

Case Study

## Developing stratified medicine

### A public dialogue on the human issues raised by the development and implementation of stratified medicine

#### Vital statistics

**Commissioning body:**

Innovate UK (formerly the Technology Strategy Board)

**Duration of process:**

17 months: April 2013 - August 2014

**Total public participants involved:**

Over 150 participants, 51 in public workshops, 38 in targeted workshops and 63 in self-facilitated workshops

**Total stakeholders involved:**

40 stakeholders at stakeholder workshop

**Total experts involved in events:**

Seven experts across the public workshops

**Cost of project:** £207,000 total,  
Sciencewise co-funding = £108,000

*Recent advances in science, particularly in molecular biology and genomics, mean that it will become increasingly possible to identify the underlying molecular mechanisms of disease. Developing diagnostic tests that indicate the molecular cause of a disease enables new treatments to be developed that can target diseases more precisely. Although these technologies are quite new, it is argued that they have the potential ability to predict which patients will respond to a given treatment.*

*This potential ability and being able to provide the exactly appropriate treatment has become known as 'stratified' medicine, indicating the division of patients into 'strata' of those who would be expected to respond to a particular medicine.*

*As the potential and implications of genomic medicine have become more apparent, the interest in stratified medicine has intensified since it began about 10 years ago. It offers the possibility of an evolution in healthcare that is based, increasingly, on early prediction and rapid prevention, rather than on later diagnosis and treatments that may come to seem, in retrospect, comparatively crude. The implications of the changes as a result of stratified medicine are not just potential improvements in healthcare, but also cultural shifts in our understanding of disease and the role of our health services in responding to it.*

*Several Government bodies and leading charities are working together to accelerate the development and uptake of stratified medicine in the UK. Innovate UK (formerly the Technology Strategy Board), the Medical Research Council (MRC), Cancer Research UK (CRUK), Arthritis Research UK (ARUK), the Department of Health (DH), the Scottish Government Health Directorate (SGHD) and the National Institute for Health and Care Excellence (NICE) have formed a partnership to take forward the Stratified Medicine Innovation Platform (SMIP). Together, they will invest around £200 million over five years in the area of stratified medicine.*

*The public dialogue project focused on identifying the human issues raised by stratified medicine, the implications for how it is delivered and what it will mean for individuals who will benefit from it and for those who will not be able to benefit from it.*

#### Policy maker view

“ We can look at this project as a model for public engagement in future to understand where trust and distrust lies with the public. ”

Innovate UK.



## Background

Even the best medicines are not equally effective in all patients. Disease processes and treatment choices can vary from person to person even though they may have similar symptoms. It is estimated that only 30% to 70% of patients respond positively to any particular drug.

Stratified medicine has been summarised as identifying the right therapy for the right patient at the right time in the right dose.

The introduction of stratified medicine would involve a number of key strategic shifts in the approach to diseases and their treatment. These have the potential to affect people in every area of healthcare – from investors in drug companies and researchers in their laboratories to general practitioners in their surgeries and patients in their homes.

Innovate UK works to stimulate innovation and economic growth in the UK. The UK is seen to have the potential to be a world leader in the development of stratified medicines due to the strength of its pharmaceutical and bioscience industries, its outstanding academic base, its single healthcare system (the NHS) and the work of many other organisations.

Healthcare is the most highly funded priority area for Innovate UK. A part of that funding is being used to promote the development and uptake of stratified medicine with a range of partners through the SMIP.

In 2011, members of the SMIP developed a roadmap that highlighted nine areas they saw as vital to support the uptake of stratified medicine. It was recognised that in many of these areas there were questions to be answered about how the public, patients and healthcare professionals would receive the proposed changes. The dialogue was designed to start to answer these questions.

## Influence on policy and policy makers

It is not yet possible to fully assess the impact of the dialogue. As one Oversight Group member said:

*“The benefit of this is not today or tomorrow, but in 18 months’ time.”*

However, Innovate UK has disseminated the dialogue results and been able to identify various immediate impacts of the dialogue. It has already changed the way that the Innovate UK staff involved communicate. In particular, they use the term ‘stratified medicine’ less and talk more about how it works and use other phrases such as ‘personalised’ or ‘tailored’. In addition, they stress that it is an evolution of established practice rather than a new concept. Innovate UK also intends to take account of the sensitivity to ethnicity identified in the dialogue and to ensure the focus is on increasing access to appropriate treatments. Further insights from

the dialogue that were useful to Innovate UK included the differing levels of trust the public had in different institutions (Government and industry), and views on the use and sharing of data.

The process also influenced the views of Innovate UK and stakeholders on working with the public in the future. Innovate UK reported that “Our colleagues are now talking about the merits of public dialogue on other topics” and an Oversight Group member commented “[Innovate UK] recognised that the public do have interesting things to say. It wasn’t too frightening”. According to another Oversight Group member, the dialogue provided “Confirmation that people are capable of making sense of complex information and, given time, that they can do it and come up with sensible opinions.”

## Key messages from the participants

The participants identified a number of challenges, which can be grouped into four main themes:

1. Definition and communication. Here, the challenges were:
  - having a clear, consistent definition of stratified medicine
  - presenting a realistic picture of stratified medicine, its pros and cons
  - continuing to engage the public and patients.
2. Implications for patients and care. Here the challenges were to:
  - support patients to make sound treatment decisions
  - support patients for whom there is no current treatment
  - provide the right facilities and training for healthcare professionals.

*“I don't like the term 'stratified medicine', it makes one think of strata in society and I think there is a danger of it being viewed as elitist.”*

Dialogue participant, London public group.

*“Some patients will really struggle with choice – having to take hard decisions could be very stressful.”*

Dialogue participant, patient group.

3. Social issues and consequences. In this area, the challenges were to:
  - understand and mitigate any implications for equality
  - define the role of the private sector in developing stratified medicine
  - develop understanding of the costs/benefits of stratified medicine.

*“What if there are more black people in the category that don't benefit from a particular medicine, does this mean other ethnicities get better medication? I would be bothered about that.”*

Dialogue participant, young patient group.

4. Research, testing and data sharing. Here the challenges were to:
  - give research participants a choice about who uses their data and how it is used
  - reconcile the role and perception of the medical research industry
  - engage the public in regulation on data sharing.

*“Pharmaceutical companies using it [data] is less trustworthy. They will use it because there is a profit to be made, they will compromise it.”*

Dialogue participant, young patient group.

## The dialogue activities

The purposes of the public dialogue were:

- To discover the diversity of public opinion about stratified medicine and in the process also to discover how best to explain what it involves, and which terms are least likely to cause confusion, misinterpretation or misunderstanding, so that stratified medicine and the issues it raises can be discussed effectively with patients, their families, and members of the public generally
- To explore the possibilities of stratified medicine through a process that enables patients and members of the public to identify advantages and disadvantages that developers and healthcare providers may be overlooking, and to think creatively about ways to amplify the former and mitigate the latter
- To identify what steps practitioners and other healthcare providers will have to take to communicate the complex information that patients and their families will need about the testing processes that stratified medicine requires, and the support that different strata of patients will require before, during and after treatment
- To establish what sort of ethical framework and practical approaches to consent for trials will build patient and public confidence in and support for the sharing of the personal data necessary to ensure the effectiveness of stratified medicine.

Good governance of the dialogue project was ensured through an Oversight Group comprising representatives from the Genetic Alliance, the Association of the British Pharmaceutical Industry, Kings College London, the University of Edinburgh, the Academy of Medical Sciences, Involve and Sciencewise. To inform the dialogue materials and processes, the dialogue contractors carried out a scoping exercise in which they interviewed six people working in and around stratified medicine, as well as reviewing significant recent literature on the topic. Results of this scoping exercise fed into the Oversight Group agreement on what the dialogue should focus on.

The dialogue project involved 19 workshops with more than 150 participants over a four-month period. It involved three main strands of events, each seeking a different perspective on the issues, using a different method, but covering the same topics and using the same materials, to enable the results to be compared and contrasted. A summary of the three main strands is in the table below.

After the workshops were completed, the results were drawn together into a headline report and discussed at a stakeholder workshop. This full-day event in January 2014 was designed to increase awareness of the project and its results, and involve stakeholders in reviewing the results and exploring the implications for the future development of stratified medicine. About 40 stakeholders from industry, academia, government and the third sector attended the event. A further 10 people from Innovate UK and the project Oversight Group also attended.

Innovate UK disseminated the dialogue results more widely. It was a measure of the success of the project for Innovate UK that it saw the results as sufficiently important to actively share them. They have now been disseminated to organisations working closely with Innovate UK including the Association of the British Pharmaceutical Industry and the Academy of Medical Sciences. Articles on the dialogue have already been published, such as in Mental Health Today on 25 April 2014, and there are plans for wider dissemination through other journals. Blogs have also been published on the Sciencewise and British Science Association websites.

	Public workshops	Targeted workshops	Self-facilitated workshops
<b>Aim</b>	To understand diversity of public views	To understand views of specific sub-sets of public	To understand diversity of public views
<b>Number of locations/groups</b>	Two locations: London and Glasgow	Four groups	Seven groups
<b>Number of participants</b>	24-27 participants per session	5-15 participants per session	5-12 participants per session
<b>Total numbers</b>	51	38	63
<b>Recruitment</b>	Purposive sampling	Via intermediary groups	Pre-existing groups
<b>Timing</b>	Two full-day sessions, two weeks apart for each location	Two evenings, about 2 hours, 2 weeks apart <sup>1</sup>	One session, about two hours <sup>2</sup>
<b>Incentives</b>	£40 each day 1 £60 each day 2	£25 session 1 £40 session 2	£80 per group, paid to the group rather than to individuals

### What worked especially well

Overall, this dialogue was very successful. Five elements of the project worked particularly well.

#### Setting up an Oversight Group before the contractor was appointed.

The Oversight Group was involved closely in framing the original purpose of the dialogue, which added to the sense that stakeholder interests were being met. The Oversight Group brought a range of perspectives to bear on the process at every stage, including on the information provided for the public, and added to the credibility of the project and its results.

#### Clear objectives, appropriate methods and good organisation.

The clarity of the objectives, clearly communicated, and the design and delivery of the process overall, worked very well throughout.

#### Sufficient time and an initial scoping review.

The dialogue project had a good amount of time available – 10 months from appointment of contractors (early June 2013) to closure (end March 2014). This allowed time to discuss things thoroughly among the delivery team, Innovate UK and the Oversight Group. It allowed the delivery contractor to hold a public pilot of the materials and make subsequent improvements, and also to try out slightly different processes via the targeted groups and self-facilitated groups. The initial scoping review clearly set out in one place the technical issues for the Oversight Group, helped frame the whole dialogue process, accelerated materials development and immersed the facilitator team in the technical content from the start.

#### Targeting groups that were likely to be more affected.

There was clear reason to suspect that some sections of the public could be more affected by stratified medicine than others. The inclusion of workshops for patients (young and adult), medical students, black and minority ethnic (BME), and patient support groups helped with triangulating the results and building the credibility of the results overall. These could not have replaced the purposively sampled public sessions, but were useful as an addition to them.

<sup>1</sup>. With the exception of one group that met on one morning for 4 hours

<sup>2</sup>. With exception of one group that met for two sessions of 2 hours and 1 hour

“The results are credible because of the wide range of ages, ethnicities, socio-economic groups and education levels, etc. involved.”

Stakeholder, final workshop.

### Stakeholder workshop at the end to discuss the results.

The inclusion of a stakeholder workshop after the dialogue events had been completed was very useful in ‘spreading the word’ about the dialogue’s existence and results, and building wider stakeholder buy-in to the dialogue process.

## What worked less well

### Self-facilitated groups.

These discussions focused on pre-existing groups, such as patient support groups and youth groups, and were a useful, but challenging, method. A volunteer from each group, who was briefed in advance by the delivery contractor, facilitated each discussion and took notes.

The Oversight Group valued this strand as it focused attention on sectors of the public that may not otherwise have been included in any numbers in the project (particularly BME communities). However, it was quite difficult to persuade a range of groups to hold a discussion and it took a while to achieve even the seven groups that did meet. The delivery contractor suggested that the following things helped or may help in future:

- If possible, invite the volunteer facilitators to the public events so they are well briefed and immersed in the technical content themselves
- Target groups of people who have a clear interest in the benefits and implications of the technologies under discussion. In this case, patient groups offered the best return
- Be ready to offer a financial incentive that is comparable to the public workshops
- It is hard for volunteer facilitators to capture a full and accurate set of notes. Following up with a debrief interview soon after the event to hear about discussions can help make the data more robust.

### Aggregated nature of reporting.

One of the Sciencewise principles is to involve participants in the reporting of their views. This is partly about the ethical requirement to be able to demonstrate to participants and others how the public input has been passed on and used, and partly so that everybody can be reassured that participant views have been captured, summarised and reported accurately. This dialogue project had four strands of workshops in all (including the stakeholder event) and one dialogue report. Brief reports on each event, circulated more quickly, may have been worth considering. However, the final report was considered to be concise, accessible and readable (including by public participants) which, given the complexity of the topic and process, was a significant achievement.

## Contact Details

### Commissioning body

Innovate UK (formerly the Technology Strategy Board)

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### Reports

Full project and evaluation reports available from Sciencewise on [www.sciencewise-erc.org.uk/cms/developing-stratified-medicine/](http://www.sciencewise-erc.org.uk/cms/developing-stratified-medicine/)