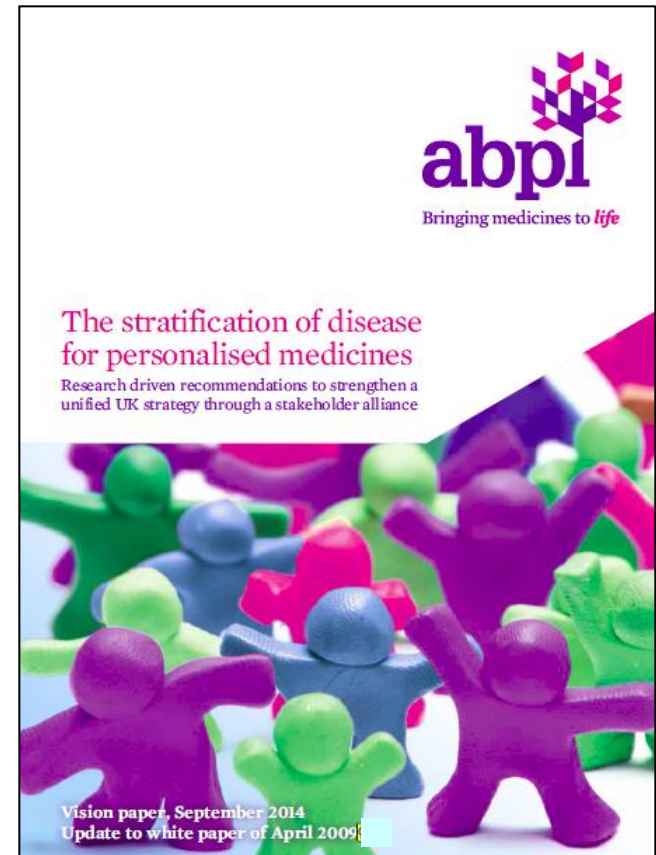
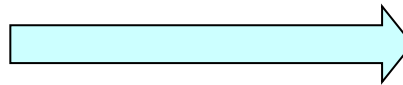
- Patients have timely **access** to stratified medicine in the NHS
- A framework for flexible and broad value assessment which enables funding and commissioning of stratified medicines
- Insights into stratification of diseases through greater precompetitive efforts with academia, NHS and diagnostic sector
- Facilitative environment in the UK to accelerate **R&D** of stratified medicine, drawing on bio-resources, well-phenotyped patient cohorts with data matched to 'omics and other biological information
- Underpinned by a strong research **culture**, data culture, collaborative culture

ABPI vision, and journey 2009 - 2014

Research-driven recommendations and rationale for a UK stakeholder alliance

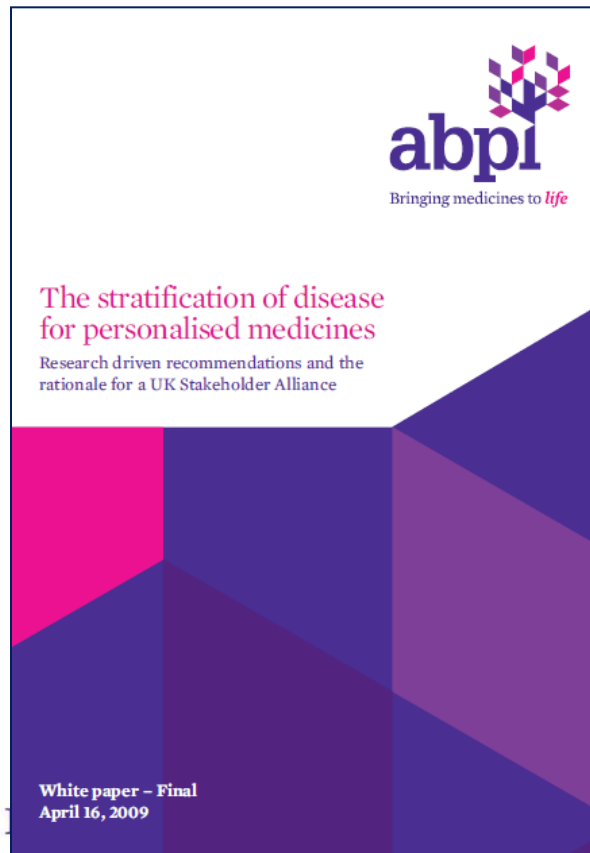
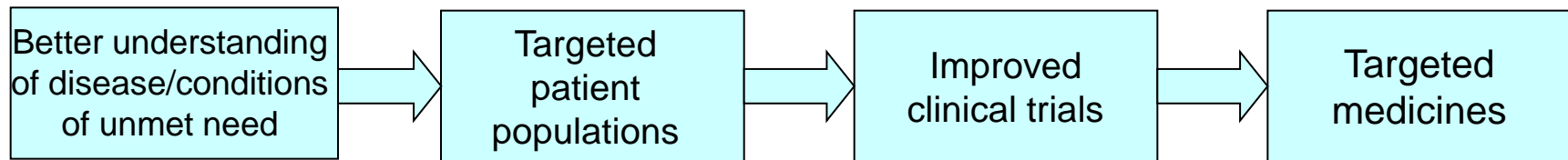


Right medicine,
right patient,
right time



How to get there?

Policy areas, 2009



Right medicine, right patient, right time

- Science and Technology
- Regulation
- Electronic health records and data linkages
- Reimbursement
- Economics and new business models
- IP
- Patient & physician engagement /education
- Privacy

<http://www.abpi.org.uk/industry-info/future/Pages/personalised-medicines.aspx>

... through a UK stakeholder alliance



Policy partnerships and brokering connections



Providing answers today and tomorrow



Technology Strategy Board
Driving Innovation



NICE



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Also globally – IMI, FP7, H2020, EC PgX, other consortia, etc

Precompetitive academic-industry consortia



Research partnerships

MRC-ABPI COPD consortium

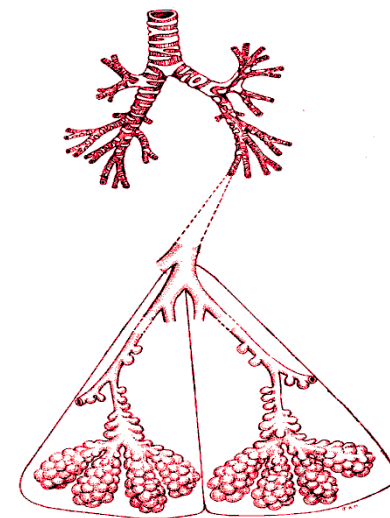
Growing global health burden, highly heterogenous disease

- Cohorts and phenotype underpin the workpackages
- Role of infection
- Tissue injury and repair
- Co-morbidities

AZ-BI-GSK-MSD-Novartis-Pfizer

Plus

- **Diabetes**
- **Dementia**
- **And more in development...**



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MRC-ABPI Rheumatoid Arthritis consortium

- Curing early arthritis
- Immunological toolkit

12 companies

An integrated UK strategy



Commitments since the ABPI White Paper and alliance building

TSB Innovation Platform

£50M -> £200M

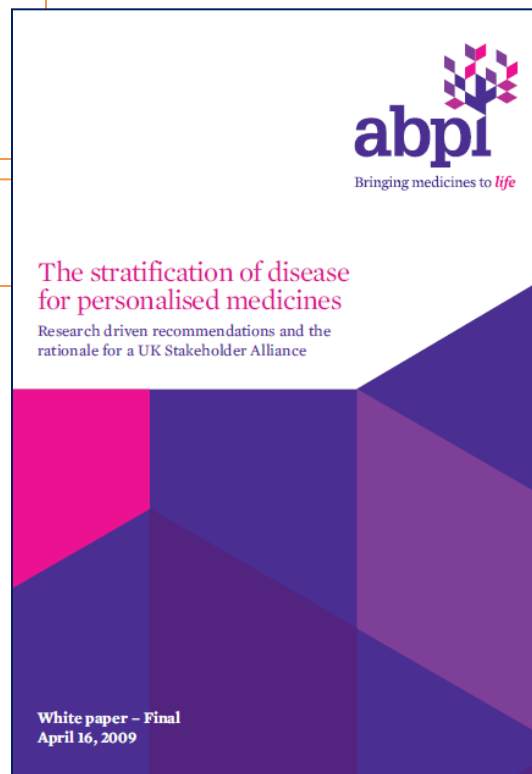
Catapult in Precision Med

Public engagement eg
Sciencewise

MISG Forum –
Regulation in Strat Med

AMS symposium – value,
business models

ABPI-BIVDA Rx-Dx networking



MRC-ABPI Immunology &
Inflammation Initiative –
COPD, RA

100K Genomes

Data linkages

CRUK SMP

MRC Molecular Pathology review

Dementia – in dev't

MRC-ABPI Diabetes
Stratification

NIHR BioResource

Asthma, ... ?

MRC Stratified Medicine call

Bio-resources for R&D

- Experimental medicine facilities £1bn
 - Access to tissues - biomarker qualification
 - STRATUM – standardisation and quality
 - UK Biobank – 500,000 now open
- + imaging + biomarker panel + genomes
- NIHR Bioresource – 75,000 volunteers on register



An experimental medicine model to support a stratified medicine approach – points to consider



100K Genome project



- 100,000 genomes: patients with rare inherited disease, common cancers and pathogens from the NHS in England
- Genomics England – owned by Department of Health
- Whole Genome Sequencing and link to clinical data in NHS
- Generate health and wealth, aiming for new treatments
- Legacy of infrastructure, human capacity and capability in NHS
- World-leaders in genomic medicine
- £100m funding over the next 5 years
- Pilot in 2014, to complete 10K genomes
- annotation and quality control
- Ethics, privacy and governance framework

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About the 100K Genome Project

We are a new company set up by the Department of Health to help deliver the 100k Genome Project first [announced by the Prime Minister](#) David Cameron in December 2012..

This project will sequence the personal DNA code – known as a genome – of up to 100,000 patients over the next five years. This unrivalled knowledge will help doctors' understanding, leading to better and earlier diagnosis and personalised care. Based on expert scientific advice, we will start by tackling cancer, rare diseases and infectious diseases.

The company will manage contracts for sequencing, data linkage and analysis, and set standards for patient consent.

“The UK will become the first ever country to introduce this technology in its mainstream health system.”

Genomics England was announced by Jeremy Hunt, Secretary of State for Health, as part of the NHS 65th birthday celebrations on 5 July 2013.

He said: “The NHS has a long track record as a leader in medical science advances and it must continue to push the boundaries by unlocking the power of DNA data.

“The UK will become the first ever country to introduce this technology in its mainstream health system – leading the global race for better tests, better drugs and above all better, more personalised care to save lives.

“Genomics England will provide the investment and leadership needed to dramatically increase the use of this technology and drive down costs.”



Prime Minister,
David Cameron



Secretary of State for Health,
Rt Hon Jeremy Hunt MP

Data linkages will facilitate discovery, development, use of SM



- Safe data
- Single health service, 60M, very diverse
- Deep history of longitudinal data, cohorts – eg Scotland, 1920s
- Enable adaptive licensing, real world data studies, responder precision

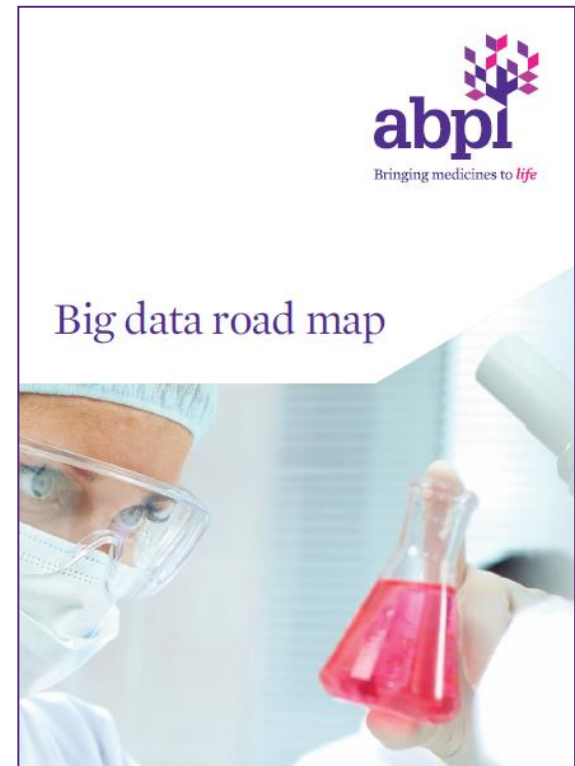
ABPI Big data roadmap, Nov 2013

Four-point plan to:

- Increase awareness
- Build capability and capacity
- Create sustainable data ecosystem
- Accelerate high value opportunities

“Data linkages for stratified medicine “

*****IN PRESS – check our website*****



Catapults – speeding businesses to products ... a case for CDx?



Inspired by the Fraunhofer institutes ...

- A technology and innovation centre where the very best of businesses, scientists and engineers can work side by side on R&D, transforming ideas into new products and services
- Catapult centres will help businesses , reduce innovating risk, offer concentrated expertise, access to cutting edge equipment and specialist facilities to develop and test ideas in reality
- Flexible model



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UK competitive position

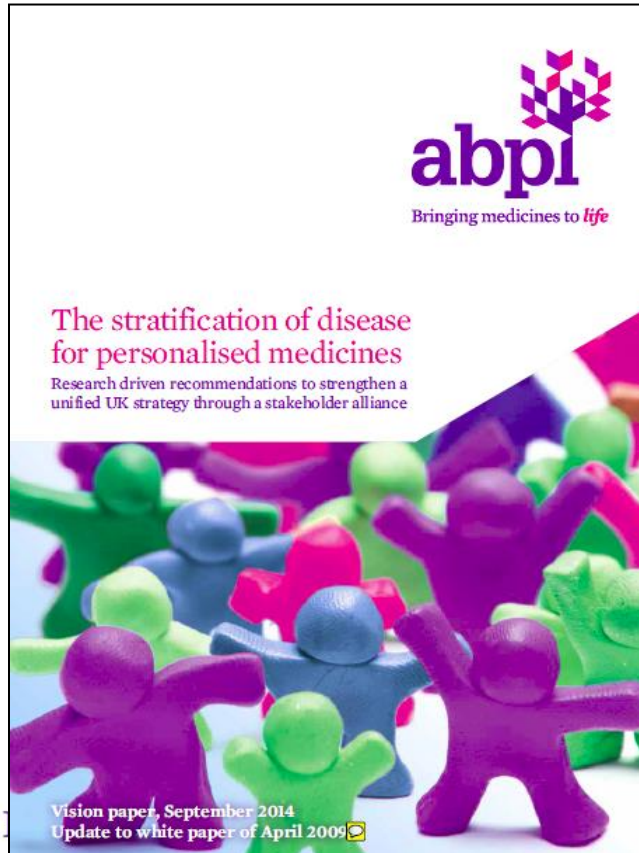
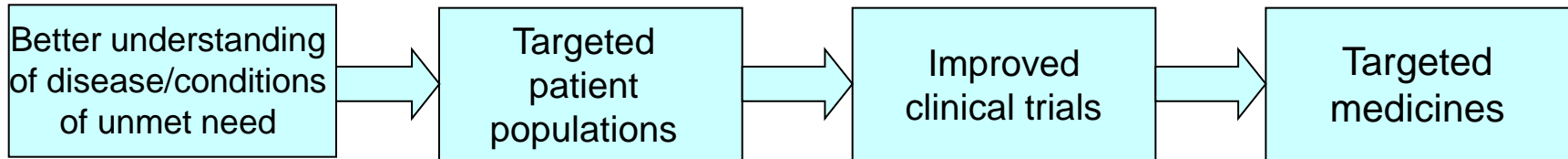


UK environment has supported stratified medicine advancement and shown international leadership

- Disease science, patient resources and faster translation
- Electronic health records, data linkages and informatics
- Genomic sequencing
- Academic clinician base , translational medicine infrastructure
- Participation in EU Innovative Medicines Initiative
- Strong third sector
- Innovate UK (TSB)

What more can the UK be doing?

Where do things stand?



ABPI White Paper – areas for action

- Science and Technology: through industry -academic -NHS collaboration
- Regulation and study design – global level but UK can lead in EU
- Electronic health records and data linkages, RWD
- Reimbursement – HTA /broader value capture and flexibility
- Economics and new business models
- IP
- Pathology, Adoption in the NHS – consistency, commissioning, structures ... Baselining project
- Patient & Physician engagement /education
- Privacy

Onwards ...



- Data and governance
- Translational medicine approaches
- Regulation
- Appropriate value assessment which delivers timely access

Implementation and use in health service

- Societal engagement

Stratified Medicine Baseline survey – use in the NHS


To take part in survey, email: john.candeto@concentra.org.uk




https://orgvuedemo.conc x

https://orgvuedemo.concentra.co.uk/Stratified-Medicines/survey/63A58A2C-CBB0-B126-9DCB-19936E0FC8

Non-Cancer Stratified Medicine¹ Baseline Survey



Bringing medicines to life



The Royal College of Pathologists
Pathology: the science behind the cure

This survey is a joint initiative between the Association of the British Pharmaceutical Industry (ABPI) and the Royal College of Pathologists (RCPATH), designed to gather professional opinion on the opportunities and challenges associated with the adoption of stratified medicine¹ in the NHS.


While there are increasing efforts to develop the evidence base for stratified medicine¹, key issues (and challenges) exist around implementation and take up into routine practice. The primary objective of the survey is to build a baseline understanding in four areas:

- Awareness
- Access
- Value
- Challenges


Your views are extremely important to us. The survey should take no more than 15 minutes to complete and your answers will remain anonymous unless you provide your email address in the introduction session of the survey.

Building an understanding of professional opinion and current practice in terms of biomarker directed strategies in real-world situations will help in overcoming some of the challenges likely to be faced by new drug-diagnostic combinations that are currently in development.

Your participation is greatly appreciated.



Stephen Whitehead (ABPI)



Ian Cree (RC Path)

¹ For this survey, stratified medicine will be defined as: "The tailoring of medical treatment to the individual characteristics of each patient. It does not literally mean the creation of drugs or medical devices that are unique to a patient, but rather the ability to classify individuals into subpopulations that differ in their susceptibility to a particular disease or their response to a specific treatment. Preventive or therapeutic interventions can then be concentrated on those who will benefit, sparing expense and side effects for those who will not."² This includes the development, validation and use of companion diagnostics to achieve the best outcomes in the management of a patient's disease or their predisposition.

² Definition from the President's Council of Advisors on Science & Technology

Start (estimated time to complete: 15 minutes)

Stratified Medicine Baseline survey – use in the NHS

To take part in survey now, email: john.candeto@concentra.org.uk



The Association of the British Pharmaceutical Industry (ABPI) and the Royal College of Pathologists (RCPATH) are conducting a survey designed to gather professional opinion on the opportunities and challenges associated with the adoption of stratified medicine in the NHS.

Please click [here](#) to access the survey.

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... and all will be revealed at:



Stratified Medicine:
Discovery to Patient – Mind the Gap
ABPI R&D Conference 20 November 2014



REGISTRATION NOW OPEN

ABPI R&D Conference 2014

**Stratified Medicine
Discovery to Patient – Mind the Gap**

Thursday 20 November
30 Euston Square, London

For more information or to view sponsorship opportunities please
Visit the [R&D Registration website](#) or contact Ivan Dennie at idendie@abpi.org.uk / 0207 747 1416

Confirmed Speakers

Professor Mark Caulfield | Genomics England
Professor Dame Sally Davies | Department of Health
Professor John Isaacs | Newcastle University
Alastair Kent | Genetic Alliance UK
Dr Louise Leong | ABPI
Professor Adrian Newland | Barts Health NHS Trust | NICE
Ben Osborn | Pfizer
Tim Pitfield | Janssen Diagnostics
Professor Sir John Savill | Medical Research Council
Professor Adrian Towse | Office of Health Economics
Dr Neil Weir | UCB
Stephen Whitehead | ABPI

A single network for the UK



<http://www.uk-pgx-stratmed.co.uk>

To join for free, email the network manager
Christine McNamee:

cjmcn@liv.ac.uk

Wolfson Centre for Personalised Medicine
University of Liverpool

Thank you