



# An evidence-based analysis to characterise the benefits of personalised medicines to patients, society and healthcare systems

July 2018

**CRA** Charles River  
Associates

## Purpose of this report

- The European Biopharmaceutical Enterprises (EBE), and the European Federation of Pharmaceutical Industries and Associations (EFPIA) has asked Charles River Associates (CRA) to conduct an evidence-based analysis of the value of personalised medicines (PM).
- In particular, the objectives are to:
  - Characterise and measure the benefit of PM to patients, society and healthcare systems
  - Identify the key enablers to the adoption of PM but also the main barriers that impede the development of PM in Europe from an economic and access perspective
  - Elaborate strategic recommendations for decision-makers to overcome these barriers and incentivise the development and adoption of PM in Europe

## This considered a range of PM technologies

- We define PM as any technology that aims to improve the prevention, diagnosis and treatment of diseases by using patients' individual characteristics to identify the most appropriate care



### Treatments

- Broadly classified into two categories:
  - **Targeted therapies:** These are therapies that act on specific molecular targets associated with a disease. These targets can arise from specific mutations associated with the disease or protein-expression targets within biological pathways
  - **Individualised therapies:** This includes modified T-cell therapies and gene therapies, which are considered ATMPs. These technologies are specifically targeted at an individual patient.



### Diagnostics

- PM refers to a process by which genetic information is used to evaluate patients at risk of developing particular diseases, or who have mutations which can be targeted by specific medicines. This includes next generation sequencing (NGS), assays for specific mutations, and gene expression profiles which characterise sections of an individual's genome

## Four tumour types were selected as cases studies to develop a fact-based landscape analysis

- CRA selected case studies in consultation with the EBE/EFPIA steering group

	Non-small cell lung cancer	Breast cancer	Ovarian cancer	Melanoma
Products / targets	<ul style="list-style-type: none"> <li>• Multiple specific mutations (ALK+, ROS+, EGFR) plus protein-expression targets (PD1)</li> <li>• Companion and complementary diagnostics</li> </ul>	<ul style="list-style-type: none"> <li>• Germline and somatic mutation-targeted therapies</li> <li>• Potential usage of advanced diagnostics (e.g. Oncotype) separate from treatments</li> </ul>	<ul style="list-style-type: none"> <li>• Introduction of PARP inhibitors</li> <li>• Use of diagnostics in screening programs for BRCA mutations</li> </ul>	<ul style="list-style-type: none"> <li>• Introduction of BRAF inhibitors (and later BRAF / MEK inhibitors)</li> <li>• Use of tumour mutation testing for treatment decision-making</li> </ul>

- To investigate the environment for each case study we chose a subset of European markets to examine in detail



Denmark



England



France



Netherlands



Poland

Countries were selected on the basis that they:

- represent different regions of Europe
- represent different reimbursement mechanism and approach to HTA
- have some level of policy activity and prioritisation for PM
- have sufficient treatment infrastructure to enable adoption of PM

- CRA conducted a set of interviews with external stakeholders to fill evidence gaps and gather different perspectives in each country (n=19)

After reviewing a range of options we agreed to focus the case studies only on oncology reflecting that this is the therapy area with the most examples to-date

# The benefits of Personalised Medicines

# The benefits of PM can be classified into three main categories



## Delivering better treatments for patients

- Improved efficacy i.e. patient more likely to receive a medicine delivering a clinical benefit
- Improvement in overall survival
- Reduced adverse events



## Delivering benefits to healthcare systems and society

- Prevention and prediction of disease
- Improvement in patient management of diseases
- Prevention or delay of more expensive care costs and allowing scarce healthcare resources to be using most efficiently
- Reduces hospitalisation



## More efficient development of novel medicines

- More effective clinical trials
- Efficient clinical trials and reduction in cost
- More ethical clinical trials

# Targeted and personalised interventions have led to better patient outcomes and optimized regimens

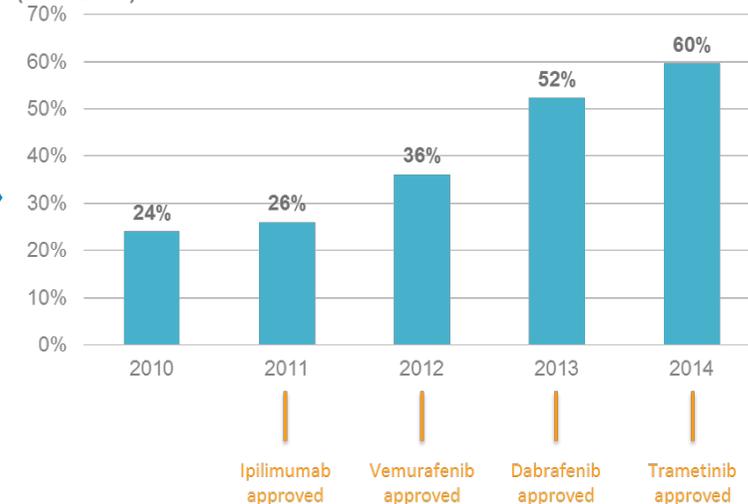
Better treatments

HC and societal benefits

Efficient development

- PM offers the opportunity to move away from 'trial-and-error' prescribing to initial prescription of optimal therapies and **deliver better response by patient**
- Progression-free survival and overall survival has increased in many cancers due to PM:
  - Alongside the introduction of immunotherapies (CTLA4 and PD1-targeting), the combination BRAF/MEK inhibitors are cited by oncologists as driving improvements in melanoma survival<sup>1</sup>
  - There has been an overall reduction in EU mortality from breast cancer and an increase in ten-year survival to 78%<sup>2</sup>**
- The introduction of PM has allowed targeting of the underlying genetic mutations in diseases, including chronic myeloid leukaemia (CML) and Cystic Fibrosis
- An analysis of 570 phase II clinical trials showed that oncology PM therapies had **4X the response rates** compared to cytotoxic therapies<sup>3</sup>
- Studies evaluating severe to life-threatening adverse events in advanced urothelial carcinoma, showed anti-PD-1 treatment **reduced frequency adverse events** from 49.4% with chemotherapy to 15.0%<sup>4</sup>

One-year survival rate for melanoma (stage IV patients), in adult women (2010-2014)



Source: Public Health England

# PM offers many possible treatment options to facilitate earlier treatment or prevention protocols

Better treatments

HC and societal benefits

Efficient development

- Molecular analysis can determine precisely which sub-phenotype of a disease a person has, or whether they are susceptible to medicine toxicities, to help guide treatment choices. This shifts the emphasis in treatment from reaction to prevention
- This has the potential to lower overall healthcare costs through early-detection, prevention, accurate risk assessments and efficiencies in care delivery



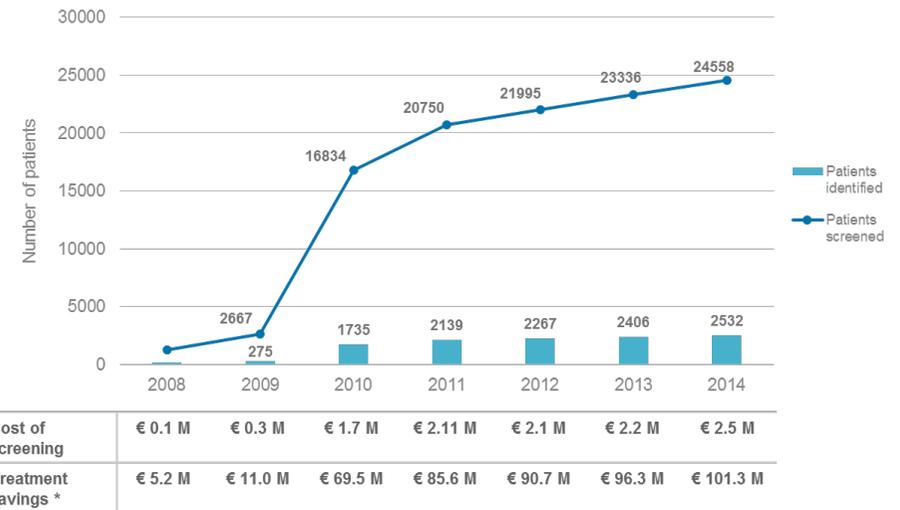
**Early identification of Familial Hypercholesterolemia (FH)** through genetic testing has led to significant savings in healthcare costs – in the UK estimated savings to the NHS are £6.9 million per year<sup>1</sup>



In France, INCa allocated an additional €1.7M to regional genetics centres across the country for EGFR testing. This resulted in substantial increase EGFR screening in patients<sup>2</sup>

- INCa concluded that this **additional investment in EGFR testing would save €69 million to the French health insurance** by identifying patients who harboured the EGFR mutation

Number of lung cancer patients screened for EGFR mutations in France



Notes: \* Treatment savings account for the spared cost of gefitinib treatment by only targeting patients more likely to respond to EGFR inhibitors

Source: CRA analysis of WIN Consortium

# Better patient management is associated with savings to healthcare systems and society

Better treatments

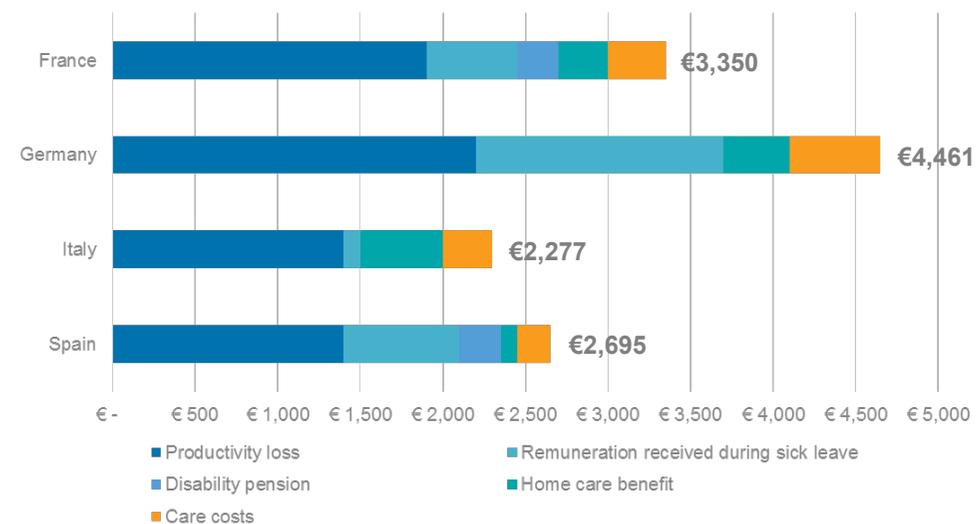
HC and societal benefits

Efficient development

## Case Study: NSCLC

- Treatment algorithms for NSCLC have changed dramatically over the last few years, following the approval of the first generation of targeted therapies
- PM is associated with more savings to society compared to standard chemotherapy in terms of increased productivity and decreased social benefits paid to patients who are able to work in France, Germany, Italy, and Spain
- **Mean incremental savings to society per patient receiving bevacizumab plus chemotherapy treatment ranged from €2,277 in Italy to €4,461 in Germany<sup>1</sup>**

Treatment savings per patient by using Bevacizumab plus chemotherapy treatment, relative to only chemotherapy (5 year cumulative savings)<sup>1</sup>



Source: Lister et al (2012)

# PM allow scarce healthcare resources to be used more efficiently

Better treatments

HC and societal benefits

Efficient development

Type of impact	Supporting evidence collected
Reduction in use of ineffective therapies for patients	 The estimated <b>cost of lost productivity</b> in early-stage breast cancer was <b>€602 lower for patients undergoing genetic testing</b> prior to starting chemotherapy <sup>1</sup>
	  <b>A 34% reduction in chemotherapy use</b> occurs if women with breast cancer receive a genetic test of their tumour prior to treatment <sup>2</sup>
	 A systematic review identified 147 studies that demonstrated the economic benefit of Oncotype DX, illustrating an impact on effective use of health resources through the <b>avoidance of unnecessary chemotherapy in breast cancer care</b> <sup>3</sup>
	 An estimated that <b>\$604 million in annual health care cost savings</b> would be realised if patients with metastatic colorectal cancer receive a genetic test for the KRAS gene prior to treatment <sup>4</sup>
	 INCa deduced that additional €1.7 million investment in EGFR testing would <b>save €69 million to the French health insurance by identifying patients with the mutation</b> , ensuring PM was only prescribed to patients who were more likely to respond <sup>5</sup>
Reduction in long-term cost of chronic diseases	 Molecular testing before first- or second-line treatment initiation in NSCLC results in better survival with limited additional costs. In the scenario the ICER was €8,308 per life years saved (LYS) compared with standard care <sup>6</sup>
	 Genetic testing to target dosing of blood thinner treatment could <b>prevent 17,000 strokes and could avoid 43,000 hospital visits</b> <sup>7</sup>
Reduction in hospital stay	 Oncologists estimate that the mean hospital stay for PM is 3-4 days, whereas it is more than a week for chemotherapy regimens <sup>8</sup>
	 In France 293,628 people were hospitalised with chemotherapy in 2013. However, with the increasing use of PM therapies, there has been a decrease in overall number of stays (public + private) by 2.7% (260,390 stays in 2012 and 253,392 in 2013) <sup>9</sup>

# Developments in PM directly impact clinical trial design and patient recruitment

Better treatments

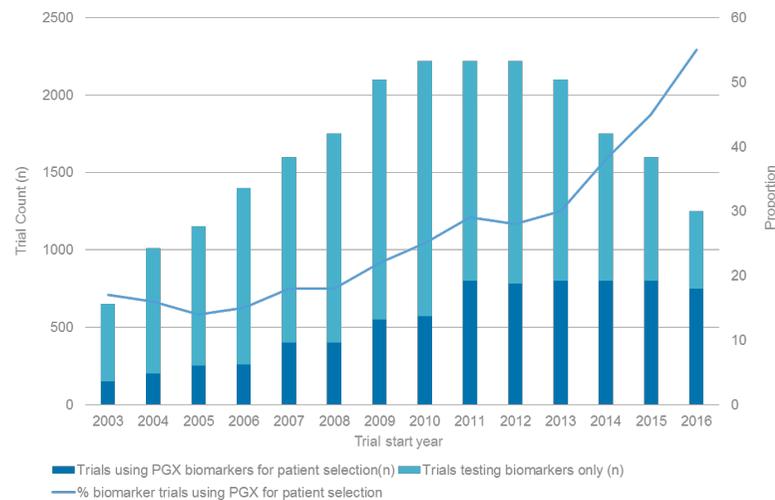
HC and societal benefits

Efficient development

- The proportion of trials using selection biomarkers continues to increase across phases

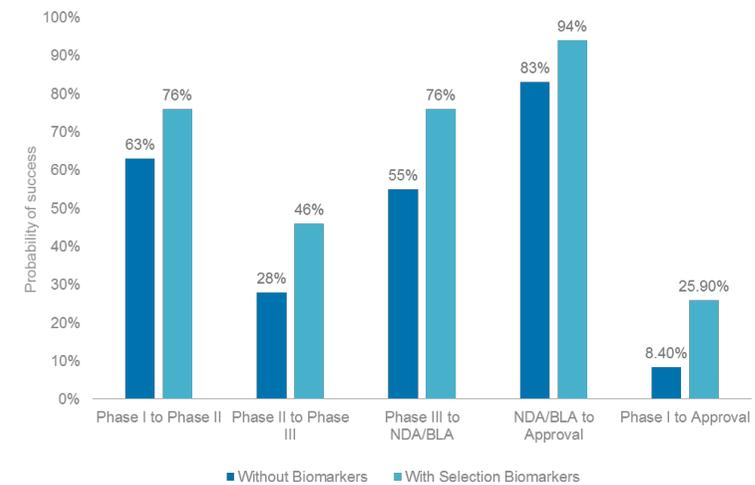
- Trials that do use selection biomarkers have a higher probability of success thus making an R&D program more cost-effective

Phase I-IV clinical trials utilising biomarkers by trial start year, 2003–2016



Source: Pharma Intelligence

Probability of regulatory approval with or without selection of biomarkers



Source: BIO (2016)

ebe  
european  
biopharmaceutical  
enterprises

efpia  
European Federation of Pharmaceutical  
Industries and Associations

CRA Charles River  
Associates

# The environment for Personalised Medicine

• Identifying **barriers**  and **enablers**  to PM

## There are a mix of approaches to prioritising PM in terms of health care policy across EU markets



- The clear benefit of having PM strategies in addition to national cancer plans (NCPs), is to allow for a **forward-looking perspective on the value of genomics to healthcare systems**; to support the testing infrastructure towards the development of whole genome sequencing (WGS) and its applicability to other conditions outside oncology
- Countries have adopted different approaches to implementation, however plans have common elements:



Denmark has implemented NCPs from an early stage relative to other European countries; the first plan was published in 2000. In 2017 Denmark opened a national genome centre for personalised medicine which will serve as a hub for integrating genomic data



England was the first to launch a dedicated program to whole genome sequencing in Europe. NHS England is supporting the integration of genomics into its services though setting up a new national network of Genomic Laboratory Hubs (GLHs) by November 2018



France initially invested centrally in molecular diagnostics and infrastructure as part of its NCP, with the development the French National Cancer Institute (INCa) in 2004. In 2016, France announced the “France Médecine Génomique 2025” program

Country	Policy prioritisation	Description
Denmark	●	<ul style="list-style-type: none"> <li>• Key focus within National Cancer Plans</li> <li>• Recent National strategy for PM (2017-2020)</li> </ul>
England	●	<ul style="list-style-type: none"> <li>• PM strategy through NHS England</li> <li>• Focus of increased integration of genomics and diagnostics into the NHS</li> </ul>
Estonia	●	<ul style="list-style-type: none"> <li>• PM program (2016–2020) managed by the Ministry of Social Affairs</li> </ul>
France	●	<ul style="list-style-type: none"> <li>• Key focus within National Cancer Plans</li> <li>• Recent investment in genomic and PM program (2016)</li> </ul>
Germany	●	<ul style="list-style-type: none"> <li>• National plan on PM that focusses on new priorities for government funding</li> </ul>
Italy	●	<ul style="list-style-type: none"> <li>• PM included within agenda for sustainable healthcare</li> </ul>
The Netherlands	●	<ul style="list-style-type: none"> <li>• Government acknowledges PM in Medicines Plan and is included in the research agenda for sustainable health</li> </ul>
Poland	●	<ul style="list-style-type: none"> <li>• No specific plan on PM</li> <li>• Access to diagnostics included as an objective in the National Cancer Plan</li> </ul>

Notes: Green – High (dedicated national plan on PM); Amber – Medium (inclusion of PM in health strategies or national cancer plans); Red – Low (no policies on PM)

## A coherent PM strategy should articulate the approach to disease profiling versus whole genome sequencing



- Countries have clearly taken account of the advances in genomic technologies and their application in clinical practise by making substantial investments in this space:

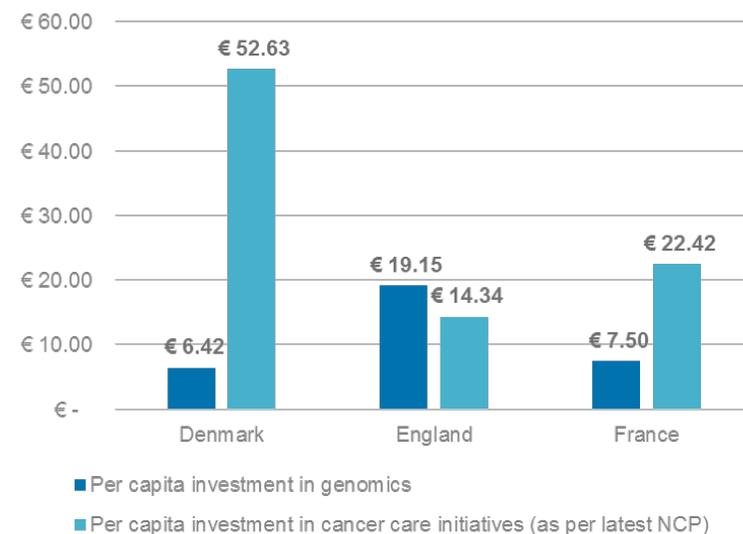
 Per capita investments in genomics and increasing diagnostic capacity is a clear priority within the NHS Five Year Forward View

 The French Genomics Plan aims to open 12 sequencing centres

 Denmark has invested heavily in genomics in previous years, which is why its latest figures are lower as there is already more developed infrastructure

- There is a question as to whether to focus on particular diagnostics test, profiling or WGS. Most countries in Europe have prioritised whole genome sequencing (WGS), rather than increasing uptake of NGS technology for more genomic profiling of tumours within current clinical pathways
- Clinical genomic profiling strategies should be better optimised to screen more patients**, using sufficiently broad targeted gene panels, rather than fewer patients with WGS. This will ensure that greater numbers of patients are more quickly identified and benefit from currently available treatments

Per capita investment in genomics compared to other cancer initiatives



Source: CRA analysis of various sources

# Centralising and increasing coordination of care is important, but should not limit PM as it is incorporated into standard of care



- Countries have varying degrees of centralisation of cancer care:



## Centralisation by tumour type

In Denmark, national cancer patient pathways results in centralisation of treatment to specialised centres. Whereas in the Netherland the degree of centralisation varies, e.g. EGFR+ NSCLC is not centralised, resulting in variation to treatment approach<sup>1</sup>



## 'Hub-and-spoke' delivery of cancer care

In England, patients benefit from a cancer management strategy formulated by a multidisciplinary team (MDT) found across cancer units in general hospitals, with specialist MDTs located in larger specialised hospitals

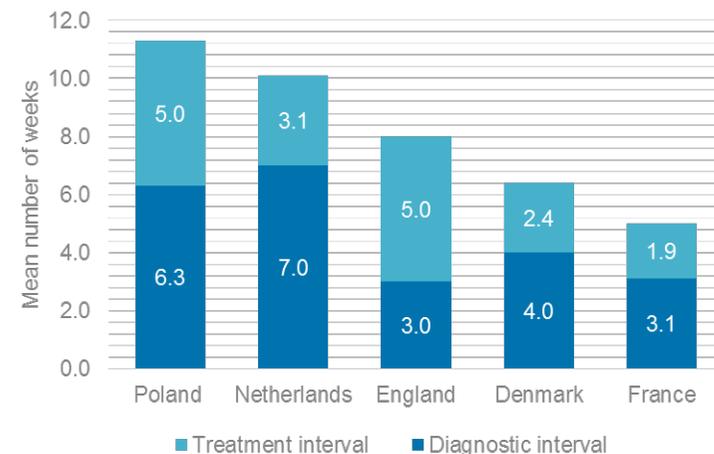


## Accredited hospital networks

INCa coordinates cancer institutions across regions to support consistency and multidisciplinary team have also been introduced in France. A similar model is being implemented in Poland

- **Concentration of expertise and infrastructure investment** in specific centres support the availability of specialised testing units to identify patients. This is particularly important for rare cancers that require specialist diagnosis
- There is evidence demonstrating that centralising rare cancer care to specialist centres of excellence **improves outcomes for patients**<sup>2</sup> Similarly, studies have also suggested that centralisation may be associated with **increased cost effectiveness** of PM<sup>3</sup>

Weeks from first symptoms to diagnosis (diagnostic interval), and diagnosis to treatment (treatment interval), in Lung Cancer



Increasingly centralised / better coordinated care

Source: CRA analysis of various sources<sup>4</sup>

## The adoption of technologies by laboratories and the factors influencing this varies depending on the technology

- The lab's decision to adopt a particular test may be dependent on the reimbursement regime for diagnostics locally. For example, if NGS panels are reimbursed and single gene tests are not, this will lead to greater use of NGS
  - While usage of NGS systems is increasing, this varies by country. approximately 17% of MoIDx labs in Europe have an NGS machine and, of those not currently running NGS, another 21% plan to acquire it in the next 5 years<sup>1</sup>
- Despite the importance of testing, there is currently **no standard metric or central public data-set which shows usage of diagnostic tests** in Europe with geographical breakdown, either in terms of biomarker testing performed by laboratories or in terms of the sales of commercial test kits and equipment
- Additionally, the degree to which diagnostics are **subject to a value assessment** and the degree to which they are integrated with the assessment of associated therapies varies across Europe:



The evaluation of diagnostics (including the impact on costs) is integrated into the NICE appraisal of PM

Estimated uptake and access to diagnostic tests across case study markets

Indication	Diagnostic test	DK	EN	FR	NL	PL
Breast cancer	HER2	Green	Green	Green	Green	Green
	BRCA 1/2	Yellow	Red	Red	Yellow	Red
Melanoma	BRAF V600 mutation	Green	Yellow	Green	Green	Yellow
	PD-L1	Green	Green	Green	Green	Yellow
NSCLC	EGFR *	Green	Green	Green	Green	Green
	ALK	Green	Green	Green	Green	Red
	PD-L1	Green	Yellow	Yellow	Green	Red
Ovarian cancer	BRCA 1/2	Green	Yellow	Green	Green	Red
Gene panel testing	NGS	Green	Yellow	Green	Yellow	Red

Notes: Green – High uptake / Full reimbursement; Amber– Medium uptake / Conditional reimbursement; Red – Limited uptake / limited reimbursement

\* Includes both ctDNA testing by liquid biopsy and traditional tumour solid biopsy approaches

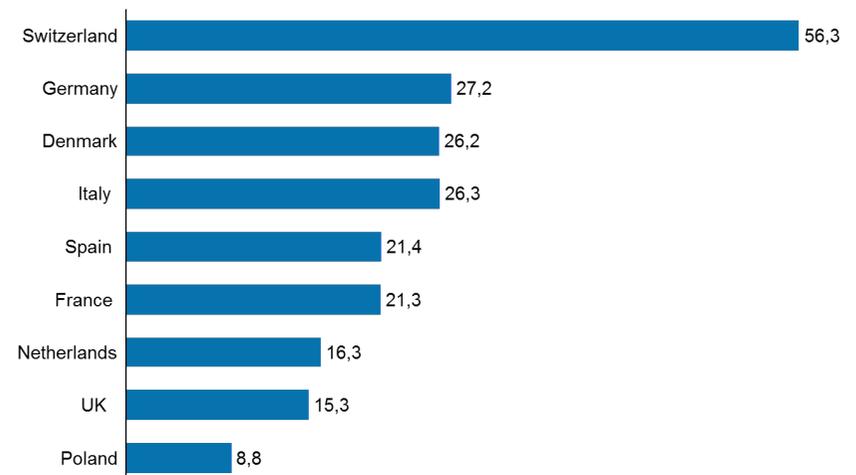
Source: CRA analysis

## The funding model should take into account infrastructure investment and the need to encourage competition between diagnostic providers

- There are wide variation in per capita expenditure on in vitro diagnostics (IVD) across selected countries in Europe
- Disease-specific funding has enabled diagnostic services to be funded as part of broader efforts to improve oncology care, this has allowed for infrastructure investment and high levels of access
  -  In France, there is good access to lab based testing services but appears to be limited access for specific diagnostic kits
- In other markets, testing services are integrated into hospital budgets and are expected to be covered through a Diagnosis-related group (DRG)-type funding
  -  HER2 breast cancer diagnostic testing in Poland is predominantly the responsibility of pathology laboratories in hospitals. This creates challenges for new tests
- Until now, investment in CDx was linked to the value of an individual medicine. Therefore access to testing could be supported by the manufacturer. England has many examples of this
  - As testing moves away from direct associations to particular products, and towards panel sequencing, individual manufacturer funding becomes no longer justified



Per capita expenditure on In vitro diagnostics (IVD) (€)



Source: MedTech Europe

## Variation in Dx testing approaches and quality may create inconsistencies in testing services both within and across countries

- A number of countries in Europe have invested heavily in molecular testing laboratory infrastructure:

 Both France and Denmark have setup a national program to support molecular testing with the establishment of regional molecular genetics centres – this has allowed for good access to newer, complex, Dx methods (e.g. NGS/ctDNA).

- In other countries, the access to Dx testing is limited by the testing environment or the coding of diagnostic tests that is required for reimbursement:

 Poland has a significant gap between demand and provision for testing in some cancers (such as Lung cancer)

 Until recently the approach to testing has been too fragmented leading to significant variation in access to diagnostics

- Various methods are being used across labs (e.g. ctDNA example data) resulting in variation in the quality of testing results**

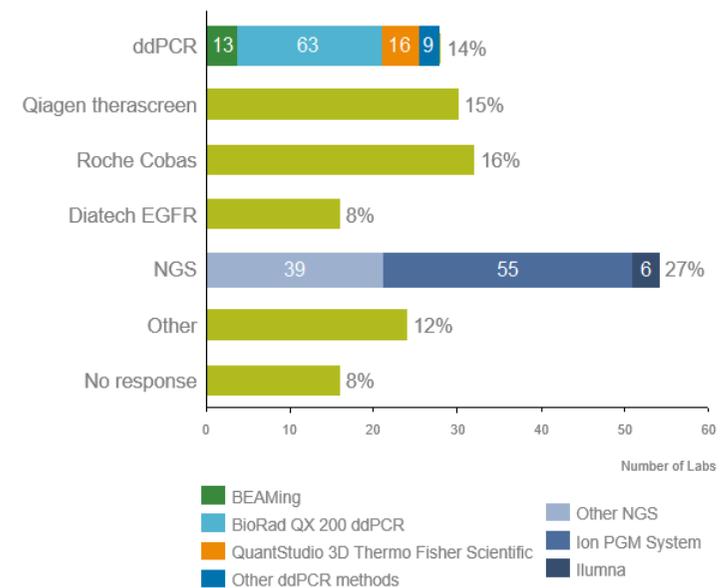
 Multiple factors may be influencing the quality of Dx testing resulting in inconsistent laboratory/test performance.

There is little evidence base for recommendations on testing methods and how to improve testing quality.

There is limited public information on lab performance or test usage. Applying External Quality Assessments (EQA) and collecting data to establish an evidence base for testing quality.



Example; frequently used methods for plasma ctDNA testing



Source: [IQN Path \(2017\)](#)

## Access to PM is restricted when countries adopt more formal HTA

- Generally, the EU5, Scandinavian and Benelux markets grant greater access to innovative therapies, whereas Central and Eastern European markets such as Poland are more likely to restrict access to manage budget impact



In England, access to personalised cancer treatments has been problematic due to challenges in meeting required cost-effectiveness thresholds to achieve positive NICE recommendations. In these cases, patient access schemes and the Cancer Drugs Fund have been important programmes in facilitating access



Countries like the Netherlands which are more pragmatic about using available evidence, or facilitating the collection of RWE through registries have better access to novel treatments

- Payer perceptions of products with CDx or specific biomarkers are generally more positive than of those without such biomarkers
- Clinical guidelines play a different role in different EU markets; in England, guidelines are integrated into HTA, whereas in consensus driven markets such as Denmark, clinical guideline development is crucial for the introduction of novel therapies



Reimbursement status of PM across case study markets

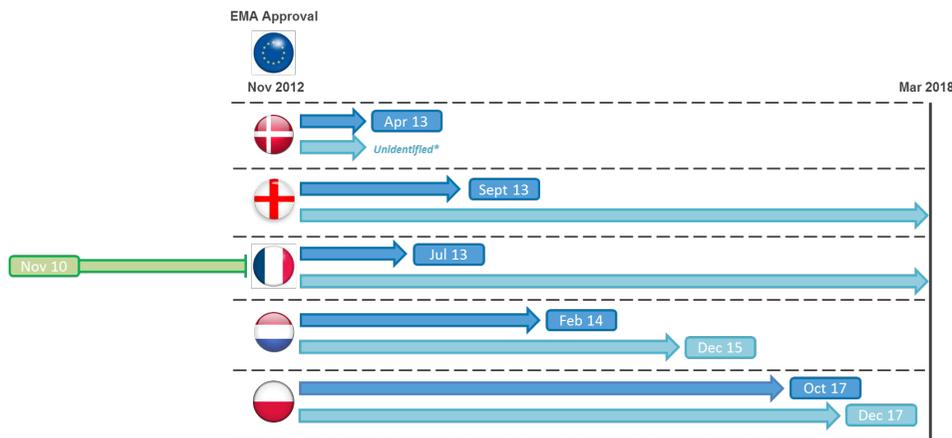
Indication	Class	Drug	DK	EN	FR	NL	PL
Breast cancer	HER2+	Trastuzumab (Herceptin)	●	●	●	●	●
	HER2+	Lapatinib (Tyverb)	●	●	●	●	●
	HER2+	Pertuzumab (Perjeta)	●	●	●	●	●
	HER2+	Ado-trastuzumab emtansine (Kadcyla)	●	●	●	●	●
Melanoma	BRAF+	Vemurafenib (Zelboraf)	●	●	●	●	●
	BRAF+	Cobimetinib (Cotellic)	●	●	●	●	●
	BRAF+	Dabrafenib (Tafinlar)	●	●	●	●	●
	BRAF+	Trametinib (Mekinist)	●	●	●	●	●
	CTLA-4	Ipilimumab (Yervoy)	●	●	●	●	●
	PD-1	Pembrolizumab (Keytruda)	●	●	●	●	●
	PD-1	Nivolumab (Opdivo)	●	●	●	●	●
NSCLC	EGFR+	Gefitinib (Iressa)	●	●	●	●	●
	EGFR+	Erlotinib (Tarceva)	●	●	●	●	●
	EGFR+	Afatinib (Giotrif)	●	●	●	●	●
	EGFR+	Osimertinib (Tagrisso)	●	●	●	●	●
	ALK+	Crizotinib (Xalkori)	●	●	●	●	●
	ALK+	Ceritinib (Zykadia)	●	●	●	●	●
	ALK+	Alectinib (Alecensa)	●	●	●	●	●
	PD-1	Pembrolizumab (Keytruda)	●	●	●	●	●
	PD-1	Nivolumab (Opdivo)	●	●	●	●	●
Ovarian cancer	VEGF-A	Avastin (bevacizumab)	●	●	●	●	●
	PARP	Lynparza (olaparib)	●	●	●	●	●

Notes: Green – Full reimbursement; Amber – Reimbursed with restrictions; Red – Limited / no reimbursement  
Source: CRA analysis

# Delays to access and updating treatment guidelines to reflect innovative treatments are clearly a challenge for PM

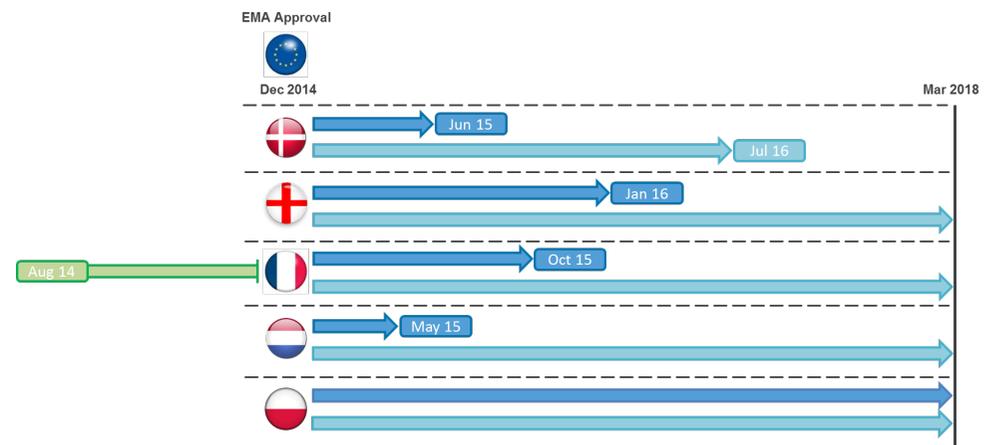


## ALK+ NSCLC: Xalkori (crizotinib)



- In France, Xalkori benefited from an early access scheme – cohort ATU – allowing it to be prescribed by oncology specialists for all patients
- In Poland, access to PM in NSCLC has been delayed and underfunded in comparison to other European markets, with reimbursement taking 5 years
- Timely updating of guidelines is also a clear barrier, with England and France yet to reflect Xalkori almost 5 years after the initial reimbursement decision

## Ovarian Cancer: Lynparza (olaparib)



- First-in-class PARP inhibitor Lynparza has seen variable access across Europe
- NICE finally backed use of Lynparza in 2016 draft guidance, but in a later line of treatment and only after legal action from the manufacturer
- As of March 2018, only Denmark has updated treatment guidelines to reflect Lynparza

However, an important determinant of access is the introduction of early access schemes in several countries

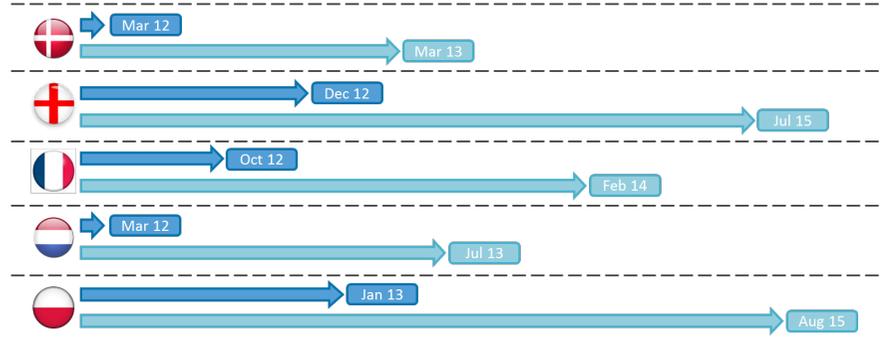


### Melanoma: Zelboraf (vemurafenib)

EMA Approval



Feb 2012



- The Netherlands and Denmark (that exempted products from HTA) have faster access, providing access within 1 month following approval
- All countries made a reimbursement decision within 1 year of approval. England and Poland took the longest, and there was a further 2 year delay to incorporate novel PM for Melanoma into treatment guidelines
- The delay in updating treatment guidelines in Poland meant Zelboraf could only be available through compassionate use or clinical trial programs

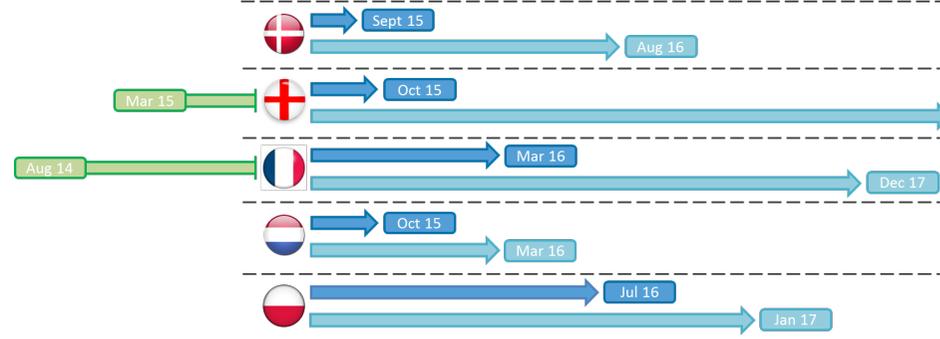
### Melanoma: Keytruda (pembrolizumab)

EMA Approval



Jul 2015

Mar 2018



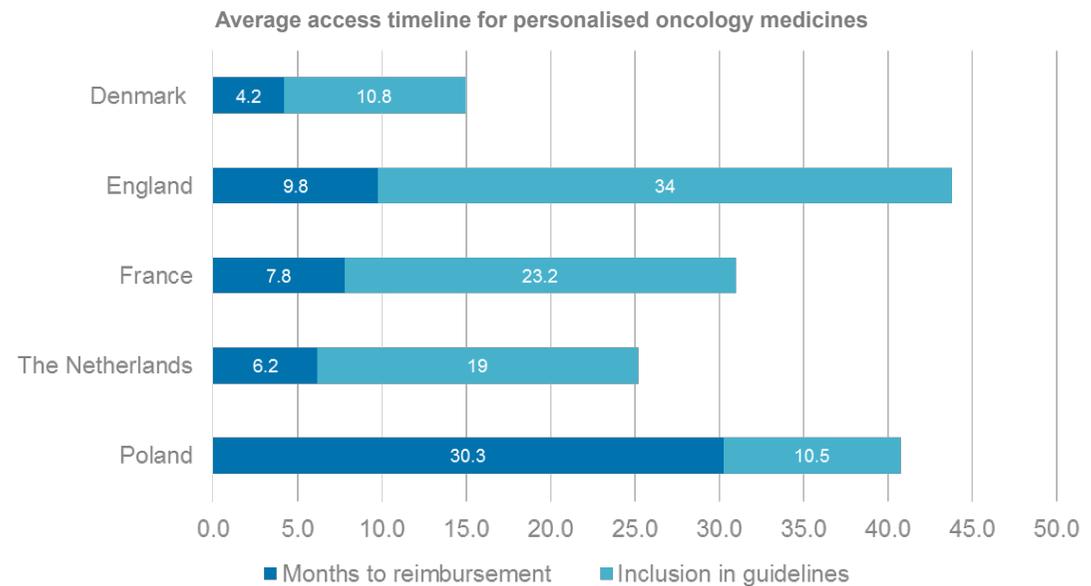
- The use of Keytruda in melanoma was the first product to be launched through the UK's Early Access to Medicines Scheme (EAMS), providing over 500 UK patients with early access. NICE have committed to start the HTA process in parallel with the MA review; earlier NICE assessment of EAMS-approved products is expected to shorten delays to reimbursement
- Indeed draft NICE guidance for Keytruda within 5 weeks of EMA approval. Though NICE is yet to update melanoma treatment guidelines to reflect Keytruda

# Tackling delays to reimbursement of new treatments will ensure more systematic and equitable access



It is clear that access to PM depends on:

1. The **existence of early access mechanisms** that take into account unmet need and provide funding for early reimbursement.
2. The approach to HTA, with countries that have a more **pragmatic approach to use of clinical and economic evidence** (or requirements for additional data collection) to assess the relative benefit of a new personalised medicine exhibit faster access.
3. A fast process for **updating treatment guidelines and care pathways**. Although this varies depending on the role of clinical guidelines, this clearly has an important impact on enabling access in countries such as Denmark and Poland.



Notes: Average access timeline from first-in-class PM in NSCLC, Melanoma and Ovarian Cancer (gefitinib; crizotinib; vemurafenib; pemprolizumab; olaparib)

Source: CRA analysis

There are important characteristics of a country's landscape that facilitates more favourable access to PM

Environment for Personalised Medicines	DK	EN	FR	NL	PL
Policy prioritisation	●	●	●	●	●
Care environment	●	●	●	●	●
Diagnostic testing infrastructure	●	●	●	●	●
Uptake of diagnostics	●	●	●	●	●
Mechanism of value assessment	●	●	●	●	●
Use of real-world evidence	●	●	●	●	●
Speed of reimbursement	●	●	●	●	●
Speed of updating guidelines	●	●	●	●	●
Funding and investment	●	●	●	●	●

Notes: Rating represents current state in the environment. England is trending green for future diagnostic testing infrastructure

## Drawing on research and interviews we have identified key barriers and enablers to accessing PM



Insufficient diagnostic testing capacity or poor quality labs limits use of novel tests	  
Delays or restricted reimbursement / access for novel personalised medicines	 
Lack of specific recognition of PM in value assessment guidelines	  
Delays to access and updating treatment guidelines to reflect innovative treatments	 
Limited level of physician exposure to current research and treatment trends	
Lack of inclusion of mutation testing in clinical guidelines	 
Restrictions on funding for specific high-priority therapy areas (particularly oncology) limits applicability beyond oncology	 
Funding availability or lack of clarity leading to insufficient funding of testing services	 



Development of a specific plan or strategy on PM with dedicated investments in novel diagnostic technologies	  
Highly specialised and coordinated management of care (including testing infrastructure and expertise)	 
Availability of high quality testing platforms and technologies, supported by quality assessment protocols	
Inclusion of PM in guidelines promotes usage and reflects the development of clinical consensus to support PM	 
Early access schemes that favour PM	 
Clear funding and value assessment mechanisms for diagnostic products, and the alignment into the assessment of medicines	
Interim funding mechanisms (e.g. CDF in England)	
Monitoring outcomes through population-based registries in order to facilitate managed entry agreements	

## We have developed policy recommendations to improve equitable access to PM



- ❖ **National policy to ensure prioritisation of PM should work hand in hand with existing health strategic plans** (e.g. National Cancer Plans).

- ❖ The level of resources and funding needs to be aligned to aspirations and the strategy should articulate the genomic profiling strategy.



- ❖ **Continued emphasis is needed on better management of care, consolidating expertise and resources to ensure the adequate 'personalisation of care'.**

- ❖ This can be achieved through a centralised approach (i.e. developing 'centres of excellence') or via cross-functional collaboration through healthcare networks.



- ❖ **National governments should continue investing and cooperating in next-generation testing infrastructure (such as molecular genetics labs) as well as developing dedicated funding pathways to ensure access to diagnostics.**



- ❖ **Collecting data to track access to diagnostics (and making this public) as well as putting a greater emphasis on External Quality Assessments (EQA) of labs will help to ensure consistent testing quality throughout Europe and allow comparison between approaches.**

- ❖ This means promoting international platforms for EQA of labs and research into quality (e.g. IQN Path) to improve diagnostics testing and make EQA participation mandatory for labs across the EU.
- ❖ This should also promote consequences for poor performance of labs, e.g. report to a supervisory authority.



- ❖ **Tackling delays to reimbursement of new treatments will ensure more systematic and equitable access. This can be improved by:**

- ❖ Supporting better alignment of data requirements between regulators and health technology assessment (HTA) bodies – this would improve evidence development and facilitate the value assessment process
- ❖ Sharing best practices on HTA methodology for PM
- ❖ Developing a more flexible approach that incorporates new technologies (e.g. NGS)
- ❖ Being pragmatic in using the available evidence.
- ❖ Introducing Interim/early access programmes