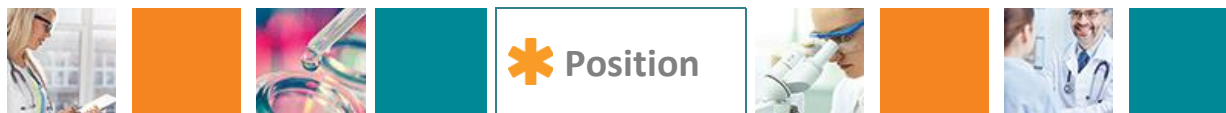


## Value-based healthcare – an industry perspective

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### Introduction

In 2017, the Health Ministers of the OECD concluded in their statement<sup>1</sup> that health systems need to become more outcomes-focused and centred around what matters to citizens and patients.

“The shift from a health system that is centred on providers to one that is centred on people’s individual needs and preferences has important implications for how we measure health system performance. /... / We need to invest in measures that will help us assess whether our health systems deliver what matters most to people. /.../ Measuring how care affects those outcomes that matter most to people and linking those with information already collected by the OECD, such as on expenditure, resources, safety and effectiveness of health care, will help us gain new knowledge on how to improve lives for all.”

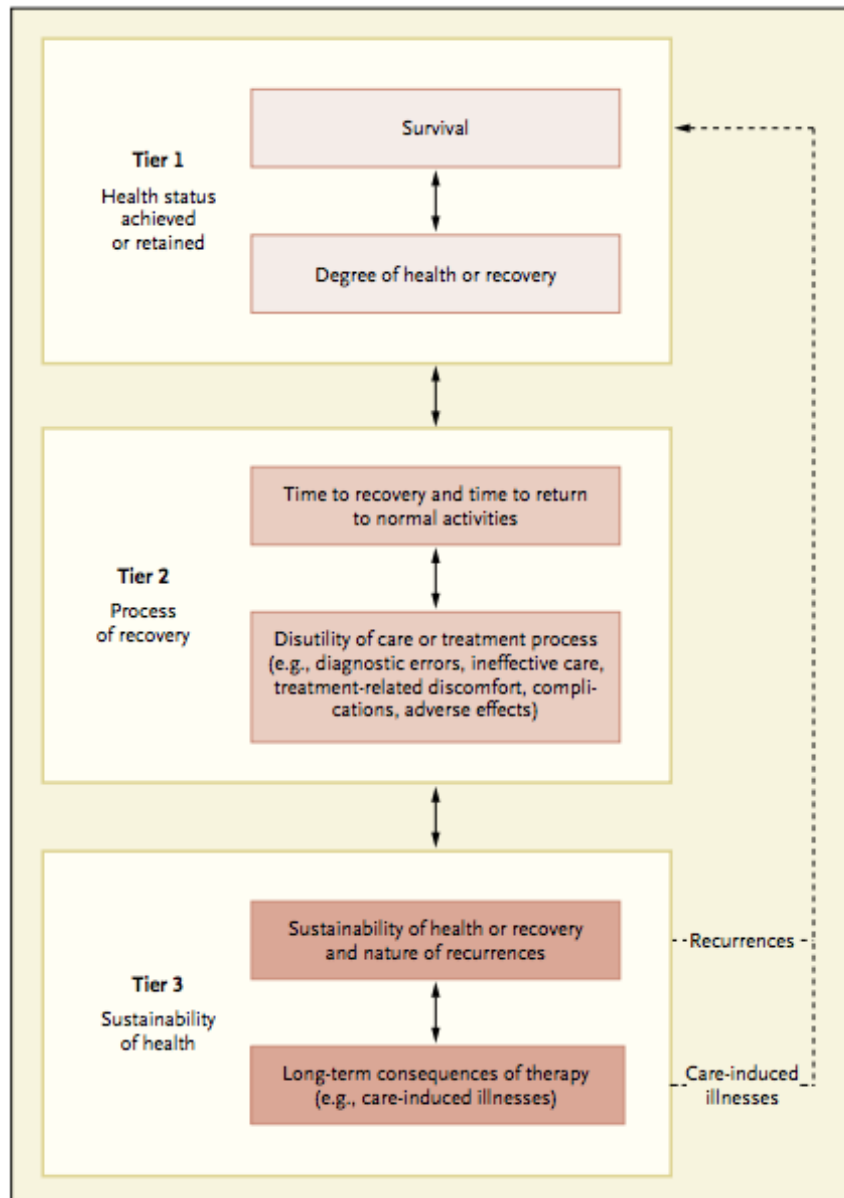
Measuring what matters to people and patients is the fundament of what is known as Value-Based Healthcare. As reflected by the Ministerial Statement, the shift towards an outcomes approach for health has for the last few years been embraced by a growing number of stakeholders as the key to transform health systems and make them both more people-centred and more sustainable in the long term. Though there is a lively debate between stakeholders on exactly how to apply value-based healthcare models in different health systems and care settings, the core mission as expressed by the OECD health ministers - to measure outcomes that matter to people - should be an objective that all stakeholders can rally around as a key enabler for health system transformation.

### How should value be defined in value-based healthcare?

The concept of value in healthcare has been debated for a long time, and there are many different “value frameworks” which take into account different dimensions of value for patients, health systems, societies and stakeholders. Though there is merit in continuously discussing and refining the value concept, it is also important to start implementing a simple, core framework which most stakeholders can agree on. Patient outcomes should be at the centre of such a framework.

<sup>1</sup> <https://www.oecd.org/health/ministerial/ministerial-statement-2017.pdf>

In the context of value-based healthcare as it is normally understood, the core of the “value definition” is outcomes divided by costs. Outcomes are here understood as health outcomes relevant for the patient, and can be both clinician-reported or patient-reported. The outcomes should be measured for the entire patient pathway of relevance, but can also be measured at specific intervals throughout the pathway. The objective is to measure the end result of care rather than the individual procedures. A commonly cited framework for categorizing patient health outcomes is the one proposed by Michael Porter (below).



Source: Porter, M. "What is value in health care?" NEJM, 2010

It should be highlighted that health outcomes are not equivalent to care quality. Quality indicators used by different health systems are in most cases process indicators, e.g. measuring if a certain guideline was followed or if the treating physician spent a certain time with the patient. Quality indicators can be important, but do not substitute for outcomes measures, as these refer to the patient-relevant end result of care, and range from mortality to Quality of Life related indicators (e.g. pain, tiredness) and functional status (e.g. ability to perform daily tasks, ability to return to work). While some outcomes measures are routinely captured by most health systems (e.g. mortality), most are only

partially captured, if at all, and often not in a standardised or quality-assured manner. This is particularly the case for Patient Reported Outcomes (PROs), as these tools are still in their infancy in terms of uptake and standardized use across disease areas, and also in terms of integration in registries and Electronic Health Records.

For the purpose of value-based healthcare, it is important that the outcomes measured can be compared over time and between providers and service, which is why standardisation of outcomes sets is of great importance. Initiatives like COMET (Core Outcome Measures in Effectiveness Trials) and ICHOM (the International Consortium for Health Outcomes Measurement) are working on establishing global standards for outcome sets across disease areas for clinical research and clinical practice respectively. As it is important to measure those outcomes that are important for patients, the development of such outcomes sets should involve patient representatives as well as clinical experts. However, the ambition to achieve standardization and comparability should not be a barrier to implementing outcomes measurement when such standards do not exist or are difficult to implement – continuous outcomes measurement can drive important improvements in care quality also on a local or provider level through continuous learning.

Costs should in a similar way be understood as the total resources required for the pathway in question, i.e. the total sum of for example the cost of individual procedures, acquisition costs for medicines and devices and time spent by healthcare professionals. It is in this context important that achieving optimal outcomes for patients should be the starting point for value-based healthcare models, not achieving high value by cutting costs.

It should be noted that there are dimensions of value that can only be captured in a fully integrated system. For example, better management of chronic disease patients through prevention and effective primary care could save resources in terms of reduced hospitalization, social care services and rehabilitation. However, whether or not these reduced costs are captured in the “value equation” will depend on whether or not the costs of these services are included in the denominator. For example, a value-based model that is only focused on the primary care service would for example not capture this dimension of value (= the savings on hospital care). Furthermore, there are dimensions of value of health that are not captured at all, for example the value to society of a patient returning to work in terms of productivity and potential reduced reliance on social care and social security benefits (as societal costs are not included in the denominator).

Patient experience of care (PREMs) is another dimension that is only included to a limited extent in this framework. This can partially be explained by patient experience being more challenging to capture in a way which is comparable and can be subject to international standards.

Despite the above-mentioned constraints, EFPIA believes that the core definition of VBHC as described above is a key tool to analyse healthcare delivery and health system performance as it:

- 1) Puts the spotlight on measurable, patient relevant outcomes, thereby being essential for achieving true patient-centred healthcare;
- 2) Focuses on the entire cycle of care, and therefore looks at healthcare delivery in a holistic perspective (and therefore can help identify ways to shift from high- to low-value care).

- 3) Should be possible for all stakeholders to accept as a starting point for a more outcomes-based assessment of health system performance (even though additional value dimensions might be preferred by different stakeholders depending on perspective).

The pharmaceutical industry is committed to increase the value of its products and services by focusing on the patient and patient outcomes. Already today, there is a growing trend to incorporate Patient Reported Outcomes in clinical trials and real world data studies. In clinical trials, using Patient Reported Outcomes as complementary endpoints to clinical endpoints can give a more holistic view of the benefit of a medicine, including relating to Quality of Life and functional status. The use of PROs in clinical trials is already well established in oncology, rare diseases and allergy, and growing in for example the autoimmune and gastrointestinal areas. Between 2012 and 2016, approximately 22% of orphan drugs approved by the EMA incorporated PROs, and in oncology, 70% of indications for 49 EMA and FDA approved drugs included PRO data in their regulatory submissions.<sup>2</sup>

With respect to Real World Data, the industry is engaged in several projects to increase the access to, and quality and usability of, Real World Data for healthcare decision-making, including through the Innovative Medicines Initiative. Projects such as HARMONY<sup>3</sup>, ROADMAP<sup>4</sup>, BigData@Heart<sup>5</sup> and PIONEER<sup>6</sup> define outcome measures and utilizes big and deep data sources to improve patient care for haematological malignancies, Alzheimer's, cardiovascular diseases and prostate cancer respectively, and the European Health Data and Evidence Network (EHDEN)<sup>7</sup> project seeks to harmonise around 100 million Electronic Health Records to create a large scale federated network for real world data research in Europe. Patient relevant outcomes measures play a central role in these projects, including in the EHDEN project where ICHOM outcome standard sets will be used to develop the OMOP Common Data Model.

The industry also works to further develop innovative medicines already on the market to improve patient experience, for example by developing longer duration of action (which decreases the number of times a patient needs to take a certain medication), less burdensome side effects or other administration forms (e.g. moving from injectable to oral administration). These types of improvements help increase adherence to treatment, which both improve patient outcomes and reduce wasteful spending. Patient support programs are another way in which pharmaceutical companies can support patients with disease understanding and management, and treatment adherence.

However, more can be done, and this requires a systematic engagement with patients throughout the life-cycle of a medicine, starting already with early research and development to understand patients' needs, all the way to the use of the medicine and fully understanding its real-world effects. The EFPIA Board in 2017 adopted the white paper "Working together with patient organisations" to set out this overall vision and further guide its members<sup>8</sup>, and EFPIA members have also initiated the PARADIGM public-private

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<sup>2</sup> IQVIA 2019. *The changing landscape of research and development*. Page 37.

<sup>3</sup> <https://www.harmony-alliance.eu/>

<sup>4</sup> <https://roadmap-alzheimer.org/>

<sup>5</sup> <https://www.bigdata-heart.eu/>

<sup>6</sup> <https://prostate-pioneer.eu/>

<sup>7</sup> <https://www.ehden.eu/>

<sup>8</sup> <https://efpia.eu/media/412524/working-together-with-patient-groups-23102017.pdf>

partnership to provide a unique framework that enables structured, effective, meaningful, ethical, innovative, and sustainable patient engagement in research priority setting, clinical trial design and early dialogue.<sup>9</sup>

## How can value-based healthcare contribute to health system transformation, and help health systems across the European Union become more effective, accessible and resilient?

Value-based healthcare has the potential to contribute to health system transformation by improving decision-making and resource allocation at several levels of the health systems.

- On a system level, policymakers will have better information on which to base decisions on health system reform and resource allocation, and tools to incentivize effective health promotion and prevention and a lifespan approach to health. Value-based decision-making will facilitate sustainable healthcare by shifting resources from low- to high-value care and the rewarding of outcomes rather than volume.
- On a provider level, the collection and feedback of outcomes data will help providers continuously improve their care quality, including through comparing and learning from others, and will be incentivized to prioritise those interventions which bring the best outcomes for patients, including through care coordination with other providers;
- On a patient level, better information on available treatment options and monitoring of patient-relevant outcomes will help the patient to take decisions on his or her own care together with the treating physician.

The sum of these decisions will help making health systems more effective, accessible and resilient.

### Effective

According to the EU State of Health Companion report, effectiveness refers to the extent to which health services achieve the desired results or outcomes at the patient or population level. However, as also noted in the report, the capture of patient relevant outcomes is an underdeveloped part of most European health systems today, as most data collection is focused on input metrics (e.g. number of doctors per capita) or process metrics (interventions, prescriptions, adherence to guidelines etc.). The core of value-based healthcare is to measure patient-relevant outcomes in a consistent way across providers, services and systems.

Although some elements of healthcare are relatively well understood in terms of value, notably medicines due to the extensive clinical trials that precede marketing authorisation, the value delivered to patients in aggregate throughout the patient pathway is often less well understood. Therefore, the implementation of standardised outcome measures is a necessary tool to achieve truly effective health systems, and organisations such as ICHOM work in a systematic way to define global standards of patient relevant outcomes for

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<sup>9</sup> <https://imi-paradigm.eu/>

specific conditions or disease areas. The systematic capture of such outcomes data throughout the health system would greatly improve the assessment of whether or not different healthcare interventions are as effective as they should, and provide evidence as to how to improve healthcare delivery and care pathways.

### **Accessible**

Value-based healthcare can facilitate the provision of timely health services, including prevention, by directing resources to interventions that bring most value for patients. Focusing on the outcomes, the end result of care, rather than the process or individual interventions, will help refocus investment towards primary, secondary and tertiary prevention, including predictive medicine and early diagnosis, as this is often the most effective way to achieve good outcomes at a lower cost. Value-based healthcare will create a clear business-model for preventive services and stimulate deployment of more innovative prevention services for different population groups.

When it comes to innovative medicines, there are many examples of how early access schemes based on outcomes can provide fast access for patients. An outcomes-based agreement, where the final payment is dependent on the achievement of specific real-world outcomes, can give a payer the necessary de-risking framework to reimburse a new medicine when there are uncertainties about the real world clinical value.

However, it should be noted that moving from fee-for-service type models of payment to more outcomes-based models could in some respect decrease access to interventions which are unnecessary or bring low value to patients. The OECD mentions caesarean sections, the use of Percutaneous Coronary Intervention in stable patients, imaging for lower back pain and headache and over-prescription of antibiotics and antipsychotics for older patients as examples of interventions associated with over-diagnosis or over-treatment. The access dimension of healthcare systems should therefore be understood in the context of high-quality services which deliver the best possible outcomes for patients. The shift towards personalised medicine, where specific diagnostics and biomarkers will help determine the appropriate treatment for each patient, will help enable access to the right treatment to the right patient and the right time, thus reducing inappropriate or low-value care.

### **Resilience**

By applying a value-based model, health managers and decision makers can identify the services, pathways and interventions which bring the best outcomes to patients relative to cost. Furthermore, by focusing on the end result of care, value-based healthcare can incentivize investment in healthcare interventions, including prevention, which brings good outcomes and potentially also savings for the system in the long term. This makes value-based healthcare more suitable as an instrument for making health systems resilient while optimising health for citizens and patients than a simple cost-cutting model which focuses on short-term savings rather than long-term sustainability.

Furthermore, the systematic measurement of standardised health outcomes combined with digital tools and data analytics will enable a cycle of continuous learning in healthcare organisations, where data on variations in outcomes is regularly analysed and fed back to physicians and other decision-makers. Applied at a larger scale, this will lead to "Learning Healthcare Systems" where quality improvement, outcomes research and dissemination of

best practices can take place at a much faster pace than today, supported by Artificial Intelligence and other decision support systems.

One key element of value-based healthcare is moving from payment and reimbursement models which are based on volume of care produced (e.g. numbers of doctor appointments, volume of surgery or prescriptions) towards models which are more closely aligned to the outcomes achieved for patients. For healthcare services in general, this implies shifting from fee-for-service type reimbursement models to bundled payments, capitation-based models and other models which reward the end result of care rather than the volume of care produced. It is important when designing these tools that patient outcomes and care quality are front and centre, as these models could otherwise become tools for cost-containment. When it comes to innovative medicines, the types of outcomes-based payment models which have been referred to above, can increase certainty for payers in terms of achieving value-for-money and minimize less effective or low-value spending.

More integrated and outcomes-based payment models can also facilitate the integration of care, as different services are incentivised to work together to achieve the best results for patients. This includes removing budget silos between different parts of the healthcare system, which can enable more efficient resource allocation focusing on achieving the best value for patients. A more holistic financing model for healthcare can enable investments that bring the best value for money in a long-term perspective, even when the return of that investment is accrued in another part of the health system.

### **Towards an actionable roadmap**

In order to accelerate the transition towards health systems more focused on outcomes and value, EFPIA calls for a multi-stakeholder dialogue to identify a concrete and actionable roadmap, building on already successful initiatives, focusing inter alia on the following elements:

- The importance of measuring patient relevant outcomes (both clinical and patient-reported);
- Standardization initiatives relating to measurement of patient-relevant outcomes;
- A “full cycle of care” approach to measuring outcomes and costs;
- The importance of publishing risk-adjusted outcomes data for comparisons, information to patients and benchmarking;
- Variation analysis and exchange of best practice;
- Integration of budgets/holistic budgeting for more effective resource allocation;
- Moving to payment models that are more aligned to patient value instead of care volumes.