



Patient access to innovative medicines in Europe

A collaborative and value based approach

January 2019

Contents

Foreword	01
Executive summary	02
Infographic	04
Part 1. Payer challenges to pharma innovation	06
Part 2. Innovating in a more challenging environment	11
Part 3. New approaches to access and pricing	16
Part 4. Succeeding in the new value-based environment	23
Part 5. Next generation market access	31
Abbreviations	36
Appendix	37
Endnotes	38
Contacts	41

Deloitte Centre *for* Health Solutions

The Deloitte Centre for Health Solutions is the research arm of Deloitte LLP's Life Sciences and Health Care practices. Our goal is to identify emerging trends, challenges, opportunities and examples of good practice, based on primary and secondary research and rigorous analysis.

The UK Centre's team of researchers seeks to be a trusted source of relevant, timely and reliable insights that encourage collaboration across the health value chain, connecting the public and private sectors, health providers and purchasers, patients and suppliers. Our aim is to bring you unique perspectives to support you in the role you play in driving better health outcomes, sustaining a strong health economy and enhancing the reputation of our industry.

In this publication, references to Deloitte are references to Deloitte LLP, the UK affiliate of Deloitte NWE LLP, a member firm of Deloitte Touche Tohmatsu Limited.

Foreword

The European environment for pharmaceutical (pharma) market access and pricing is changing rapidly. European payers are struggling to meet the health demands of their populations. Consequently, health care providers are looking for ways to optimise their expenditure on health care services, and pharma products represent a clearly visible cost payers can attempt to control.

Over the past decade European payers have strengthened their rhetoric on pharma pricing and have implemented policies and pricing arrangements at a regional, country-specific and European level. The ways in which pharma prices are being controlled are many, but overall there is an increasing requirement for pharma companies to demonstrate the budget impact and clinical effectiveness of its products through health technology assessments, budget impact tests and value-based care arrangements.

At the same time, pharma companies are continuing to develop more personalised innovative therapies that can significantly improve patient outcomes. However, this has significant implications for the one-size-fits-all approach to medicating patients. These more innovative therapies have led to increased R&D costs, largely due to the growing complexity of clinical trials and R&D returns are reducing as the potential number of patients a product can treat successfully is smaller. In addition, a shortening of regulatory approval times, through accelerated access schemes for the latest breakthrough innovations, requires pharma companies to generate optimal and robust evidence that satisfies the needs of both regulators and health technology assessment authorities.

For market access initiatives to succeed in this more challenging environment, the pharma industry will need to be more constructive in their dialogue, communicative in their intentions for a product, collaborative with stakeholders, and innovative in the business models they deploy. Pharma can also be a key driver of the move to value-based care by offering new forms of contracting that share cost and risk based on patient outcomes and the quality of services the pharma industry provides. This requires pharma to engage in earlier dialogue with regulators and payers to develop a shared understanding of the clinical and economic benefits of new products, and improve evidence generation during and after clinical trials, through the use of real-world evidence.

Ultimately our report highlights the need for pharma to become a collaborative partner in developing new therapies, build trust and acquire new skills and capabilities to be more agile, technology driven, and tailored in their approach to market access. At the same time, payers need to explore how these new collaborative approaches with the industry can help them use funds more effectively and facilitate better patient outcomes.

Hanno Ronte
Partner
Monitor Deloitte

Elizabeth Hampson
Director
Monitor Deloitte

Karen Taylor
Director
UK Centre for Health Solutions

Executive summary

European payers are responding to significant financial and societal challenges

Pharmaceutical (pharma) companies wishing to sell their medicinal products in Europe are required to obtain authorisation from the EU's European Medicines Agency (EMA). However, that is only the first step. Europe represents a unique challenge for pharma market access and pricing activities. Unlike single regulator countries such as the US and Japan, each European country has its own market access and health care system, with different backgrounds, population sizes and epidemiological factors, as well as different payer systems. These differences affect decisions on whether a product will be approved and at what price it will be reimbursed.

Since the 2008 financial crisis, Europe has endured a decade of financial austerity and cost-containment measures. On average across 16 European countries, the GDP spent on health care has increased slightly from 9.52 per cent in 2010 to 9.74 per cent in 2016, while the percentage of GDP spent on pharmaceuticals has decreased from 1.50 per cent in 2010 to 1.36 per cent in 2016. At the same time, the burden of an ageing population, increases in chronic diseases and increasing pressure from patients and pharma to fund products for rare diseases has increased significantly, putting pressure on European health care systems.

Efforts used to control drug pricing

As a high percentage of health care costs are fixed and difficult to tackle in the short term, governments have adopted a number of aggressive pricing strategies to exert downward pressure on consumables, including the price they are willing to pay for new and existing drugs. All governments have introduced policies aimed at changing prescribing behaviour, including generic substitution and prescribing by International Non-proprietary Names (INN). They are also increasing the use of health technology assessments (HTAs). The table below highlights a number of other efforts being used to control pharma spending.

patient co-payments	price cuts
rebates and clawbacks	managed entry agreements
budget caps for individual and groups of products	reimbursement controls
tendering and selective contracting	reference pricing
limiting medications to specific sub-populations	changes to distribution margins

Increasingly, these policies are being used to emphasise the value of pharma products (for example, value-based care (VBC)) by requiring pharma to demonstrate outcomes-effectiveness and cost-based improvements.

Pharma innovation is facing a more challenging market access environment

Fuelled by small-molecule blockbuster drugs, pharma has traditionally approached the European market with a volume-based business model which aims to reach as many patients in a target population as possible. However, scientific advances have enabled a more personalised approach to prescribing. These advances, particularly in genomics and other 'omics', are allowing patient populations to be stratified, through the use of bio-marker diagnostic tests, to those who will or will not respond to a treatment. Chimeric antigen receptor T (CAR-T) therapies are the latest example of such highly personalised treatments.

Additionally, pharma products have become more complex. Biologic products (large-molecule formulations) are able to treat difficult diseases such as cancer and autoimmune disorders better than traditional therapies. These highly innovative biologics (such as immunotherapies) are often priced at a premium.

To increase patient access to pharma innovation, the European Medicines Agency (EMA) has implemented an accelerated approvals process for breakthrough medicinal products. This programme allows pharma to enter the European market conditionally (for example, using continuous post-approval safety assessments), after demonstrating adequate levels of safety in the clinical trials process (typically around phase 2). However, the shorter length of trials have created challenges for payers and HTA authorities who prefer longer-term data to reduce uncertainty around a pharma product's efficacy and cost impact. Innovative pharma products are also facing increasing delays to reach patients; the average length of time from market authorisation to reimbursement in Europe increased from 233 days (2007 to 2009), to 318 days (2014 to 2016).

With pricing at the forefront of recent political and societal debate, the payer challenges and other factors discussed above make the market access and pricing environment in Europe increasingly challenging for pharma.

Value-based approaches to market access and pricing

Approaches to market access and pricing therefore need to change. There are a variety of value-based contracting models (financial, outcome and service based), of particular interest to some payers. These agreements, which can be based on cost, evidence and risks, require a more collaborative relationship between pharma, payers, providers, physicians and patients, in which all members work together to improve patient outcomes and the performance of health care. In some of these approaches pharma provide health care providers with services that decrease the administrative burden, improve operational and financial performance, and improve patient outcomes. However, payers often prefer discount-based agreements.

Succeeding in the new market access and pricing environment

In order to succeed in the European environment, pharma need to communicate their intentions for a product more effectively, be more collaborative with stakeholders, and innovative in the models they deploy for market access and pricing. More specifically:

- improve understanding of the scientific and economic considerations of both regulators and payers
- utilise early access schemes to gather evidence on a product's safety and efficacy, and partner on services and technologies in order to develop solutions that clearly demonstrate value 'beyond the pill'
- develop regulatory and technical standards and skills to ensure data privacy and interoperability, including registries that allow tracking of outcomes and multi-indication pricing.

Next generation market access requires pharma to evolve

Continued success depends on the pharma industry's ability to adapt and tailor their approaches to pricing and market access. Pharma companies need to be more agile and enhance their core organisational capabilities to support market access and pricing in a proactive way throughout the life cycle of their products. Moreover, pharma need to understand the new skills and talent organisations will need to succeed in market access endeavours in the future. These core organisational capabilities are:

- **Earlier launch planning focussed on dialogue:** to understand the needs of and collaborate with payers to understand their disease and cost burden; earlier in the R&D process, including early dialogue with payers and collaboration with patients to identify unmet needs and real life experiences.
- **Innovative contracting:** to design contracting and service solutions that meet the genuine needs of the system, payers and patients, deploying multi-disciplinary teams with first-class collaboration and interpersonal skills backed by strong evidence of health system understanding to support the future health system sustainability.
- **Real-world value dossier creation:** using RWE to develop an in-depth understanding of system challenges, physician and patient experiences and the benefits of products and services including the ability to demonstrate value in a more holistic way.
- **Build trust and understanding:** demonstrate that you are a collaborative partner in your therapy area by providing patients and providers with the tools to understand and comply with treatments, and assurance on data use, security and privacy. Develop stronger relationships with payers based on increased transparency.
- **Build the skills and expertise needed for the future:** adopt new ways of working and deploy multidisciplinary teams that engage early in the R&D process and have deep technical skills such as data analytics, health economics and actuarial modelling skills; combined with creative problem solving, advocacy and communication / engagement expertise.

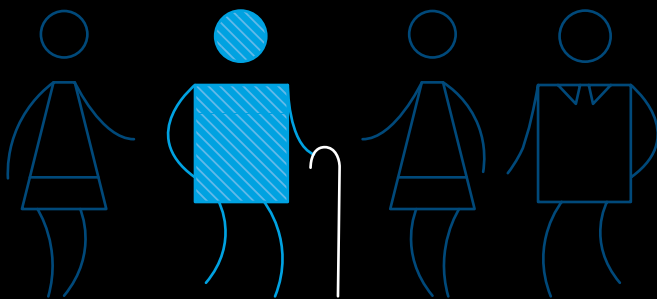
Conclusion

In an environment where payers may soon know as much about real-world medicine effectiveness as pharma, the burden of proof for outcome delivery is shifting to pharma companies, and the bar is rising. However, to solve the challenges facing the health care system, a collaborative approach is required where all players adapt their skills and move away from historic methods of engagement. Pharma should build a tailored approach to contracting and market access discussions, using new technical and communicative skills. At the same time, payers need to leverage pharma's significant skills and commercial capabilities to solve their challenges. It is to both sides' advantage to engage in more dialogue, communicate, share evidence, and take a value-based approach to the use of medicines across Europe. Without taking these steps, patient access to innovations may be undermined, and the health care system risks becoming unsustainable.

The pharma market access and pricing environment in Europe is rapidly changing

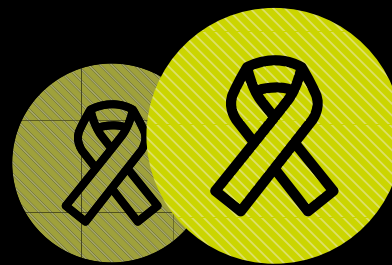
Payers are responding to:

Aging populations



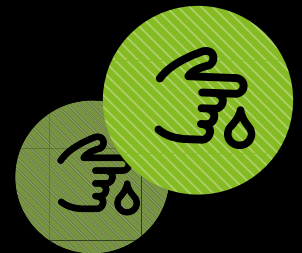
By **2030** **25%** of the European Union's (EU) population will be aged **65 and over**, up from **19%** in 2015.

Chronic diseases

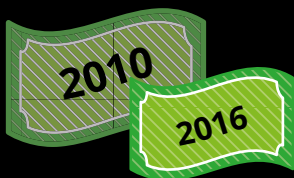


From **2018** and **2040** the incidence of **cancer** is predicted to increase by **23%**.

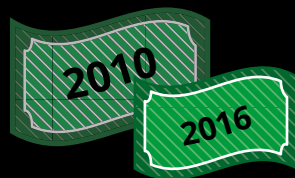
From **2017** and **2045** the number of people with **diabetes** (aged 20-79) is projected to increase by **16%**.



Constrained health care budgets are impacting pharma spending*



GDP spent on the **health care** has increased from **9.52%** in 2010 to **9.74%** in 2016



GDP spent on the **pharmaceuticals** has decreased from **1.50%** in 2010 to **1.36%** in 2016

Increasing pressure to fund drugs for rare diseases



From 2007 to 2017:

- the **EMA** has given **1544** orphan drug designations
- the **FDA** has given **2707** orphan drug designations.

Worldwide, it is estimated that **orphan drug sales** will total **\$216 billion** by **2022**, up from **\$125 billion** in 2017.

Governments in Europe have tightened policy towards reimbursement and pricing

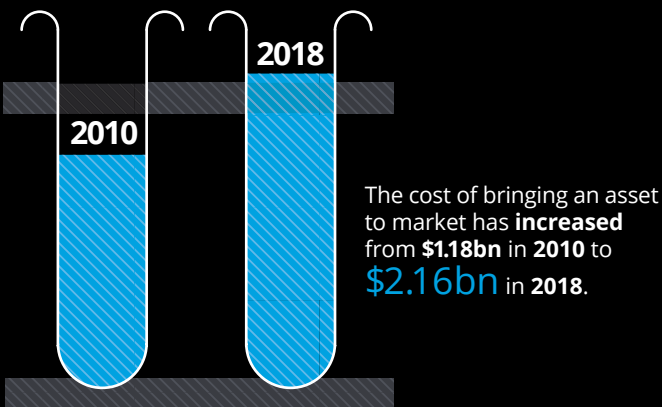
2:1 ratio of **unfavorable** to **favorable** policies.



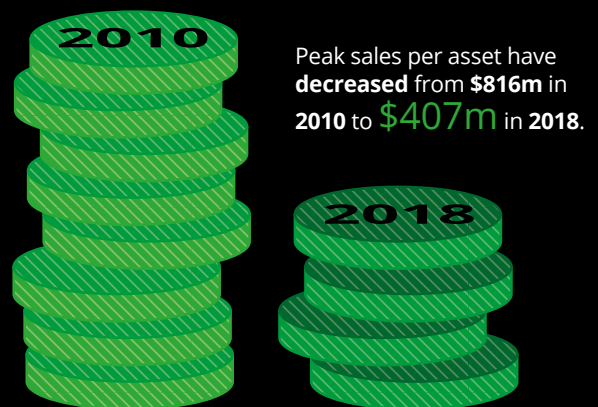
Note:* 16 European countries were included in this analysis; Austria, Belgium, Finland, France, Germany, Greece, Ireland, Italy, Netherlands, Norway, Poland, Portugal, Spain, Sweden, Switzerland and The UK.

Pharma are responding to:

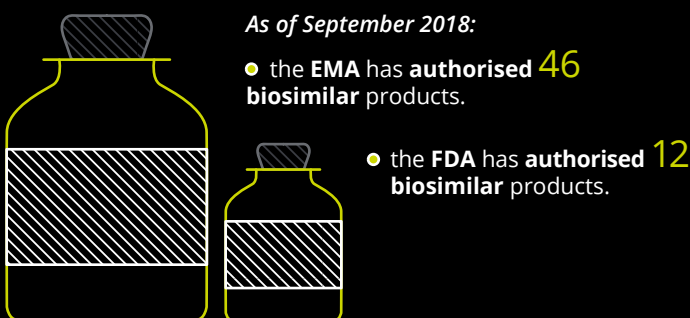
Increasing R&D costs**



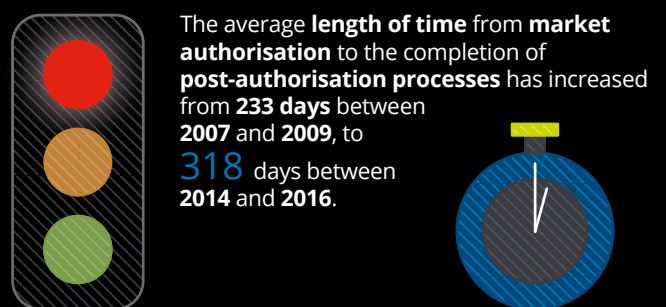
Falling peak sales per asset**



Increasing number of biosimilars entering the European market



Delays in patient access following market authorisation



Pharma should enhance their core capabilities:

- Earlier launch planning focused on dialogue:** Understand payer needs earlier in the R&D process through earlier dialogue with payers, providers, physicians and patients
- Innovative contracting:** Design contracting and service solutions that meet the genuine needs of the system, payer and patient, and support its sustainability

- Real-world value dossier creation:** Use RWE to develop a true understanding of system challenges, physician and patient experiences and the benefits of your products and services
- Build trust and understanding:** Be a collaborative partner in your therapy areas and build trust
- Build the skills and expertise needed for the future:** Consider the skills gap you have between technical and communicative expertise

Note:** Information taken from Deloitte's annual report, *Measuring the return from pharmaceutical innovation 2018*. Figures presented are for the original cohort of 12 large market capitalisation biopharma companies.

Part 1. Payer challenges to pharma innovation

Europe is the second largest biopharmaceutical (pharma) market in the world, with the 28 European Union (EU) countries accounting for \$211 billion (18 per cent) of the total \$1,133 billion market in 2018.¹

While countries such as the US and Japan are essentially single markets, Europe provides a unique and distinct challenge for the pharma industry. Within the EU, once the European Medicines Agency (EMA) has clinically approved a pharma product, each pharma company has to navigate a range of diverse European health care systems (largely split between Social Health Insurance and National Health Service frameworks); and local regulatory, market access, reimbursement and cost containment mechanisms before it can launch its products successfully across the continent. These approaches to reimbursement vary from country to country and, in some instances, even within countries. This affects the way pharma products are reimbursed and priced and can lead to pricing variations and disruption to market access for the same product across Europe.

The financial constraints on pharma innovation

The market context is changing fast as payers and providers seek to respond to increasing demands from ageing populations living longer with chronic non-communicable diseases (NCDs) such as cancer and diabetes. The burden of NCDs in Europe is expected to grow as the population continues to age. In Europe, the incidence of cancer is expected to increase by 23 per cent between 2018 and 2040.² Similarly, between 2017 and 2045 the number of people (aged 20-79) with diabetes is expected to increase by 16 per cent.³ This increasing demand is occurring at the same time as countries are attempting to control health care spending. It also follows a decade of prolonged economic austerity and cost containment policies implemented by countries across the EU. According to the OECD, in 16 European countries, the percentage of GDP spent on health care has slightly increased from 9.52 per cent in 2010 to 9.74 per cent in 2016 (see Appendix).⁴

For health care providers and payers, expenditure on pharma products represents a visible category of cost that they can measure and control. Governments have introduced a number of policies to manage the cost of pharma products more effectively. As a result, and despite a rising cost per patient for new treatments, the percentage of GDP spent on pharmaceuticals has decreased, as an average for 16 European countries, from 1.50 per cent in 2010 to 1.36 per cent in 2016 (see Appendix).⁵

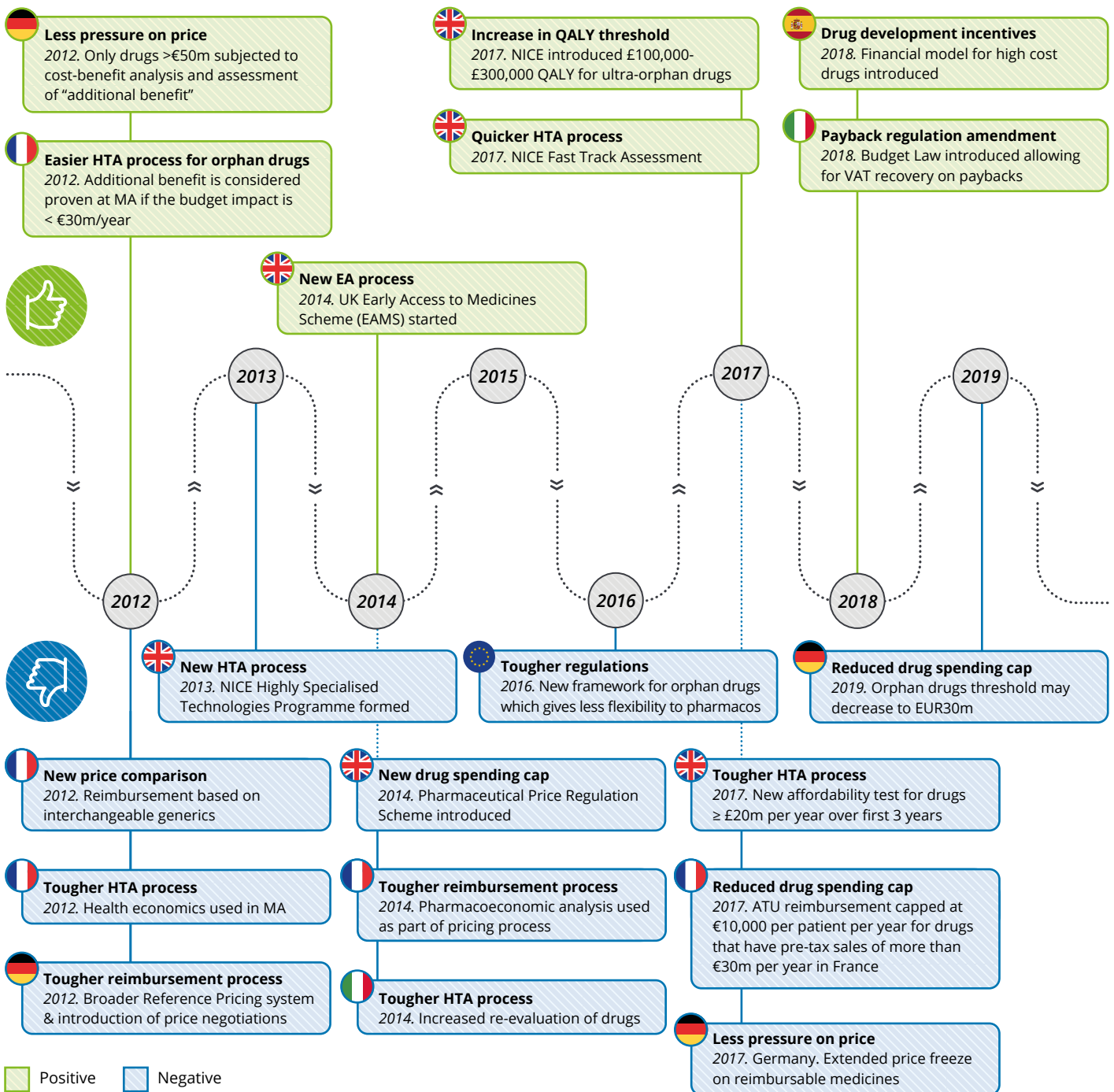
In contrast, the average launch price of innovative pharma products is rising due to increasing molecular complexity, changing modality of treatments (such as, combination therapy) and decreasing size of the targeted population (for example, precision therapies). Moreover, the launch prices of cancer drugs have more than doubled over the last 20 years. The escalating cost of pharma innovation, coupled with EU payers becoming more budget conscious, is increasing pressure on drug manufacturers to demonstrate evidenced-based cost-effectiveness and value through value-based care (VBC) initiatives that shift a higher level of responsibility and risk to manufacturers.

However, pharmaceutical cost containment policies across Europe are creating points of tension between manufacturers of innovative products, payers and providers. This risks stifling innovation and could reduce the access patients have to new therapies.

Reimbursement and pricing

Over the past seven years, there have been numerous policy developments across Europe aimed at influencing pharma pricing while improving access to new medicines. However, the balance of these changes is more heavily weighted towards tightening the control and toughening the rhetoric of pricing and reimbursement at a country-specific and European level (See Figure 1). Our analysis of the wider European market identified a ratio of 2:1 in terms of unfavourable to favourable policies, as the evidence requirements for reimbursement increase and the tolerance for imperfect clinical data declines.

Figure 1. Ongoing changes to the pharma market access and pricing environment across the EU5



Note: Policies covered in the figure are not representative of all changes occurring across Europe
Source: Deloitte LLP, 2019

Cumulatively, these shifts place pressure on how pharma products are priced and how pharma budgets are managed within each country. Indeed, a wide range of policies have been implemented across Europe, these include:

- changes to prescribing behaviour including generic substitution and prescribing by International Non-proprietary Names (INN)
- price cuts
- managed entry agreements
- reimbursement controls
- reference price changes including increases in frequency and changes in the basket of countries
- changes to distribution margins
- rebates and clawbacks
- tendering and selective contracting
- budget caps for individual and groups of products
- patient co-payments
- limiting medications to specific sub-populations.

There has also been an increasing use of health technology assessments (HTA) to appraise products' clinical and economic value in order to limit reimbursement levels. Examples include:

- In 2014, the UK government introduced its latest Pharmaceutical Price Regulation Scheme (PPRS), which allows the Association of the British Pharmaceutical Industry (ABPI) to negotiate the prices of branded drugs for a fixed 5 year period on behalf of the Department of Health (DH). Pharma organisations voluntarily sign up to the PPRS. Pricing control practices employed by the PPRS include budget caps for individual and groups of products. Between 2014 and 2017, the scheme has limited the growth in spending by NHS England on medication to an average of 0.9 per cent annually (0 per cent in 2014 and 2015, and 1.8 per cent in 2016 and 2017).⁷

- In 2014, the Dental and Pharmaceutical Benefits Agency (TLV) in Sweden implemented the 15-year rule, which cuts the price of drugs by up to 7.5 per cent for those with little generic competition and on the market for 15 years or more. Moreover, the rule is based on the date when a drug (i.e. active ingredient and form) was first introduced to the market, meaning that it also applies to drugs that have been on the market for fewer than 15 years.⁸
- In 2017, the National Institute for Health and Care Excellence (NICE) in the UK introduced the £20 million budget impact test for medicines which are predicted to cost more than £20 million in any of their first three years of use. Reaching the threshold results in discussion between NHS England and the manufacturer to limit the impact of the medication on NHS England's budget.⁹
- In 2017, through its yearly Social Security Finance Act (LFSS), France introduced a reimbursement cap of €10,000 per patient, per year for drugs with pre-tax sales of €30 million per year.¹⁰

Countries are also increasing their individual capacity and capability to negotiate with pharma on pricing (See Case study 1).



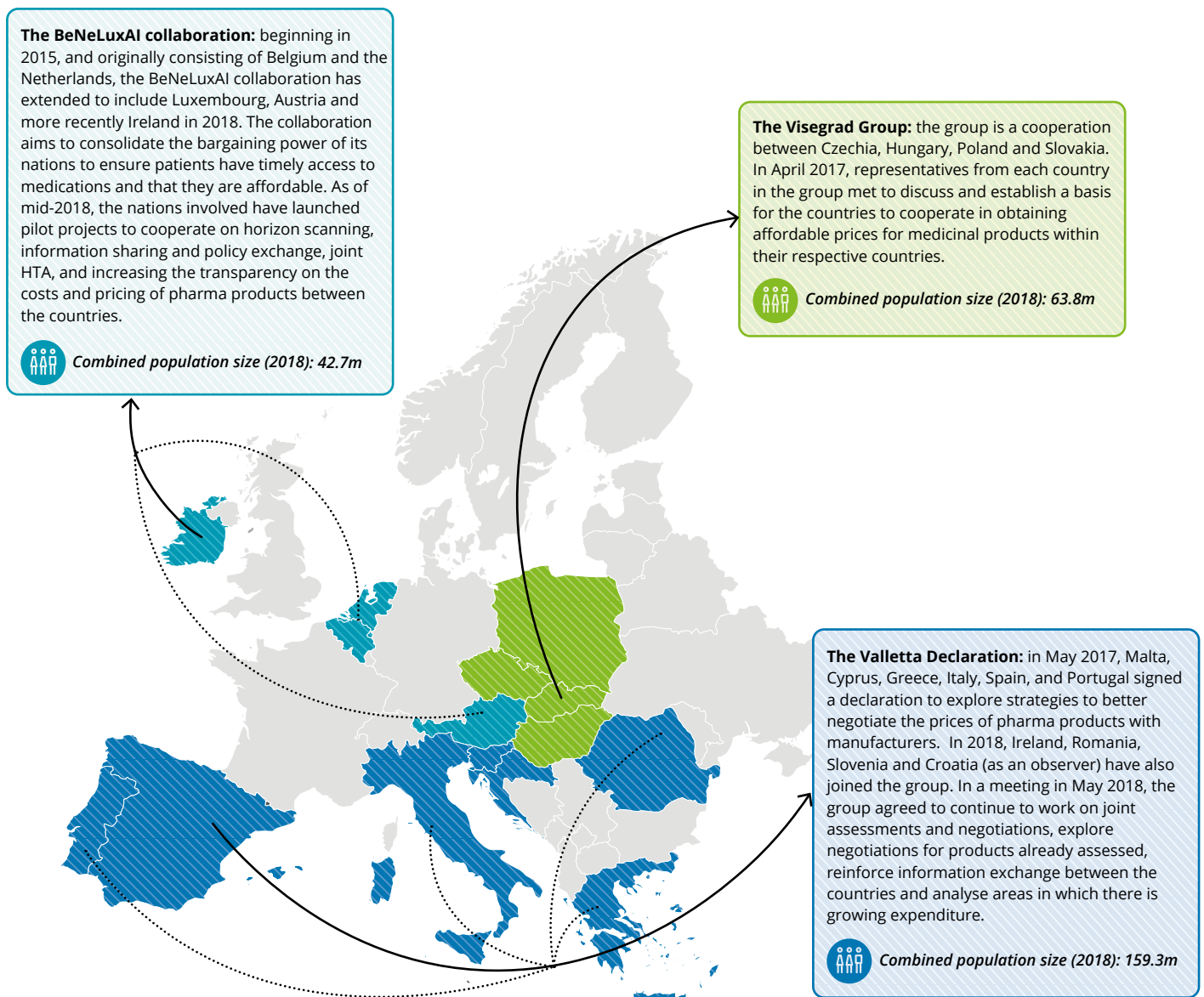
Case study 1: The Commercial Medicines Unit (CMU)

providing procurement and tendering expertise to NHS England

The UK's Commercial Medicines Unit (CMU), formally part of the Medicine, Pharmacy and Industry Group of the UK Government's Department of Health, moved into NHS England's Specialised Commissioning team in 2017.¹¹ The CMU works in partnership with NHS England and helps the health service with the contracting of medicines used within England, particularly for secondary care. Moreover, the CMU provides analysis of expenditure and procurement support to providers for a range of medicinal products, including branded and generic medicines. The CMU has a range of tools that it utilises to provide transparency on the tendering and procurement process. These tools include an online pharmacy catalogue and an eTendering system to track activity and contract negotiations.¹²

Additionally, smaller European countries have banded together – or are beginning to – in order to increase their bargaining power and share scientific knowledge and methods for economic appraisals (See Figure 2).^{13, 14, 15, 16}

Figure 2. Cross-country collaborations formed to negotiate on pharma pricing

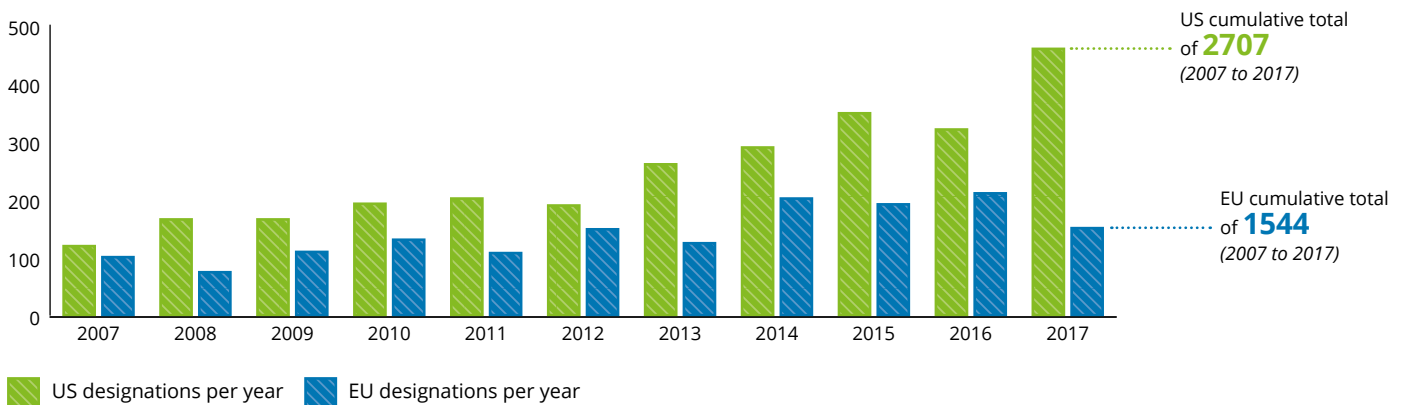


Source: BeNeLuxA, 2018; Euactive, 2018; Infarmed, 2018; Visegrad, 2017; Worldometers, 2018

For pharma companies looking to access the European market, the pricing environment is becoming significantly more challenging, especially in terms of achieving a balance between market access and obtaining reasonable prices for their products. This is particularly true for highly innovative products such as personalised medicines and combination therapies. Moreover, the banding of countries also raises a number of questions on how negotiations will be conducted in the future and whether they will take into account different pricing reforms and reimbursement strategies wanted by individual countries.

The increasing number of orphan drugs on the market, coupled with an expected rise in drugs granted orphan designation, are resulting in payers spending an ever larger proportion of the drug budget on 'rare' diseases that, in aggregate, can no longer be considered rare. Orphan drugs represent a high cost area for health care and a lucrative opportunity for pharma. For example, in 2017 the average cost per patient (based on list prices) of an orphan drug in the US was \$147,308 compared to \$30,708 for a non-orphan drug.¹⁸ However, many health care providers do not pay these prices due to negotiated discounts.

Figure 3. The number of orphan drug designations in the US and EU, 2007 to 2017



Note: The EMA defines an orphan drug as a medicine for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition that is rare (affecting not more than five in 10,000 people in the European Union) or where the medicine is unlikely to generate sufficient profit to justify research and development costs. The FDA defines an orphan drug as a drug intended to treat a condition affecting fewer than 200,000 persons in the United States, or which will not be profitable within 7 years following approval by the FDA.

Source: EvaluatePharma, 2018

A rise in the number of products for rare diseases raises a dilemma for payers

Rare diseases are market segments with high unmet need and a lack of treatment options. Historically, pharma has sought to develop drugs that can target as wide a patient population as possible. However, rapid advances in genotyping and DNA sequencing have enabled the development of more advanced and targeted treatments, increasing the focus on developing orphan drugs to treat rare disease. Cumulatively, 1,544 orphan drug designations have been approved in the EU over the last 10 years (See Figure 3).¹⁷

Worldwide, it is estimated that orphan drug sales will continue to grow, with the market totalling \$216 billion by 2022, up from \$125 billion in 2017.¹⁹ Additionally, orphan drugs are estimated to account for 55 per cent of the cumulative value of the European pharma pipeline through to 2022.²⁰ The current and forecast costs attributed to orphan drugs are creating further pressure on health care systems in Europe. As a result, payers in Europe are requiring pharma manufacturers to provide a stronger evidence base over a longer time period to justify the prices of rare disease products.

Part 2. Innovating in a more challenging environment

Pharma medical innovations over the last 40 years have been crucial to improving life expectancy and health outcomes for patients across the world. Traditionally, pharma has used a volume-based business model, whereby it produced small-molecule products that could treat as many patients as possible. This model produced numerous blockbuster drugs (a drug whose revenues exceed \$1 billion annually), and in the 1990s and early 2000s was highly successful at producing treatments for large patient populations.

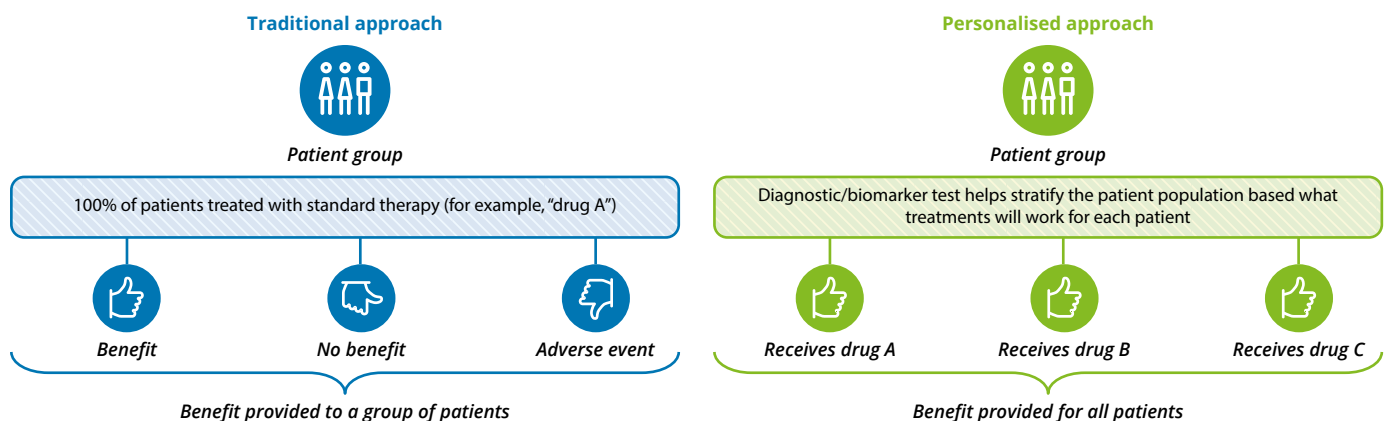
The 2000s saw pharma's R&D focus shift towards large-molecule formulations (biologics) due to their enhanced selectivity in treating disease and improved impact on patient outcomes. However, biologics, which are derived from living cells, are inherently more complex and expensive for pharma to discover, develop and manufacture than their small-molecule counterparts.²¹ Advances in genetics allow patient populations to be further stratified to those who will or will not respond to a treatment. Consequently, R&D costs for pharma are increasing at the same time as the potential target population for new products is reducing, meaning companies have to recoup R&D investment from smaller patient populations. Treatment regimes are also changing from the use of single drugs to combination therapies, and the use of companion diagnostic tests to identify responsive patients early on in the treatment process.

Indeed, findings from Deloitte's annual report, Measuring the return from pharmaceutical innovation 2018, indicate that the cost of bringing an asset to market has increased from \$1.18 billion in 2010 to \$2.16 billion in 2018 for 12 large-cap pharma companies. Over the same time, peak sales per asset have decreased from \$816 million to \$407 million.²² The average length of time from the discovery of a new medicine to its approval has remained at 10-12 years, largely due to the complex regulatory requirements needed for approval.²³

Reimbursement thresholds for personalised medicines

Personalised medicine is a treatment and prevention approach that takes into account a person's genetics, lifestyle and diagnosis. It allows doctors and researchers to predict more accurately, through the use of bio-marker diagnostic tests, which treatment strategies for a particular disease will work and for which groups of people. It contrasts with the traditional 'one-size-fits-all' approach in which disease treatment and prevention strategies are developed for the average person, with less consideration for differences between individuals (See Figure 4).

Figure 4. Traditional vs personalised approaches to the treatment of patients



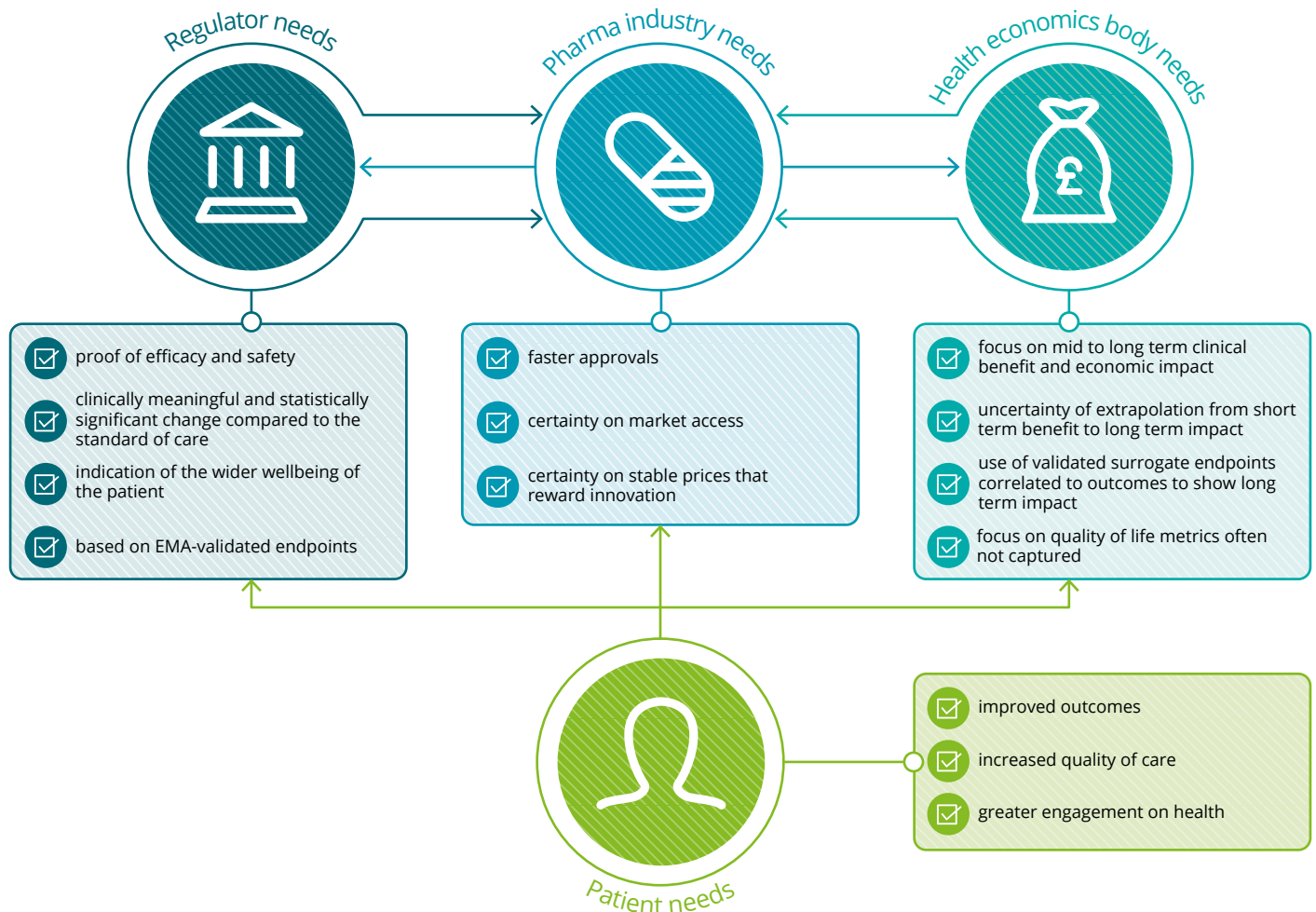
Note: This is an illustrative example not based on any particular personalised medicine product available on the market
Source: Deloitte LLP, 2019

Given the treatment's complexity and smaller treatment population, the cost of research, manufacturing and downstream market prices of precision medicines tend to be higher.²⁴ Moreover, the clinical and budget impact requirements of payers and HTA authorities are resulting in some manufacturers struggling to find an equitable balance between budget constraints and uptake of innovation, despite therapies showing improved patient outcomes. For example, high cost precision medicines may sometimes fail to meet the cost-effectiveness criteria to qualify for reimbursement. This issue is exacerbated in Europe by a lack of consensus on appraisal methods, reimbursement thresholds and what price to outcomes ratio is deemed cost-effective. This is an issue European countries are working together to resolve, as discussed in this report.

More certainty on efficacy over the longer term

The regulatory, reimbursement and health economics frameworks in Europe have differing evidence requirements when appraising new pharma products. These differences can result in a product receiving regulatory approval but not reimbursement approval within a particular market. Around the world, including Europe, regulators have sought to speed up their assessment processes through shorter clinical trials to enable patients with unmet medical needs access to the the latest pharma innovations. However, the increase in accelerated assessments has created a situation where the amount of evidence captured may not be substantial enough to satisfy the needs of HTA appraisers (See Figure 5).

Figure 5. The needs of patients, the pharm industry, regulators and health economics bodies



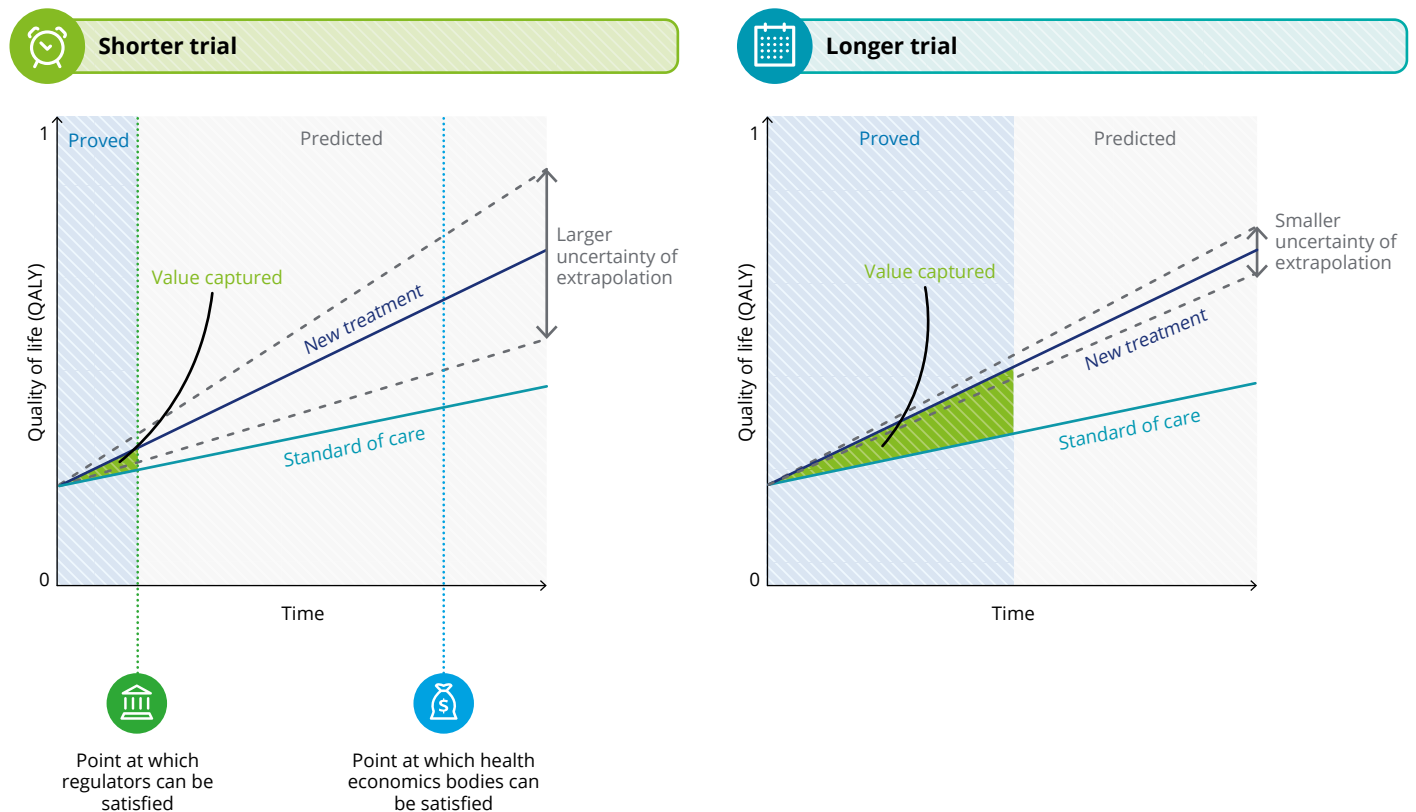
Source: Deloitte LLP, 2019

The use of shorter trials can result in smaller differences captured between new and existing products, which is at odds with the longer-term data required by payers and HTA authorities to demonstrate stronger differences between products and reduce uncertainty about a product's efficacy. Therefore, innovative products going through accelerated approvals, without additional data collection strategies, may fail to meet the impact thresholds needed to obtain reimbursement. Figure 6 highlights the differences between shorter and longer-term trials in capturing and supporting the value of a pharma product. With the increasing use of accelerated approvals, a balance is needed to satisfy both regulatory and economic appraisal requirements, requiring more constructive dialogue between regulators and HTA authorities.

The use of adaptive trials, innovative contracting models and data acquisition based on real-world evidence (RWE) can have a number of positive impacts that serve all stakeholders, which we believe include:

- a stronger correlation between surrogate endpoints and long-term impact
- reduced time to gain market access
- reduction in the health economics body's level of uncertainty over the efficacy and safety of a product
- increased confidence in the product by health care providers and patients
- increased likelihood of reimbursement.

Figure 6. Longer trials can allow pharma to demonstrate the true value of their products



Source: Deloitte LLP, 2019

More price competition for the most innovative products

Biologics unlike their small-molecule counterparts, are able to interact in specific ways with biological systems, thereby providing more effective treatments for patients.^{25,26} In comparison to traditional therapies, biologics are able to treat difficult diseases such as cancer and autoimmune disorders more effectively, and in some instances are able to offer cures, such as recently approved CAR-T immunotherapies to treat childhood leukaemia.²⁷ Biologics represent an increasing proportion of pharma's R&D pipeline, totalling 38 per cent of the pipeline in 2017, up from 25 per cent in 2010.²⁸ Biologics are more expensive than small molecule drugs and tend to maintain a price premium for longer after loss of exclusivity due to less competition. Indeed, a small number of branded large molecule formulations constitute a large proportion of prescription drug revenue, making biologics an increasingly important category for pharma. In 2016, a leading biologic product produced 63 per cent of its parent company's revenues.²⁹

Similar to the generics boom in the 2000s, biologic therapies face their own competitive and pricing pressure through biosimilar medications.³⁰ However, biosimilars are not identical copies to the originator biologic molecule and, unlike generics, are more complicated to manufacture, require more rigorous testing and research than simply copying the original drug, and tend to be authorised and approved at a slower rate. They are therefore much more costly to bring to market than generics; this means there have been fewer competitors and historically have obtained smaller discounts compared to the originator, leading to fewer incentives for health care providers to switch patients.³¹

Nevertheless, following the publication of the EMA's Guidelines on similar biological medicinal products in 2004, there has been an increasing emphasis on the use of biosimilar products due to the savings they can provide for the continent's health care systems. As of September 2018, the EMA has authorised 48 biosimilars for use in Europe.^{32,33} By comparison, the US FDA implemented a regulatory framework much later, in 2010, with final guidance issued in 2015. As of September 2018, the FDA has approved 12 biosimilar products.³⁴ One such biosimilar entry was reported to have saved €85 million annually across 17 EU countries since 2011.³⁵

However, health care payers in Europe have struggled to convince physicians and providers to switch to prescribing biosimilars instead of biologic medications. More recently, individual European countries have started to support the use of biosimilar medications in order to push both cost savings and changes in physician prescribing behaviours. Examples include:

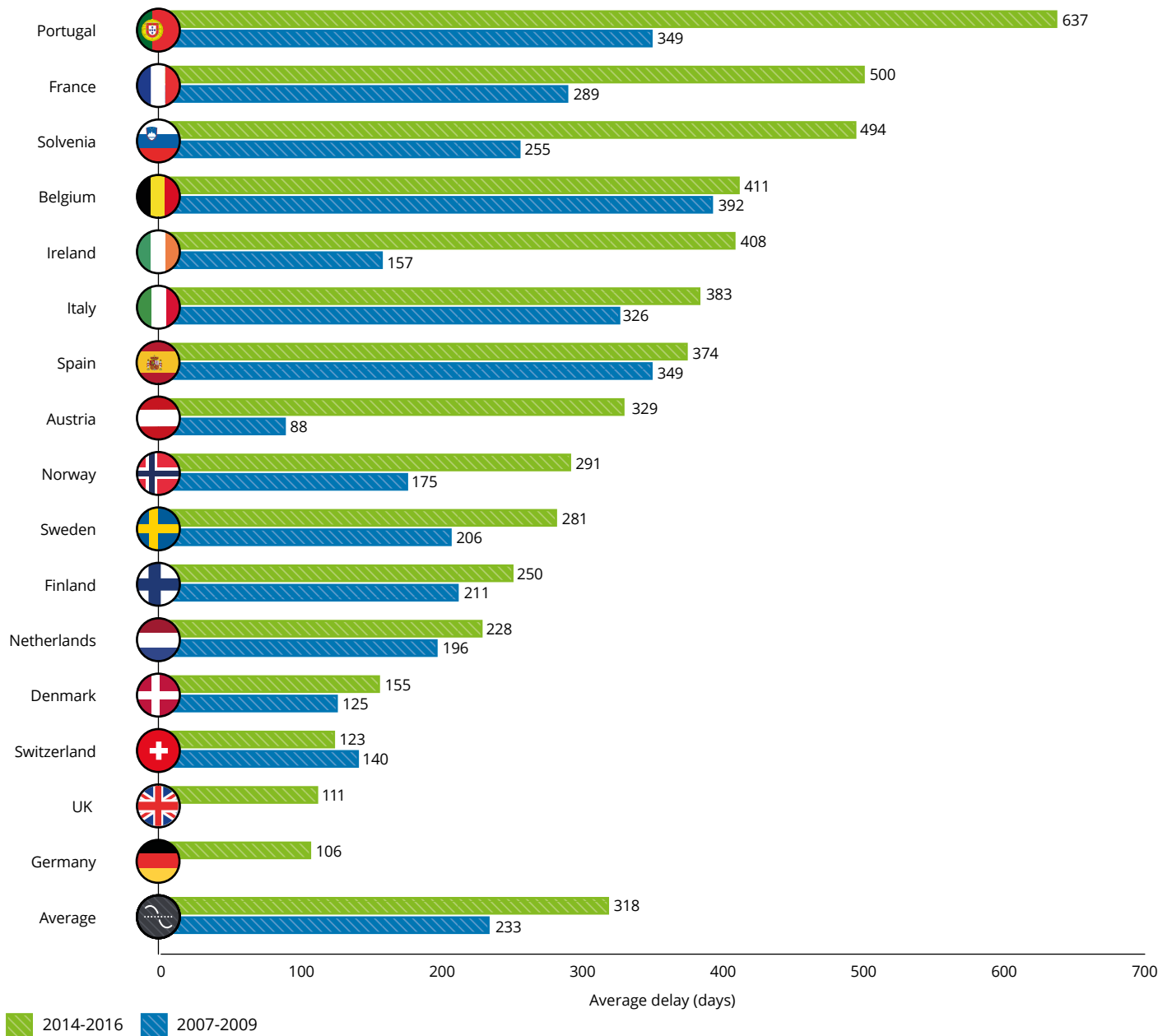
- In March 2017, the Austrian parliament adopted a new pricing regime for generics and biosimilars. On market entry of a generic or biosimilar, the manufacturer of the original product will be requested to reduce the price by up to 30 per cent.³⁶
- In September 2017, NHS England issued new guidance which aims to have 90 per cent of new patients prescribed the best value biological medicine within 3 months of the launch of a biosimilar medicine, and at least 80 per cent of existing patients within 12 months. By 2020/21, the UK has targeted potential savings of at least £200-300m per year.³⁷
- In February 2018, as part of the France's 2018-2022 National Health Strategy, the country aims to reach 80 per cent biosimilar penetration by 2022 – which represents an increase from the previous year's 70 per cent target.³⁸

As a result, it is becoming more difficult for pharma in Europe to sustain stable prices for their biologic originator products after patent expiry and recover the higher R&D costs required to produce them.

Innovation struggles to reach patients

In Europe, pharma products are also struggling to reach patients quickly, with the gap between market authorisation and patient access widening. Research conducted by the European Federation of Pharmaceutical Industries and Associations (EFPIA) analysed the length of time it takes pharma products to reach patients after obtaining EU marketing authorisation across a range of European countries between 2007-2009 and 2014-2016.^{39,40} Deloitte analysis of the data set found that of the countries covered in both EFPIA analysis (i.e. the 2007-2009 and 2014-2016 data set), the average number of days it takes to complete post-marketing authorisation processes has increased from 233 days for products covered in the 2007-2009 analysis, to 318 days for those covered from 2014-2016. The largest increase in the number of days it takes complete these activities was seen in Portugal (an increase of 288 days), Ireland (an increase of 251 days), and Austria (an increase of 241 days) (See Figure 7).

Figure 7. The average length of time between pharma market authorisation and patient access is increasing, 2007-2009 and 2014-2016



Note: 2014-2016 average is of those countries included in the chart. 2007-2009 average excludes the UK and Germany due data not being available for the countries in older reports produced by EFPIA. With the reason cited in the 2007-2009 analysis by EFPIA being “For the purpose of the “Patients W.A.I.T. Indicator”, it is considered that Germany and the UK allow access to new medicines upon marketing authorisation – in these countries, no pricing / reimbursement process needs to be completed before new medicines can be prescribed to patients. However, other hurdles to access – which are not within the scope of this analysis – may apply in these countries.” Data in the chart is arranged by longest length of delays as per the 2014-2016 EFPIA data set.

Source: EFPIA, 2010 and 2018

Part 3. New approaches to access and pricing

Because of the challenges presented by the current European market, the approaches to pharma market access and pricing are changing. The market context dictated by regulators and payers requires pharma to demonstrate the long-term value of its products and contribute to the health system in clinical, operational and financial outcomes, thereby fueling a greater emphasis on VBC.

In turn, regulators and payers in some markets have become more receptive to new models of regulatory approval and pricing and how innovation can reach patients faster.

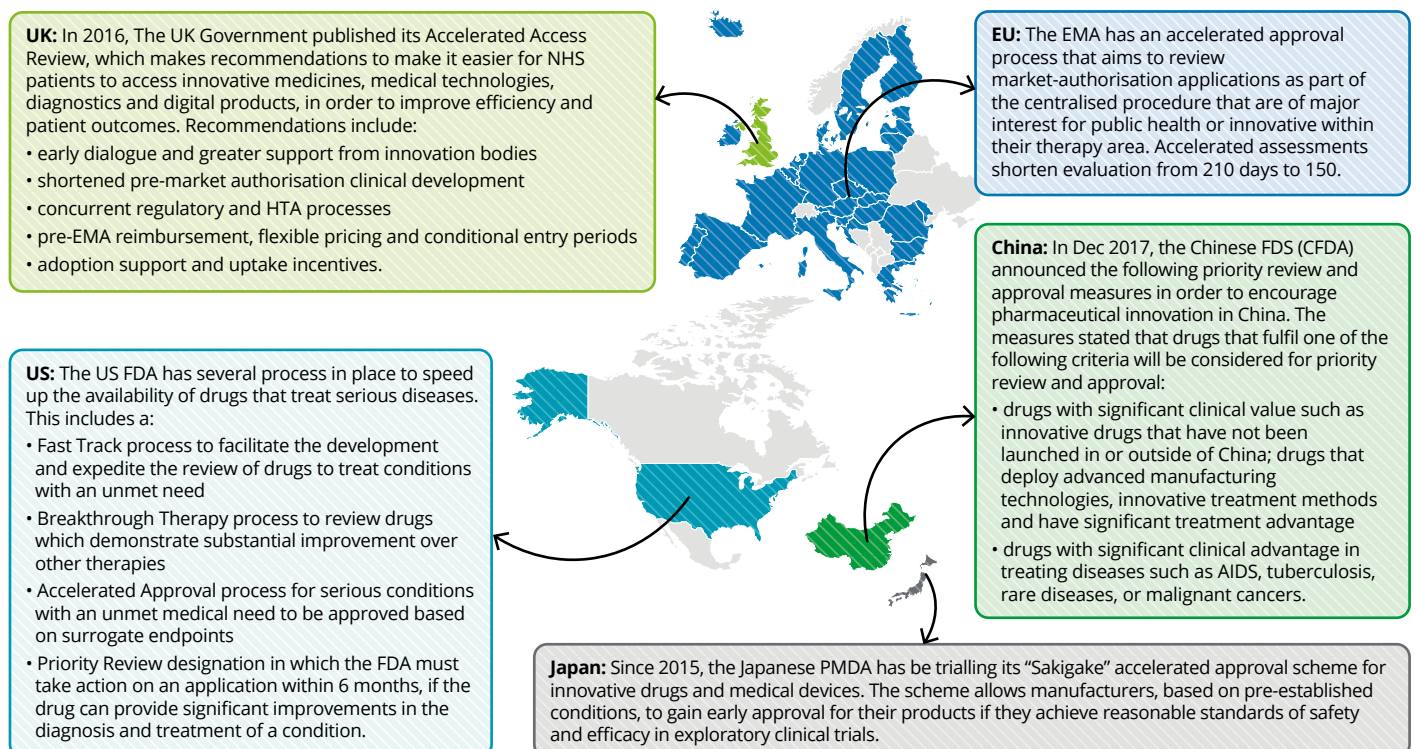
Accelerated approvals allow pharma to derive revenue faster

Leading regulators across the world have implemented processes that speed up the appraisals, approvals and market access for pharma products (See Figure 8).^{41,42,43}

For pharma products, these include:

- accelerated approvals for breakthrough products
- new models of contracting
- a requirement for greater service participation.

Figure 8. Key pharma accelerated access schemes across the world



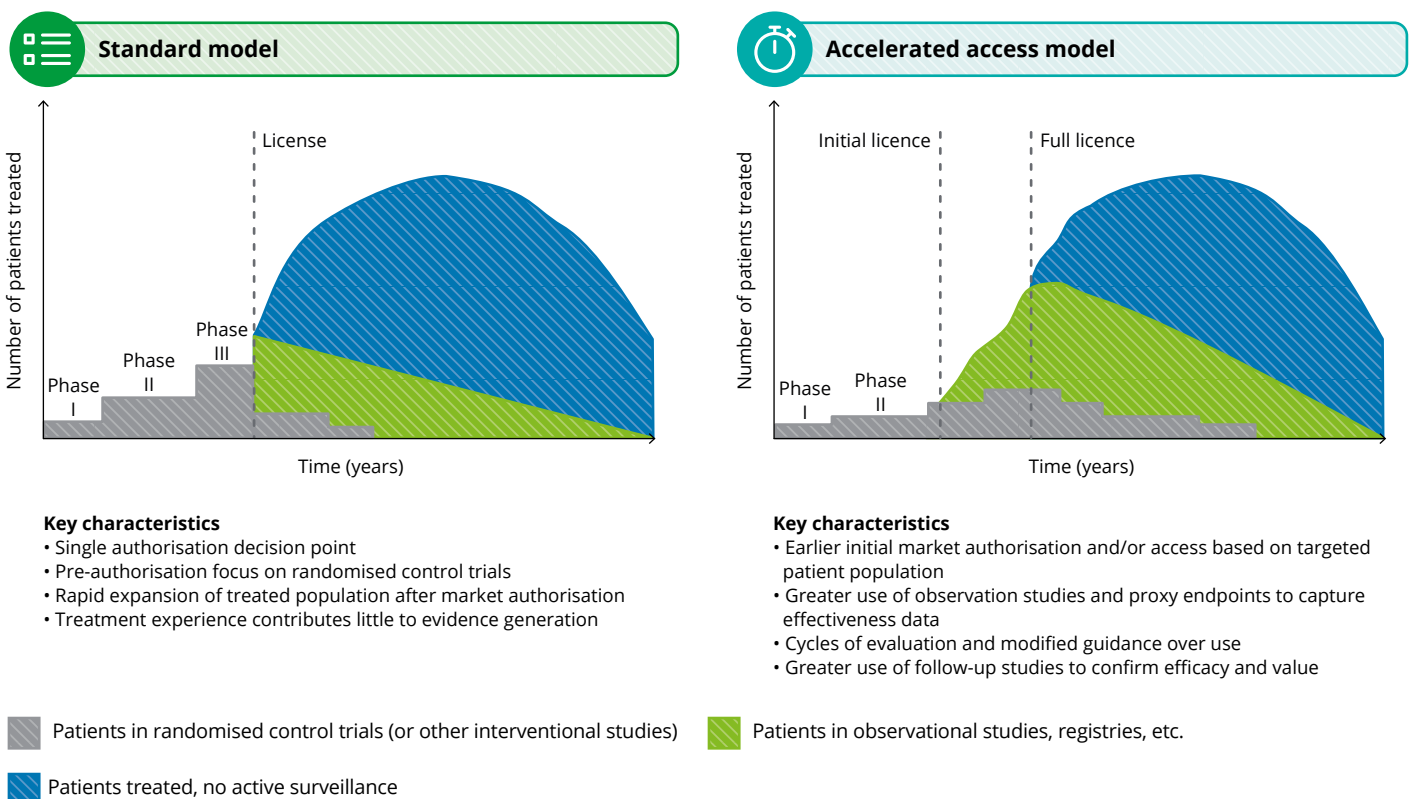
Source: Deloitte LLP, 2019; FDA, 2018; EMA, 2018; Global Forum, 2018; PMDA, 2017; Accelerated access review, 2016

The underlying aim is to increase patient access and adoption rates for new therapies developed by the pharma industry by using the approvals process usually reserved for breakthrough products or those with an unmet medical need. Accelerated pathways fundamentally change the model for approvals from one that is rigid in its approach to one that is agile, adaptive, iterative and allows pharma to generate early revenue if they successfully navigate the process (See Figure 9). In these schemes regulators are willing to assess clinical efficacy and safety data during exploratory trials and grant manufacturers' access to the market based on conditions, such as being able to continue to assess efficacy and safety data while the product is on the market.⁴⁴ These market access conditions are important as manufacturers and payers can negotiate prices and reduce long-term risk based on a product reaching certain milestones: for example, providing better patient outcomes in comparison to a competitor or reducing payer and provider costs.

Interest in and use of accelerated approval schemes has been growing among manufacturers due to the early market access and revenue they can provide. For example, between 2014 and 2017:

- the EMA received 78 requests for accelerated appraisal, accepted 50, with a peak of 17 approvals in 2015, and denied 28. Moreover, the agency recommended 52 per cent (26 pharma products) of these approved appraisal requests as medications⁴⁵
- the FDA, through its Breakthrough Therapy scheme (combining data from the FDA's Center for Drug Evaluation and Research and Center for Biologics Evaluation and Research) received 500 requests for appraisals, accepted 191, with a peak of 59 in 2017, and denied 248. Moreover, the agency recommended 49 per cent (94 pharma products) of these approved appraisal requests as medications.⁴⁶

Figure 9. Key characteristics of the standard and accelerated access models of pharma regulatory approval



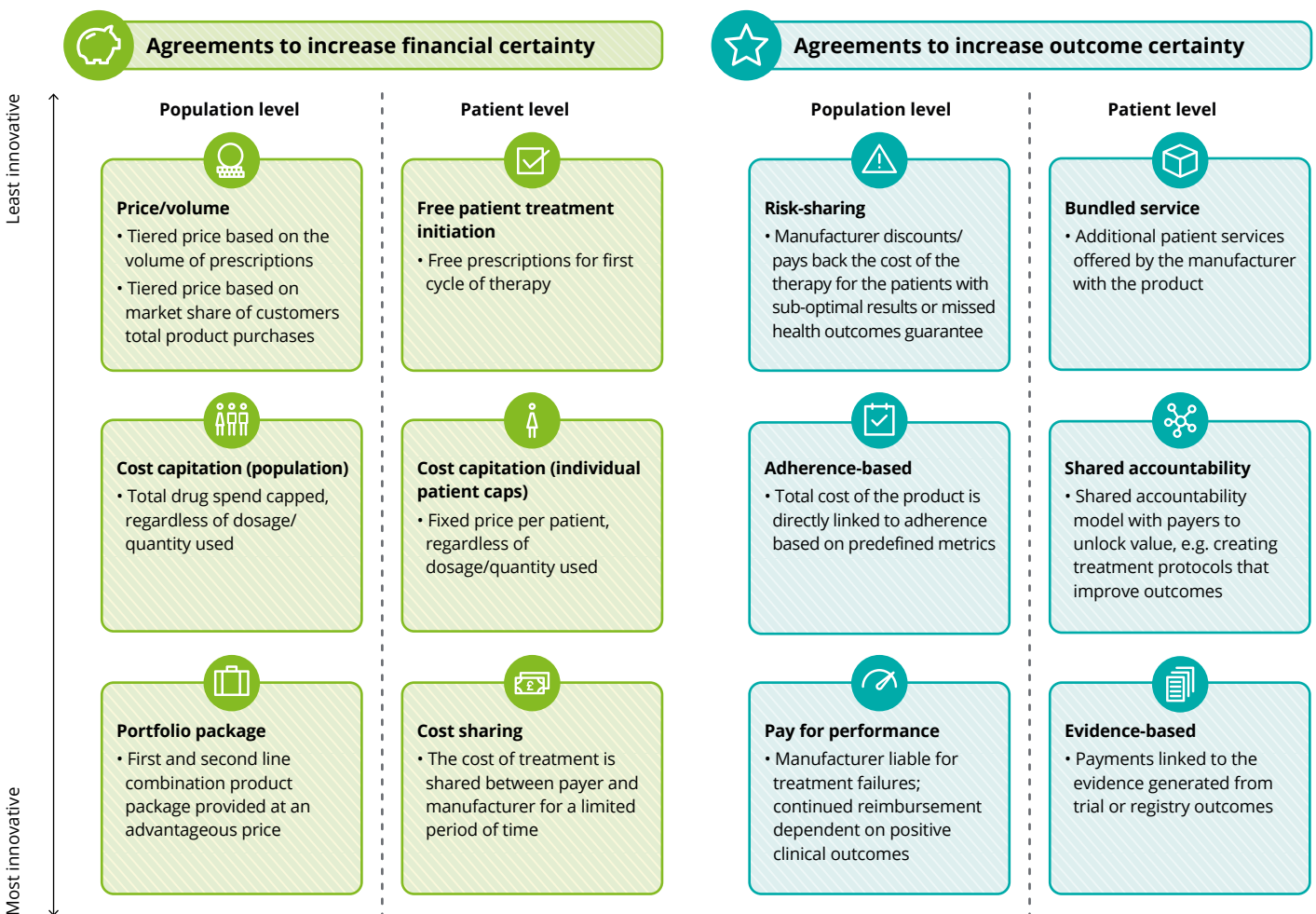
Source: Eichler et.al, 2016; Deloitte LLP, 2019

Innovative contracting

Innovative contracting is an evolving paradigm in pharma pricing which facilitates VBC, of which there are a variety of different models (see Figure 10). Globally, from 1995 to 2018 the number of innovative contract cases submitted totalled 477, with 283 (60 per cent) of these in Europe.

The number of cases submitted in Europe was highest in Italy (88), followed by the UK (70) and then Sweden (68), and lowest in Slovenia (1), followed by France (3), and Belgium (4). Innovative contracting was primarily used to support therapies in oncology, endocrinology and neurology, accounting for 50, eight and six per cent, respectively, of submitted cases.⁴⁷

Figure 10. Types of innovative value-based contracts



Source: Deloitte LLP, 2019

Given the significant financial constraints on European payers, there has been increasing emphasis to move away from the traditional volume-based pricing of pharmaceuticals to ones based on health outcomes, risk sharing and cost containment. Analysis by the Economist Intelligence Unit in 2016, commissioned on behalf of Medtronic, evaluated the presence of VBC systems across 25 countries.

In Europe, the study showed that there has been a gradual shift in alignment towards aspects of VBC, in particular towards outcome-based payment approaches, albeit to varying extents in the countries covered (see Figure 11).⁴⁸ These variations may be a reason why the uptake of innovative contracting has been slow and generally occur at a local rather than national level.

Figure 11: Alignment with value-based care, by country

	Alignment with value-based healthcare	Enabling context, policy and institutions for value in healthcare	Measuring outcomes and costs	Integrated and patient focused care	Outcome-based payment approach
France	Moderate	Moderate	Moderate	Moderate	Very high
Germany	Moderate	High	Moderate	Low	Very high
Poland	Moderate	Moderate	Moderate	High	High
Spain	Low	Low	Moderate	Moderate	Low
Sweden	Very high	High	Very high	Very high	Very high
United Kingdom	High	High	High	Very high	High
Australia	Moderate	Moderate	Moderate	Moderate	High
China	Low	Low	Moderate	Moderate	Low
India	Low	Low	Low	Moderate	Low
Japan	Moderate	Moderate	Moderate	Very high	Low
United States	Moderate	Moderate	High	Moderate	Moderate

Source: Economist Intelligence Unit, 2016

Approaches to contracting vary in their scope and execution and can be tailored to the specific needs of the product and the launch market. The rise of innovative contracts shows that payers, providers and manufacturers are developing closer relationships and more constructive dialogue, and are moving away from traditional price/volume agreements. The success of the more innovative contracting models relies on the ability of the manufacturer to provide solutions that address a genuine challenge for the payer, are easy to administer, and built on aligned interests and use realistic payment terms. The more progressive approaches range from the bundling of additional services on top of products in order to support providers in achieving efficiency improvements and pathway cost reductions,⁴⁹ and the use of outcomes-based approaches that link reimbursement to the health outcomes of a patient (See Case study 2).

Although a variety of contract models have been proposed by pharma, payers may still be unwilling to participate in discussions. Factors that may detract from the feasibility of VBC and innovative contracting include a lack of transparency and trust between pharma organisations and payers, the administrative burden associated with VBC, and a perception that increased contractual complexity increases payer risk.

Moreover, payers should also consider indication-based pricing (IBP), whereby a pharma product can have different prices depending on the value it provides for specific medical indications. Within the US a sense of the value IBP can offer payers and the pharma industry is gaining traction. However, within Europe there has been little discussion on IBP.



Case study 2: Examples of outcomes-based payment approaches

There are a variety of outcomes-based approaches implemented across the world. Below are a few examples of some of the contracting schemes. These include:

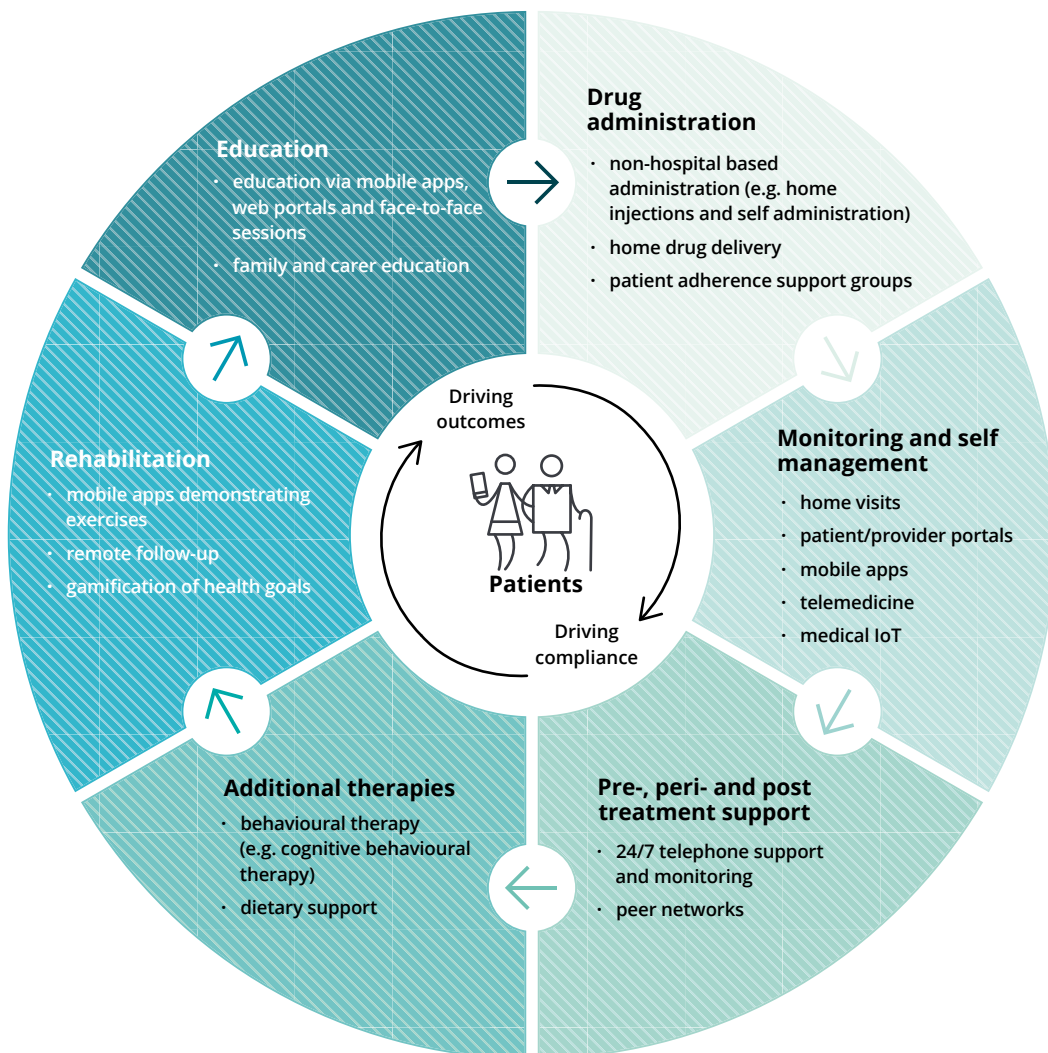
- **Cost-sharing agreements:** where full or partial discounts are offered for the initial cycle of treatments, such as the use of Sunitinib, a drug used for patients with metastatic renal cell carcinoma treated by the NHS. Under this scheme, the first treatment cycle of 6 weeks (costing an average of £3,139 per patient) is provided free via a patient access scheme. Subsequent cycles are funded by the NHS until disease progression.^{50,51}
- **Risk-sharing agreements:** where a partial or full price discount is offered if patients do not respond to treatments. For example, through the use of a genetic test, a manufacturer was able to offer a non-small cell lung cancer drug to Spanish payers based on a risk-sharing agreement. In a study involving 41 patients, the agreement used genetic-testing to define the patient population, where patient outcomes were evaluated at week eight and week 16 of the treatment. If the treatment failed, the total cost would be reimbursed to the payer. Compared to traditional payment methods, the payer saved 4.5 per cent on overall treatment costs when assessed at week 16 of treatment and saved €36,000 in total (approximately €880 per patient).⁵²
- **Payment-by-results:** where payments to the manufacturer increase or decrease depending on patient outcomes. For example, in 2018, AstraZeneca, a global pharma manufacturer, and Harvard Pilgrim Health Care, a large not-for-profit health services company in the US, signed a deal for an outcomes-based approach over their asthma and chronic obstructive pulmonary disease drug, Symbicort. The agreement sees Harvard Pilgrim monitoring whether asthma-related symptoms for patients on Symbicort are in line with the clinical trial results provided by AstraZeneca. If the occurrence of worsening symptoms/exacerbations requiring medical intervention exceed predetermined thresholds based on the clinical trials data, Harvard Pilgrim will be charged a lower amount.⁵³ Also in 2018, Harvard Pilgrim reached a outcomes-based agreement with Alnylam Pharma to provided its rare disease therapy, ONPATTRO, for patients with polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis.⁵⁴

Requirement for greater services participation

As demonstrated in our 2018 report, *Medtech and the Internet of Medical Things*, the successful implementation of services can transform an organisation from a supplier of innovative products into an innovative partner for health care delivery, rewarded for improving health care performance.⁵⁶ With the rhetoric around VBC continually developing, the life sciences industry as a whole is being required to build closer relationships with payers that go beyond purely supplying products.

According to data from Eurostat, 49 per cent of the EU population self-reported the use of prescription medicines in 2014, with the highest usage among those 75 years of age and above, at 87 per cent.⁵⁷ Given the pervasiveness of pharma products among European populations, there is a significant opportunity for the industry to offer services that support patients and health care providers with drug administration, monitoring and self-management, pre-, peri- and post-treatment support, complementary therapies, and patient rehabilitation and education programmes (See Figure 12).

Figure 12. Services can be used to support patients and health care providers in a number of ways



Source: Deloitte LLP, 2019

A number of pharma companies already provide services that deliver additional value to the organisations they are working with. However, due to the different requirements from country to country and sometimes between country-specific providers, initiatives often occur locally or regionally, and rarely at national level. Service design requires pharma to not only think differently about how they charge for their products but also about the way in which they collaborate both internally and externally. It also requires a greater level of understanding of the local health care system and the partnership pathways available (See Case study 3).

...the successful implementation of services can transform an organisation from a supplier of innovative products into an innovative partner for health care delivery, rewarded for improving health care performance.



Case study 3: Louisiana Department of Health seeking Netflix type subscription model for hepatitis C drugs

In the US, the state of Louisiana has a high burden of hepatitis C, with an estimated 35,000 people living with the disease and the state's spend on treatment circa \$35 million annually. Given their current budget, the state has enough to cover fewer than 3 per cent of Medicaid patients and 1 percent of prisoners with the disease.

Some of the most innovative treatments for hepatitis C can cure the disease but come at a high per-patient cost. It would cost an estimated \$760 million for the state of Louisiana to provide its hepatitis C population with the latest treatments. As result, the state reserves these treatments for the most severe cases.

The state is seeking alternative contracting models to improve patient access to innovative new treatments, improve patient outcomes and get the most out of its hepatitis C budget. The proposed model sees the selected pharma organisation providing unlimited access to its hepatitis C medication in exchange for an annual fee. The Louisianan Department of Health is aiming to have the model in place within the next year and a half, following federal approval of the scheme.

Ten other US states have also expressed interest in mirroring the subscription model should Louisiana be successful in gaining approval.⁵⁸

Part 4. Succeeding in the new value-based environment

For pharma to succeed in the current European market, the industry needs to be more constructive in their dialogue, communicative in their development intentions and market access strategies for a product, collaborative with stakeholders, and innovative in the models they deploy for market access and pricing.

Early dialogue can allow pharma to understand better the needs of regulators, payers, patients and HTA authorities

Given the differing evidence requirements of the EMA, HTA and payer authorities (such as NICE in the UK), products that have been approved for the market by regulators sometimes fail assessments used to decide reimbursement. Early dialogue allows pharma to understand better the scientific and economic considerations of the regulator and HTA and payer authorities early on, and to build and capture these parameters within the design of a clinical trial and downstream pricing negotiations. Early dialogue allows manufacturers to:

- increase the likelihood of product approval
- form a stronger basis for downstream discussions on pricing and reimbursement
- provide better planning for a product's life cycle
- promote the collection of high-quality data
- build a better view of sustainable value
- reduce the burden of a clinical trial on the patients involved.

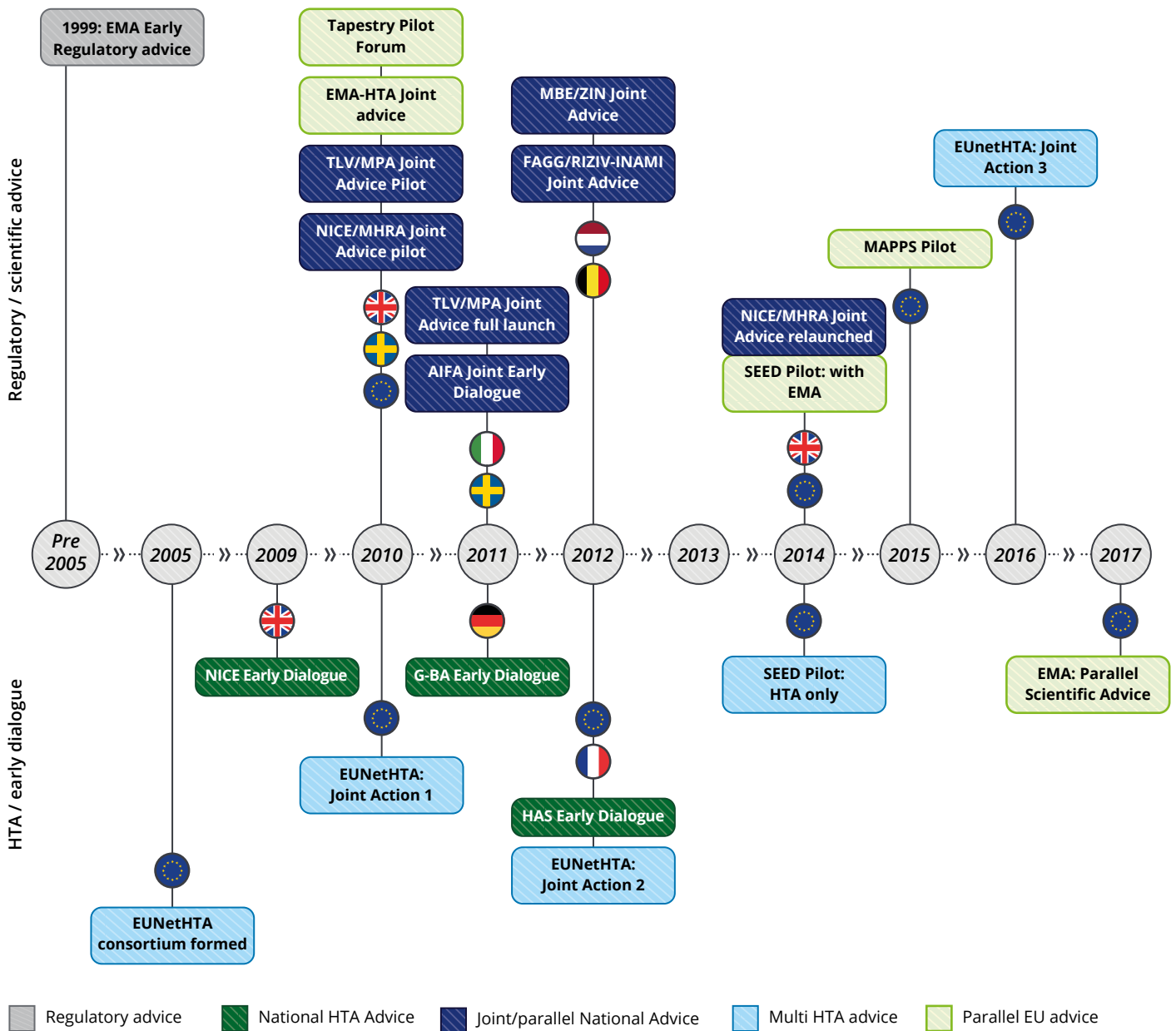
Early dialogue, especially with the regulator, is an established method pharma has used to increase the likelihood of market approval. Between 2013 and 2017, the EMA saw a 33 per cent increase in the number of requests for scientific advice, with 62 per cent of scientific advice applicants receiving positive opinions of their product in 2017.⁵⁹

Achieving early dialogue discussions with HTA and payer authorities is a more difficult and nuanced task due to the differing assessment criteria used across European countries and the past requirement for manufacturers to contact appraisal authorities separately when seeking parallel scientific advice with the EMA. However, in 2017 this was changed, and the EMA introduced parallel consultations to replace the scientific advice procedure, allowing consultations to occur at the same time between the regulator and the economic assessor. It is too early to assess the effectiveness of this initiative but it does represent a significant step forward.

Prior to this, individual countries worked on a number of initiatives and pilots to support early dialogue, including the establishment of the European Network for Health Technology Assessment (EUnetHTA) in 2005. The EUnetHTA is a network of 80 organisations representing the 28 EU countries. The network collaborates on initiatives such as facilitating the efficient use of resources available for HTA, creating a sustainable system of HTA knowledge sharing, promoting good practice in HTA methods and processes, and supporting the use of early dialogue discussion with pharma and medtech across the EUnetHTA network (See Figure 13 overleaf).^{60,61}

Early dialogue allows pharma to understand better the scientific and economic considerations of the regulator and HTA and payer authorities early on, and to build and capture these parameters within the design of a clinical trial and downstream pricing negotiations.

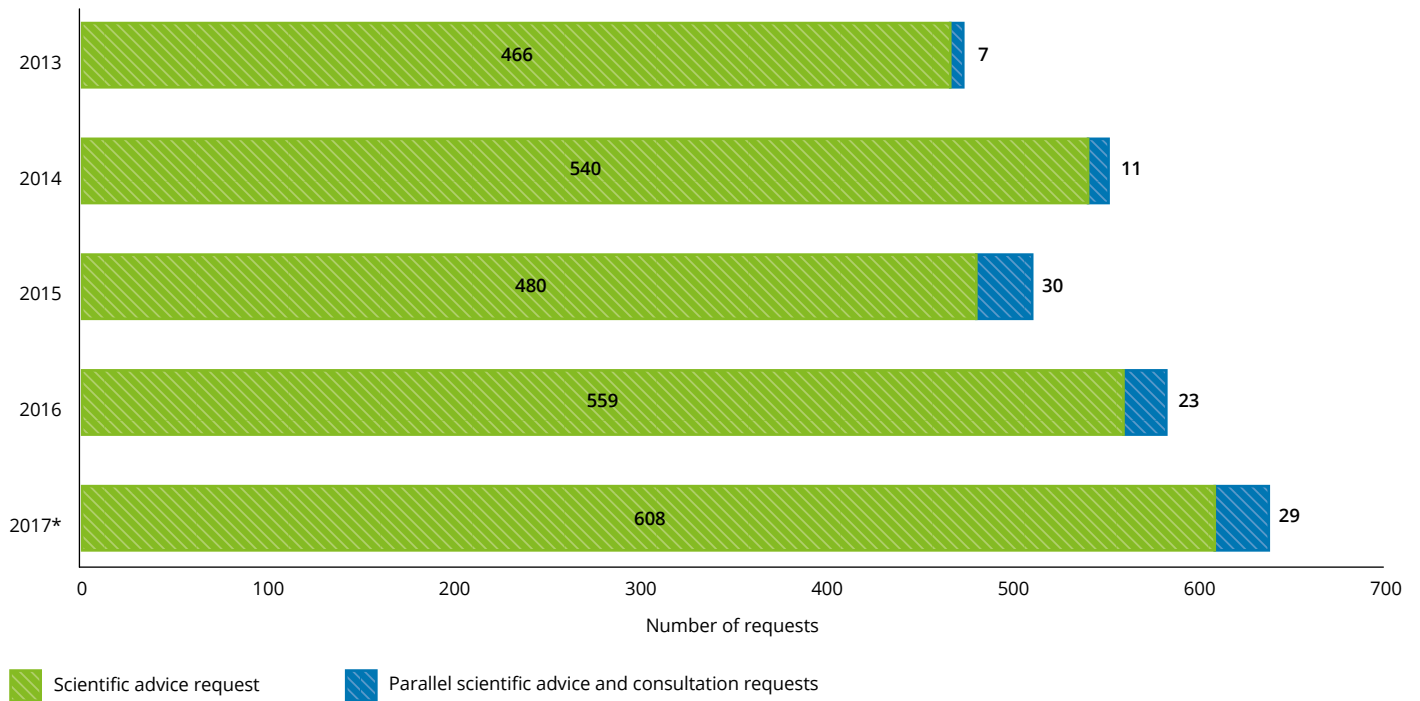
Figure 13: Early dialogue and joint advice initiatives occurring across Europe



Note: date shows year in which process initiated
 Source: Deloitte LLP, 2019

However, the number of requests for parallel scientific advice and consultation remain low (see Figure 14), and when early dialogue is initiated, manufacturers may still be opting to seek dialogue with individual HTA and payer authorities.^{62, 63}

Figure 14. The number of scientific , and parallel scientific and consultation requests received by the EMA, 2015 to 2017



Note: Parallel scientific advice and consultations have been combined for 2017, of which there were 22 and 7, respectively. The parallel consultation procedure was introduced in 2017 therefore there is no data on parallel consultations prior to this year.
 Source: EMA, 2018

There are a number of early dialogue options for pharma at a national, joint-national and multinational level (See Figure 15), and, as discussed earlier, a number of innovative contracting models that pharma can use.

Figure 15: Early dialogue options available to pharma

Responsible authority	Negotiation Level	Advantage	Disadvantage	Considerations on when to engage	Who to engage (e.g.)
HTA authority	National	<ul style="list-style-type: none"> Depth of advice to tackle country-specific issues Ability to change standard of care Close work with agency to set out criteria 	<ul style="list-style-type: none"> Small market proportion Companies unlikely to change development programs to suite one market May be difficult to engage if a well renowned assessor due to time constraints (i.e. NICE and G-BA) May lack specific therapeutic knowledge 	<ul style="list-style-type: none"> Influence HTA authority has over other agencies Quality of advice Cost of engagement Availability of HTA assessor 	<ul style="list-style-type: none"> UK: NICE Germany: G-BA France: HAS Italy: AIFA Sweden: TVL
Regulator and HTA authorities	Join-national (parallel)	<ul style="list-style-type: none"> Depth of advice to tackle country-specific issues Potential to drive convergence between regulatory and HTA requirements Potential to save time by combining meetings 	<ul style="list-style-type: none"> Discussions may be dominated by regulator or one HTA body No guarantee of the convergence of requirements 	<ul style="list-style-type: none"> When there is expected to be a difference between evidence requirements between the regulator and HTA body 	<ul style="list-style-type: none"> UK: NICE and MHRA Germany: G-BA and BfArM France: HAS and ANSM Italy: AIFA Sweden: TVL and MPA EMA
Regulator and HTA authorities	Multi-National	<ul style="list-style-type: none"> Potential for large market penetration Able to provide a broad perspective on evidence requirements across multiple countries Ability to engage multiple HTA assessors at once saving time and expense Ability to understand areas in which there is divergence between HTA bodies and what is driving them 	<ul style="list-style-type: none"> Due to so many participants there is may be little time to discuss specific details important to individual agencies Potential for small HTA assessors to lose their voice and follow the stricter assessment criteria used by more established assessors 	<ul style="list-style-type: none"> For multi national product launches For rare disease products as benefits will be similar across all nations 	<ul style="list-style-type: none"> EUNetHTA and EMA through initiatives such as the Shaping European Early Dialogues (SEED) project and parallel consultations process, respectively

Source: Deloitte LLP, 2019

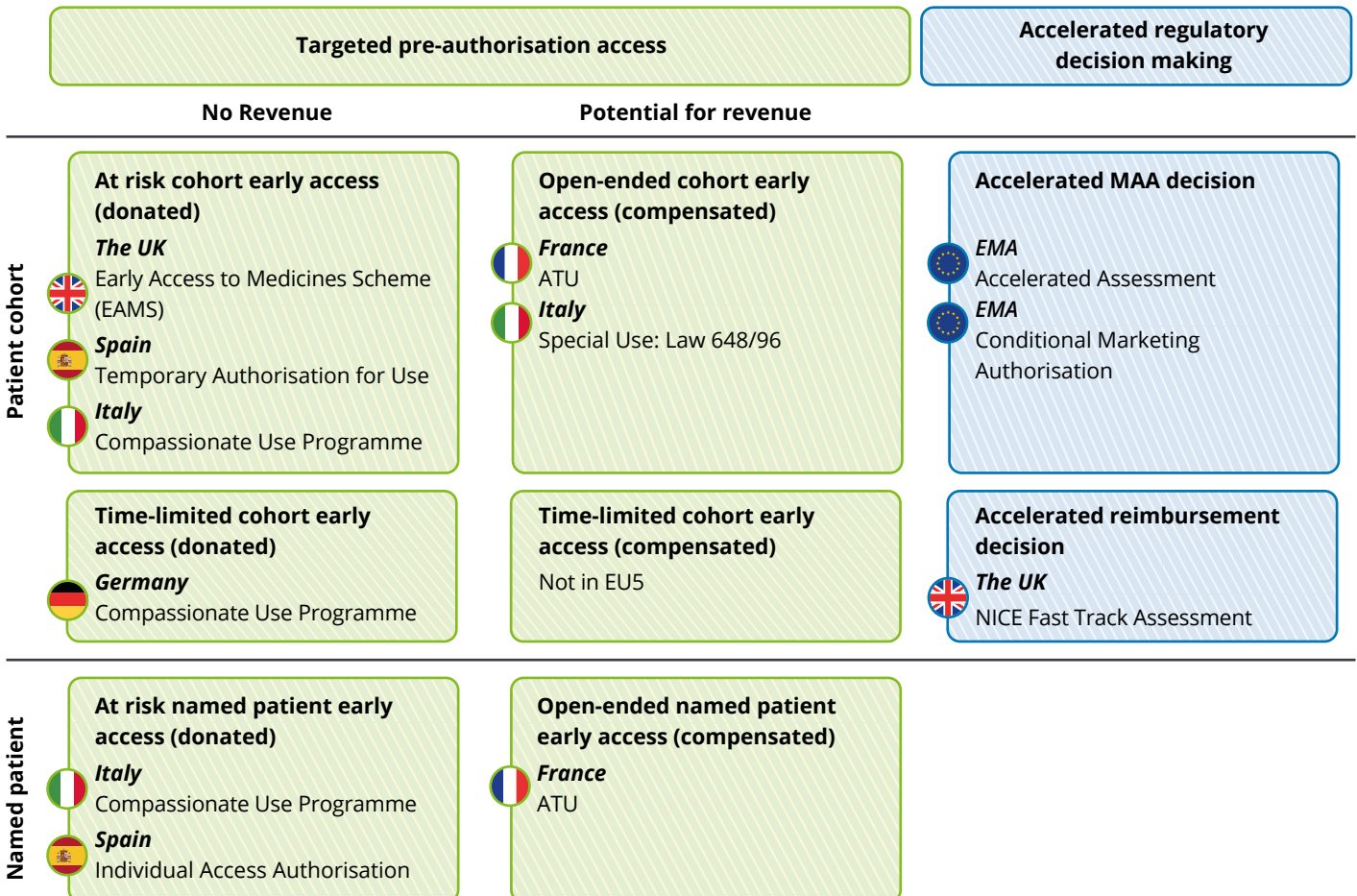
Better use of early access schemes can improve pharma market access

There are also a number of approaches that pharma can use to gain access to a market pre-authorisation. These are often aligned to the compassionate use of a product at both an individual and cohort level. Eighteen of the 28 EU member states have nationalised regulations in place that are well-defined.⁶⁴

Some of these initiatives allow pharma to access markets through the donation of medication for at-risk groups of patients, such as the UK's Early Access to Medicines Scheme and the German Compassionate Use Programme, but these initiatives carry no revenue for the donating organisation.^{65, 66}

Other schemes in Europe allow the pharma industry to access a market pre-authorisation and derive revenue from a product, though usually for a short period of time. For example, the French Temporary Utilisation Programme (ATU), which can be used for individuals or cohorts of patients, allows the manufacturer to set the price of the product freely for one year – an extension of one year is also possible. However, if the product is launched, and a difference in price is found, the difference must be paid back. An overview of the schemes can be found in Figure 16.⁶⁷

Figure 16. Types of early access schemes available in the 5EU



Source: Deloitte LLP, 2019

Given the increasing amount of personalisation in pharma products and the smaller number of patients targeted for specific therapies, the use of pre-authorisation access schemes can allow pharma to gather evidence on a product's safety and efficacy, gauge a market's receptiveness towards adoption early on in a product's life cycle, and provide patients with access to the latest medications. In Europe, there are a variety of schemes available, and there is widespread recognition of their importance to patients. However, to use them successfully, pharma should:

- collaborate more closely with patient advocate groups and doctors early in the development of a medicine to better understand the medicine's appeal
- define outcomes measurements that can be captured through early access which can be used to support accelerated regulatory approval and economic assessments used by HTA and payer authorities
- define inclusion criteria for compassionate use with patients, doctors, regulators and payers (if applicable)
- accept that entering into early access schemes has risks such as the continuation of a programme should reimbursement be declined and increased transparency in a product with health care providers and patients, although potential benefits include possibly reducing the long-term risk associated with a product.⁶⁸

Service and technology partners can help pharma demonstrate value beyond the pill

In some European countries there are greater requirements for pharma organisations to provide services that better support patients, providers and payers. To this extent, the pharma industry has developed, or is looking to develop, partnerships with technology organisations which can provide services beyond the pill, as outlined in our 2017 report, *Pharma and the connected patient*.⁶⁸

With VBC and RWE becoming increasingly prominent, partnerships with technology organisations are occurring at all junctions of the pharma value chain, from drug discovery to sales and reimbursement.

Some partnerships have led to the creation of mobile apps that are able to support patients better with their illness while simultaneously providing live feedback on their condition and behaviours to them, their carers and clinicians coordinating their care (See Case study 4).



Case study 4: Voluntis collaborations with pharma on mobile technologies

Voluntis is a mobile technology developer that has collaborated with a range of large pharma organisations to create clinically approved mobile applications for patients and physicians. The therapeutic areas in which apps have been produced include:

- **Diabetes:** Voluntis collaborated with Sanofi and the French diabetes research institute (CERITD) to create a mobile application to better treat patients suffering with type 1 and 2 diabetes on a basal-bolus regimen. CE approved in 2013,⁷⁰ the application, Diabeo, provides patients with decision making support and helps calculate personalised doses of insulin.⁷¹ The app also allows patients to be remotely managed through connections via telemedicine with healthcare providers.⁷² Clinical evidence shows that the technology provides a substantial improvement to metabolic control in chronic, poorly controlled type1 diabetic patients without requiring additional medical time and higher costs.⁷³ It also created Insulia, for people with type 2 diabetes on a basal regimen, that also has the CE Mark and was FDA-cleared for distribution in the United States.
- **Oncology:** Theraxium Oncology is a solution that is designed to help empower patients to self-manage their symptoms through a mobile application and allows care teams to follow the progress of patients through a web application. The clinical algorithms supporting decision making are consistent with US and EU guidelines for symptom management in oncology. The mobile application allows patients to identify, qualify and report their symptoms, take action to self-manage their symptoms through personalised recommendations, know when they need to contact their care team, and gain knowledge about their condition. For care teams, the data fed to the web application allows them to quickly identify the patients who need the most attention, take quick action when needed, arrange follow-up meetings with patients and coordinate care more effectively with other team members. Voluntis' oncology solutions have been used by both AstraZeneca and Roche to support patients undergoing treatment for ovarian and breast cancer, respectively.⁷⁴

Other partnerships focus on the application of big data analytics and artificial intelligence (AI) to improve internal decision-making. For example, in 2016 IBM Watson and Pfizer partnered to use IBM's cognitive technologies (i.e. machine learning and natural language processing) to accelerate drug discovery by allowing them to analyse and test hypotheses from large amounts of disparate data sources rapidly.⁷⁵ Other opportunities arise from the need to improve operational and financial performance of a health care system by the life sciences industry collaborating on services, such as programmes to reduce prescribing errors (Case study 5).

Partnerships with technology and service-based companies have the potential to disrupt the market and fuel change; they also offer a number of benefits to the pharma industry and health care stakeholders alike that can improve market access and pricing discussions if developed early on in the life cycle of a product (See Figure 17). These collaborations require not only that external organisations come together but also that internal business units work towards the same goal.



Case study 5: Pfizer and University of Leicester Hospitals collaborate to reduce prescribing errors

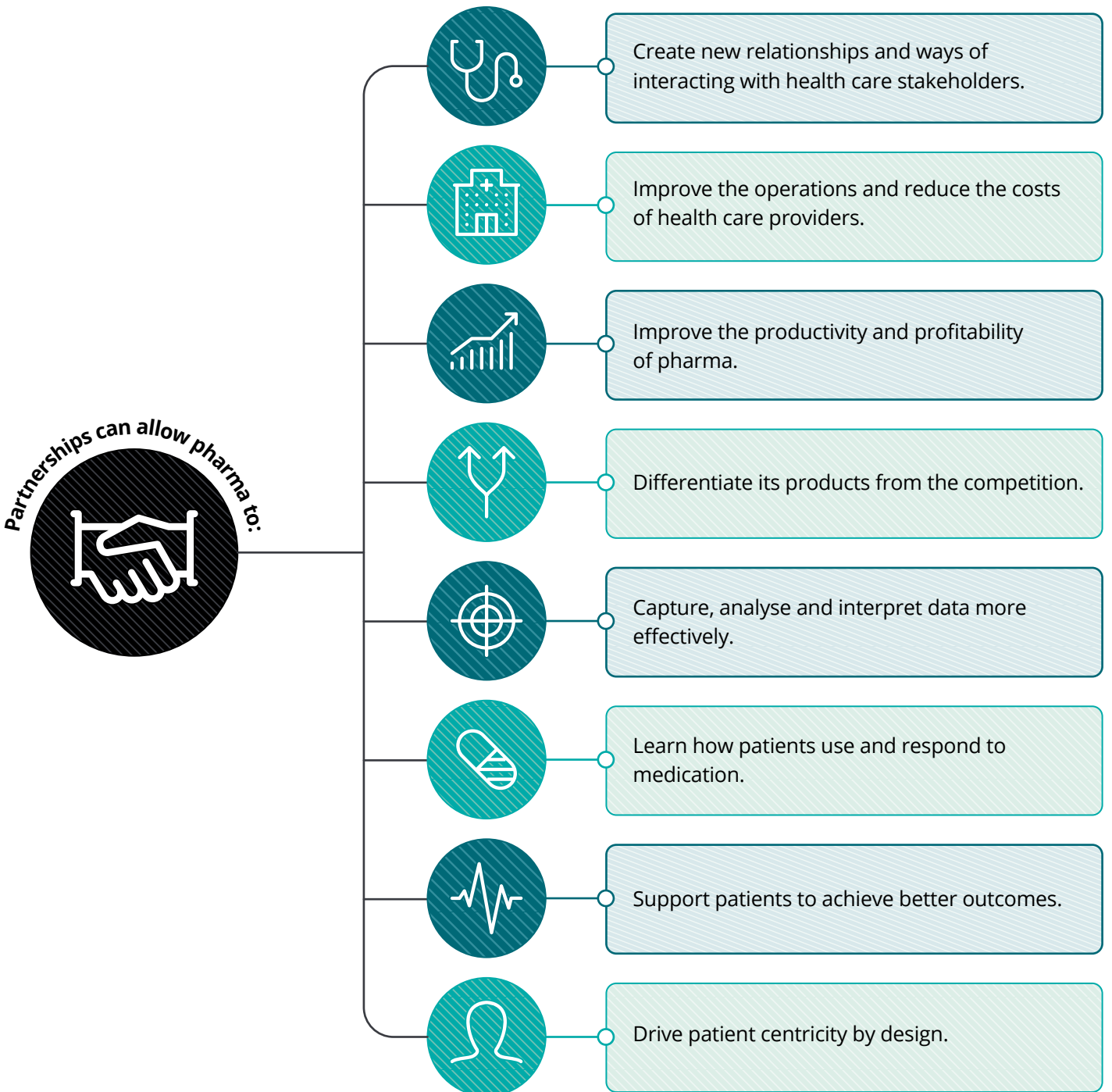
The National Patient Safety Agency (NPSA) estimates that avoidable harm from medication costs the NHS over £750 million annually in England. A 2009 study funded by the General Medical Council (GMC) found that junior doctors in their first and second years in practice make twice as many errors in prescribing as consultants, nurses or pharmacists. In 2013/14, Pfizer and University of Leicester Hospitals collaborated on the Effective Performance Insight for the Future (EPIFFANY) project, which aimed to improve prescribing competence, performance and attitudes towards safe prescribing and patient care among junior doctors. Two cohorts of junior doctors were rotated through the programme which comprised of four teaching components that incorporated real clinician and patient feedback.⁷⁶

Results from the trial showed:

- a 50 per cent decrease in medication errors made by the junior doctors who took part
- by the end of the four month trial, the junior doctors showed the same performance improvements as those with 12 months of clinical experience
- the potential to achieve £308,928 cost saving from avoidable medication errors
- the potential to avoid 489 inpatient bed days.⁷⁷

Following the success of the project, EPIFFANY is preparing for a national upscale across three further sites in the East Midlands, in order to evaluate the programme and to see ways in which it can be improved.⁷⁸

Figure 17. Tech and service based partnerships can provide a number of benefits to all health care stakeholders that can aid pharma product pricing



Part 5. Next generation market access

The unique challenges facing European payers will determine the extent to which pharma are able to adapt and tailor their approaches to market access and pricing and continue to thrive. Pharma companies should be more agile and develop and enhance their core organisational capabilities to support market access and pricing in a proactive way throughout the life cycle of their products. This includes, developing a deeper understanding of payer needs, using RWE to capture a product's value, managing patient data effectively, and building trust with patients and payers.

Earlier launch planning focussed on dialogue

Pharma should have a greater understanding of the specific needs and priorities European payers have when considering product development. For example, understanding at the outset of product development the disease and cost burden individual European payers are facing. Pharma should, at critical milestones in the R&D phase, plan and discuss the clinical need for a new product with payers across Europe. Engaging with payers earlier will allow them to target the right markets for their products and engage with patients and providers in much more constructive, collaborative and valuable ways. This should also help pharma develop products that meet the priorities of the health care system and the unmet needs of patients.

Innovative contracting

Understanding the clinical and financial needs of payers early on in the R&D process allows pharma to model, test and negotiate a variety of potential contracting solutions. Not all payers in Europe will be looking for innovative outcomes-based contracts when reimbursing a product, as they may favour more financially driven contracts such as volume- and portfolio-based contracts. Only through constructive dialogue can pharma adapt their pricing strategies to meet the needs required by the market they are looking to access. The success of innovative contracting will be contingent on pharma developing and deploying multi-disciplinary teams that can collaborate effectively with European payers to solve their most pressing issues and support their long-term sustainability.

Real-world value dossier creation: use RWE to understand system challenges, physician and patient experiences and the benefits of products and services

RWE represents a shift in the way pharma products are assessed for both clinical- and budget-effectiveness. At its best, and through working closely with payers and regulators, RWE can improve the access patients have to new medications, while simultaneously improving outcomes and allowing a pharma company to assess the value of its product outside tightly controlled clinical settings (See Case study 6).

Moreover, RWE is a technology and data-driven solution that also allows pharma organisations to gain an in-depth understanding of health system challenges, physician and patient experiences, and the benefits of their products and services, to the patient populations they serve, including the ability to demonstrate value in a more holistic way.

Understanding the clinical and financial needs of payers early on in the R&D process allows pharma to model, test and negotiate a variety of potential contracting solutions.



Case study 6: GlaxoSmithKline (GSK) utilising RWE to speed up patient access and improve the health of Manchester's population

Running from 2012 to 2016 the Salford Lung Studies (SLS), funded by GSK (a global pharma company) and made possible by the use of an integrated primary and secondary electronic health record developed by North West EHealth (a clinical trials platform service provider), investigated the impact of its asthma and COPD medication Relvar in a 12 month phase IIIb clinical study (pre-licence). Utilising early dialogue discussions with NICE and the Medicines and Healthcare products Regulatory Agency (MHRA), the study team were able to design a trial, that if successful, would satisfy the safety and data collection needs of both stakeholders.⁷⁹

In a move away from traditional highly controlled studies, GSK worked with 80 primary care providers in Salford and South Manchester and took a RWE approach to the study. Asthma and COPD patients were eligible based on a GP diagnosis without the need for spirometry and without stringent monitoring of whether a patient was taking their medication correctly.

Through this open study design the trial was able to capture 2,800 patients, including over a quarter of Salford's COPD patient population and 4233 asthma patients. Additionally, the study used no placebo (the comparison was against the patient's usual medication), the patient's own physician as the clinical investigator and an electronic monitoring system (linking primary and secondary care electronic health records) to notify physicians of serious adverse events. This real-world population based approach to the study resulted in a number of key improvements over traditional methods of clinical trials, including:

- increasing patient retention with only 7 per cent of COPD patients dropping out compared to up to 30 per cent seen in traditional studies of similar duration⁸⁰
- improving patient access to new medications.

The study was also able to show how effective Relvar was in real life by reducing the annual rate of acute exacerbations of COPD by 8.4 per cent when compared to the standard treatment and improving control in asthma patients, where the odds of achieving or improving control was double in the group initiated on Relvar compared with the usual care group.⁸¹

Build trust and understanding: be a collaborative partner in your therapy areas and build trust

The corporate reputation of pharma has been poor historically and remains a challenge. Research by PatientView, who conduct an annual study into the corporate reputation of pharma at a global and selected country level, found that in the UK, the percentage of patient groups who rated the pharma industry's corporate reputation as "excellent or "good" reached an all-time high of 29 per cent in 2017, up from only 25 per cent in 2016.⁸² A poor reputation among patients and patient groups can detract from patient engagement initiatives. Indeed, research from our 2017 report, *Pharma and the Connected Patient*, showed that patient groups trust in apps produced by different developers ranked apps developed by pharma and biotech companies as the least trusted. They were also less willing to share data from their health apps with pharma compared to other developers. Despite this, patient

groups highlighted a willingness to collaborate in the creation of apps with pharma. However, only 15.1 per cent of the patient groups we surveyed had been involved in co-creating a pharma health app to date.⁸³

Improving early patient engagement and feedback through meaningful collaboration with patients and physicians is a key capability required by pharma to ensure they create products truly needed by the end user. The most innovative of these approaches sees pharma organisations providing patients and health care providers with the tools to understand, manage and seek treatment for the conditions that are affecting them or their patients better (See Case study 7 overleaf). Moreover, pharma needs to ensure that data acquired from patients are handled in an ethical way, patient privacy is protected and data are stored securely as to not undermine patient trust and engagement.



Case study 7: Leo Pharma and the Leo Innovation Lab building therapeutic trust through technology

Leo Pharma is a global pharmaceutical company that specialises in developing products and services for patients with skin conditions such as psoriasis. In 2015 it established the Leo Innovation Lab, an independent unit of the company that is primarily focused on making a difference to people living with skin conditions by developing e-Health and add-on devices solutions.⁸⁴

With Leo Pharma and subsequently the Innovation Lab wholly owned by the Leo Foundation, the organisation has a corporate structure without shareholders and profits are directly reinvested into developing new solutions. This allows the organisation to be more agile and fosters an environment in which innovation can thrive and ideas be rapidly prototyped, allowing closer relationships with the users of their digital products to be built.⁸⁵ The Leo Innovation Lab has developed a number of solutions for people with chronic skin conditions. These include:

- **Imagine:** a digital platform to improve prevention, monitoring and diagnosis of chronic skin conditions. By taking pictures with their smartphone, the users can track their skin lesions as they change over time through an app. This enables users to draw correlations with lifestyle triggers, assess the efficacy of treatments and consequently find the optimal treatment and life style change as rapidly as possible. The project is also harnessing image data to develop sophisticated AI that can assess flare-ups of the condition and within seconds determine the nature of a skin lesion. Currently, the platform has succeeded in diagnosing psoriasis with an accuracy of 91 per cent.
- **Studies&Me:** a digital recruitment and qualification platform for clinical studies. The platform is aimed at people living with a skin condition and allows them to find a clinical study based on information in their user profile and store-and-forward evaluations carried out by a qualified dermatologist.⁸⁶
- **PsoHappy:** a global study that aims to measure the happiness levels of people living with chronic conditions, including psoriasis and atopic dermatitis.⁸⁷ The initiative provides reports on the research to date, with the latest released in September 2018.⁸⁸
- **Flaym:** an online community and mobile app for psoriasis sufferers with over 5,000 members currently registered. It allows those with psoriasis to share experiences on living with their condition.⁸⁹
- **HelloSkin:** an ecommerce platform designed to provide patients with skin conditions such as psoriasis, eczema, acne and dry skin with transparent information about the most effective ingredients for their condition, and access the best skin products on the market to treat their conditions. The app allows patients to shop by need, type and ingredient, and the information and advice provided through the app are vetted by medical experts.⁹⁰
- **Klikkit:** an Internet of Things tracking and monitoring system, designed to help patients follow treatment plans. The Klikkit platform is built from a smartphone app (available on both iOS and Android), a range of attachable smart buttons, and a remote monitoring Klikkit dashboard. The platform aims to help users overcome challenges such as forgetfulness or the fear of side effects. It also enables payers and providers to gain real-time insights on adherence to medication and remote interventions on patient populations.

Moreover, pharma should build stronger relationships with payer authorities in order to move away from transaction based interactions to ones that are based on long term partnerships, which promote collaboration and improve patient access to medicines. To be successful, this requires increased transparency between pharma and payers that clearly outlines the needs and constraints of both organisations.

**Build the skills and expertise needed for the future:
Consider the skills gap you have between technical and communicative expertise**

As the volume of health data grows exponentially, the success of pharma's commercial activities will require new and enhanced skills and capabilities and embrace new ways of working. Specifically, pharma needs to develop capabilities in collecting, storing and analysing patient data and use this information to develop solutions tailored to patient needs.

To access the necessary skills and talent will require pharma companies to partner with others to jointly create and demonstrate value across a spectrum of areas, from deep technical expertise to communication and advocacy (See Figure 18). Pharma should also consider whether capabilities can be carried out internally, locally or centrally and which must be outsourced to partner organisations.

The success of pharma market access strategies will be contingent on the development and deployment of multi-disciplinary teams that engage early in the R&D process and have deep technical, problem solving, advocacy and communicative expertise to tackle country specific health care challenges.

On the technical side, the generation, collection, processing and analysis of RWE to generate payer insights requires:

- analytical skills such as data cleansing, data analytics, machine learning and AI in order to capture, analyse and generate insights in real-time that can be used to support individual or groups of products, and support patients, health care payers and providers
- health economics and actuarial population modelling skills to enable more constructive discussions with HTA and payer authorities, design solutions for particular populations, and develop risk-based innovative contracts.

On the advocacy and communication side, deep listening skills, empathy and design skills are needed to:

- listen to payer concerns, and generate, identify and capture health care insights on a European and country-specific level, and understand therapeutic alignment to an organisation's expertise
- design evidence-based contracting strategies and market access options tailored to the specific needs of European payers
- scenario plan and progress through pricing negotiations to create win-win solutions
- work and engage effectively with a broader range of stakeholders, such as service and technology providers, to collect evidence to understand key payer challenges, and develop and deliver solutions that meet patient and system needs.

Realising these core capabilities will allow pharma to better define the strategic, tactical, technical and societal steps their organisations should take to ensure value is delivered across the life cycle of their products.

As the volume of health data grows exponentially, the success of pharma's commercial activities will require new and enhanced skills and capabilities and embrace new ways of working.

Figure 18: A value map to improve pharma market access and pricing strategies



Source: Deloitte LLP, 2019

Abbreviations

AIFA: Italian Medicines Agency (Agenzia Italiana del Farmaco)

ANSM: French National Agency for Medicines and Health Products Safety (Agence Nationale de Sécurité du Médicament et des Produits de Santé)

ATU: Temporary Authorisation for Use (France)

BfArM: The Federal Institute for Drugs and Medical Devices (Bundesinstitut für Arzneimittel und Medizinprodukte)

EMA: European Medicines Agency

FAGG: The Federal Agency for Medicines and Health Products (Federaal Agentschap voor Geneesmiddelen en Gezondheidsproducten)

G-BA: The Federal Joint Committee (Der Gemeinsame Bundesausschus)

HAS: The High Health Authority (La Haute Autorité de santé)

MAPPs: Medicines Adaptive Pathways to Patients

MBE: Medicines Evaluation Board

MHRA: The Medicines and Healthcare products Regulatory Agency

MPA: The Medical Products Agency

NICE: National Institute for Health and Care Excellence

QUALY: Quality-adjusted life year

RIZIV-INAMI: National Institute for Sickness and Disability Insurance (Institut national d'assurance maladie-invalidité)

SEED: Shaping European Early Dialogues for health technologies

TVL: The Dental and Pharmaceutical Benefits Agency (Tandvårds- och läkemedelsförmånsverket)

ZIN: National Health Care Institute (Zorginstituut Nederland)

Appendix

Within this report, when the average of 16 European countries is discussed, the countries included are Austria, Belgium, Finland, France, Germany, Greece, Ireland, Italy, Netherlands, Norway, Poland, Portugal, Spain, Sweden, Switzerland and the UK. For the European average relating to pharmaceutical expenditure as a percentage of GDP in 2010, data from 2013 was selected for the UK as it was the earliest available year.

Endnotes

1. World industry outlook: Health care and Pharmaceuticals, The Economist Intelligence Unit, 2017. See also: [http://www.eiu.com/industry/Health care](http://www.eiu.com/industry/Health%20care)
2. Globocan 2018: Estimated Cancer Incidence, Estimated number of incident cases, Globocan, 2018. See also: http://globocan.iarc.fr/Pages/burden_sel.aspx
3. International Diabetes Federation. IDF Diabetes Atlas, 8th edn. Brussels, Belgium: International Diabetes Federation, 2017. See also: <http://www.diabetesatlas.org>.
4. Health spending, OECD, 2018. See also: <https://data.oecd.org/healthres/health-spending.htm#indicator-chart>
5. Pharmaceutical spending, OECD, 2018. See also: <https://data.oecd.org/healthres/pharmaceutical-spending.htm>
6. The Imperative of Addressing Cancer Drug Costs and Value, National Cancer Institute, 2018. See also: <https://www.cancer.gov/news-events/cancer-currents-blog/2018/presidents-cancer-panel-drug-prices>
7. Pharmaceutical Price Regulation Scheme (PPRS) 2014: payment percentage for 2018, Department of Health, 2017. See also: https://assets.publishing.service.gov.uk/government/uploads/system/uploads/attachment_data/file/668673/PPRS_Payment_percentage_2018.pdf
8. Price reduction according to the 15-year rule, TLV, 2017. See also: <https://www.tlv.se/lakemedel/prissankning-enligt-15-arsregeln.html>
9. Changes to NICE drug appraisals: what you need to know, NICE, 2017. See also: <https://www.nice.org.uk/news/feature/changes-to-nice-drug-appraisals-what-you-need-to-know>
10. Market access in Europe: balancing access and affordability, PMLive, 2017. See also: http://www.pmlive.com/pharma_intelligence/market_access_in_europe_balancing_access_and_affordability_1184275?SQ_DESIGN_NAME=2
11. Commercial Medicines Unit – move to NHS England, NHS Procurement, 2017. See also: <https://nhsprocurement.org.uk/commercial-medicines-unit-move-to-nhs-england/>
12. Commercial Medicines Unit (CMU), UK Government, 2011. See also: <https://www.gov.uk/government/collections/commercial-medicines-unit-cmu>
13. BeNeLuxA collaboration, Beneluxa, 2018. See also: <http://www.beneluxa.org/collaboration>
14. Visegrad Bulletin 5, Vicegrad Group, 2017. See also: <http://www.visegradgroup.eu/visegrad-bulletin-5-2>
15. Sarantis Michalopoulos, Euactive, 2018. See also: <https://www.euractiv.com/section/health-consumers/news/eu-southern-alliance-on-drug-pricing-expands/>
16. Press release - La Valletta Declaration Committee met in Lisbon, Infarmed, 2018. See also: http://www.infarmed.pt/web/infarmed-en/home/-/journal_content/56/281/2614977
17. EvaluatePharma Orphan Drug report, Evaluate Ltd, 2018. See also: <http://www.evaluategroup.com/public/Reports/EvaluatePharma-Orphan-Drug-Report-2018.aspx>
18. Ibid
19. EvaluatePharma World Preview 2018, Outlook to 2024, Evaluate Ltd, 2018. See also: <http://www.evaluategroup.com/public/Reports/EvaluatePharma-World-Preview-2018.aspx>
20. Rare diseases. Orphan drug market, Pharma World, 2018. See also: <http://www.pharmaworldmagazine.com/rare-diseases-orphan-drug-market/>
21. Will Biologics Surpass Small Molecules In The Pharma Race? BiopharmaTrend, 2018. See also: <https://www.biopharmatrend.com/post/67-will-small-molecules-sustain-pharmaceutical-race-with-biologics/>
22. A new future for R&D? Measuring the return from pharmaceutical innovation 2017, Deloitte, 2017. See also: <https://www2.deloitte.com/uk/en/pages/life-sciences-and-health-care/articles/measuring-return-from-pharmaceutical-innovation.html>
23. Biopharmaceutical Research & Development: The Process behind New Medicines, PhRMA, 2015. See also: http://phrma-docs.phrma.org/sites/default/files/pdf/rd_brochure_022307.pdf
24. The Economics of Precision Medicine, International Risk Governance Center, EPFL, Lausanne, 2018. See also: [https://irgc.epfl.ch/files/content/sites/irgc/files/Publications/IRGC%20\(2018\).%20The%20economics%20of%20precision%20medicine.%20Workshop%20report.pdf](https://irgc.epfl.ch/files/content/sites/irgc/files/Publications/IRGC%20(2018).%20The%20economics%20of%20precision%20medicine.%20Workshop%20report.pdf)
25. Biologics & Biosimilars, PhRMA. See also: <https://www.phrma.org/advocacy/research-development/biologics-biosimilars>
26. Realizing the Promise of Biologics, Harvard Health Policy Review, 2017 (Volume 17, Issue 1). See also: <http://www.hhpronline.org/articles/2017/4/9/realizing-the-promise-of-biologics>
27. Biologics: Driving Force in Pharma, Pharma's Almanac, 2017. See also: <https://www.pharmasalmanac.com/articles/biologics-driving-force-in-pharma>
28. Pharma R&D Annual Review 2018, Pharmaprojects® | Informa, 2018. See also: <https://pharmaintelligence.informa.com/resources/product-content/pharma-rd-annual-review-2018>
29. AbbVie's Humira Revenue Up 15% (Despite Patent Cliff Fears, Seeking Alpha, 2017. See also: <https://seekingalpha.com/article/4066896-abbvies-humira-revenue-15-percent-despite-patent-cliff-fears>
30. Drantisaris et.al., Drug tendering: drug supply and shortage implications for the uptake of biosimilars, 2017. See also: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5628685/>
31. Biosimilars action plan: Balancing Innovation and Competition. FDA, July 2018. See also: <https://www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/TherapeuticBiologicApplications/Biosimilars/UCM613761.pdf>
32. Guideline on similar biological medicinal, European Medicines Agency, 2005 (2014 revision). See also: http://www.ema.europa.eu/docs/en_GB/document_library/Scientific_guideline/2014/10/WC500176768.pdf
33. European public assessment reports, European Medicines Agency, 2018. See also: [https://www.ema.europa.eu/en/medicines/download-medicine-data#european-public-assessment-reports-\(epar\)-section](https://www.ema.europa.eu/en/medicines/download-medicine-data#european-public-assessment-reports-(epar)-section)

34. Biosimilar Product Information, FDA, 2018. See also: <https://www.fda.gov/drugs/developmentapprovalprocess/howdrugsaredevelopedandapproved/approvalapplications/therapeuticbiologicapplications/biosimilars/ucm580432.htm>
35. Drantisaris et.al., Drug tendering: drug supply and shortage implications for the uptake of biosimilars, 2017. See also: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5628685/>
36. New price cap for medicinal products, International Law Office, 2017. See also: <https://www.internationallawoffice.com/Newsletters/Health-care-Life-Sciences/Austria/Preslmayr-Attorneys-at-Law/New-price-cap-for-medicinal-products>
37. Commissioning framework for biological medicines (including biosimilar medicines), NHS England, 2017. See also: <https://www.england.nhs.uk/wp-content/uploads/2017/09/biosimilar-medicines-commissioning-framework.pdf>
38. Reprint with permission. GaBI Online – Generics and Biosimilars Initiative. France aims to reach 80% biosimilar penetration by 2022. [www.gabionline.net]. Mol, Belgium: Pro Pharma Communications International; [cited 2018 Sep 28]. Available from: HYPERLINK "http://www.gabionline.net/Policies-Legislation/France-aims-to-reach-80-biosimilar-penetration-by-2022" www.gabionline.net/Policies-Legislation/France-aims-to-reach-80-biosimilar-penetration-by-2022
39. EFPIA Market Access Delays Analysis, EFPIA, 2018. See also: <https://efpia.eu/media/412416/market-access-delays-2017-final-140318.pdf>
40. Patients W.A.I.T Indicator, EFPIA, 2010. See also: <https://www.efpia.eu/media/15510/patients-wait-indicator-report-2010.pdf>
41. Fast Track, Breakthrough Therapy, Accelerated Approval, Priority Review, FDA, 2018. See also: <https://www.fda.gov/forpatients/approvals/fast/default.htm>
42. Accelerated assessment, EMA, 2018. See also: http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000955.jsp&mid=WC0b01ac05809f843a
43. Regulatory Update from MHLW/PMDA, PMDA, 2017. See also: <https://www.pmda.go.jp/files/000221880.pdf>
44. PMDA Puts Regulatory Approval on a Fast Track, Global Forum, 2018. See also: <https://globalforum.diaglobal.org/issue/may-2018/pmda-puts-regulatory-approval-on-a-fast-track/>
45. Deloitte analysis of EMA Annual reports 2014, 2015, 2016 and 2017
46. Frequently Asked Questions: Breakthrough Therapies, FDA, 2018. See also: <https://www.fda.gov/RegulatoryInformation/LawsEnforcedbyFDA/SignificantAmendmentsstotheFDCA/FDASIA/ucm341027.htm>
47. Performance Based Risk Sharing Database (PBRs). The Comparative Health Outcomes, Policy, and Economics (CHOICE) Institute, University of Washington School of Pharmacy, August 2018 See also: <http://depts.washington.edu/pbrs/>
48. Value-based Health care: A Global Assessment, Economist Intelligence Unit (Commissioned by Medtronic), 2016. See also: <http://vbhcglobalassessment.eiu.com/>
49. Integrated Health Solutions, Medtronic, 2018. See also: <http://www.medtronic.com/us-en/health-care-professionals/services/integrated-health-solutions.html>
50. Sunitinib for the first-line treatment of advanced and/or metastatic renal cell carcinoma, NICE, 2009. See also: <https://www.nice.org.uk/guidance/ta169/chapter/3-the-technology>
51. Adamski, Jakub & Godman, Brian & Ofierska-Sujkowska, Gabriella & Osirńska, Bogusława & Herholz, Harald & Wendykowska, Kamila & Laius, Ott & Jan, Saira & Sermet, Catherine & Corinne, Zara & Kalaba, Marija & Gustafsson, Roland & Garuoliene, Kristina & Haycox, Alan & Garattini, Silvio & Gustafsson, Lars. (2010). Risk sharing arrangements for pharmaceuticals: potential considerations and recommendations for European payers. BMC Health Service Research. 10:153. 10.1186/1472-6963-10-153. See also: <https://www.researchgate.net/publication/202314971/download>
52. Clopes et al, Financial consequences of a payment-by-results scheme in Catalonia: gefitinib in advanced EGFR-mutation positive non-small-cell lung cancer, Journal of Medical Economics, 2016. See also: <https://www.tandfonline.com/doi/full/10.1080/13696998.2016.1215991>
53. Harvard Pilgrim signs outcomes-based contract with AstraZeneca for Symbicort, Harvard Pilgrim Health care, 2018. See also: https://www.harvardpilgrim.org/public/news-detail?nt=HPH_News_C&nid=1471916509161
54. Alnylam Announces Alignment on Value-Based Agreements with Leading Health Insurers and Launches Comprehensive Patient Support Services for ONPATTRO™ (patisiran), Alnylam, 2018. See also: <http://investors.alnylam.com/news-releases/news-release-details/alnylam-announces-alignment-value-based-agreements-leading>
55. Flume M et.al, Feasibility and attractiveness of indication value-based pricing in key EU countries, 2016. See also: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4864834/>
56. Medtech and the Internet of Medical Things: How connected medical devices are transforming medtech, Deloitte, 2018. See also: <https://www2.deloitte.com/content/dam/Deloitte/global/Documents/Life-Sciences-Health-Care/gx-lshc-medtech-iiomt-brochure.pdf>
57. Self-reported use of prescribed medicines by age, Eurostat, 2017. See also: [https://ec.europa.eu/eurostat/statistics-explained/index.php?title=File:Self-reported_use_of_prescribed_medicines_by_age_2014_\(%25\).png](https://ec.europa.eu/eurostat/statistics-explained/index.php?title=File:Self-reported_use_of_prescribed_medicines_by_age_2014_(%25).png)
58. Louisiana seeking 'subscription model' for hepatitis C drugs, AP News, 2018. See also: <https://apnews.com/50d4713d213640318d71b12c5173f41a/Louisiana-seeking-'subscription-model'-for-hepatitis-C-drugs>
59. Annual Report 2017, European Medicines Agency, 2017. See also: http://www.ema.europa.eu/docs/en_GB/document_library/Annual_report/2018/04/WC500248201.pdf
60. About EUnetHTA, EUnetHTA, 2018. See also: <https://www.eunethta.eu/about-eunethta/>
61. Early Dialogues, EUnetHTA, 2018. See also: <https://www.eunethta.eu/services/early-dialogues/>
62. Ibid
63. Multi Stakeholder: Late & Early Dialogue, European Medicines Agency, 2017. See also: http://www.ema.europa.eu/docs/en_GB/document_library/Presentation/2017/10/WC500236324.pdf
64. Balasubramanian et.al, An overview of Compassionate Use Programs in the European Union member states, 2016. See also: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5116859/>

65. Early Access to Medicines Scheme (EAMS), ABPI. See also: <https://www.abpi.org.uk/what-we-do/collaboration-and-partnership/value-and-access/early-access-to-medicines-scheme-eams/>
66. Compassionate Use Programmes, Federal Institute for Frugs and Medicinal Products, 2018. See also: https://www.bfarm.de/EN/Drugs/licensing/clinicalTrials/compUse/_node.html
67. Early Access Programs in Europe: Differences and Opportunities in Germany, France and the UK, Health Advances Blog, 2018. See also: <https://healthadvancesblog.com/2018/06/07/early-access-programs-in-europe-differences-and-opportunities-in-germany-france-and-the-uk/>
68. Early Access to Medicines In Europe: How to make compassionate use become a reality for all in need, Eurordis, 2017. See also: http://www.ema.europa.eu/docs/en_GB/document_library/Presentation/2017/10/WC500237756.pdf
69. Pharma and the connected patient: How digital technology is enabling patient centricity. Deloitte, 2017. See also: <https://www2.deloitte.com/content/dam/Deloitte/global/Documents/Life-Sciences-Health-Care/gx-lshc-pharma-and-connected-patient.pdf>
70. Our Digital Therapeutics, Voluntas, 2017. See also: <http://www.voluntas.com/>
71. Ibid
72. The Diabeo Software Enabling Individualized Insulin Dose Adjustments Combined With Telemedicine Support Improves HbA1c in Poorly Controlled Type 1 Diabetic Patients, Charpentier G et al, Diabetes Care, 2011. See also: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3041176/>
73. Charpentier G et al, The Diabeo Software Enabling Individualized Insulin Dose Adjustments Combined With Telemedicine Support Improves HbA1c in Poorly Controlled Type 1 Diabetic Patients, Diabetes Care, 2011. See also: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3041176/>
74. Theraxium Oncology, Voluntas, 2018. See also: <http://www.voluntas.com/oncology/>
75. Pfizer to Accelerate Research with Watson for Drug Discovery, IBM. See also: <https://www.ibm.com/products/watson-drug-discovery/features>
76. Leicester's ePIFFany project successfully reduces junior doctor prescribing errors, University of Leicester, 2014. See also: <https://www2.le.ac.uk/offices/press/press-releases/2014/may/leicester2019s-epiffany-project-successfully-reduces-junior-doctor-prescribing-errors>
77. Joint Working: EPIFFANY –Preparation for National Upscale, Pfizer, 2017. See also: <https://www.pfizer.co.uk/joint-working-epiffany-preparation-national-upscale#2>
78. EPIFFANY (Effective Performance Insight for the Future) education programme, The AHSN Network, 2017. See also: <http://atlas.ahsnnetwork.com/epiffany-effective-performance-insight-for-the-future-education-programme/>
79. Collier S et.al, Monitoring safety in a phase III real-world effectiveness trial: use of novel methodology in the Salford Lung Study, 2016. See also: <https://onlinelibrary.wiley.com/doi/full/10.1002/pds.4118>
80. GSK's real world study exemplifies trial design of the future, Center Watch, 2017. See also: <https://www.centerwatch.com/news-online/2017/03/06/gsk-real-world-study-exemplifies-trial-design-future/>
81. GSK announce positive results from the COPD Salford Lung Study published in the NEJM and presented at European Respiratory Congress, GlaxoSmithKline(GSK), 2016. See also: <https://www.gsk.com/en-gb/media/press-releases/gsk-announce-positive-results-from-the-copd-salford-lung-study-published-in-the-nejm-and-presented-at-european-respiratory-congress/>
82. UK patient groups say that pharma should do more on being transparent, PatientView, 2018. See also: <http://createsend.com/t/j-2EB334546F8C1B6C2540EF23F30FEDED>
83. Pharma and the connected patient: Deloitte, July 2017, see also: <https://www2.deloitte.com/content/dam/Deloitte/uk/Documents/life-sciences-health-care/deloitte-uk-pharma-and-the-connected-patient.pdf>
84. Leo Innovation Lab, About, 2017. See also: <https://leoinnovationlab.com/about/>
85. Ibid
86. How it works, Studies&Me, 2018. See also: <https://studiesandme.com/how-it-works>
87. Psohappy, 2018. See also: <https://www.psohappy.org/>
88. Psohappy, January Blues. See also: <https://www.psohappy.org/january-blues>
89. Flaym, Feature, 2018. See also: <https://www.flaym.io/features>
90. About HelloSkin, HelloSkin, 2018. See also: <https://helloskin.co.uk/>

Contacts

Authors

Elizabeth Hampson

Director, Monitor Deloitte
Tel: +44 20 7007 8169
Email: ehampson@deloitte.co.uk

Karen Taylor

Director, UK Centre for Health Solutions
Tel: +44 (0) 20 7007 3680
Email: kartaylor@deloitte.co.uk

Amen Sanghera

Analyst, UK Centre for Health Solutions
Tel: +44 20 7007 4559
Email: asanghera@deloitte.co.uk

Contacts

Thomas Croisier

Partner, Life Sciences and Health Care, Monitor Deloitte, France
Tel: +33 1 58 37 92 87
Email: tcroisier@deloitte.fr

John Haughey

UK and NWE Life Sciences and Health Care Leader
Tel: +44 20 7303 7472
Email: jhaughey@deloitte.co.uk

Dr. Gregor Elbel

Partner, Life Sciences and Health Care, Deloitte, Germany
Tel: +44 (0) 20 7007 2540
Email: gelbel@deloitte.co.uk

Alexander Mirow

Partner, Life Sciences and Health Care, Monitor Deloitte, France
Tel: +33 1 58 37 96 09
Email: amirow@deloitte.fr

Gonzalo Casino Fernandez

Director, Deloitte Consulting, Spain
Tel: +34 932533695
Email: gcasinofernandez@deloitte.es

Hanno Ronte

Partner, Life Sciences and Health Care, Monitor Deloitte, UK
Tel: +44 (0) 20 7007 2540
Email: hronte@deloitte.co.uk

Mike Standing

EMEA Life Sciences and Health Care Leader
Tel: +44 20 7007 3178
Email: mstanding@deloitte.co.uk

Colin Terry

Partner, Life Sciences R&D Advisory, Deloitte MCS Ltd, UK
Tel: +44 20 7007 0658
Email: colterry@deloitte.co.uk

Elisabeth Aloy

Director, Life Sciences and Health Care, Monitor Deloitte, Switzerland
Tel: +41 58 279 6831
Email: elialoy@deloitte.ch

Federico Rigato

Senior Manager, Deloitte Consulting, Italy
Tel: +39 0283323218
Email: frigato@deloitte.it

Acknowledgements

Ushma Soneji, Emma Dabbs and Juan Salcines Gomez-Pardo of Monitor Deloitte, and Mark Steedman, Francesca Properzi, Matthew Thaxter and Pratik Avhad of the Deloitte Centre for Health Solutions UK. Special thanks to the valuable contributions provided by Deloitte practitioners and teams across the world.

Contact information

Deloitte UK Centre for Health Solutions, 1 New Street Square, London EC4A 3HQ



Important notice

This document has been prepared by Deloitte LLP for the sole purpose of enabling the parties to whom it is addressed to evaluate the capabilities of Deloitte LLP to supply the proposed services.

Other than as stated below, this document and its contents are confidential and prepared solely for your information, and may not be reproduced, redistributed or passed on to any other person in whole or in part. If this document contains details of an arrangement that could result in a tax or National Insurance saving, no such conditions of confidentiality apply to the details of that arrangement (for example, for the purpose of discussion with tax authorities). No other party is entitled to rely on this document for any purpose whatsoever and we accept no liability to any other party who is shown or obtains access to this document.

This document is not an offer and is not intended to be contractually binding. Should this proposal be acceptable to you, and following the conclusion of our internal acceptance procedures, we would be pleased to discuss terms and conditions with you prior to our appointment.

Deloitte LLP is a limited liability partnership registered in England and Wales with registered number OC303675 and its registered office at 1 New Street Square, London EC4A 3HQ, United Kingdom.

Deloitte LLP is the United Kingdom affiliate of Deloitte NWE LLP, a member firm of Deloitte Touche Tohmatsu Limited, a UK private company limited by guarantee ("DTTL"). DTTL and each of its member firms are legally separate and independent entities. DTTL and Deloitte NWE LLP do not provide services to clients. Please see www.deloitte.com/about to learn more about our global network of member firms.

© 2019 Deloitte LLP. All rights reserved.

Designed and produced by The Creative Studio at Deloitte, London. J16678