

Exploring a new social contract for medical innovation

June 2015

Report of a workshop held on the 3 June 2015 hosted by the Academy of Medical Sciences and the British Academy, in association with the Medical Innovation Academic Consortium

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The Medical Innovation Academic Consortium

The Medical Innovation Academic Consortium (MIAC) is the academic arm of the Centre for the Advancement of Sustainable Innovation (CASMI), a partnership between Oxford University and University College London, whose aim is to address the issues that have led to current failures in the translation of basic bioscience into affordable and widely adopted new treatments. Drawing together expertise from medicine and medical science, behavioural and social science, economics, ethics and law, MIAC aims to gain a better understanding of the drivers of, and barriers to, the adoption and diffusion of medical innovation.

Disclaimer

This document reflects the views of participants expressed at the meeting and does not necessarily represent the views of all participants, the Academy of Medical Sciences, the British Academy or the Medical Innovation Academic Consortium. For further information, please contact Rachel Quinn, Director of Policy at the Academy of Medical Sciences (rachel.quinn@acmedsci.ac.uk, (0)20 3176 2163).

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Contents

Summary	5
Introduction	7
Cross-cutting themes	11
Emerging priorities	17
Next steps and conclusions	35
Appendix I Programme	37
Appendix II Delegate list	38

Summary

Medical innovation plays a key role in the delivery of a sustainable healthcare system through improvements in diagnostics, therapeutics, medical devices, data analytics and e-health technologies. Yet despite these advances, there are increasing concerns that medical innovation is not helping patients fast enough. This comes at a time when there is growing concern that the current UK healthcare system is unsustainable as it faces challenges such as an ageing population and rising rates of non-communicable diseases.

Stakeholders' perceptions of risk and value contribute to the slow and costly rate of adoption of medical innovation. Stakeholders need to be satisfied that they can expect the benefits they value to outweigh the risks that concern them for medical innovation to be adopted. Failure to meet these expectations will lead to a breakdown of the implicit 'social contract'; an agreement whereby all stakeholders work together to achieve a common good, in this case an effective and sustainable health system, of which a key aspect is the adoption of medical innovation.

On 3 June 2015, the Academy of Medical Sciences and the British Academy, in association with the Medical Innovation Academic Consortium, hosted a joint workshop entitled 'Exploring a new social contract for medical innovation' to explore what is known – and what needs to be known – about how the value and risks of medical innovation are perceived and acted upon throughout society, and if a new social contract for medical innovation is needed.

Discussions centred around the perceptions of value and risk of medical innovation in future healthcare delivery, the importance of the NHS (in the UK context) in any social contract for the delivery of health, and the centrality of public engagement with patients and broader society to any dialogue about the future of medical innovation. In particular, the following priorities emerged:

- **Innovation:** Speeding up access to medical innovation was viewed as a key priority at the meeting. To do so, a better understanding of the underlying drivers and barriers to the adoption of innovations at the individual and organisational level will be essential.
- **People:** The UK has a diverse population that has a range of attitudes to health. This diversity should be embraced in the development of any new social contract and will most likely require the development of a range of healthcare solutions. Patient empowerment was viewed as an asset for health, although a better understanding of the effects of empowerment and individualism on solidarity within the healthcare system, and the influence of lifecourse on decision-making, was thought to be necessary.
- **Data:** The opportunities of big data are considerable. However, barriers associated with data ownership, usage, storage, security, analysis, skills and quality will need to be addressed in order to realise the full potential of the data revolution.
- **Communication/provision of information:** In order to more effectively communicate the risks and benefits of treatments to support informed decision-making and patient engagement, innovations in methods of communication will be required alongside innovation in treatments and technologies. To do so, further research into the ways in which individuals perceive risks and values will be needed.

- **Public engagement:** Giving citizens and patients a voice that is listened to will be essential to ensure that any new social contract for medical innovation is shaped by the views of the wider society. Public dialogue and engagement can be done in different ways at both local and national levels, and healthcare organisations will need to be better prepared to listen to and take account of these views.
- **Costs/financial concerns:** New methods of assessing value will need to be developed to reflect what is most important to individuals beyond the traditional cost-benefit analysis. Tensions between funding healthcare initiatives with long-term goals as opposed to immediate gains will need to be resolved to ensure that investment resulting in better healthcare is appropriately distributed.

Taking into consideration the priorities highlighted above, the following cross-cutting themes also emerged:

- **Trust:** Developing practices that merit trust will be important at a number of different levels, including: in the information that is communicated (i.e. its accuracy and availability, and the ways in which it is communicated); in data usage (i.e. for scientific benefit as opposed to income generation); and in science and its capacity (i.e. being open about what science can and cannot achieve). Further research into the development and maintenance of trust will be important in this regard.
- **Importance of the NHS:** A culture of innovation is needed within the NHS and will help to ensure more consistent and widespread adoption of innovation where this will add value. Encouraging the engagement of individuals with NHS services when they are healthy - in order to minimise periods of future ill-health - will become a priority, particularly as the NHS has finite resources, which will limit the extent to which it is capable of adopting expensive innovations as they emerge.
- **Values:** Understanding the social values that determine attitudes to medical innovation, and which underpin healthcare systems, is essential. Such values need to be better understood to enable effective dialogue and engagement around the costs and benefits of medical innovation.
- **Rights and responsibilities:** Defining, and thereafter communicating, the rights and responsibilities of individuals under any social contract will be difficult, and raises further questions regarding individuals' expectations of the health system, and the impact of individuals failing to meet their responsibilities on their healthcare rights. Engaging society in discussions around their rights and responsibilities will be vital.

Participants felt that future work should focus on: normative issues surrounding the development of a framework for a new social contract that is relevant to both medical innovation and a sustainable healthcare system; and empirical research into individuals' perceptions of risk and value, how these affect behaviour, and how to improve the communication of risks and benefits. Public engagement must underpin these areas of research to ensure that stakeholder views are appropriately taken into consideration for future medical innovation and healthcare systems.

Introduction

There are growing concerns that in the coming years healthcare systems will be unable to meet the increasing demands that will be placed on it, for example by an ageing population in which there are rising rates of non-communicable diseases such as obesity and diabetes.

Medical innovation can help to address these concerns but its current rate of adoption is slow and there are concerns that the adoption of innovation of marginal incremental benefit may be contributing to the accelerating costs of health service provision. Reasons for this relate to the perceptions of risk and value by the stakeholders in this process who need to be satisfied that they can reasonably expect the benefits they value to outweigh the risks that concern them for the current situation to change. Failure to meet these expectations will lead to a breakdown of the implicit 'social contract'; an agreement whereby all stakeholders work together to achieve a common good, in this case an effective and sustainable health system, of which a key aspect is the adoption of medical innovation.

These issues were further explored in a one-day workshop entitled 'Exploring a new social contract for medical innovation' (see Appendix I for the programme) convened by the Academy of Medical Sciences (AMS) and the British Academy, in association with the Medical Innovation Academic Consortium (MIAC).¹ We are grateful to all those who participated in the meeting, in particular the session and breakout group Chairs and the speakers; and to Dr Andrea Hodgetts (MIAC) for leading the drafting of the meeting report.

The workshop featured key stakeholders from across the medical innovation landscape (see Appendix II for a list of delegates), spanning health economics, social sciences, humanities, patient advocates and experts in public deliberations, to:

- Explore the perceptions of risk and value in relation to medical innovation from the perspectives of individuals, organisations and society.
- Understand the interactions between individuals, organisations and society and how each group's actions and needs might influence, or be influenced by, the actions and needs of the others.
- Consider how to engage all groups of stakeholders (in particular individuals who tend to be underrepresented) in dialogue about the future of medical innovation.

The discussions were particularly timely given the Government's current Accelerated Access Review into how NHS patient access to innovative drugs, devices and diagnostics can be accelerated.²

To structure and stimulate discussions, the framework of 4P medicine (predictive, pre-emptive, personalised and participatory – see Box 1) was presented as it is an emergent framework that would catalyse an increasing need to address perceptions of risk and

¹ MIAC is the academic arm of the Centre for the Advancement of Sustainable Medical Innovation (CASMI) www.casmi.org.uk

² <https://www.gov.uk/government/organisations/accelerated-access-review>

barriers to innovation. Workshop participants were divided into three groups to discuss the following stakeholder perspectives:

- **Individuals:** those stakeholders who can make decisions and act independently of others (e.g. citizens and patients).
- **Organisations:** the stakeholders who have a particular purpose in innovation and the delivery of healthcare (e.g. industry, commissioners/payers, NHS Trusts).
- **Society:** those who develop the normative frameworks/policy principles which the other spheres would work to (e.g. NHS England, NICE).

Delegates then reconvened in a plenary discussion session to explore ways in which a wider dialogue about developing the social contract for medical innovation can be initiated.

Box 1 4P medicine

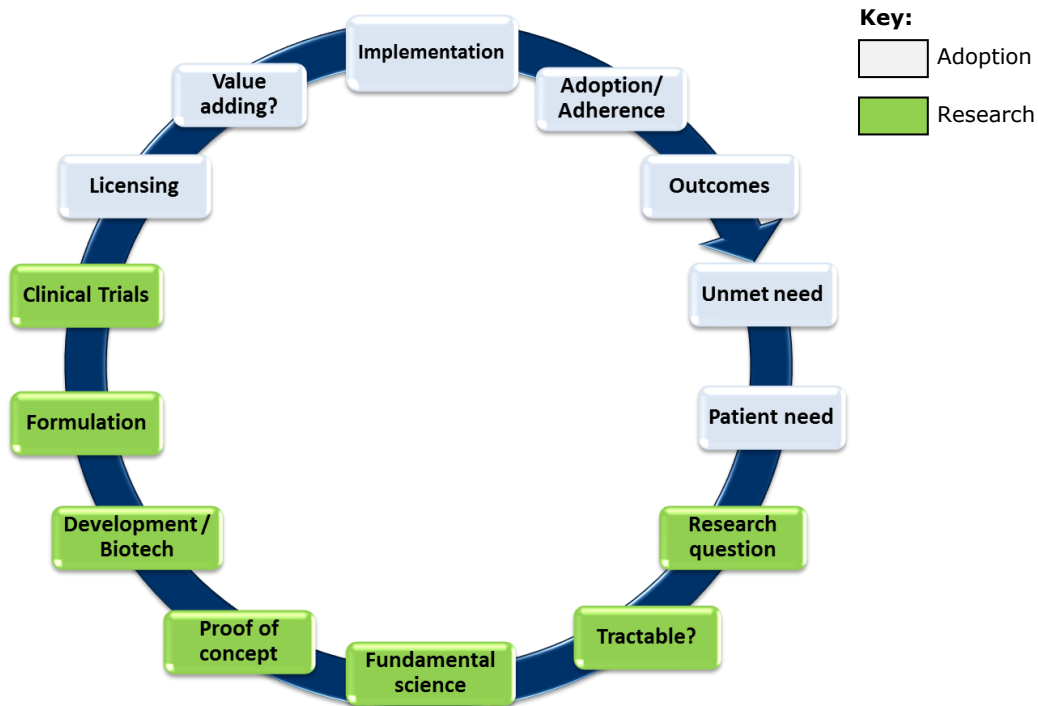
4P medicine, that is predictive, pre-emptive, personalised and participatory, is a healthcare model which seeks to focus on the maintenance of health, rather than just the treatment of illness. It was first described in 2008 by Dr Elias Zerhouni, Former Director of the National Institutes of Health (NIH), USA, with the 4Ps defined as:³

- **Predictive:** Having the ability to determine how, when and in whom a disease will develop.
- **Pre-emptive:** Employing strategies to delay disease onset before symptoms emerge.
- **Personalised:** Using the genotypic, phenotypic and behavioural characteristics of individuals (or their disease) to deliver the right treatments to the right people at the right time.
- **Participatory:** Engaging individuals, communities and healthcare providers in a proactive manner as early as possible and throughout the natural cycle of a disease process.

The model of 4P medicine was used in the workshop as an exemplar of a healthcare model whose delivery will be dependent on medical innovation, but which raises a range of issues regarding acceptability and value that society will need to address.

Medical innovation was taken broadly to mean any proven medical advance that would be required to deliver a healthcare system which aims to prevent as well as treat illness. It was not limited to pharmaceuticals and included, amongst others: new models of care; diagnostics; technologies to monitor health and lifestyle; and ways of engaging the public. The innovation process (Figure 1) was depicted as a continuum with individuals being the key link between research and adoption. Indeed, they are both the 'push' and 'pull' drivers of innovation, stimulating the research that is required to meet unmet clinical need, engaging with the output and delivery of the research, and further informing research needs.

³ Fiscal Year 2008 Director's Budget Request Statement. <http://www.nih.gov/about/director/budgetrequest/fy2008directorsbudgetrequest.htm>

Figure 1 The innovation cycle

The concept of a social contract was introduced at the meeting as a way of reconciling diverse interests in the pursuit of the common good. The idea of a social contract is often just used as figure of speech to refer to any social arrangement or set of institutions on which there is a general consensus as to their workings. When used within political theory, the term social contract carries a number of connotations; there is no universal agreement on what these are, but one strand of thinking includes that the social contract should be multi-lateral, must represent gain for all parties, and that it should establish the obligations on each of the parties as well as providing benefits. With this background, questions that should be asked of a social contract for medical innovation would include:

1. Will the contract be of mutual advantage to all parties? Citizens, patients, professionals, industry, commissioners/payers, professional groups, providers such as the NHS all need to find something of value to them.
2. Who will represent the relevant interests in any social contract? This is particularly important when there are organised interests (the NHS and industry) on one side and the diffuse interests of patients, now and in the future, on the other.
3. Will the parties to the contract be able to make a credible commitment to one another to abide by its terms?

There appeared to be some uncertainty regarding the usefulness of the concept of a social contract and whether the current one had broken down to the extent that a new one is needed. In addition, 4P medicine did not seem to be the most effective paradigm within which to discuss the social contract issue. As a result, rather than focusing on the social contract and medical innovation, much of the discussion considered the challenges faced in ensuring that the NHS applied existing knowledge in order to improve service delivery. This was seen to be as much a priority as thinking about future innovation.

This report is a summary of the discussions that took place on the day and does not necessarily represent the views of the AMS, British Academy, MIAC, nor of all participants present at the meeting. It is divided into two parts: the first describes the broad themes that emanated from the day, namely the importance of the NHS, trust, values, rights and responsibilities, and complexity; the second explores in more detail the main topics that emerged from the discussion, including innovation, data, communication and financial concerns.

Cross-cutting themes

A series of cross-cutting themes emerged throughout the workshop. These were issues that arose in relation to multiple topics and are described below.

Trust

In what is being communicated

A shift from treating sickness to maintaining health will require the provision of new sources of information with respect to health maintenance strategies and lifestyle recommendations. To ensure that it is useful and comprehensible to those accessing it, a better understanding of the practice of communicating healthcare information is needed. Understanding how people receive and interpret health information and what motivates them to do their own research will be just as important as what is being communicated.

In how data will be used

Exploiting patient data for financial gain sits uncomfortably with the public. Participants felt that individuals will want to know that their data are being used for scientific benefit and not treated as a commodity for income generation through its sale.

The 'quantified self' (i.e. data generated from personal health monitoring) could produce data including information on individual adherence to health maintenance strategies. Participants also stressed that there is a fine line between patients voluntarily providing data on, for example, adherence, and the feeling that they are being monitored. If this line is crossed, individuals may feel less comfortable sharing their data. It will be important to respect individuals' privacy preferences.

That there is honesty over what science is capable of

Participants felt that science is very much part of how our society operates, not a separate entity. It is driven by, and is a driver of, health and wealth, and openness to ensure society understands its capabilities is therefore required. There were three aspects in particular where participants thought that transparency would be important:

1. What is not yet achievable in science, so that scientists are not accused of withholding information or misleading the public.
2. The lack of certainty in science, to ensure that the public understand that as theories are tested, and new evidence discovered, there might be variations or changes to information they are given.
3. The rationale for deciding not to provide or to withdraw an innovation, and clear information and supporting evidence on the necessity and benefits of additional actions.

Developing trust

Research into the way trust is engendered and how relationships develop is needed. It was also felt that healthcare professionals should be aware that if trust is built in one part of the system (e.g. between a patient and their GP), this does not necessarily translate to

trust in another part of the system (e.g. between a patient and a researcher accessing their data).

The importance of the NHS

The NHS as a driver of medical research and an adopter of medical innovation

The Health and Social Care Act (2012) resulted in changes to the structure and responsibilities of the NHS.⁴ It is now a legal obligation to use and support research in the NHS. This has been facilitated by the establishment of the National Institute for Health Research (NIHR) and implementation of Academic Health Science Centres (AHSCs) and Networks (AHSNs) to drive research and the adoption of evidence-based practice. By engaging with research, and implementing its findings, the NHS can demonstrate the importance of research and adoption of innovation. However, participants commented that some areas are better at this than others. For innovations to be adopted across the sector and not just within discrete sections, widespread engagement will be required. In addition, to ensure that innovations are adopted uniformly across sectors and geographical boundaries, participants agreed that a culture of innovation adoption needs to be developed within the NHS.

Participants also highlighted that differences in NHS Trusts and Clinical Commissioning Groups' (CCGs) decisions may result in variations to the service provided. Patients can currently choose their referral hospitals with decisions based on multiple factors (e.g. level of care, clinical expertise, proximity, car parking, local reputation etc.). Where choice is made on the basis that innovative treatments are used, options should be available to all centres to equilibrate the level of service offered to patients. Participants thought that there may be lessons to be learnt in this respect from geographical variations in healthcare provision and from devolved administrations. In addition, it was recognised that the devolved administrations could make different decisions regarding healthcare provision.

It is also important to remember that although there is significant potential for innovation, there may be boundaries beyond which the NHS cannot expand to accommodate additional innovation given its finite resources. The NHS will undoubtedly face tough decisions when deciding on how to reconcile tensions generated from increased demand on its service, due in part to an ageing population, with the need to invest in innovation. Participants asked whether the NHS could realistically grow to encompass all of the new possibilities or if CCGs will face increasingly difficult choices when deciding which innovations to adopt.

Are people recipients of, or engagers with, the support provided by the NHS?

Personalised medicine may challenge the current perception of the health service, changing from a disease service to one with which patients engage and feel supported to remain well. To achieve this, individuals will need to have a higher level of engagement with their health. In the current system, engagement when individuals are well is

⁴ Health and Social Care Act (2012).
http://www.legislation.gov.uk/ukpga/2012/7/pdfs/ukpga_20120007_en.pdf

possible, albeit infrequent (e.g. through Foundation Trust membership and online information sources such as NHS Choices). There may be reluctance by those who have taken care of their health to finance treatment of those who have not. However, participants were clear that minimal engagement in maintaining health when well should not reduce the level of service received when ill. Rather, a better understanding is needed of how to change attitudes to encourage engagement when people are well in order to minimise their periods of ill-health. The fundamental principle of the NHS is that everyone has the right to treatment relevant to their needs regardless of income, family background, ethnicity, etc. If a move is made towards personalisation, such equity could be hard to maintain as personalised treatments appropriate for the diversity of patients with different disease drivers (e.g. genetic mutations) might not exist. Systems for how to deal with this will need to be developed.

Values

Recognising that values can change

Values held by individuals, organisations and society are varied and are constantly changing. Medical innovation is also diverse and there is therefore likely to be a variety of views regarding the benefits that innovations bring and how these are valued. Furthermore, as new technologies are developed, new issues might arise and lead to the development of new values.

Social values were central to the founding of the NHS.⁵ Its establishment was underpinned by a desire to treat patients based on diagnosis rather than on what individuals could afford. This was a significant shift in values. It was thought at the workshop that society may be moving towards a situation where the fundamental values underpinning healthcare might change again.

Understanding values to enable effective communication and engagement

For communication to be effective, it needs to be meaningful to the recipient. Individuals' values bring them together, for example in religious groups and other value-based organisations. Participants suggested that such social structures may offer a portal through which it would be possible to engage, and thereby improve communication around medical innovation.

Rights and responsibilities

Participants discussed the rights and responsibilities of individuals under any social contract and raised the following issues:

- How do individuals perceive the existing and any future social contract, and what would its stakeholders gain from it?
- What level of compromise would its stakeholders be expected to undertake? This includes changes needed to secure a common good (e.g. vaccination and screening).

⁵ <http://www.nhs.uk/NHSEngland/thenhs/about/Pages/nhscoreprinciples.aspx>

- Would certain aspects of a social contract be voluntary, while others would be mandatory?
- How would these mandatory requirements be perceived (e.g. something that was of benefit to the individual or to the system)? What level of support would be required to achieve this?

There is no job description for being a citizen and as such, participants recognised the difficulties in deciding and communicating what is expected of an individual with respect to healthcare, for example the level of commitment or self-reliance that is expected which with a new social contract could change from that laid out in the current NHS constitution.⁶ Participants also questioned who should decide on what is required of individuals, what individuals can expect from the health system (see Box 2), and what rights individuals have if they do not meet their responsibilities (see Box 3).

Box 2 What can individuals expect from the health system?

If the healthcare system is moving to a situation where individuals have greater responsibility for their own health, the responsibilities of the health system may need to evolve to make that achievable (for example in terms of support, advice, treatment, etc.)

In these conversations, participants raised difficult ethical questions, including:

- Should there be unlimited access to NHS care or restrictions placed on it relative to individuals' input?
- Should the goal of the NHS be to provide the same level of care to everyone or to offer the treatment that maintains the same level of health?
- Should the NHS provide an end of life support system for those who failed to engage in a healthy lifestyle?

Participants felt that most individuals are reassured in the knowledge that the NHS is available to them should they fall ill. However, most are keen to keep their independence when healthy. This has implications for screening, for example, which encroaches on this independence and is a reminder of mortality and illness.

Box 3 What rights do individuals have if they do not meet their responsibilities?

Participants also questioned individuals' rights if they fail to meet their responsibilities, for example:

- Does a failure to take personal responsibility for health become something blameworthy?
- If individuals choose not to share their data, can they trust that they will still receive the appropriate level of care or will failure to participate be penalised?
- Is there a duty on individuals to try to remain healthy, given that the NHS will be more cost-effective the fewer people who use it?
- Would those that adhere or participate (e.g. through participation in vaccination programmes or by maintaining a healthy lifestyle to combat obesity) get more from the healthcare system while non-adherence would be penalised?

⁶ <http://www.nhs.uk/choiceintheNHS/Rightsandpledges/NHSConstitution/Pages/Overview.aspx>

- What is the health level target to achieve (i.e. how 'healthy' do individuals have to be)?
- How can the level of responsibility in terms of provision of care be measured? For example, can adherence to certain criteria be required for certain treatments to be available?

Participants recognised that defining the rights and responsibilities of the stakeholders would be challenging and to do so would require engaging with all parties to understand what they would be both capable of and agree to doing.

Complexity

The message that surfaced repeatedly throughout the workshop was the complexity of the issues being discussed. The interplay between each of the topics and the underlying themes prevents any of the issues being treated in isolation and demonstrates the difficulties that could be encountered in achieving consensus. What was also evident, however, was the recognition of the need to begin the process of understanding the changes that will be needed to support the sustainability of the healthcare system.

Emerging priorities

Innovation

Diversity in innovation

Medical innovation is often thought about in terms of new pharmaceuticals or medical products. For the purpose of this workshop, participants were asked to take a broad view of medical innovation and include advances such as: new methods of care; timing or method of delivery; treatment tailoring according to individuals' values and their circumstances; advances in lifestyle advice; access to 'big data'; and mobile health technologies.

Accelerating regulation of pharmaceuticals

Schemes to accelerate patient access to new medicines need innovative forms of regulation to protect those involved in the process, be they trial participants, patients or the clinicians prescribing the medication. However, reducing the time to undertake clinical trials (see Box 4) could be viewed with suspicion by the public: reduced periods of testing may be misconstrued as an attempt by industry to get their products to market sooner, thereby generating greater profits, without undertaking rigorous care in product development. To ensure that the changes designed to improve the current regulatory system are not met with scepticism and undue resistance, workshop participants stressed that the need for change should be appropriately communicated.

Box 4 Clinical trials and evolving trial design

Clinical trials represent the process by which the safety and efficacy of medicinal products are assessed. They are subject to strict rules and regulations, including the EU Clinical Trials Directive, which will be superseded by the Clinical Trials Regulation (CTR) when it becomes applicable (no earlier than 28 May 2016).⁷

There is a range of different trial designs depending on the drug to be tested, but all trials are costly and lengthy. In an effort to improve timely access for patients to new medicines, alternative approaches to trial design are emerging. Examples of these are the adaptive pathways approach and Early Access to Medicines scheme.

Adaptive pathways approach⁸

Clinical trials follow a series of phases, going from first in human studies (Phase 0), through the safety and efficacy phases (Phases I-III) to post-marketing stages (Phase IV). They follow a specific trial protocol that is agreed prior to the start of the trial. In the adaptive pathways approach (formerly known as 'adaptive licensing'), trial designs are adapted in response to the outcomes of the early phases. Adaptive studies still abide by the current regulatory frameworks but balance the need for timely access to the medicine with the importance of providing adequate evolving information on its benefits and risks.

⁷ Clinical Trials Regulation (CTR) EU No 536/2014. http://ec.europa.eu/health/human-use/clinical-trials/regulation/index_en.htm

⁸ http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000601.jsp

Examples of this approach include granting initial approval in a well-defined patient subgroup with a high medical need and subsequently widening the indication in a larger patient group, or giving conditional approval for a medicine and collecting post-approval data to improve knowledge of efficacy. Such variations to the standard processes allow trials designed using this approach to be optimised whilst on-going rather than being restricted to a rigid trial protocol.

Early Access to Medicines Scheme (EAMS)⁹

The main aim of EAMS is to provide patients who have life-threatening or seriously debilitating conditions access to new medicines where there are no other viable treatment options. Companies with data from the safety and efficacy phases of their clinical trial can apply for a medicine to be entered into the EAMS. Under the scheme the Medicines and Healthcare products Regulatory Agency (MHRA) will give a scientific opinion on the benefit/risk balance of the medicine which, if favourable, will allow the medicine to continue through the scheme and receive conditional approval for its use in an NHS setting. Licensing of the medicine is still required but the scheme will allow R&D companies to access specific patients to determine the treatment's true clinical value and give patients for whom current treatments are ineffective access to innovative medicines.

The Government's recently launched 'Accelerated Access Review' is further exploring how access to innovative drugs, devices and diagnostics for NHS patients can be accelerated.¹⁰

Innovation in care delivery

Existing models of care delivery often create organisational and professional boundaries that fail to support innovation. Participants suggested that these will need to be more flexible in the future. For example, the separation of health from care services and their disconnect from users' daily lives, is sometimes a barrier. The current healthcare system is subdivided into a series of specialisms, often based on organs or systems. However, as understanding of the genetic basis of disease and its effects on multiple systems grows, disease treatment will likely require greater collaboration across medical teams. This is likely to have implications for professional training and support, in-so-much as healthcare professionals will need to have a much broader understanding of disease processes as opposed to a more specialist, focused knowledge of a particular organ. In addition, whilst the NHS is a public body, a number of its services are delivered by private providers. Private sector provision of healthcare services has always been controversial but the boundaries between private and public providers are rarely as distinct as they are perceived to be. For example, a magnetic resonance imaging (MRI) scanner used to diagnose a disease could be provided by a private company, whilst interpretation of the data it generates and any further treatment that may be needed is undertaken by the NHS. The relationship between private and public healthcare provision and any changes that may result from the adoption of innovation need to be widely understood in order to identify and address concerns more precisely.

⁹ <https://www.gov.uk/apply-for-the-early-access-to-medicines-scheme-eams>

¹⁰ <https://www.gov.uk/government/organisations/accelerated-access-review>

Adoption of innovation

To deal with the issues raised above, the adoption of innovation at a pace that will facilitate the delivery of an efficient and sustainable healthcare system will be vital. However, participants agreed that, when compared to other industries, the medical system is slow at adopting innovation. The reasons for this are unclear but participants suggested that they could include: financial reluctance; professional conservatism and vested interest; historical precedent; or poor articulation of the reasons for change.

To fully understand the process of innovation adoption, an understanding of its underlying drivers and barriers is needed. For example, starting from the perspective of a healthy person, our knowledge of how, when and why they make choices about their healthcare is still limited. In this workshop, the model of 4P medicine was used, part of which supports the prevention of disease. It was suggested that people who have become ill and been treated might fully appreciate the benefits of preventing disease onset, whereas healthy individuals may underestimate the value of preventative care, which (if successful) would mean ill-health would not affect them. Participants highlighted that people often do not react to information about risk susceptibility by changing behaviour. As a result, it was suggested that there is little incentive or market for prevention. A better understanding of why individuals make certain decisions, utilising research into behaviour change, could thus lead to an enhanced understanding of how to incentivise innovation adoption.

Similarly, denial of symptoms is a common response in individuals experiencing health issues. Yet disease treatments often have a greater chance of success, with an associated reduction in both financial and personal cost, if started as early as possible in the disease process. Understanding how to overcome human denial of symptoms or illness is needed to inform ways of ensuring individuals' full engagement.

People**Population heterogeneity**

The UK has a diverse population within which there are differences in cultural background, levels of education, wealth (including income) and attitudes to health. This diversity poses challenges in terms of reaching a sufficient degree of consensus to establish a social contract for medical innovation. Participants discussed the cultural theory of risk, which suggests that there are a limited number of individual approaches to how risks and value are distributed with only four major groupings of behaviours that will need to be accommodated (see Box 5).¹¹ Many participants thought that a social contract that is acceptable to these four behaviour groups would likely be acceptable to most tranches of society. Ensuring the population participates in the maintenance of their health is, and will be, the most challenging aspect of healthcare. Indeed a 'one size fits all' approach cannot be adopted and a range of solutions will need to be developed to accommodate the diverse population. Participants stressed that fully acknowledging cultural and societal variation will be important to prevent any perceived or actual prejudice. If a new social

¹¹ Douglas M & Wildavsky A (1982). *Risk and Culture: An Essay on Selection of Technological and Environmental Dangers*. California University Press. Berkeley.

contract is developed, it should therefore not strive for consensus too soon but take all these factors into account.

Box 5 Cultural theory of risk¹²

People do not know all the risks that affect them, and for the risks they are aware of, they may not fully understand their nature, seriousness or consequence. Managing individuals' reaction to risk relies on understanding their awareness of risks, their perception of the severity of those risks, and their likely response to them. Cultural theory of risk seeks to describe how individuals respond to risk and the conflicts that arise due to the different reactions. The framework it uses subdivides people into four groupings: hierarchists, individualists, fatalists and egalitarians, with their approaches to risk described as:¹³

- **Hierarchists:** Accept risk as long as the decisions regarding them are made by experts.
- **Individualists:** See risk as an opportunity – with no risk, there would be no opportunity for personal reward.
- **Fatalists:** Do not knowingly take risks. They would only get hurt and there is little prospect of reward.
- **Egalitarians:** Wary of risk. The good of the group is more important than the good of the individual, and risk-taking could upset the equity within the group.

Whilst differing views are held by the four groups it should be remembered that individuals rarely sit entirely within a grouping and each group needs the other groups to either make up for its deficiencies, to exploit or to define itself against.

Understanding the different ways in which the groups perceive risk and the flexibility of their reactions could help to achieve the consensus on perceived risk acceptability that would be necessary for a new social contract.

Importance of individualism

The importance of individualism was widely recognised at the workshop with the understanding that each individual has the right to make their own decisions and exert their autonomy. Participants were in agreement that individualism is not the same as being selfish and does not preclude them being concerned about society.

Patient empowerment

Patient empowerment was argued to be an asset for health, with new technology and innovation enabling empowerment. The rise in mobile technology to support personal health monitoring and increased provision of health and lifestyle information all serve to educate and advise people on how to maintain their health. In return, the healthcare system can learn and improve practices from data fed into these apps (see section below on data for more information). Participants agreed that whilst the NHS should recognise and facilitate this innovation, the appropriate regulation/systems need to be in place to

¹² Douglas M & Wildavsky A (1982). *Risk and Culture: An Essay on Selection of Technological and Environmental Dangers*. California University Press. Berkeley.

¹³ Strictly speaking, the classification is fivefold, as it also includes the Hermit's way of life, in which individuals draw back from all four groupings.

ensure that information is accurate and appropriate, with the necessary support provided so that any decisions individuals make are from an informed standpoint.¹⁴

Participants stressed that patient empowerment should not be seen as a substitute for care or as a means to offer a minimal health system. The aim would be for healthcare services and individuals to work synergistically to optimise health outcomes.

Influence of the lifecourse on decision-making

Supporting individuals to develop their attitude to both solidarity with others who need health services (see Box 6) and also their stake in sustainable medical innovation may depend on the point they are at in their lifecourse, rather than in a snapshot of an acute episode of care. There was widespread agreement that the stage an individual is at in their lifecourse may influence decision-making processes, and that a better understanding of this influence is necessary. The perception of one's own health is likely to change throughout their lifecourse. Indeed, individuals tend to suffer from a 'present bias', meaning that they might not adopt health behaviours that are fully consistent with long-term aims, which might impact on their willingness to engage in preventive health measures.¹⁵ Additionally, it was suggested that long-term health behaviours might be more likely to be adopted by younger individuals who have longer to benefit from them, whereas their value may not be perceived to be as great in the elderly. Periods of ill-health were also thought to impact on decision-making in terms of appetite for risk and willingness to engage.

Box 6 The consequences of patient attitude on solidarity within the healthcare system

One definition of solidarity describes it as the shared practices reflecting a collective commitment to carry costs (financial, social, emotional, or otherwise) in order to assist others.^{16,17} When applying this to healthcare, the sustainability of the healthcare system may depend on a shared belief that it is for a common good, and a willingness to enter into an 'insurance pool' in which everyone agrees to pay knowing that only some might need to make major demands on the services. Workshop participants raised questions about the potential consequences for the healthcare system should such feelings of solidarity break down. Two scenarios where this might occur were envisioned:

- Those who are healthy and do not need to access healthcare services could eventually perceive the NHS as being effectively for someone else's good but not their own. This could impact on individuals' willingness to contribute and ensure its sustainability.
- Alternatively, knowledge of disease susceptibility may lead to feelings of solidarity with current sufferers, raising individuals' inclination to contribute – for personal rather than societal gain.

¹⁴ Academy of Medical Sciences (2014). *Health apps: regulation and quality control*. <http://www.acmedsci.ac.uk/viewFile/552cc937dcfb4.pdf>

¹⁵ O'Donoghue T & Rabin M (2000). *The economics of immediate gratification*. *Journal of Behavioral Decision Making* **13**, 233–250.

¹⁶ Nuffield Council on Bioethics (2011). *Solidarity: reflections on an emerging concept in bioethics*. http://nuffieldbioethics.org/wp-content/uploads/2014/07/Solidarity_report_FINAL.pdf

¹⁷ <http://nuffieldbioethics.org/report/solidarity-2/definition-of-solidarity/>

For further information on this concept, please see the Nuffield Council on Bioethics' report on solidarity.¹⁶

Issues of equality

Participants stressed that there is an inherent unevenness in individuals' abilities to both find and assimilate information that will assist in decision-making. Understanding how to support and engage individuals who are less able to carry out these activities is fundamental to building equality. Personal wealth is also uneven and is not randomly allocated – often with individuals with higher levels of education being wealthier. Wealthier individuals are more likely to invest in preventative care, and are better able to afford care and treatment not offered by the NHS.¹⁸

Participants expressed concern that Intervention Generated Inequalities (IGIs – see Box 7) exist which could be exacerbated by some technologies, especially if access to them is not equitable. This is not a new problem and the current system of care is known to be uneven. However, for the construction of a social contract that all groups have a reason to sign up to, it is important that the construct is equitable.

Box 7 Intervention Generated Inequalities

In introducing healthcare interventions, care needs to be taken that they do not result in increased health inequalities. This happens when an intervention being implemented is of greater benefit to advantaged groups (that are at lower risk) than to disadvantaged groups for whom the risk is higher.¹⁹ These are known as Intervention Generated Inequalities (IGIs) and can arise at a number of points in the implementation process including the assessment of need for an intervention, its advertisement, the point at which it is delivered, or in the way that it can be accessed. IGIs may serve to either introduce a health inequality or widen one that already exists.

An example of an IGI could be the use of a survey in a needs-assessment for a particular healthcare service. Socio-economic variations are known to impact on the response to surveys, with those of a lower socio-economic position (SEP) being less likely to respond.²⁰ As such, using this method of assessment means the need for the service could be underestimated in that population purely due to lack of response rather than lack of need, with subsequent provision being mistakenly directed towards those of higher SEP. In practice, attempts to address this will be made that focus on weighting responses so that they reflect the population in which they are being measured.

¹⁸ Scholz JK & Seshadri A (2012). *Health and Wealth in a Life-Cycle Model*. <http://www.mrrc.isr.umich.edu/publications/papers/pdf/wp224.pdf>

¹⁹ Lorenc T, *et al.* (2013). What types of interventions generate inequalities? Evidence from systematic reviews. *Journal of Epidemiology and Community Health* **67**, 190-193.

²⁰ Turrell G (2000). *Income non-reporting: implications for inequalities research*. *Journal of Epidemiology and Community Health* **54**, 207-214.

Data

Issues associated with big data

The opportunities presented by big data are immense (see Box 8). Participants acknowledged, however, that barriers associated with big data still remain and that these would need to be overcome in order to fully realise its potential.²¹ Issues discussed include the following:²²

- **Data ownership:** participants felt that there is a lack of clarity over who owns and manages data. Within the NHS, GPs are regarded by some as having a protective attitude to patients, which extends to patient data. However, data are not only held within government systems. As mobile technology and health-related apps become more popular, data privacy conditions may be overlooked and users may be unaware that companies not directly involved in healthcare provision (e.g. Google, Apple and/or others not yet in existence) may ultimately decide on what happens to data within their contractual arrangements with their users.
- **Data usage:** there is a central issue of trust in how patient data is used. Whereas strict regulations are in place within NHS systems, data from the 'quantified self' (i.e. data generated from personal health monitoring), health apps and other technologies may be subject to less strict policies. Workshop participants identified a range of issues arising from this, including:
 1. Whether data should be treated as a commodity.
 2. User awareness of health app data policies.
 3. Whether informed consent has been given for sharing data.
 4. How to balance the issues raised above with the potential health benefits of using such data.
- **Storage capacity:** medical innovation is likely to be driven by data and therefore large volumes of data will be generated and greater storage capacity will be required.
- **Security:** questions were raised about how appropriate levels of security could be achieved (for example, anonymisation, generation of very large datasets where individuals' data are effectively 'hidden' in amongst the volume of data, etc.).
- **Analyst skillset:** analysis of the data requires an adequately skilled workforce, which will have implications for training and capacity building.²³
- **Quality:** to allow for meaningful research, the quality of data and the analytical techniques used to mine them will need to be sufficient, and mechanisms put in place to account for potential disparities in data quality.

Participants also highlighted the need to carefully manage patient expectations in situations where tests are unavailable, inappropriate or could give ambiguous results. Additionally, the risk of overpromising through diagnostics and genetics exists and care must be taken to ensure individuals understand that identification of disease susceptibility

²¹ Academy of Medical Sciences (2014). *Health apps: regulation and quality control*. <http://www.acmedsci.ac.uk/viewFile/552cc937dcfb4.pdf>

²² These issues are also relevant to so-called 'real world evidence', a subset of big data, and are being explored by the Academy of Medical Sciences: <http://www.acmedsci.ac.uk/policy/policy-projects/real-world-data/>.

²³ See the British Academy (2015) *Count Us In* report, which sets out the need for better skills in handling data, especially in order to take advantage of the opportunities presented by big data: http://www.britac.ac.uk/policy/count_us_in_report.cfm

and adoption of mitigating lifestyle factors does not completely remove the risk of that disease.

To embed the acceptance of big data and what it can achieve, participants suggested that embracing technology should be on an incremental basis. Initially the use of mobile technology to keep people healthy could be encouraged and then, as this becomes commonplace, data sources could expand incrementally (for example, over the counter diagnostic tests could be fed into the system enabling diagnostics to be linked with longer term health data to support improved health outcomes).

Box 8 Big data and its opportunities

The Parliamentary Office of Science and Technology defines big data as '*data with characteristics that make data collection, processing, analysis or interpretation a challenge; often requiring the use of innovative techniques*'.²⁴ The types of data that are included within this definition vary according to the context in which the term is employed and can comprise data from large databases (e.g. search engines, social media activity, loyalty cards, etc.) to data from more specialised databases (e.g. in the biomedical sphere, the Clinical Practice Research Datalink database, human genome data, biobanks, electronic health records, etc.).²⁵

Big data has been identified both in the UK and the EU as having the potential to drive future economic growth. As such, both the UK Government and the European Commission are investing in research and infrastructure to support the use of big data.²⁶ Opportunities for big data are wide-ranging spanning multiple sectors including business, security, energy consumption, public health, education, construction and transport.

Participants discussed various opportunities for use of big data within the biomedical landscape, including the following:

- **For the public:** The data revolution has massive potential to change current professional/healthcare structures. Indeed, the rise in the use of NHS Choices, which asks a series of questions to identify a person's ailment, shows that individuals will seek information from online sources.²⁷ There may be a role for peer-review algorithms in the future. For example, the computer system Dr Watson (IBM) analyses the scientific literature every 15 minutes and self learns.²⁸ Individuals input symptoms and get a diagnosis. It is currently being tried at the Mayo Clinic and has the potential to revolutionise care. Some may argue however, that these types of technologies should not be a substitute for personal care.
- **For researchers:** There are many instances where analysis of real world, big data could result in benefits to patients, ranging from, but not limited to the identification of

²⁴ POSTnote (2014). *Big Data: An Overview*.

<http://researchbriefings.parliament.uk/ResearchBriefing/Summary/POST-PN-468#fullreport>

²⁵ <http://www.cprd.com/intro.asp>

²⁶ European Commission (2014). *Towards a thriving data-driven economy*. <http://eur-lex.europa.eu/legal-content/EN/TXT/PDF/?uri=CELEX:52014DC0442&from=EN>

²⁷ <http://www.hscic.gov.uk/article/5161/One-billion-visits-to-NHS-Choices-website>

²⁸ <http://www.ibm.com/smarterplanet/us/en/ibmwatson/what-is-watson.html>

genetic mutations, detecting patterns in disease progression or analysing trial data and effectiveness of treatments. Where groups of individuals choose not to share their data (for example in particular ethnic or demographic groups), this may limit the applicability of the analysis.

Communication, information and understanding

Understanding and perception of benefits and risks

Whilst individuals need to have a good understanding of risk for informed consent to be given, participants stressed that this is sometimes done at the detriment of communicating the benefits. They suggested that participation could be increased if the two were better balanced and that conversations should focus on the benefit/risk balance, rather than solely discussing potential risks.

Although individuals' engagement is strongly influenced by their own perception of risk and value, currently little is understood about how risks and values are perceived by individuals. For example, it is often assumed that stratified/personalised medicine will be well received, but it is not actually known if this is the case.^{29,30} Patients' engagement with treatment is influenced by their own perceptions (e.g. necessity, beliefs and concerns) but little is understood about how information about the risks and benefits on treatment impacts on beliefs and behaviours.³¹

Individuals' implicit evaluations of the risk and benefits of prescribed treatments might appear irrational from the medical perspective, but research has shown that these are often linked to perceptions of treatment that have a 'common-sense rationality' even though they may not be medically accurate (for example the patient who stops taking an essential medicine that relieves symptoms because they believe their treatment is no longer necessary when they feel better).^{32,33} Initiatives to communicate the benefits and risks of interventions may be more effective if patients' beliefs and behaviours are taken into account. There was widespread agreement among participants that more research is needed into how the risks and benefits of current and future treatments can be best communicated to facilitate informed treatment choices and optimal engagement with appropriate prescriptions.³⁴

²⁹ Technology Strategy Board (2011). *Stratified Medicine in the UK Vision and Roadmap*. <https://connect.innovateuk.org/documents/2843120/3724280/Stratified+Medicines+Roadmap.pdf/fb639848-282e-4619-a960-51e3a16ab893>

³⁰ Green D, Horne R & Shephard EA (2013). *Public perceptions of the risks, benefits and use of natural remedies, pharmaceutical medicines and personalised medicines*. *Complementary Therapies in Medicine* **21**, 487-491.

³¹ Horne R, et al. (2013). *Understanding patients' adherence-related beliefs about prescribed medicines: a meta-analytic review of the Necessity-Concerns Framework*. *PLoS ONE* **8**, e80633.

³² Horne R, et al. (2007). *Patients' perceptions of Highly Active Antiretroviral Therapy in relation to treatment uptake and adherence*. *Journal of Acquired Immune Deficiency Syndromes* **45**, 334-341.

³³ Cooper V, et al. (2009). *The influence of symptom experiences and attributions on adherence to Highly Active Anti-Retroviral Therapy (HAART): a six month prospective, follow-up study*. *AIDS Care* **21**, 520-528.

³⁴ The Academy of Medical Sciences has recently launched a new workstream on 'How does society use evidence to judge the risks and benefits of medicines?' to further explore some of these issues: <http://www.acmedsci.ac.uk/policy/policy-projects/how-does-society-use-evidence-to-judge-the-risks-and-benefits-of-medicines/>

Innovations in methods of communication were deemed to be necessary alongside innovation in treatments and technologies. Participants suggested that the effect of communication on the perception of risk and value should be investigated with questions including:

- How do individuals and healthcare professionals understand risk?
- Is there a common language of risk between healthcare professionals and individuals?
- How can information about risk be communicated effectively?

Participants proposed that healthcare professionals should be thought of as brokers of information that guide individuals through all the information they encounter.

Sources of information

The availability of information is important for patient empowerment and shared decision-making. Questions were raised about the accuracy and reliability of information, how it could be monitored, and how to deal with conflicting sources or misleading information. The need to understand how the accuracy of the information is ascertained by both patients and healthcare professionals was recognised, along with how individual preferences are understood. Participants also asked what the consequences of acting against patients' wishes might be in instances where the patient's demand may be inappropriate, considering that this may occur as a result of increased patient engagement with their own health.

Understanding adherence to treatments

The language used regarding adherence to treatments was recognised as being particularly important, as small changes in adherence could have a major impact on the success of treatments and/or behavioural/lifestyle changes in preventive health strategies.³⁵ Whilst non-adherence may be unintentional, it can also represent a choice by a patient not to engage with the treatment.³⁶ Such intentional decisions are often related to individuals' beliefs about illness and treatment. As such, a better understanding is needed of how to take account of these beliefs when presenting and communicating treatments.

Public engagement

Participants explored ways in which a wider dialogue about developing a new social contract for medical innovation could be initiated, and how to ensure that future discussions and recommendations are shaped by the views of the wider society, during a panel session with the following experts:

- **Professor Sarah Cunningham-Burley**, Professor of Medical and Family Sociology, University of Edinburgh (**public perspective**)

³⁵ The Academy of Medical Sciences held a workshop to consider issues pertaining to patient adherence to medicines. A full meeting report summarising the main discussion points is available at: <http://www.acmedsci.ac.uk/viewFile/552f6b3fdab3a.pdf>

³⁶ Horne R, et al. (2006). *Concordance, Adherence and Compliance in Medicine Taking: A conceptual map and research priorities*. Report for the National Co-ordinating Centre for NHS Service Delivery and Organisation R&D: http://www.netsec.ac.uk/hsdr/files/project/SDO_FR_08-1412-076_V01.pdf

- **Mr Derek Stewart OBE**, Associate Director for Patient & Public Involvement , National Institute for Health Research Clinical Research Network and a patient advocate (**patient perspective**)
- **Dr Sarah Castell**, Director, Ipsos MORI (**dialogue practitioner perspective**)
- **Ms Alyson McGregor**, Director, Altogether Better (**community perspective**)

The infographic on pages 28 & 29 provides a visual summary of these discussions.

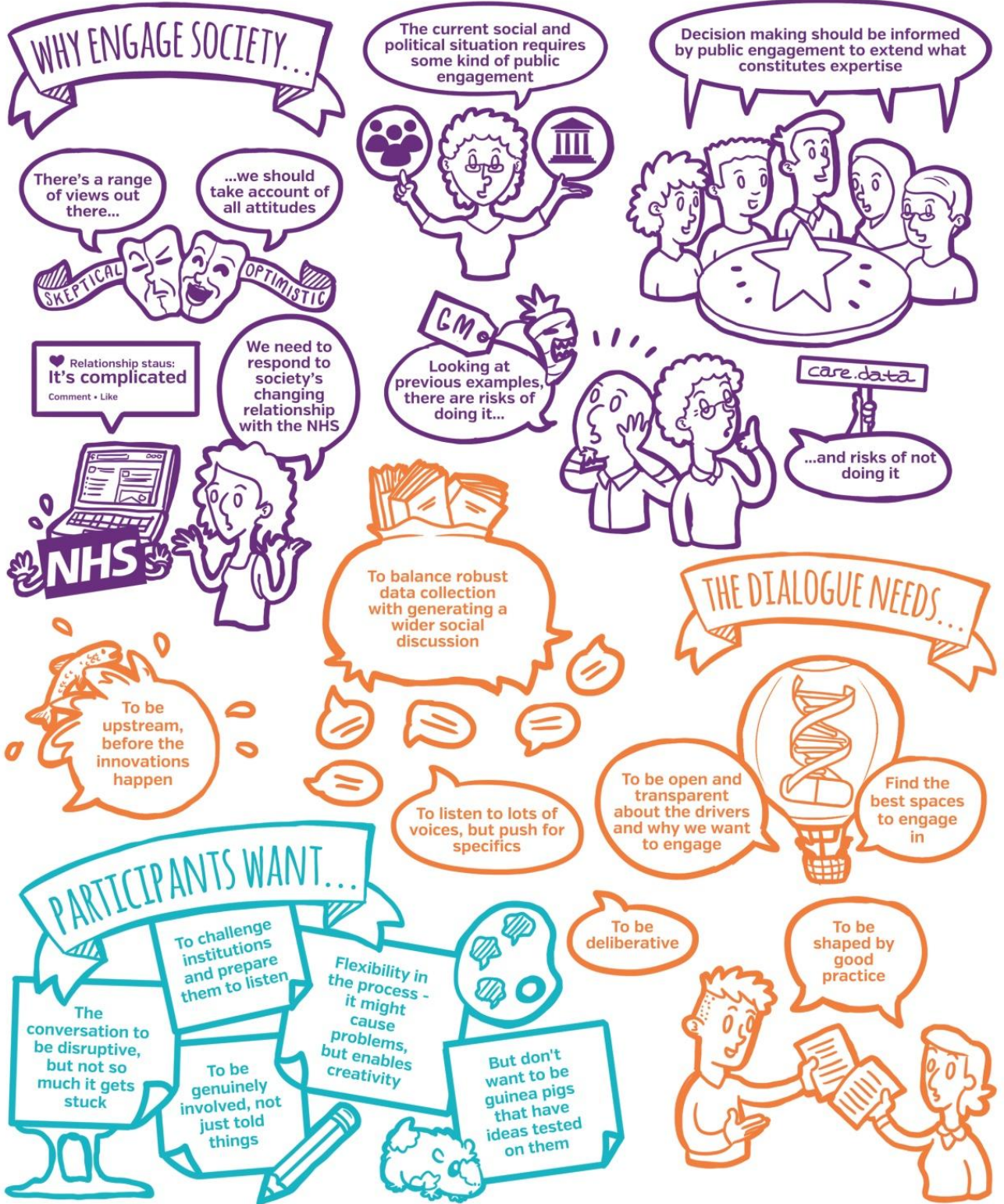
During this session, Professor Sarah Cunningham-Burley emphasised the importance of public engagement, particularly in the current social and political climate. She stressed that the whole range of public attitudes needs to be taken into account to inform decision-making. As such, it is important to think about the timing and location of engagement so that a broad range of views are considered, not just those that are most prominent or easily represented. Professor Cunningham-Burley recognised that engagement may at times be a double-edged sword. Indeed, past examples illustrate the challenges of both pro-actively engaging with the public, as in the case of mitochondrial donation, and those associated with not engaging sufficiently, as was the case for care.data (see Box 9). She also highlighted that dialogue needs to be deliberative and nuanced, and should ideally occur at the beginning of the innovation process in order to better understand the implications for society, by exploring how people's views and values will determine what innovations citizens want. This is key to facilitating implementation. She outlined three different forms of dialogue:

1. **Awareness-raising:** to provide information to drive trust.
2. **Consultative:** to gain an insight into individuals' views.
3. **Empowering:** where the public play a role in the decision-making or research process.

Box 9 Dialogue around the research process, the healthcare system and the use of patient data

It was thought that better communication and wider discussions about the research pathway, how the NHS and treatments are funded (e.g. the Cancer Drugs Fund), and how an individual's personal choices can impact both on themselves and on the wider society (e.g. the loss of herd immunity resulting from parents choosing not to vaccinate their children) were required. A recurring example was the furore surrounding care.data. Many participants felt this could have been avoided if citizens were engaged earlier in discussions to explore public views and values on the risks and benefits to the individual and society as a whole.

There is little understanding of the nature of public concerns about the collection and use of patient data. Without creating opportunities for citizens to consider these issues together with health professionals and policymakers, future communications regarding any potential benefits to individuals and society cannot not be optimised. There is need for greater public dialogue about, and research into, these issues to create an evidence base for communicating future initiatives that hope to make use of patient data.



act for medical innovation

THE FUTURE OF MEDICAL INNOVATION



A WORKSHOP CONVENED BY THE ACADEMY OF MEDICAL SCIENCES AND THE BRITISH ACADEMY, IN ASSOCIATION WITH THE MEDICAL INNOVATION ACADEMIC CONSORTIUM

The importance of giving patients and the public a voice, particularly in the development of a new social contract, was echoed by Mr Derek Stewart OBE. He felt that the motto '*every patient a research patient*' resonated with the public and that involvement in research can lead to positivity about the process. Individuals generally want to feel genuinely involved, and do not appreciate simply being told things or having ideas tested on them. It was acknowledged that the NIHR has set up and funds 'Involve' an organisation whose aim is to support public involvement in NHS, public health and social care research and plays an important role.³⁷ Participants agreed that dialogue needs to be ongoing and deliberative with shared decision-making: there is still considerable progress to be made to achieve the Government's position of '*no decision about me without me*'. Involvement was also felt to be an opportunity to raise awareness of the pressures on research and the NHS, and to have an open dialogue about the costs of healthcare.

Ms Alyson McGregor further highlighted the pivotal role citizens and patients can play in the design and generation of new models for general practice that deliver better healthcare and health outcomes. She described Altogether Better's network of volunteer health champions who work with their communities to transform health and well-being.³⁸ She stressed the need to more effectively involve patients and citizens in discussions around healthcare delivery in GP practices, which should be more open, reflective and engaging. She highlighted that such conversations can be very effective: indeed, they had resulted in a reduction in the number of GP appointments and accident and emergency attendance, and increased registration at a GP practice she had previously worked with.

Dr Sarah Castell gave participants an insight into how public dialogue on medical innovation and a new social contract could be led. She outlined a spectrum of types of engagement, from robust data collection to generating a wider social discussion. The type of engagement will have a profound influence on the methods, structure, design, costs, outputs and stakeholders to involve in the process. In any dialogue work, it is important to clearly identify the aims of the study and the research questions upfront, and to focus on the specifics to avoid unnecessary duplication of previous work.

However, participants questioned whether most institutions involved in healthcare and health research were configured to take account of citizens' views. Some organisations are making progress on this front, for example NHS Citizen has been set up to gather feedback on potential improvements to the NHS, and NICE's Citizens Council provides a public perspective on moral and ethical considerations when producing guidance.^{39,40} It was thought that public dialogue should play a more central role in the activities of the National Academies, which should explore how to make better use of their Fellowships in conversations with the public. Participants also acknowledged that the costs associated with public engagement can sometimes be prohibitive, and that any type of dialogue needs to be constructive, not purely used as a complaint mechanism.

³⁷ <http://www.invo.org.uk/>

³⁸ <http://www.altogetherbetter.org.uk/home.aspx>

³⁹ <http://www.nhscitizen.org.uk/>

⁴⁰ <https://www.nice.org.uk/get-involved/citizens-council>

Costs/financial concerns

Interpreting and measuring value

An important component of the workshop discussions surrounded the issue of how to measure 'value'. One of the most important factors in innovation and related topics is the Value Proposition, defined as outcomes/cost. The standard approach taken in health economics, and often adopted by decision-making authorities, is to apply cost-benefit analysis (CBA). CBA aims to translate a wide set of considerations relating to the impact of a particular project into monetary measures of its costs and benefits to society, thereby providing a guide for choosing between competing projects and for evaluating whether individual projects provide a net gain to society. CBA is derived from so-called utilitarian welfare analysis which weights together the welfare of different individuals in a linear manner and it typically uses market prices to estimate the value and costs of goods.

It is well-recognised that standard CBA may be problematic when applied to valuing human life, and authorities such as NICE therefore use non-monetary variants of CBA, such as Quality Adjusted Life Years (QALY). This allows the application of a common criterion to all patients when assessing the value of investing in treatment – while reflecting the fact that some might feel the benefit of that treatment for longer than others.

However, while participants recognised that CBA has provided a very valuable tool for decision-making, they also highlighted that there might be a need for broadening of concepts going beyond traditional CBA. One particular issue concerns the measurement of value. It may often be hard to place a monetary value on a situation affecting individuals' circumstances such as dignity in death, which typically cannot be priced in the market. Patient empowerment was mentioned as another relevant complication, since empowerment affects individuals' welfare. But again, it is not easily translatable into a commodity that can be priced and therefore incorporated in a CBA.

Participants felt that if there is to be a change to the social contract, new methods for assessing value will need to be developed that go beyond quantitative measures of costs and gains.

Externalities

Traditional applications of CBA are built on the assumption that market prices can be used to evaluate costs and benefits. A difficulty relates to the fact that individuals or organisations may often be affected by actions taken by others which are not priced in the market, so-called externalities. Externalities can be positive (individuals may for example appreciate living amongst healthier neighbours creating a positive externality from health provision) or negative (such as the impact of smoking on non-smokers). A common difficulty is that the external effects by their very nature are very hard to price precisely because they most often are not traded in the market. An additional difficulty is the interrelation between projects – an investment in one drug or in one type of treatment can have important consequences for the attractiveness of other projects. Such interrelations may not be confined only to different drugs or different treatments but also to other parts of society. For example, delaying the onset of dementia can impact on the

attractiveness of pension reforms as it affects individuals' ability to partake in an active work-life. Both these types of considerations are important for health related issues and complicate considerations when applied to decision-making of particular projects in isolation, unless effort is made to take the broadest possible view thereby facilitating the inclusion of external effects.

A key question that stemmed from this theme was that if the extended welfare analysis is to be included when measuring value, then who should be responsible for applying it appropriately? NICE uses cost effectiveness analysis using QALY as a measure of benefit. Taking a broader view, which may encompass effects not accounted for by the QALY, whilst important, may produce a less transparent principle for decision-making which could be problematic in terms of accountability.

The report has already touched on 'present bias' (see page 21) and 'common-sense rationality' (see page 25), issues that can have a significant effect upon long-term policy planning. If individuals fail to behave rationally, it might be important to consider how to implement health policies in a way that incentivises individuals to reap their fullest benefits for minimal economic impact. Examples of this, such as 'nudging' are already applied in practice (albeit subject to debate regarding its efficacy) and there was a view that such considerations perhaps should be given more weight.⁴¹

Financial models

A range of issues associated with financial modelling of innovation were raised. The first of these related to the impact of externalities mentioned above. External effects are important for many issues related to health because health outcomes impact on other sectors of the economy and because health issues depend on other policies. For example, social services may incur costs for adaptations to a person's home to make the environment safer, but the NHS reaps the benefits from the reduction in falls or other accidents in the home. In the current system, it is not customary to take a broader view thereby allowing decision-makers to take full account of such external effects. When applied to innovations in healthcare, without understanding the impact of externalities, the true costs and benefits of these interventions will not be known.

A second key issue relates to the 'financial model' for industrial drug development. Participants recognised the need for having a variety of different financial arrangements available as different medicines may require different funding schemes. The financial model for a therapeutic agent that could be taken by a large proportion of the population (e.g. statins), would need to be very different than for a new antimicrobial where industry will be rewarded for a treatment that either will not be used or would be used very sparingly.

A key issue of concern in terms of financing relates to truly transformative treatments, in particular if they cater for only a small number of people. It is hard to evaluate the potential impact of transformative treatments because their potential impact is by definition unknown. The private sector may be unwilling to invest in such innovations because the perceived risk is high while society could potentially gain a lot. This would

⁴¹ Thaler RH & Sunstein CR (2009) *Nudge*. Penguin. London.

seem to indicate a potential role for public financing or for public private partnerships (PPP), but this requires high expertise on the part of the public sector in terms of project evaluation. When these transformative innovations cater for very few there are added complications. Participants used the example of ivacaftor for the treatment of cystic fibrosis (see Box 10) to illustrate some of the ethical questions that might arise as more personalised medicines of benefit to restricted patient populations emerge, including the following:

- How might an expensive treatment that is truly transformative to only a small proportion of patients impact on the larger patient population?
- Would scarce resources be better spent on developing drugs aimed at extending life expectancy for a larger proportion of patients, even if these have only marginal benefits?

Investment in long-term goals

Participants noted that the difficulties in investing in innovations with long-term impact on health may be considerable. CCG budgets are often allocated on a year-to-year basis which creates an incentive to invest in projects which pay off relatively quickly. A similar bias towards investing in projects with a more immediate pay off may come from democratic institutions due to electoral cycles. Thus it seems likely that ambitious projects with high long-term benefits but lower immediate returns are underfunded. Provisions need to be made for assuring that high return long-term health projects are properly funded.

Box 10 Ivacaftor and the treatment of cystic fibrosis

Ivacaftor (trade name: Kalydeco), produced by Vertex Pharmaceuticals, is prescription medicine for cystic fibrosis (CF) patients.⁴² It has transformed the treatment of this condition, for which there was previously limited therapeutic options. It works by enabling chloride channels in lung cells to open more frequently, thereby allowing more chloride ions to move in and out of cells. The movement of chloride ions is thought to help balance salt and water content in the lungs, resulting in improved lung function. However, it is only effective in patients that have one of the following mutations in their CF gene: G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, or S549R. Patients with such mutations only account for a very small proportion (approximately 4%) of all CF sufferers, and treatment with ivacaftor costs over £180,000 per patient per year.⁴³

⁴² <http://www.kalydeco.com/>

⁴³ NHS Commissioning Board (2012). *Clinical Commissioning Policy: Ivacaftor for Cystic Fibrosis*. <http://www.england.nhs.uk/wp-content/uploads/2013/04/a01-p-b.pdf>

Next steps and conclusions

The workshop was entitled 'Exploring the social contract for medical innovation' but, as discussion evolved during the workshop, no distinction was made by participants between medical innovation and the sustainability of the current healthcare system. There appeared to be some uncertainty regarding the concept of a social contract. Despite participants being asked directly if they thought there was a requirement for a new social contract for medical innovation, there was no clear indication that they thought the current one had broken down. As such, it is difficult to draw firm conclusions without further consideration of the following points:

1. Whether medical innovation and a sustainable healthcare system are actually separate issues or aspects of the same one.
2. Whether they are competing alternatives or co-dependent.
3. Whether this is a case of different timescales, one concerned with immediate problems (healthcare system sustainability) and one with a longer term horizon (medical innovation).

In addition to understanding these issues, participants broadly supported three related workstreams arising from the workshop, as described below.

1. Normative issues

The first workstream on normative issues would deal with the norms and values that underpin the framework upon which a new social contract will be based, relevant to both medical innovation and developing a sustainable healthcare system. Focusing on issues such as the rights and responsibilities of the stakeholders, this workstream would seek to answer the key questions relating to the expectations placed on stakeholders, examples of which are outlined in Boxes 2 and 3 (page 14).

2. Empirical research

The second workstream would require empirical research to understand perceptions of risk and value, how these impinge on behaviour, and how to apply this understanding to improving the methods by which treatments are presented and communicated. Specific questions for this workstream would include the following:⁴⁴

1. How are risks and values perceived by individuals and what impact does this have on their acceptance of medical innovation?
2. How are the risks and benefits of treatments best communicated to facilitate informed treatment choices and optimal engagement with appropriate prescriptions?
3. What motivates people to seek out additional information?
4. How do patients and healthcare professionals ascertain the accuracy of their information sources?

⁴⁴ The Academy of Medical Sciences' new workstream on 'How does society use evidence to judge the risks and benefits of medicines?' aims to explore some of these issues: www.acmedsci.ac.uk/evidence-for-medicines.

5. How is trust engendered and how do relationships between individuals and the health service/healthcare professionals develop?

3. Public engagement and dialogue

Linking the first two workstreams, the importance of public engagement and dialogue in this work cannot be overestimated. Engaging the relevant stakeholder groups in the research for the two former workstreams will be vital in ensuring all sectors have the opportunity to contribute to, and play their part in, the developing healthcare system and a new social contract that may be needed to ensure its sustainability.

Appendix I Programme

Wednesday 3 June 2015 at the Academy of Medical Sciences

Hosted by the Academy of Medical Sciences and the British Academy, in association with the Medical Innovation Academic Consortium

09.00	Registration with refreshments
09.30	Welcome <i>Professor Albert Weale CBE FBA, Professor of Political Theory and Public Policy, University College London</i>
09.35	Overview of '4P' medicine and the social contract concept <i>Professor Sir John Tooke PMedSci, President, Academy of Medical Sciences</i>
10.15	Simultaneous breakout groups <ul style="list-style-type: none"> • Breakout 1 – Perceptions of risk & value of individuals (citizens, patients and professionals) <i>Chair: Dr Russell Hamilton CBE, Director of Research and Development, Department of Health</i> • Breakout 2 – Perceptions of risk & value of organisations (industry, commissioners/payers, professional groups, providers such as the NHS) <i>Chair: Professor Sir John Tooke PMedSci, President, Academy of Medical Sciences</i> • Breakout 3 – Perception of risk & value by society and regulators and other bodies whose decisions affect both individuals and organisations <i>Chair: Professor Sir Alasdair Breckenridge CBE FRSE FMedSci, Former Chair of the MHRA</i>
12.30	Lunch
13.30	Feedback from groups and discussion session <i>Chair: Professor Sir John Tooke PMedSci, President, Academy of Medical Sciences</i>
15.00	Refreshment break
15.15	Engaging society in dialogue about the future of medical innovation? <i>Chair: Professor Jonathan Montgomery, Professor of Healthcare Law, University College London; Chair, Nuffield Council of Bioethics</i> The session will explore ways in which the Academy can initiate a wider dialogue about developing the social contract for medical innovation. It will explore ways in which the Academy can ensure any future discussions and recommendations are shaped by the views of the wider society. Short presentations followed by a plenary discussion session: <ul style="list-style-type: none"> • Public perspective – Professor Sarah Cunningham-Burley, Professor of Medical and Family Sociology, University of Edinburgh • Patient perspective – Mr Derek Stewart OBE, Associate Director for Patient & Public Involvement, National Institute for Health Research Clinical Research Network • Practitioner perspective – Dr Sarah Castell, Director, Ipsos MORI • Community perspective – Ms Alyson McGregor, Director, Altogether Better
16.45	Summary and conclusions <i>Professor Sir John Tooke PMedSci, President, Academy of Medical Sciences</i>
17.00	Close

Appendix II Delegate list

- Dr Mark Bale**, Deputy Head of Health Science and Bioethics, Department of Health
- Professor Richard Barker OBE**, Director, Centre for the Advancement of Sustainable Medical Innovation
- Ms Jessica Bland**, Principal Investigator, Policy and Research Team, Nesta
- Professor Sir Alasdair Breckenridge CBE FRSE FMedSci**, Former Chair, Medicines and Healthcare products Regulatory Agency; Council Member, Academy of Medical Sciences
- Ms Sophie Broster-James**, Public Affairs and Communications Manager, Medical Research Council
- Mr Simon Burall**, Director, Involve
- Dr Sarah Castell**, Director, Ipsos MORI
- Ms Victoria Charlton**, Head of Policy, Academy of Medical Sciences
- Mr Robin Clarke**, Dialogue and engagement specialist, Sciencewise
- Dr Claire Cope**, Senior Policy Officer, Academy of Medical Sciences
- Mr Philip Creasy**, Patient and University College London Hospital Foundation Trust member
- Professor Sarah Cunningham-Burley**, Professor of Medical and Family Sociology, University of Edinburgh
- Ms Vanessa Cuthill**, Deputy Director for Evidence, Impact and Strategic Partnerships, Economic and Social Research Council
- Professor Alex Faulkner**, Professor of the Sociology of Biomedicine & Healthcare Policy (International Relations), University of Sussex
- Dr Russell Hamilton CBE**, Director of Research and Development, Department of Health
- Dr Shahid Hanif**, e-Health Data Development Manager, Association of the British Pharmaceutical Industry
- Mr Nick Hillier**, Director of Communications, Academy of Medical Sciences
- Mr Mike Hobday**, Director of Policy, British Heart Foundation
- Ms Hannah Hobson**, Policy Intern, Academy of Medical Sciences
- Dr Andrea Hodgetts**, Partnerships and Projects Coordinator, University College London
- Professor Stephen Holgate CBE FMedSci**, MRC Clinical Professor of Immunopharmacology, University of Southampton
- Professor Christopher Hood CBE FBA**, Gladstone Professor of Government and Public Administration, University of Oxford
- Dr Joshua Hordern**, Associate Professor of Christian Ethics, Lecturer in Theology, University of Oxford
- Professor Rob Horne**, Professor of Behavioural Medicine, University College London
- Professor Peter Johnson FMedSci**, Chief Clinician, Cancer Research UK
- Dr Janine Jolly**, Head of Patient, Public and Stakeholder Engagement, Medicines and Healthcare products Regulatory Agency
- Dr Kate Knobil**, Senior Vice President, Value Evidence and Outcomes, GlaxoSmithKline
- Ms Jan MacDonald**, Head of Patient Information Quality, Medicines and Healthcare products Regulatory Agency
- Professor David Mant OBE FMedSci**, Emeritus Professor of General Practice, University of Oxford
- Dr Fiona Marshall**, Community In-Reach Research Fellow, University of Nottingham

Professor Theresa Marteau FMedSci, Director of the Behaviour and Health Research Unit, University of Cambridge

Dr Natasha McCarthy, Head of Policy, British Academy

Professor Barry McCormick CBE, Director, Centre for Health Service Economics and Organisation at Nuffield College, University of Oxford

Ms Alyson McGregor, Director, Altogether Better

Professor Jonathan Montgomery, Professor of Healthcare Law, University College London; Chair, Nuffield Council of Bioethics

Dr Liam O'Toole, Chief Executive Officer, Arthritis Research UK

Sir Nick Partridge OBE, Deputy Chair, Health and Social Care Information Centre

Dr Rachel Quinn, Director of Policy, Academy of Medical Sciences

Professor Morten Ravn, Professor of Economics, University College London

Ms Holly Rogers, Communications Officer, Academy of Medical Sciences

Mr Adrian Sieff, Assistant Director, Health Foundation

Mr Sam Smith, Coordinator, medConfidential

Mr Derek Stewart OBE, Associate Director for Patient & Public Involvement, National Institute for Health Research Clinical Research Network

Ms Victoria Thomas, Associate Director for Public Involvement Programme, National Institute for health and Care Excellence

Professor Sir John Tooke PMedSci, President, Academy of Medical Sciences

Professor Adrian Towse, Director, Office of Health Economics

Sir Mark Walport FRS FMedSci, Government Chief Scientific Adviser

Dr Geoff Watts FMedSci, Freelance Journalist

Professor Albert Weale CBE FBA, Professor of Political Theory and Public Policy, University College London

Mr Giles Wilmore, Director, Patient and Engagement Directorate

Dr James Wilson, Senior Lecturer of Philosophy, University College London

Professor Patricia Wilson, Professor of Primary and Community Care, University of Kent

Professor Lucy Yardley, Professor of Health Psychology, University of Southampton



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