The benefits of personalised medicines to patients, society and healthcare systems

Charles River Associates was asked by EFPIA/EBE to undertake an evidence-based analysis of the benefits of personalised medicine (PM) to patients, society and healthcare systems. We found that there is considerable evidence of the potential benefits of PM for patients, clinicians and the healthcare system; however, significant barriers continue to hinder the adoption of PM in Europe. We composed five policy recommendations based on what is needed to encourage the development of PM and incentivise more equitable uptake.

Our conclusions are based on a case study approach focusing primarily on oncology PM, examining experiences in five European markets (Denmark, England, France, the Netherlands and Poland), supplemented by 19 interviews with payers, policymakers and healthcare professionals to understand the access landscape for PM.

The benefits of personalised medicine

We classify the benefits of a personalised medicine into three main categories: (1) delivering better treatments to patients; (2) delivering benefits to healthcare systems and society; and (3) more efficient development of new medicines (see Figure 1). In each area we found convincing evidence of benefits today, although we also note that the evidence base is stronger in the US than in Europe.

Figure 1: CRA categorisation of the benefits of PM

<table>
<thead>
<tr>
<th>Delivering better treatments for patients</th>
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<tr>
<td>• Improved efficacy i.e. patient more likely to receive a medicine delivering a clinical benefit</td>
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<tr>
<td>• Improvement in overall survival</td>
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<td>• Reduced adverse events</td>
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<table>
<thead>
<tr>
<th>Delivering benefits to healthcare systems and society</th>
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<tbody>
<tr>
<td>• Prevention and prediction of disease</td>
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<tr>
<td>• Improvement in patient management of diseases</td>
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<td>• Prevention or delay of more expensive care costs and allowing scarce healthcare resources to be used most efficiently</td>
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<td>• Reduces hospitalisation</td>
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<table>
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<tr>
<th>More efficient development of novel medicines</th>
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<tr>
<td>• More effective clinical trials</td>
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<td>• Efficient clinical trials and reduction in cost</td>
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<td>• More ethical clinical trials</td>
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Source: CRA analysis

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1 We selected four tumour types—non-small cell lung cancer (NSCLC), breast cancer, ovarian cancer and melanoma—to identify different challenges associated with PM technologies.
Firstly, PM has delivered better treatments for patients as targeted therapies have improved the likelihood of a desired clinical effect, a better outcome and a reduced risk of adverse events. Although it is not always possible to disentangle the specific impact from personalisation, according to the interviews the impact on patients in certain therapy areas has been significant.

Secondly, there are significant benefits to the healthcare system and society from improvements in patient management and costs offset by reduced use of ineffective treatment, reduced cost of chronic conditions and reduced hospital stays.

Thirdly, there is also evidence that PM facilitates more efficient development of medicines. PM has improved the efficiency and effectiveness of running clinical trials. These benefits are growing and will be even more significant in the future.

The environment for personalised medicine

We have identified nine areas critical for the encouragement of PM:

1. policy prioritisation;
2. the care environment;
3. diagnostic testing infrastructure;
4. uptake of diagnostics;
5. mechanism of value assessment;
6. use of real-world evidence;
7. speed of reimbursement;
8. speed of updating guidelines; and
9. the level of funding and investment in PM.

We find that Denmark and France are markets that are most supportive to PM. These are countries that have prioritised PM, invested in testing infrastructure, and ensured that patients have access to both medicine and diagnostics. However, even in these markets the environment is getting more challenging, with changes to the funding of diagnostics (from a centralised to a hospital-tariff-based approach) and the introduction of a more formalised value assessment framework. Given changes in technology, there is a choice as to whether to invest in a particular diagnostics test, gene profiling or whole genome sequencing (WGS). As shown in Figure 2, a few countries have made large investments in genomics technologies. Most countries in Europe have prioritised WGS rather than increasing uptake of next-generation sequencing (NGS) technology for more genomic profiling of tumours within current clinical pathways. The fragmented reimbursement process for diagnostics is a significant barrier to uptake and the current approach appears unsustainable given the trends towards profiling and NGS.

Figure 2: Per capita investment in genomics compared to other cancer initiatives

![Figure 2: Per capita investment in genomics compared to other cancer initiatives](image)

Source: CRA analysis of various sources

Figure 3: Weeks from first symptoms to diagnosis (diagnostic interval), and diagnosis to treatment (treatment interval), in lung cancer

![Figure 3: Weeks from first symptoms to diagnosis (diagnostic interval), and diagnosis to treatment (treatment interval), in lung cancer](image)
The level of coordination of care is also a key enabler of PM. Of particular importance is the concentration of expertise and infrastructure investment in specific centres to support the availability of specialised testing units to identify patients. There is evidence demonstrating that centralising cancer care to specialised centres of excellence improves outcomes for patients. Similarly, studies have also suggested that centralisation for some tumour types may be associated with increased cost-effectiveness of PM.

Conclusion and policy recommendations

Based on our assessment of PM as well as input from the external interviews, we have composed a set of recommendations based on what is needed to incentivise the development of PM and improve equitable access:

1. A coherent prioritisation of personalised medicine that goes hand-in-hand with existing health strategic plans;
2. A continued emphasis on better management of care, coordination of expertise and allocation of resources to ensure an adequate “personalisation of care”;
3. Continued investment and cooperation in next-generation testing infrastructure (such as molecular genetic laboratories) as well as development of dedicated funding pathways to ensure access to diagnostics;
4. A consistent diagnostic testing infrastructure throughout Europe; and
5. Better alignment of data requirements between regulators and health technology assessment (HTA) bodies to improve evidence development and facilitate the value assessment process.

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