Challenges in personalised medicine

Personalised medicine will require the development of targeted medicines.

For personalised medicine to make progress, new findings from molecular research, and new technologies (such as ‘omics’ technologies), must be translated (adapted) for use in medicines development and approved therapy.

However, an advantage of developing targeted medicines is the increase in the efficiency of clinical trials. Fewer new medicines should fail at each stage of the development process if they are targeted at a known cause of the disease, and the use of biomarkers will be central to personalised medicine. Validation of biomarkers as unique and predictive for the outcome of treatment has to be in place before medicines developed in this way can be authorised.

Companion diagnostics

Often new medicines are authorised together with an accompanying diagnostic test to ensure that the medicine is appropriate for the patient being treated. If a diagnostic test is not properly validated, the medicine may not work or give side effects. Therefore, proper design and validation of the diagnostic test is essential to get the best outcome from the medicine and for the patient.

Companion diagnostics are necessary tests that select patients before a medicine is given. They may:

- show who is likely to respond to the medicine (‘responders’ and ‘non-responders’)
- identify patients at high risk for adverse reactions

- help the doctor to select an appropriate dose that is both safe and effective.

Companion diagnostics may include tests directly on the patient such as electrocardiography (ECG) or diagnostic imaging such as MRI. Tests carried out on samples taken from a patient (such as DNA tests) are generally considered by regulatory authorities to provide the most valid evidence.

Companion diagnostics should have an ‘intended use’ or ‘indications for use’. They are often both referred to under the umbrella term of ‘intended use’ (of a treatment). They should generally include the following elements:

- The target population for whom the test is intended (such as individuals with particular genotypes (genetic make-up) or phenotypes (characteristics)).
- Why the measurement is being done – the ‘clinical purpose’ (such as to help with diagnosis, assessing how the disease is likely to develop (prognosis), and monitoring)
- What is measured, identified, or detected (such as a specific gene or protein)
- What kind of measurement the test makes, including whether the test is qualitative (looks at observations and descriptions), or semi-quantitative and quantitative (looks at numbers)
- The sample type and where it is taken from (e.g. whole blood, cerebrospinal fluid)
- The setting in which the diagnostic device is meant to be used (in a laboratory or at ‘point-of-care’) and what type of equipment is required to perform the test
- The target condition (a particular disease, disease stage, health status, or any other identifiable condition or event)

Challenges

There are particular ethical challenges for researchers who work with genetic and genomic data. It is important that they consider privacy and keeping data confidential, and making sure participants in trials understand what they are agreeing to (informed consent).

Even with medicine targeted for a specific sub-population of patients, it is still possible that patients with the “same disease” but in different sub-groups might be prescribed the medicine. This could lead to:

- a lack of response to the treatment
- a delay in the patient receiving a treatment that is better for them
- a waste of the medicine itself
- an adverse reaction that could have been avoided.

Patient experience

Personalised medicine can provide much more information about the health of an individual person, which can have personal implications and potentially change their way of living. Are patients ready for this? And are healthcare professionals skilled enough and ready to communicate about it with their patients?

With personalised medicine, the patient with a disease should have more reassuring information prior to treatment:

- that a medicine is likely to work well for them, and
- that they are unlikely to suffer from side effects with a particular treatment.

Where side effects are unavoidable, better knowledge about them and how severe they might be should make it easier for the patient to decide and plan for the treatment and to fit it

into their daily life.

Patient-doctor communication

In general, with personalised medicine, there may exist more or different information about the available treatment options for the patient and doctor to understand and discuss. Patients who find this difficult will need good support from their doctors.

It may be necessary to do more tests than we are used to in order to make personalised medicine possible. Blood tests are generally seen as routine, but biopsies (where pieces of tissue are removed) require an anaesthetic and can take longer to be analysed. Patients and their doctors will need to discuss the pros and cons when making decisions about testing.

If a test predicts how likely a patient is to respond to a medicine, the result might be expressed for example as ‘odds’ (1 in 3) or as percentage (33 %). People have different ways of interpreting risk, and doctors will need to support them in interpretation of risks.

Further resources

- Genomics England
- Nuffield Council on Bioethics (2010). Medical profiling and online medicine: the ethics of ‘personalised healthcare’ in a consumer age.
- Corpet, A. & Almouzni, G. (Dec 2006-Jan 2007). *Sciences et Avenir*, 149

Attachments

- Challenges-in-Personalised-Medicine-v1_EN
  Size: 396,441 bytes, Format: .pptx

A presentation describing the challenges in personalised medicine, which can be adapted for own use.

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