

Healthier future

The case for outcomes-based,
sustainable healthcare



efpia

European Federation of Pharmaceutical
Industries and Associations



Healthier future

Shaping sustainable, outcomes-driven
healthcare in Europe



Outcomes driven, sustainable healthcare has been the subject of discussions across the healthcare community for many years. We recognise that an outcomes based system may lead to lower prices for pharmaceuticals that do not deliver as much value as we hoped, and that there is therefore risk involved for the pharmaceutical industry in this context. However, at least such a system would also fairly reward true breakthrough products, and the reduction of waste in the system as a whole would create headroom to bring innovation to patients faster and broader.

We believe an outcomes based system will do a better job of stimulating and rewarding real innovation. The innovation that benefits patients most, and supports health system sustainability.

As an industry, we believe we can contribute to a more sustainable future by developing new pricing models, such as outcomes based or value based contracts. This is happening in a number of countries, but it is in its infancy and will require partnering with patients, healthcare providers, payers and industry to create real breakthrough.

Healthier future is our voice in this important dialogue.

Contents

Section 1
Foreword
Page 4

Section 2
European Healthcare:
challenge and opportunity
Page 6

Section 3
Analysing our current
approach to managing
healthcare
Page 9

Section 4
Outcomes driven,
sustainable healthcare
Page 14

Section 5
Case studies
Page 16

Section 7
Healthcare Data: The key to
outcomes-based healthcare
Page 22

Section 6
What are the barriers in navigating
towards an outcomes-based
healthcare system?
Page 19

Section 10
Being part of the
outcomes-based dialogue
Page 30

Section 9
Paying for outcomes
- medicines pricing in
an outcomes-focused
systems
Page 26

Section 11
Afterword from
the *Healthier future*
Advisory Panel
Page 33

Section 8
Moving along the
outcomes-based
spectrum
Page 24

Section 1: Foreword

Nathalie Moll, EFPIA Director General

We live in an era of unprecedented breakthroughs in medical technologies. Advances in biomedical sciences, greater understanding of the human genome and disease pathways combined with a sustained investment in research & development by the pharmaceutical industry are delivering new, innovative therapies across all disease areas. Immuno-oncology and targeted therapies are transforming the outlook for cancer patients step by step, new cell- and gene therapies are being designed to repair or replace faulty cells and genes causing a range of diseases from hemophilia to diabetes, and the tireless search for new ways to target neurological disorders such as Alzheimer's goes on. Serious and previously deadly viral infections such as HIV/AIDS and Hepatitis C are now possible to treat and even cure in the case of Hep C.

In addition to bringing the promise of longer and better quality lives for patients suffering from disease, many innovations can help to reduce costs in other parts of the health and social care system, for example by reducing the need for hospitalisations, expensive surgery, or long-term care.

Despite these fantastic advancements, most healthcare systems are not delivering to the

fullest of their capacity, and many are struggling to cover an ever-increasing demand of healthcare with limited budget resources. As the ageing of our populations continue, with growing incidence of chronic diseases as a result, the pressures on our health- and social care systems will continue to grow. This has health and finance ministries in Europe and across the world worrying about the long-term sustainability of public healthcare expenditure. How are we going to prepare for the future?

The answer is twofold: we must start to see health both as an investment and as a pre-requisite for a healthy and growing economies, not only as a cost. And we must be ready to scrutinize our healthcare systems and the way we organize and invest our resources in a much more systematic and thorough way. Are we doing those things that actually make patients healthier and/or better prevent people from getting ill in the first place? It might seem like an obvious question – but in the complex and rapidly changing world of our healthcare systems, the answer is far from a given. In reality, according to most estimates, around 20% or more of our healthcare expenditure is wasted on less effective, or even harmful, interventions, money that could be put to much better use. This is an equation that we cannot afford.

Luckily, we live in an age when we can harness the power of data and digital solutions to record and track which healthcare interventions deliver most value to patients and society. Instead of measuring our healthcare systems in terms of inputs (numbers of doctors or hospital beds) or procedures (number of screenings or pills prescribed), we must start to select and measure those health outcomes that are the end result of the various procedures. Through agreeing which outcomes we want to achieve for each condition and patient group, and then measuring these in a standardized way, we can start comparing and contrasting different procedures, providers, regional care and even countries, and learn from each other's best and worst practices. A growing body of evidence shows that healthcare institutions that systematically track outcomes to holistically refine their patient pathways from prevention to rehabilitation, not only improve the outcomes for patients over time but also save costs.

Furthermore, healthcare systems must also start rewarding healthcare providers for the health outcomes they deliver, rather than for the quantity of services or products. This would ensure that resources are invested in the interventions that deliver the most value, and would also send a powerful stimulus to

all actors in the system to develop innovations that deliver true value. We already have positive examples of primary care providers moving from fee-for-service towards pay-for-performance type schemes, and agreements where innovative medicines are reimbursed based on outcomes rather than just volume. More needs to be done in order to make these systems work at scale, both in terms of agreeing on which outcomes to measure and in setting up the infrastructure for collecting and analysing the data necessary for these models to work.

EFPIA and its member organisations are committed to doing our part in changing how healthcare is provided in Europe. This transformation requires dialogue and collaboration between all healthcare system stakeholders, including patients, healthcare professionals, providers, payers, policy makers and the life science industries. If we work together, we can set European healthcare systems on a new path towards a more outcomes-based and sustainable healthcare. Together, we can create a Healthier Future for Europe.

Section 2: European Healthcare: challenge and opportunity

By 2025, the global population is expected to increase by 1 billion people, with half a billion more people over 50 years of age¹. These significant demographic changes are reflected in Europe, where life expectancy has risen by nearly a decade over the last 50 years. According to the Organisation for Economic Co-operation and Development (OECD), the percentage of the European population over 80 years of age has risen from 2% in 1980 to over 5% in 2016 and is projected to rise to 12,7% by 2080².

As the population continues to age and grow, so does the prevalence of chronic diseases such as diabetes, cardio-vascular disease and cancer.

Between the ages of 45 and 65, the incidence of heart disease more than doubles, and over a quarter of people aged 85 years and over live with dementia.

The result of these significant demographic changes is that Healthcare systems across Europe are facing unprecedented challenges. Demand for health and social-care services is rising rapidly, driven by the ageing population and increased prevalence of chronic disease. Managing these conditions already accounts for 75% of healthcare expenditure in Europe, a figure that is set to rise as demographic trends persist. Spending on healthcare as a whole has risen faster than GDP, and projections show that the expenditure on long-term care alone as a percentage of GDP could double by 2060³.



Over a quarter of people aged 85 years and over live with dementia

Europe's history of health is a story of success in which improved socio-economic conditions, lifestyle changes, better prevention and public health strategies coupled with advances in treatment and better patient care have all played their part. But as the continent is gradually starting to reinvest in healthcare after the strict cost-cutting during the financial crisis, healthcare systems and governments across Europe are facing difficult choices as they allocate resources to manage the health and social care needs of their citizens.

Despite the challenges, there are many reasons to be optimistic about a *Healthier future* for Europe. With over 7000 medicines in development, an exciting, new wave of innovation will play a key role in addressing the challenges faced by patients, healthcare systems, and society. This pharmaceutical innovation is mirrored by developments in medical devices, diagnostics, imaging and data science.

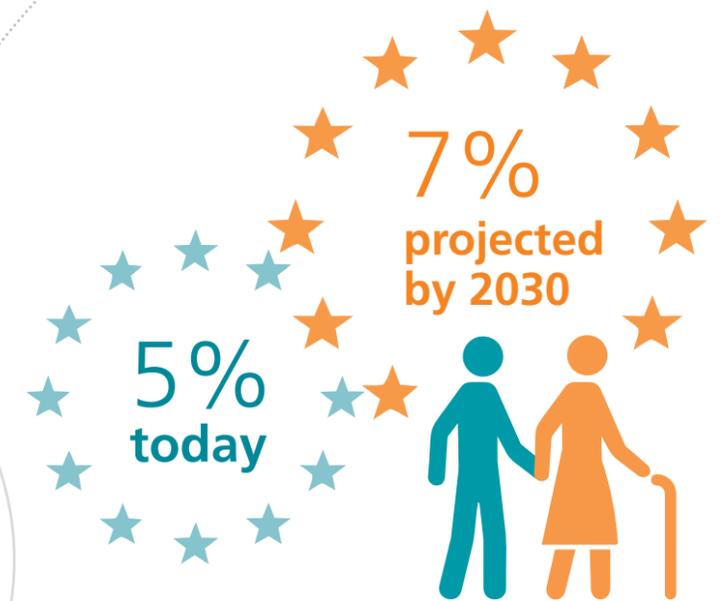
The potential of this exciting new wave of innovation is enormous. We already experience the beginning of a revolution in cancer treatment by exploiting the ability of the body's immune system to locate and eradicate cancer cells. Advanced therapies such as cell and gene therapies are giving new hope to patients with previously untreatable rare or chronic diseases. Significant advances in the vaccines sector are targeting global health issues such as malaria, Ebola, HIV, and Cancer.

Genomic research and new data analytics techniques are giving researchers powerful tools to study how multiple genetic factors impact on disease development. This offers a springboard towards the creation of specifically targeted, personalised medicines.

But patients can only benefit from this innovation if it is affordable now and sustainable in the future. In the context of ageing populations and chronic disease, the adoption of innovation places additional pressure on resources. How we manage the rising healthcare demand and capitalise on the new medical innovation in a sustainable way, is the foremost question for many stakeholders in European healthcare.



Between the ages of 45 and 65, the incidence of heart disease more than doubles



The percentage of the European population over 80 years of age has risen from 2% in 1980 to 5% today and is projected to rise to 7% by 2030



Section 3: Analysing our current approach to managing healthcare

Europe provides some of the best healthcare in the world. It has long been a centre of excellence for medical research, education and clinical practice. But conscious of the challenges outlined in section 2, many stakeholders are questioning the way healthcare is organised, performance is measured and rising healthcare demand is managed.

Despite the progress of public health across countries in Europe, significant health inequalities persist. This is not simply a question of economics; the evidence suggests significant variation in treatment outcomes for patients between countries but also within countries, which cannot be explained by different levels of investment in health and healthcare⁴.

So what lies behind the variance in patient outcomes across Europe and across national healthcare systems within Europe?





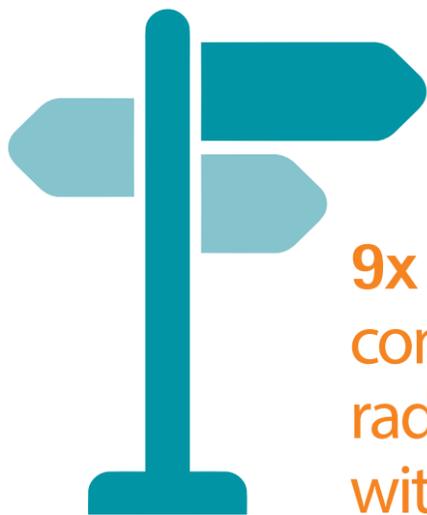
7x variation in breast cancer re-operation rates within a country



3x variation in COPD admission rates within a country



4x variation in lung cancer 5-year survival across OECD



9x variation in complication rates from radical prostatectomies within a country

Variations in clinical practice

The fragmented nature of healthcare systems across Europe means we often see significantly different care pathways for patients and large variations in clinical practice. Healthcare providers may have received different training, use different treatment guidelines, adopt innovation or new clinical developments at varying rates, provide care in multiple settings and have a wide range of political, social and healthcare priorities. Some variation is natural since medical science is constantly in development, and some is desired because certain patients may require a different treatment, for example due to different tolerability. However, too often care practices that deliver a less than optimal outcome continue to be used despite better methods being available.

Complex care pathways

Healthcare systems are often large and complex. A patient with multiple chronic diseases often has to deal with a whole range of providers, from primary care physicians to specialists in hospitals and physiotherapists in rehabilitation centres. Many times these different providers don't coordinate well with each other, leaving the patient alone to navigate a complicated system. Uncoordinated care can also lead to unnecessary duplication, when the patient has to undergo the same test several times. Similarly, it can result in under-treatment, when patients "fall in the gaps" between providers, and important interventions are not undertaken.

This puts the long-term sustainability of healthcare in Europe at risk. It also provides a clue to how we can re-orientate the management of healthcare to improve outcomes for patients and make them more sustainable.

Data fragmentation

The fragmentation in care is also mirrored in the data systems intended to capture a patient's health data. While many systems record data on procedures and interventions, models for measuring and collecting data on health outcomes are under-developed. As such, the data available to scrutinise the effectiveness of different health interventions and identify best practice is limited. Systems that do not systematically track interventions and health outcomes of patients, are lacking the basis for a root-cause analysis that would tell them why one patient is achieving a better outcome than another. With many systems lacking such a comprehensive evidence base, they struggle to make effective decisions.

Transaction-based incentives

In any field of activity, targets and incentives drive behaviours. Healthcare systems have tended to focus on targets, measures and incentives based on inputs and procedures rather than the outcomes they deliver. Success is often defined by transactional measures such as the number of tests completed, patient visits to a Doctor or waiting times rather than by the patient's outcomes that result from these interventions. An inevitable consequence of this approach is that priorities and resources are focused on meeting these input targets with little reference to the impact they have on patients.

The cumulative effect of variations in clinical practice, complex care pathways, data fragmentation and transaction-based incentives is variation in outcomes for patients, waste and inefficiency in the system. A recent OECD report estimates that around one fifth of health expenditure make no or minimal contribution to health outcomes. In the context of ageing populations, increasing prevalence of chronic disease, and constrained resources, this puts the long-term sustainability of healthcare in Europe at risk. It also provides a clue to how we can re-orientate the management of healthcare to improve outcomes for patients and make them more sustainable.

The cumulative effect of variations in clinical practice, complex care pathways, data fragmentation and transaction-based incentives is variation in outcomes for patients, waste and inefficiency in the system.



4x variation in (male) hip replacement across OECD

What are outcomes?

Outcomes are the results of treatment that patients care about most.

Outcomes are not “outputs”; they are not lab results; they are not technical details. They’re real-world results, like physical functioning or level of pain. Unfortunately, today, in healthcare systems around the world, evaluation efforts take into account a number of clinical indicators, structural metrics, and even reputation – but they tend to ignore outcomes.

International Consortium for Outcomes Measurement, 2016



Section 4: Outcomes driven, sustainable healthcare

By re-orienting healthcare systems on outcomes, many stakeholders, including EFPIA, believe that we can put healthcare systems in Europe on a more sustainable path.

The principle behind outcomes-based healthcare is that healthcare systems should focus on delivering health outcomes, rather than on delivering interventions. Focussing on outcomes addresses the central problem that healthcare systems today do not incentivise what actually matters: better health for patients.

Instead of paying for hospital beds, visits to the doctor, pills, screenings and surgical interventions, our focus should be paying for better health and longer lives. By determining exactly what type of intervention brings the best health outcome for each patient, and directing our resources to those specific measures facilitates better health outcomes and quality of life for patients. A focus on delivering outcomes also results in more value for money and can contribute significantly to healthcare system sustainability by identifying and discontinuing interventions that do not deliver superior or no patient outcomes. By eliminating spending on ineffective interventions, a focus on outcomes can free up the resources required to address the healthcare needs of an ageing population and fund those innovations that deliver positive results for patients and value for systems. The potential for waste reduction is significant. It is estimated that around 20% of healthcare spending is currently wasted on ineffective interventions⁵.

Sustainable healthcare

Improve Outcomes

The starting point is to focus on improving patient outcomes

Reduce overall costs

Better quality of care is often less expensive over the long-term

Increase value

Better quality care at equal or lower cost leads to higher value in the system

$$\frac{\text{Health Outcomes}}{\text{Cost of delivery}} = \text{Value}$$

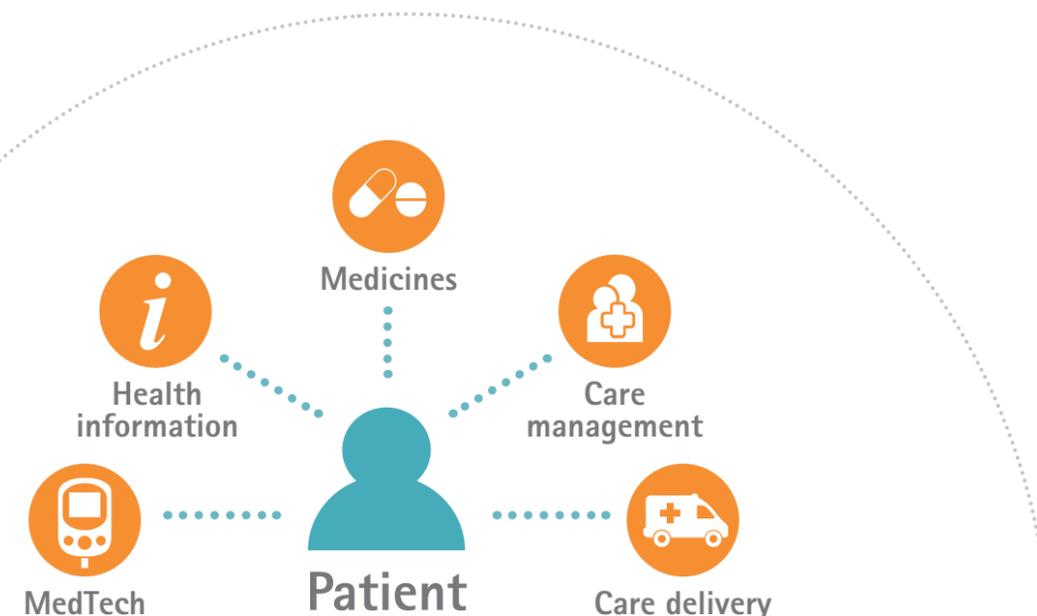
True value-based healthcare, in which systems guide their decision based on the ratio of outcomes to cost, is still in its infancy. Healthcare systems across Europe are still grappling with how to make the concept a reality and what kinds of tools are needed to make it work. Like other potentially paradigm-shifting concepts such as big data or mobile health, the initial excitement is followed by the realisation that system-wide change is immensely challenging. But at EFPIA we believe that the goal, a healthier, more sustainable future, can be realised step-by-step, in partnership with stakeholders across the system.

This transition will take both time and investment, and most of all political will. Member States need to invest in integrated health information systems for tracking health outcomes – with disease registries and

Electronic Health Records as key components – and standardise outcomes metrics that will make it possible to compare health outcomes across providers, regions and even countries. Patient involvement is key when agreeing on these outcomes metrics, but equally so is the involvement of healthcare professionals, since a continuous evolution of evidence based clinical practice is at the core of an outcomes-based approach to healthcare.

The good news is that we don't have to wait for the perfect system to be in place, change can be implemented step-by-step. Even though reduced waste and better value for money will be one of the rewards, the main driver for change must be better health outcomes and putting the patient at the centre of healthcare management.

The key is to learn from each other and spread the use of models that have been proven to work – the next chapter explores just a few of the inspiring examples from across Europe.



Section 5: Case studies

Sweden

The Swedish Childhood Cancer Registry has captured the diagnosis, treatment and outcomes of patients since the 1970s. At 80%, Sweden now has the highest childhood cancer survival rate in Europe, with no significant regional variation in the survival rates⁶

Sweden

Since 1998, Sweden's National Cataract Registry has collected nationwide data on postoperative endophthalmitis (PE), which, although rare, results in blindness in 30 to 50% of patients⁷. The Registry enables the identification of specific risk factors⁸, which has contributed to a decline in PE from 0.11% of all cataract surgery cases in 1998 to 0.02% in 2009⁷

England

The Radiotherapy Innovation Fund secured £23 million of government funding to support the roll-out of advanced radiotherapy treatment, with the aim to ensure 24% of radical treatments used 'inverse planned' Intensity Modulated Radiography Therapy (IMRT)²¹. In just six months, the number of patients receiving the more effective IMRT increased to over 22%²¹. This means that annually, 5,800 more patients in England can receive advanced radiotherapy, leading to better clinical outcomes and a better quality of life for patients²²

The Republic of Ireland

The STOP-HF study demonstrated the benefit of screening with brain-type natriuretic peptide testing¹¹, showing that it is possible to reduce the incidence of new onset heart failure by 50% and deliver improved patient outcomes at no extra cost¹²

England

Launched in 2016, the NHS Diabetes Prevention Programme (DPP) seeks to identify those at risk of diabetes and refer them onto an evidence-based behaviour change programme. By 2020, 100,000 referrals are expected to be available annually⁹. This provision through the DPP will help tackle the 80% of type 2 diabetes cases which could be prevented by simple cost-effective interventions, such as identifying people who are most at risk¹⁰

The Netherlands

Within 10 years of the onset of Rheumatoid Arthritis (RA), at least half of patients in developed countries are unable to hold down a full-time job¹⁹. Successful implementation of early aggressive treat-to-treat strategies among patients with a clinical diagnosis of very early RA included in the DREAM remission induction cohort study, demonstrated that remission in daily clinical practice is a realistic outcome for patients²⁰

The Netherlands

In the Netherlands, healthcare professionals have helped to develop the outcome indicators for registries of quality and outcome data. In exchange for reported data, hospitals are provided with a weekly dashboard to help identify best practice and how outcomes can be improved. Outcomes have include a ~30% decrease in mortality after resection in colorectal cancers between 2010 and 2012¹³

Spain

In Catalonia, the use of an expert patient group has resulted in a 22% increase in self-care and 12% uplift in patient quality of life. For the healthcare system, the programme led to a ~40% reduction in hospitalisations, as well as fewer physician appointments and ER visits¹⁸

Germany

A focus on transparent outcomes at the Martini Klinik Centre of Excellence for Prostate Cancer in Hamburg resulted in one-year erectile dysfunction falling to 35%, from a hospital average of 76%. The focus on outcomes has also seen a compounded annual growth rate of 18% in prostatectomies since 2004¹⁷

Spain

The Ribera Salud Group's Alzira model combines public funding, ownership and control with private healthcare provision. This integrated model tracks performance and creates incentives for better performance through financial reward at an individual and system level. Implementation of the model has led to reduced waiting times for diagnostics such as MRI (88% reduction), a 34% reduction in patient re-admission and a 26% increase in levels of patient satisfaction^{15,16}

Germany

Since 2006, Gesundes Kinzigtal (GK) has been contractually accountable for the whole health care service budget for ~50% of the 69,000 people in the town of Hausach. Through targeted planning, which incorporates a focus on prevention and wellness, and continuous evaluation, GK has reduced the per person healthcare cost by €151 and reduced the mortality rate by 53%¹⁴



Section 6: What are the barriers in navigating towards an outcomes-based healthcare system?

Better outcomes for patients and more sustainable healthcare systems are common goals for all stakeholders in the European healthcare landscape. Outcomes-based approaches have been around for over a decade but system-wide change is problematic and notoriously difficult. This section explores some of the barriers to a system-wide transition to a more value and outcomes-based approach and can be grouped into technical, structural, financial and political aspects.





Technical barriers

Outcomes-based healthcare relies on delivering value, measured as health outcomes divided by costs. It is based on the ability to capture, analyse and utilise outcomes (and financial) data, with standardised definitions of outcomes at the core.

Today, the measurement of outcomes is not common practice. Many providers and healthcare systems do not know which outcomes they achieve in which disease area. Those healthcare systems and providers within one system that are measuring outcomes, often use different outcomes measures, making comparisons and evidence-based service design difficult. At a more basic level, the availability and usability of IT systems in clinical settings varies significantly between providers. For clinicians operating under pressure, the capture of the necessary outcomes data can quickly become a burden. Even where outcomes data is captured effectively, the fragmented nature of IT infrastructure and lack of interoperability between IT systems, mean that sharing data across providers and between healthcare services is often not possible. Without a comprehensive evidence base, the analysis of clinical data to identify where best outcomes are being achieved and why is burdensome and costly at best, and technically unfeasible at worst.

A consequence of these technical issues and a further barrier to an outcomes based approach is that outcomes-based reimbursement models, that can help drive a more value-based approach, are often considered too burdensome to administer. A lack of readily available data and analytical capability needed to support an outcomes-based contract adds to the investment cost and uncertainty for entering into such arrangements. Providers will revert to a more volume-based approach if they do not have the systems and administrative capacity in place to measure and ultimately reward outcomes.

Structural barriers

The most significant structural barrier is the fragmentation of healthcare systems. Individual organisations within a healthcare system often have different definitions of outcomes, different incentives and targets, and alternative preferred care pathways. They also collect different data within their own IT systems. This is a major barrier to the development of outcomes-based healthcare at national or pan-European level.



Financial barriers

Instead of rewarding the long-term improvement of a patient's health, fiscal incentives tend to reward process related measures like adherence to clinical guidelines, the number of times a doctor talks to his or her patients about prevention and healthy lifestyles, the number of patients of a certain category that are referred to a specialist or prescribed a certain medication. Or something else related to process rather than actual outcome. Where overall resources are under pressure, it is natural that fiscal incentives will drive action.

There are additional barriers in how healthcare systems are financed. Budgets are often separate for different types of interventions. For example, there are separate budgets for hospital treatment, medical devices, pharmaceuticals, and outpatient care. This is why downstream savings from an intervention are often realised in a different part of the healthcare budget to where the initial investment in the new treatment was made. Sometimes, these savings even accrue outside healthcare budgets like in social care budgets, for example when medical interventions allow a patient to continue to work and contribute to social welfare systems. Similarly the benefits from a healthcare intervention may be realised over the lifetime of the patient, whereas the costs of the intervention may already be incurred in the year the treatment is administered. Introducing flexible approaches to financing healthcare such as outcomes-based reimbursement models, overcoming budget siloes and developing systems to allocate savings across financial years are key milestones in transitioning towards a more outcomes-based approach to healthcare.

Political barriers

System-wide, transformational change is challenging, it requires strong political commitment over a number of years to make it happen. Implementing some outcome-based decisions such as concentrating highly specialized care or transitioning from inpatient to outpatient and community care can invoke strong reactions from local stakeholders who are attached to particular local services.

The concept of outcomes-based healthcare is intellectually attractive but its implementation can include some difficult, sometimes politically unpopular decisions.

In the highly emotive and politicised healthcare sector, policy makers need a strong evidence base, case studies of successful implementation, national and international support to facilitate the change to an outcomes and value-based healthcare system at pace and scale.



Section 7: Healthcare Data: The key to outcomes- based healthcare

As healthcare begins to embrace the digital revolution, the potential of data to change the way we deliver healthcare, improve patient outcomes and shape future research is an important new frontier. The availability of healthcare data is increasing exponentially, providing an opportunity to explore new ways to capture and analyse healthcare data and to accelerate the transition to an outcomes-based approach.

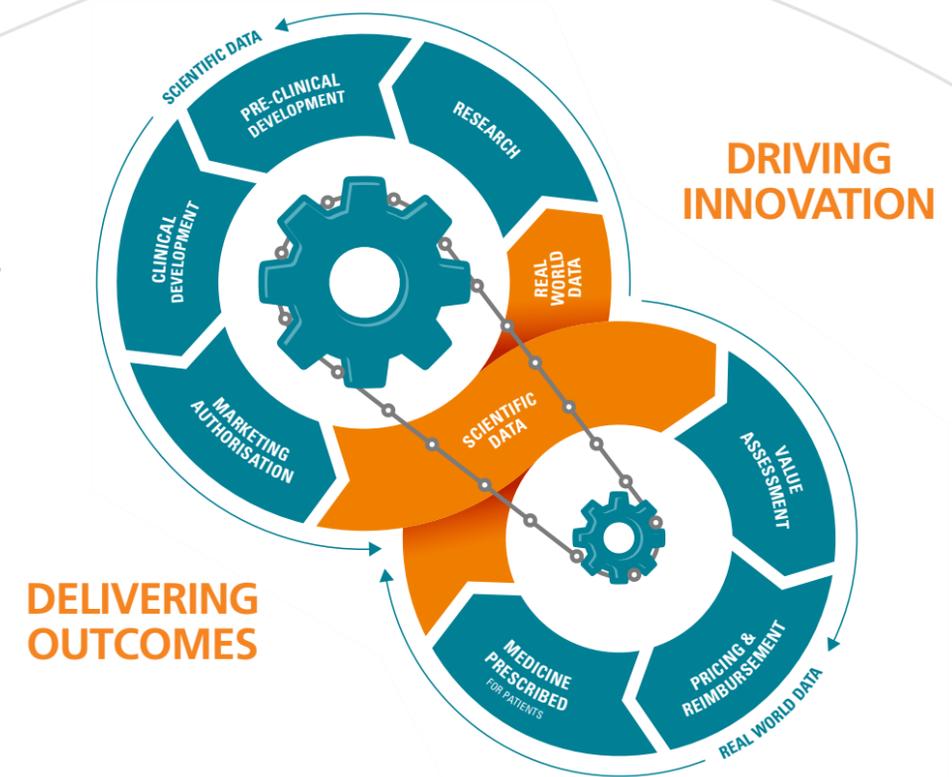
From a pharmaceutical perspective, for several decades, the clinical trial has been the gold standard of measuring safety and efficacy. In the future, clinical trials will continue to play an important role in generating data and evidence to shape healthcare. But with the advent of the digital revolution encompassing electronic health records, disease registries, patient reported outcome measures, mobile health apps and advances in data analytics, our healthcare eco-systems are generating unprecedented amounts of “real world data” that can complement clinical trial data in important ways.

Real world data is all health data that is generated and collected in real clinical practice right across our healthcare systems. Combining pre-clinical and clinical data generated by industry with real world data collected in clinical settings and beyond we can drive medical innovation and improve patient care. With more and better data, clinicians can better target interventions to the patients where they will have the most impact, improving patient outcomes but also increasing efficiency.

Data collection and analysis can help us better understand the side effects of medicines and improve patient safety. Genomic science is facilitating a revolution in personalized medicines and crucially, all of this data can inform and shape tomorrow’s breakthrough innovations.

But maximizing the potential of all this data for patients requires a new level of collaboration to address a number of challenges.

The medicines life-cycle
From innovation to outcomes



In reality, much of our RWD is scattered across the healthcare system without any easy way of bringing this data together. They are noted down in individual patient’s records, recorded in registries kept by small groups of specialists for tracking a specific disease in a selected number of patients, or collected for reimbursement purposes for a specific product and then discarded. Often, data privacy regulations limit the ability to re-use existing data, despite its potential to deliver important insight into clinical practice and patient outcomes.

Data should be generated according to the same standards in order to be comparable. E-health systems should be made compatible between hospitals or countries, with a single, electronic health record for individual patients, with all health data collected in one place. There should be a means of linking all data collected for a variety of purposes. Regulation needs to facilitate data being transferred between systems and used for research or quality improvement.

The situation, though, is changing rapidly. Considerable efforts are now being made both by public and private stakeholders to capture, consolidate and most importantly utilise this real-world data to benefit patients, science and our healthcare systems. Trust is critical in ensuring a new health data ecosystem can thrive. It is vital to be cognoscente of the sensitive nature of healthcare data. Patients need clarity on how and why their data will be used. Systems need high standards of technical data protection, recognising that as we move into a world of many different data sources and opportunities, these standards will need to evolve.

With more information about how different interventions actually compare in terms of health outcomes for patients, healthcare managers and policymakers will be able to take much more informed decisions on implementing clinical practice and resource allocation, creating not only better health outcomes for patients but also getting more value from every Euro spent on healthcare. Progress will require dialogue, collaboration and investment but as the key to an outcomes-based, more sustainable future driving the healthcare data agenda, it is a critical success factor.

Section 8: Moving along the outcomes-based spectrum

No two healthcare systems in Europe are at the same point on the journey to a more outcomes-based approach. Some have advanced examples of good practice, others are beginning to look at outcomes-based reimbursement models, others simply do not have the infrastructure in place to base clinical decision-making and service design on outcome measures. However, at whatever point a healthcare system is on the journey towards an outcomes-based approach there are a number of actions that can be undertaken to support change.

Understand the healthcare challenges

Understanding the epidemiological, structural, technical, financial and political challenges can help inform strategies to move to outcomes-based model of healthcare.



System Readiness Assessment

Using structured analysis of stakeholder awareness, data infrastructure, proof of concepts, and enablers in a local system to obtain insight in to areas to develop and invest.

Define health outcomes measures

Defining standardised sets of health outcomes measures for all diseases and conditions, together with patients, that will allow for systematic measurement and comparisons across providers and countries



Analyse variation

Data analytics provides the key to identifying variances in care and their impact on outcome, to detect sources of waste and inefficiencies in the system



Identify best practice

Standardised outcomes measures, quality and transparent outcomes data, coupled with the use of data analytics will facilitate the identification of best practice for replication across health systems



Promote proof of concepts

Successful pilots of improving outcomes in a specific patient population build trust in the merits of an outcomes-based approach and provide important clues into the practicalities of implementing outcomes-based healthcare



Develop integrated health information systems

Tools such as electronic health records, disease registries and user-friendly data capture systems all contribute to developing an outcomes based system



Build a health data eco-system

To spark, develop and deliver change, data needs to be of high quality and shared across the healthcare system for quality improvement and research



Feedback and learn

As clinical practice and service delivery changes, real world evidence and data analytics provide a mechanism for real-time learning and continuous development



Remove budget siloes and reward quality of care

Establishing flexible and holistic finance systems that promote care integration, and payment models that reward good health outcomes for patients can help facilitate change

Section 9: Paying for outcomes - medicines pricing in an outcomes-focused systems

The promise of medical innovation has never been greater, but there are legitimate concerns about affordability and access for patients. As we look to the future, spending on healthcare interventions should be considered in the context of the outcomes they deliver and their impact on the wider healthcare system and society.

New ways need to be found to reward the value that is created, whether incrementally or through a step change in treatment, while ensuring access and overall affordability.

Tying incentives and payment to outcomes is not just appropriate for some medicines and therapy areas but for healthcare systems as a whole. Medicines account for around one fifth of total healthcare spending in Europe. Applying outcomes-focused thinking to medicines alone will not be sufficient to ensure the long-term sustainability of our healthcare systems. However, as we transition to more outcomes-focused systems, industry has a role to play in working with payers, clinicians and patients to establish more flexible and outcomes focused payment models that support the development of a sustainable, efficient, high-quality health care system that also rewards innovation.

This is not without risk for industry. If a product – or for that matter a health care service - does not deliver on its clinical promise, society should not continue to pay for it if there are other healthcare interventions that deliver more value for money. Equally, if the product delivers more value than was expected, this extra value

must be rewarded. We believe that the long-term benefits outweigh the risks and the initial investment, and that an outcomes-focused approach provides much-needed support to health systems and patients. In the end, it's about paying for what we all ultimately want our health systems to deliver: good health outcomes for patients.

What are outcomes-focused pricing models and how do they work?

There are already concrete examples of such schemes in operation in Europe, for example, Managed Entry Agreements (MEAs). MEAs is a term commonly used to describe an agreement between a medicines manufacturer and a payer to provide medicines at a certain price under certain conditions, and are often used when introducing new medicines where there is some uncertainty about the budget impact or exact clinical value of the medicine. In some of these agreements, pharmaceutical companies and payers have agreed to adopt outcomes-focused reimbursement models in a real world setting.



In the UK bortezomib pioneered a risk-share scheme whereby the **NHS only paid for those patients with a complete or partial response** after 4 cycles



A number of other examples exist where companies **rebate payers up to 100% for sub-optimal or non-responders**, for example in Spain with nilotinib for chronic myeloid leukemia and in Italy with ruxolitinib in myelofibrosis, or where **re-imburement is suspended after a specific period if no clinically meaningful improvement is achieved**, for example with a scheme agreed in Switzerland for pasireotide in Cushing's Disease



Examples also exist where companies have offered **discounts if post-market data demonstrated that a product did not achieve pre-specified performance standards**, for example with pazopanib in the UK or sacubitril/valsartan where discounts are offered to payers if expected hospitalization rates and related cost do not meet expectations.



Companies have even been willing to **take on the risks of failure for a therapy**. For example in a scheme for an osteoporosis drug in Germany, a company has proposed paying for drug, hospitalization and rehabilitation costs of patients failing on therapy and having a bone fracture within 12 months of starting therapy.

Today there is limited scope for outcomes focused agreements because of the technical, structural, financial and political barriers to outcomes-focused systems. However a number of examples exist at a national level in Europe where outcomes-focused approaches are used systematically to agree prices for new medicines and to facilitate improved patient access.



In **France pricing contracts are reviewed every five years** to assess the performance of new medicines in real life settings.



Italy has a comprehensive infrastructure of patient registries for cancer care that allows an almost personalized reimbursement of new oncology drugs on the basis of actual patient response. This system has resulted in prices that are more affordable, accelerated approval times and improved patient access.



Through the Early Access Medicines Scheme, the **UK is actively exploring how to accelerate pre-approval regulatory pathways** and how this can then be integrated into commissioning decisions by the NHS.

Committing to change – Moving from experimentation to operating at scale

The pharmaceutical industry is ready to work with governments, patient groups, healthcare professional associations and all relevant stakeholders across Europe to help accelerate the development of outcomes-focused pricing models at a national level. This comes with a commitment to respond to the challenges of affordability and to move towards outcomes-focused payment models as part of our aim to be a trusted partner in the delivery of improved health outcomes.

Outcomes-focused approaches for medicines pricing should be founded on a number of common principles agreed between stakeholders, including:

- 1 **Affordability and sustainability** of medicines prices
- 2 **Reward for Innovation**
- 3 **Timely and equitable access** for patients that will benefit from new therapies
- 4 **Ability to pay** specifically considering different national incomes levels across Europe, prompting different national pricing
- 5 **Distribution of the economic value** created by continued incentives for innovation recognizing the important role of IP
- 6 **Evidence-based** and accompanied by qualitative and solid real-world data collection and generation
- 7 **Appropriate levels of transparency in the design of outcomes-focused models within each country** to ensure that the interests of all parties are protected
- 8 **Mutually agreed-upon and internationally standardized** outcomes measures (recognizing that outcomes might be challenging to measure in some therapeutic areas)
- 9 **Value based**, there needs to be a recognition that a 'cost plus' payment or 'lowest price procurement based' approach is inappropriate for valuing new technologies and creating an environment that will encourage on-going innovation
- 10 **Aligned** incentives to ensure an integrated system, and that it is not just medicines carrying the burden for change
- 11 **Optionality**, in some cases these models will not be appropriate because simpler pricing schemes may be better

Over time, such an approach will allow the phasing out of redundant pricing mechanisms.

Today, practices such as inter alia external reference pricing (currently used by 25 of 28 member states), parallel trade incentives or therapeutic tenders for patent protected products, act as a disincentive to the implementation of an outcomes-focused approach. In the future, these could cease to be necessary as cost control mechanisms, as outcomes-focused models will deliver superior value.

Roadmap for change

It will take time for the industry and for our health system partners to adapt to a more outcomes-focused approach to medicines pricing. However we believe that it is realistic to target meaningful change over the next decade and propose the following goals to be delivered by joint stakeholder action:

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GOAL 1
Agree on a sustainable framework of value with stakeholders that reflects the opportunity for outcomes-focused payment models
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GOAL 2
Reduce and/or remove disincentives created by external price referencing through gradual implementation of outcomes-focused pricing models on a European wide basis
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GOAL 3
Ensure the development of interoperable outcomes data capture mechanisms that can support outcomes-focused pricing models at scale, based on standardised sets of outcomes measures
- 

GOAL 4
Establish an agreed approach to horizon scanning providing health planners with forward visibility of emerging technologies
- 

GOAL 5
Showcase robust case studies of outcomes-focused pricing models, operating at scale across European countries, showing how prices can change over time depending on the value delivered

The industry can make a valuable contribution to each of these goals by participating and cooperating in the development of solutions and proposing pilot candidates for new schemes.

This vision is ambitious and we owe it to the patients and citizens that we serve, to start immediately and work together to develop workable and sustainable solutions that will ensure that any investments in healthcare and new technologies are focused on delivering improved outcomes for our health systems, our patients and our societies.

Section 10: Being part of the outcomes-based dialogue

The transition to outcomes-based, sustainable models of healthcare is a multi-stakeholder endeavour. It requires dialogue, partnership and collaboration across the healthcare ecosystem. There are many organisations, healthcare systems and individuals engaged in the debate adding different perspectives and expertise to all aspects of the debate. Below we have listed a few for information:

International Consortium for Health Outcomes Measurement (ICHOM)

www.ichom.org

ICHOM is a non-profit organization with the mission to unlock the potential of value-based healthcare by defining global standard sets of outcome measures that really matter to patients for the most relevant medical conditions and by driving the adoption and reporting of these measures worldwide. By the end of 2017 global standard sets of outcome measures for 24 medical conditions covering 54% of the disease burden had been made available

Innovative Medicines initiative (IMI)

www.imi.europa.eu

Established jointly by the European Commission and EFPIA, the Innovative Medicines Initiative (IMI) is the world's largest public-private initiative in life sciences. The IMI brings together the latest research and cutting-edge technology to boost innovation and entrepreneurship in the pharmaceutical sector, to meet the challenges faced by patients and society as a whole. The IMI sets a platform for various stakeholders to share data and pool resources and knowledge at an unprecedented scale in a collaborative effort to improve health outcomes, by addressing some of the most pressing and complex issues impacting healthcare systems.

Big data for better outcomes (BD4BO)

www.http://bd4bo.eu/

Big Data for Better Outcomes (BD4BO), a programme under IMI2, aims to catalyse an evolution towards value-based, outcomes-focused, sustainable healthcare systems in Europe. By exploiting the opportunities offered by the wealth of data, BD4BO can inform improved methodologies which contribute to better health policy and practice. BD4BO's objectives are to maximise the potential of large amounts of data from a variety of sources and provide a platform for the development of a transparent outcomes framework.

Value of Health

www.valueofhealth.eu/

Created by a multi-stakeholder group, under the auspices of FIPRA, "Value of health: improving outcomes" lays out a vision for the role outcomes measures can play in the improvement of performance and associated efficiencies of European health systems. The initiative advances arguments for a central thesis that health outcomes should be included among the priorities for any new or revised sustainable growth policy adopted at European level, for greater transparency and accountability for health system performance. The final report,

published in September 2018, put forward 3 key recommendations for advancing this agenda in Europe:

1. Continue and expand the OECD PaRIS project (Patient Reported Indicator Survey)
2. Integrate more health outcomes data in EU-level health system performance assessments
3. Support the development of health information infrastructure which can support the collection of outcomes data

Core Outcome Measures in Effectiveness Trials (COMET) Initiative

www.comet-initiative.org

Founded in 2010 by the Medical Research Council (MRC) Hub for Trials Methodology, the Core Outcome Measures in Effectiveness Trials (COMET) Initiative brings together a multidisciplinary group of healthcare experts and stakeholders engaged in the development and application of an agreed standardised sets of outcomes, known as the 'core outcome set' during the healthcare research process. By introducing endpoints that are meaningful for patients, the COMET initiative seeks to address difficulties in the research process caused by the heterogeneity between clinical trials.



Section 11: Afterword from the *Healthier future* Advisory Panel

Most commentators would agree that focusing on measuring and improving outcomes for patients is preferable to the continued emphasis on measuring and rewarding inputs that dominates the European Healthcare landscape.

But to make that change, there are a number of cultural, structural, technical, and political barriers to address in order to translate the desire to base our healthcare on outcomes into a reality.

Standardising definitions of outcomes, agreeing measures and putting in place the infrastructure to capture, measure and analyse healthcare data to support outcomes-based decision making is a long and complex process. To facilitate change requires dialogue, partnership and collaboration from across the healthcare spectrum. Often it will require attitudinal change at an individual and institutional level. System-wide transformation of healthcare is a bold and ambitious goal but central to a more sustainable and *Healthier future*.

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