Chapter 1

New health technologies: Managing access, value and sustainability

by
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This chapter presents an overview of the analytical report prepared by the OECD Secretariat for the 2017 Health Ministerial on "New Health Technologies: Managing Access, Value and Sustainability". The report discusses the need for an integrated and cyclical approach to managing health technology to mitigate clinical and financial risks and to ensure acceptable value for money. This synthesis chapter considers how health care systems and policy makers should adapt in terms of the development, assessment and uptake of health technologies. Following a brief examination of the past adoption and impact of medical technology, this synthesis chapter focuses on opportunities linked to new and emerging technologies as well as current challenges faced by policy makers. It concludes with a suggested new governance framework to address these challenges.

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Introduction

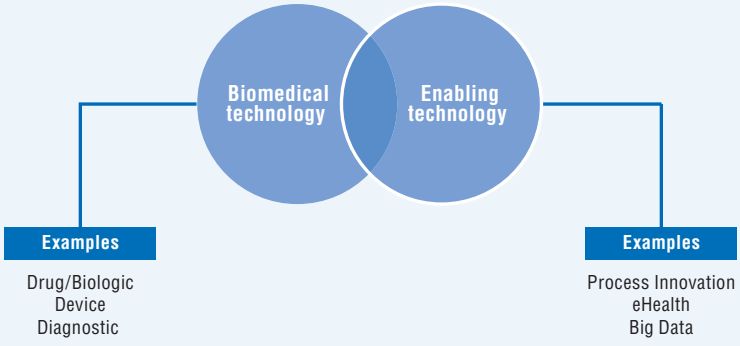
Technology has profoundly affected the way medicine is practised and health care delivered. Thanks in large part to innovations in medical technology, modern health service is virtually unrecognisable from a few decades ago. While technology has delivered undisputable benefits to human health, however, it has done so at considerable cost. As such, the value – the health benefits compared to the costs[1] – of health technology is often called into question. Seen in these terms, not all technology, new or existing, may be worth the expenditure.

The health technology landscape is continually changing, with innovation moving in new directions: artificial intelligence, remote sensors, robotics, 3D printing, "Big Data", genomics, stem cells and more (Box 1.1). Introduction of these new technologies into health care systems sometimes represents disruptive changes in processes, relationships and resourcing. In a context of limited resources as well as rising public expectations for effective and affordable health care, policy makers must think pro-actively about the potential impact of new technology on sustainability, health gains and costs. Changing

Box 1.1. **Health technology – a basic taxonomy**

Health technology and innovation is defined as the application of knowledge to solve practical clinical and health problems, including products, procedures and practice styles that alter the way health care is delivered. Such a definition includes biomedical technology – such as medicines, medical devices and diagnostics (Dx) – as well as enabling technology such as mobile health (mHealth) and "Big Data". The definition also includes innovations in processes and care delivery. Process innovation is addressed in this report when it is a product of, or related to, the development and introduction of other types of technology. For example, single-day surgical procedures were enabled through development of medical equipment that permitted minimally invasive access to internal bodily structures, while digital technology has driven process redesign across all care settings.

Figure 1.1. **Health technology – a basic taxonomy**



market dynamics for health technology necessitate new regulatory models and incentives. Existing institutions, regulatory pathways and reimbursement systems may no longer be fit for purpose.

This report considers how health care systems and policy makers should adapt in terms of the development, assessment and uptake of health technologies. The ultimate objective of health policy is to improve population health, often under budget constraints. To act towards this objective, policy makers need to:

- encourage development and adoption of technologies that help improve population health,
- ensure equitable access to these technologies, and
- promote the sustainability of health care systems.

This implies that technologies should be delivered at a price that offers value for money and is affordable. These principles guide the discussion and recommendations of this report.

Following a brief examination of past adoption and impact of medical technology, this synthesis chapter focuses on opportunities linked to new and emerging technologies as well as current challenges faced by policy makers. The chapter then suggests a new framework to address these challenges. The overarching theme is the need for an integrated and cyclical approach to managing health technology to mitigate clinical and financial risks and ensure acceptable value.

1. Impact of health technologies on health and health spending: Lessons from the past

The past provides some lessons for the development of policies to harness both emerging and existing technologies to achieve the objectives listed above. Progress in medical science has resulted in major advances in society's understanding of disease and its ability to develop and improve treatments. Numerous examples exist of immense health benefits derived from medical technology. While the costs of these innovations vary, most have delivered a decent return on the resources invested in their development and use (i.e. value). But some innovations have delivered little or no health benefit (but incurred considerable costs) and some were even harmful.[2]

Technology has influenced how health care is delivered in many ways: by expanding the number of treatable conditions and patient types; by substituting for existing interventions or targeting them more accurately; by intensifying the level of treatment for given conditions; and by changing processes of care delivery. The diffusion of health technology in concert with other factors such as income levels, reimbursement systems, medical culture and demographic change – has been a strong driver of the remarkable rise in health care expenditure in OECD countries since the mid-20th century. Depending on the approach used, attempts to estimate the direct impact of health technology on expenditure range from one-fifth to as high as 70% (Chernew and Newhouse, 2012). Given the differences between health care systems and the incentives they provide to actors and stakeholders, no single figure can be applied across all health systems. However, given the rising share of national income spent on health care across OECD countries, any point within the range of estimates is likely to be considerable. As health spending invariably displaces other areas of expenditure that also generate welfare, such as education, housing and infrastructure, the opportunity cost of expenditure driven by the adoption of health technology must be considered.

Based on research focusing on a subset of high-impact illnesses such as cardio-vascular diseases (CVD), cancer and infectious diseases in the United States, the additional cost of introducing technology in the past appears to have delivered acceptable levels of value and can therefore be deemed "worth it". Overall, the resources devoted to the development and application of health technology have yielded satisfactory results, generally measured through longevity gains and survival. However, this research is constrained by: 1) assumptions around attributing the health effect of the technologies examined against other, non-medical factors influencing human health; and 2) the absence of quality data on patient and population health outcomes extending beyond mortality into dimensions such as quality of life and function. Nevertheless, recognition is growing that in more recent decades, the escalating expenditure on technology-enabled therapies may not be matched by commensurate health gains. The cost-benefit function may be trending towards unfavourable territory, suggesting that a more prudent approach to implementation and adoption of technology is required in the future.

The impact of technology on patients, populations and health care systems is highly variable depending on the technology, its application, the disease or patient group, and the context in which it is used. Seen through the lens of value, health technology can be grouped into three types (Chandra and Skinner, 2008, 2012). The first type is technology that is effective in achieving its therapeutic aim and delivers high value. Cheap, "low-tech" technologies that can be broadly applied across populations feature strongly in this group. Costly interventions can also deliver considerable value if they are effective and their target population is clearly defined. Well-defined indication is a common characteristic of the costlier technologies of this type. Examples include the aseptic technique, vaccines, beta-blockers combined with aspirin, and antiretroviral treatment for HIV.

The second type includes technologies that, while effective in some indications, are prone to expanding their application across a population and to cases where their clinical utility is diminished. The decreasing marginal benefit dilutes the value derived from these technologies. Many diagnostic technologies (e.g. radiology and endoscopy) feature in this category. Cardiac catheterisation and angioplasty are other examples of a medical technology proven to benefit a certain category of patient, but whose application crept into patient types that could be better managed in other, often more conservative and less costly ways. Considerable geographic variation in the use of these technologies is often observed, partly driven by factors other than population health need. This is one of the reasons why even technologies that are cost-saving at individual level end up having an expansionary effect on aggregate expenditure: they are eventually applied to cases where they produce little benefit, thus undermining value.

The final type comprises technologies for which evidence of therapeutic benefit is weak or non-existent, and that are clinically equivalent to "watchful waiting" or less complex, conservative interventions. Many such interventions are costly in financial terms as well in the clinical risk posed by iatrogenic harm. They include some spinal surgery, a range of diagnostics such as liver function testing, and devices such as those that measure pulmonary artery pressure. Remarkably, provision (and reimbursement) of these interventions continues, despite decades of evidence for their lack of effectiveness in some cases.

The past indicates that the value of health care technology is undermined by its suboptimal and inappropriate application, diffusion and implementation. Similar benefit at lower cost could be generated from the therapeutic arsenal at society's disposal if more appropriate use was encouraged. Chapter 2 provides a number of examples. For example,

wide variation in admissions to intensive care is observed, with little effect on clinical outcomes but a considerable inflation of costs. Aggressive medical interventions at the end of life can impose great financial costs with not only little benefit but – in many documented cases – disutility and suffering for patients and loved ones. Another example is antimicrobial resistance (AMR), to a large extent the result of unfettered application of the "miraculous" technology of antibiotics. Had more effort been made to ensure appropriate and prudent use of this technology – in both human and agricultural domains – the world would now perhaps not be facing the considerable cost of AMR.

The lesson for the future is that technology must be developed and applied intelligently, in a way that is based on evidence and with health benefits for individuals and populations the principal objective. The right policy settings can help maximise value derived from health technology. This will be critically important to ensure the financial and institutional sustainability of health care systems as more complex – and potentially costly – technology comes on stream in the next few years and decades. Enabling technology such as ICT (information and communications technology) is urgently needed to collect and provide better information for more rational deployment of treatment, interventions and health care system resources more generally.

2. Promises and challenges of new and emerging technologies

The flow of new technologies comes with many promises of future benefits for patients but also a number of challenges for policy makers. Some technologies blur the traditional frontier between medicines and medical devices or integrate digital technologies, requiring new regulatory pathways. Some are marketed at very high prices, impairing access to treatment and threatening the sustainability of current financing models.

2.1. New types of technologies challenge regulatory pathways

In the past, medical technologies were distinct from one another and used at discrete points of the care pathway. Today, technology categories increasingly converge in ways that profoundly alter the delivery of health care. Many of these technologies challenge regulatory systems, which traditionally address a single type of technology (medicines, medical devices).

Treatments are increasingly tailored to individual patients

Precision medicine (PM) holds the potential to radically transform medicine. Current research initiatives in this field are increasing the medical community's knowledge and capacity to predict, prevent and treat diseases (Box 1.2). So far, PM has mainly found concrete applications in the development of personalised or stratified medicines, which provide safer and more effective treatments to patients.

PM challenges regulatory pathways in many ways. First, new designs of clinical trials are tested out. In oncology for instance, trials where patients' treatment is selected according to the molecular characteristics of their tumour sometimes replace the traditional randomised controlled trial (RCT), which compare a treatment to a placebo. These trials have so far produced heterogeneous results, which suggests that prospective studies are still needed. In some cases, target populations are very small, trials cannot recruit hundreds of patients, and results must be inferred from very small samples. In addition, personalised medicines often target severely debilitating or life-threatening conditions for which no treatment is available. As a result, regulators are often under pressure to provide quick access to these medicines.

Box 1.2. **Precision medicine: some definitions**

Precision medicine (PM) is defined by the United Kingdom's Programme Coordination Group as "[refining] our understanding of disease prediction and risk, onset and progression in patients, informing better selection and development of evidence-based targeted therapies and associated diagnostics. Disease treatment and other interventions are better targeted to take into account the patient's genomic and other biological characteristics, as well as health status, medications patients are already prescribed and environmental and lifestyle factors" (Innovate UK, 2016). PM holds the potential to radically transform medicine, with a change of paradigm from "a medicine of organs (heart, liver)" to a medicine targeting cells, molecules, genes, etc. As an example, a few decades ago, blood cancers were grouped in five categories: chronic leukaemia, acute leukaemia, preleukaemia, indolent lymphoma and aggressive lymphoma. Today, medical science recognises 94 types of blood cancers (WHO, 2016), a refinement that contributed to the development of treatments that have improved five-year survival rates from virtually zero to as high as 82% for some subtypes (American Cancer Society, 2016).

Personalised or stratified medicines are pharmaceutical products whose approval is linked to the use of a biomarker[1] diagnostic test to determine the target population. Such a test is used to identify before or during treatment patients who are most likely to benefit from the corresponding medical product or patients likely to be at increased risk of serious adverse reactions. It is essential for the safe and effective use of the product. It is performed with an in vitro *companion diagnostic* device, whose use is stipulated in the instructions for use in the labelling of both the diagnostic device and the corresponding therapeutic product.

While biomarker diagnostics have been thought of so far in terms of "one test – one therapeutic strategy", the landscape is changing with the development of next-generation sequencing (NGS). NGS refers to a number of different modern sequencing technologies to sequence DNA and RNA much more quickly and cheaply than before. *Multiplex tests* – testing several biomarkers at the same time – are also being developed. For instance, three diagnostic tests in breast cancer now allow simultaneous testing for 12, 21 and 70 genes. NGS is expected to become more effective and potentially more cost-effective than current biomarker tests (Bücheler et al., 2014; Van den Bulcke et al., 2015) and may be preferred to individual biomarker tests associated with select treatments.

Whole genome sequencing (WGS – sequencing a person's entire genetic code) and *whole exome sequencing* (WES – limiting investigation to 1% of the genome) are also developing. In contrast with other types of tests, these tests are not designed to capture pre-defined data points (Evans et al., 2015). They can be used for several purposes and may also reveal incidental findings (information that was not sought), including "actionable" information (i.e. information that can be used to prevent or treat a disease). In France, the National Cancer Institute projects that by 2019, single gene tests will be totally replaced by multigene approaches for oncology patients (INCa, 2014).

1. A biomarker is a biological molecule found in blood, other body fluids, or tissues that is a sign of a normal or abnormal process, or of a condition or disease.

While controlled, comparative trials will likely remain the gold standard for pre-market evidence generation, these changes invite the development of new methods to assess the safety and efficacy of new medicines.

Second, as the safety and efficacy of personalised medicines depends on the performance and predictive value of the diagnostic test mentioned in their label, the approval of such medicines needs to take the latter into account. Today, regulatory

requirements for the approval of biomarker diagnostic tests differ across countries but also depend on who develops and performs the test. In Europe and the United States, commercial in vitro diagnostics (IVD) need regulatory approval while laboratory-developed or in-house tests are not subject to the same level of requirements (Garrison and Towse, 2014). Without streamlined regulatory oversight of the quality and performance of all tests, health care systems may in turn struggle to effectively evaluate the costs and benefits of tests coming from varied sources and settings of care.

Finally, the development of multiplex tests and whole genome sequencing in clinical practice will require a number of adaptations to address technical and ethical challenges, such as: How will regulators and Health Technology Assessment (HTA) agencies determine the clinical utility of such diagnostic tools? What sort of patient consent should be sought and who is the owner of the information? Who will be responsible if "actionable" information provided by the test is not used to prevent or treat a disease in a given patient?

Mobile health applications are flooding the market

According to one estimate, more than 165 000 health apps were available in 2015, a figure that has doubled since 2013. These apps perform a constellation of functions: medication reminders, tracking movement and activity, monitoring fertility and progress of pregnancy, and analysing a person's speech to help in the management of mental health problems. Mobile health (mHealth) has the potential to improve health care by: continuous monitoring and timely response; interactions between patients and health professionals beyond traditional settings; and communication with systems that can provide real-time feedback along the care continuum, from prevention to diagnosis, treatment and monitoring. Such potential is welcome at a time of rising prevalence and incidence of chronic diseases and multimorbidity. As people's contact with the health care system shifts from short episodes of acute care to more sustained, long-term monitoring and management that requires a team-based approach, the utility of smartphones and portable devices will rise. In addition, mHealth favours patients' empowerment and engagement in the management of their own conditions. mHealth has the ability to put people at the centre of managing their health, to bring care closer to them, and to connect them with the right information, services and institutions at the right time.

But existing frameworks, processes and institutions are not adequately equipped to address these new technologies. Passive adoption of mHealth will not guarantee success in terms of either clinical outcomes or value for money. Successful integration of mHealth in health care systems requires a number of adaptations: the performance and clinical utility of mobile applications must be assessed for reliable and efficient use in health care, and financial incentives are needed to encourage take-up of mobile applications that are effective and cost-effective. In addition, exchanges of information must be protected by appropriate levels of security, and the expected individual and societal benefits balanced with privacy and security risks. Chapter 4 examines mHealth in more detail.

Combination products increasingly blur the line between drug and device technology

Many emerging medicines are "smart" combinations of drug and device technology. Examples include drugs containing nanotechnology to target tumours or clots, or "digital medicines" that deliver information on patient adherence. The common aim is to improve targeting of treatment with medicines, to enable them to reach the right area of the patient's body, for example, and to improve safety and effectiveness.

Combining the benefits of medicines and medical devices is not without risk. Evaluating such risks and benefits requires specialised expertise, which is why many countries have separate regulatory authorities for each technology type, or separate offices within the same agency. Evaluating evidence on a hybrid product therefore requires additional co-ordination and collaboration within and between health care systems.

Wearable devices and sensors employ digital communication tools

Traditional medical devices such as implantables (e.g. pacemakers) are employing digital communication tools to deliver and/or receive data, for example via a mobile application on patients' or providers' smartphone. Wearable devices and sensors can continuously transmit people's vital signs to their providers in real time, permitting more effective and tailored management of their health problems.

Such technologies combine the existing challenges in regulating medical devices with the emerging regulatory challenges surrounding mHealth, each discussed above. In particular, the performance of digital communication tools is paramount, as is adequate training and monitoring of users (providers and/or patients). This is true for any input to clinical decision making, but has become amplified as such treatment decisions become automated.

"3D printing" of devices is underway and bioprinting is emerging

3D printing is already commonly used in health care (for example, in dental care and joint replacement). 3D printing enables providers to create devices matched to a patient's anatomy, which in turn affects that device's safety and effectiveness. This causes disruption in the supply chain of such products, challenging not only the economic business model of the medical device industry, but also the regulation of these devices.

Issues around 3D bioprinting, currently in development, are even more challenging. 3D bioprinting applications engineer tissue from human cells. The ultimate goal of 3D bioprinting is seen as replacing damaged neurological tissue and entire organs to help meet the growing public health crisis of transplant organ shortages. However, this technology has other potential clinical applications – regenerative scaffolds and bones, bridge to transplant, in situ printing of cells directly onto a wound, or even potential cosmetic applications. While all bioprinted tissue is still currently experimental for human implantation, some tissues are beginning to enter clinical trials. A market is growing for bioprinted tissues to aid in research and development (R&D) – for example, studies of liver toxicity using 3D bioprinted liver tissue could be an eventual replacement for pre-clinical animal testing. This could potentially significantly reduce costs in the R&D process.

Regulatory considerations for 3D printing and bioprinting will largely hinge on the chosen model of dissemination. For example, in the case of 3D bioprinting, a key concern is defining the "product": is it the printer, the bioprinted tissue, or part of a surgical intervention? Most stakeholders expect that the existing regulatory pathway for cell/tissue products will apply, but the level of evidence required, and the detail to which the product is specified, need to be clarified as this technique moves towards human treatment.

2.2. The proliferation of high-cost medicines questions current pricing models

Payers are increasingly confronted with medicines with high price tags requested by manufacturers. Pharmaceutical spending is concentrating on specialty medicines.[3] While

many specialty drugs offer considerable therapeutic value to patients and represent significant improvements over alternative treatment options, they usually have a much higher price than traditional drugs. A treatment for multiple sclerosis, for instance, now costs USD 60 000 per year, about ten times what it cost ten years ago (Hartung et al., 2015). A new gene therapy (Glybera®) entered the German market in 2014 at USD 1 million per cure. Notably, clinicians are refraining from using it because of its cost (Regalado, 2016).

Trends in oncology are particularly worrisome in this regard. The number of approvals for oncology indications is on the rise, with many more oncology drugs in the pipeline, while the prices of oncology treatments are soaring. In Australia for instance, the average reimbursement price per anticancer prescription drug increased by 133% in real terms between 1999 and 2012, while the price of all other prescription drugs increased by only 37%. As similar trends are observed in other OECD countries, the sustainability of current pricing models is questionable.

Trends in the orphan drug[4] market are also a subject of concern. The United States, the European Union, Australia and Japan have implemented policies to encourage development of medicines for rare diseases. These policies are a mix of incentives, such as tax credits on R&D expenditures, extended market exclusivity, regulatory assistance for clinical trials protocols, or reduced user fees for regulatory procedures. These incentives have undoubtedly fostered the development of orphan medicines, which now account for up to half of new molecular entities approved by the US Food and Drug Administration (FDA) every year. Orphan drugs, however, typically enter the market with very high prices, often exceeding USD 100 000. As a result, they are not available to all patients who need them. Among 60 orphan medicines with a marketing authorisation in Europe in 2010, almost all were available in France, the Netherlands and Denmark; two-thirds were available in Belgium, Hungary and Italy; but only one-third were available in Spain and Greece (Eurordis, 2010).

High-cost medicines do not always deliver commensurate health outcomes. The prices of medicines used for very severe conditions and/or diseases with no alternative treatment are too often disconnected from the health benefits they bring to patients. Many of these drugs are not cost-effective, according to standard thresholds.[5] A landmark study looking at 58 oncology medicines approved between 1995 and 2013 in the United States found that the average survival benefit was a little less than six months, while the treatment cost per life year gained – adjusted for inflation – increased by 10% per year (i.e. by USD 8 500 each year) to reach USD 207 000 in 2013. And these costs do not include the costs of other medicines or treatments used in combination nor the costs of dealing with adverse effects (Howard et al., 2015). For orphan medicines, incremental costs per quality-adjusted life year (QALY) gained often exceed USD 100 000 and even EUR 1 million in extreme cases (Schuller et al., 2015).

The approval of new treatments for hepatitis C in 2013 and 2014 raised a novel type of challenge in all OECD countries. These medicines represent a great medical advancement for patients, reaching cure rates of 95% or higher for specific population targets. Despite high prices, these medicines were assessed as cost-effective. However, the immediate budget impact of treating the entire population affected proved to be unaffordable for OECD countries and all payers decided to limit access to the most severely affected patients. For some countries, rationing access to highly effective treatments was a new practice and generated protests from both patients and clinicians. Beyond lack of access, the pricing strategy of the company marketing sofosbuvir (Gilead) raised a number of questions (see Box 1.3).

Box 1.3. **What is wrong with new treatments for hepatitis C?**

Gilead's pricing strategy raised legitimate questions and led to an investigation from the US Senate. Sofosbuvir (Sovaldi®) was initially priced at USD 84 000 for a standard 12-week course of therapy and sofosbuvir/ledipasvir (Harvoni®), launched a few months later by the same company, was priced at USD 94 500. In the United States, these two products contributed to a 12.2% increase in US prescription drug spending in 2014, in spite of access restrictions imposed by all payers. Yet only 2.4% of infected Medicaid beneficiaries got access to these treatments and the situation was even worse in prisons: while one-third of the 2.2 million prisoners are infected by hepatitis C, only 222 of them got access to these treatments in 2015 (Kapczynski and Kesselheim, 2016). In 2015, the list ex-factory price of a 12-week course of sofosbuvir across 26 OECD countries ranged from USD 48 999 in Japan to USD 84 000 in the United States. When adjusted for purchasing power parities, list prices appeared to be particularly high in Poland, Turkey, the United States and the Slovak Republic. By contrast, the lowest list prices were observed in Nordic European countries, Switzerland and the United Kingdom. Treating the entire population in these countries – assuming a 23% rebate in all of them – would cost from 10.6% of total pharmaceutical spending in the Netherlands to more than 150.0% of total pharmaceutical spending in New Zealand or Poland (Iyengar et al., 2016). While the price actually paid in each country is not transparent, treating the whole population would clearly be unaffordable in many countries, even with a 50% discount.

The US Senate report estimates the outlay for research and development for sofosbuvir at between USD 125.6 million and USD 942.4 million (estimates provided by Pharmasset – the initial developer of sofosbuvir – and Gilead, respectively). In return, Gilead earned USD 26.6 billion in the first 21 months of marketing for Sovaldi® (Kapczynski and Kesselheim, 2016), more than 25 times the initial R&D outlay.

Though Gilead made notable efforts to make these treatments available in low-income countries at highly discounted prices, affordability in high- and middle-income countries is a real issue. Even though countries may not want to treat all patients with a drug whose long-term effects are not yet known, current access sounds far too restrictive to doctors and patients. Many stakeholders condemn Gilead and believe that the company could reduce its price to widen access while still earning a sufficient return on investment. Though this reasoning seems at odds with the logic of value-based pricing (the medicine is cost-effective by the usual standard at the proposed price), it holds if one considers that the drug would be even more cost-effective at a lower price and that the total value created would be better shared between the company and society.

Debates on drug pricing mechanisms are flourishing on the international scene. Payers, doctors and patients increasingly question the rationale of companies' pricing strategies, which not only impair access but also do not seem sustainable. Whatever the perspective adopted, be it "fairness" or "value" (for patients and the general public), the outlook is discouraging. Well-meaning stakeholders acknowledge that trust between the pharmaceutical industry and other parts of society needs to be restored and pricing mechanisms revised.

2.3. Health care systems struggle to "pay for value"

As stated earlier, the ultimate objective of health care systems is to improve population health. Policy makers often act towards this objective under a budget constraint, which is

more or less imposed on them. In addition, they are often expected to take into account the interest of the biomedical industry, whose knowledge-based activities are considered a strong economic asset in many OECD countries. This report primarily focuses on health policy. It considers that health policy should: 1) encourage the development and adoption of technologies (products and processes) that help improve population health; 2) ensure equitable access to these technologies; and 3) guarantee the sustainability of health care systems. This implies that technologies should be paid for at a price that offers value for money and is affordable.

Increasing pressure on public health spending, growing demand for health care, and the high pace of innovation require adaptations to the decision-making process to fund new technologies. Basically, societies cannot pay for everything and choices have to be made. If choices are not explicit, they might take the form of local rationing, the arbitrariness of which results in inefficiencies and inequalities. Therefore, policy makers need to ensure that they pay for new technologies that deliver value to patients, health care systems and societies.

Indeed, OECD countries increasingly refer to "value" to make decisions on coverage[6] and financing of health interventions. They increasingly use HTA to inform funding decisions and make public choices explicit. This is not, however, without ambiguity about the meaning of the term "value". In the extra-welfarist approach commonly used in health economics, value can be defined as the health outcomes achieved per dollar spent. In the pharmaceutical sector, for instance, value-based pricing[7] is envisaged as an interesting option to combine static efficiency (paying for good health outcomes today) and dynamic efficiency (providing the right incentives for future innovation). However, value-based pricing has proved difficult to implement in practice. In some market segments, such as oncology or rare diseases, prices are set at very high levels without commensurate benefits (Paris and Belloni, 2013). For medical services, providers' payments usually depend on the amount of resources engaged to produce them, without any reference to value. At best, "outcome-based payments" account for a small fraction of providers' payments (OECD, 2016).

The definition of value is a crucial issue. The underlying questions are: Do decision makers reflect "public preferences" when paying high prices for medicines that are not cost-effective? Is value limited to "health benefits related to incremental costs" or is it more than that? The response to these questions is ambiguous and depends on the perspective adopted (health care system or societal).[8] In the case of orphan medicines for instance, the extent to which the general public supports such decisions – reflecting a higher willingness to pay for patients with rare diseases – is not clear.

Researchers and stakeholders are exploring new methods to make more explicit the criteria and inputs used to determine value. In Europe, a range of stakeholders (payers, industry, experts, etc.) proposed a specific "value framework" to help assess the value of orphan medicines for reimbursement and pricing purposes (MoCA-OMP, 2014). This framework considers four criteria: the availability (or not) of therapeutic alternatives; the clinical effect of the medicine; the response rate; and the degree of uncertainty attached to evaluation. The framework suggests qualitative and quantitative benchmarks to assess the value of orphan medicines. More recent research, not specific to orphan medicines, also explores the possibility of using multicriteria decision analysis (MCDA) to make reimbursement and pricing decisions (Kanavos and Angelis, 2013). Such tools could

potentially contribute to making coverage decisions, and the criteria on which they are based, more transparent and explicit. However, they do not have the ability to solve specific problems of unbalanced negotiation powers in certain therapeutic classes or affordability issues.

3. Appropriate diffusion and funding of value-adding technologies

To encourage appropriate diffusion of valuable technologies, OECD countries should: better prepare for new technologies; provide quick access to promising technologies for high unmet medical needs without compromising patient safety; strengthen the regulation of medical devices; adapt regulation to new health products; and use the potential of ICT to improve the safety and performance of new technologies and health care systems.

3.1. Co-operative horizon scanning can be used to better prepare for new technologies

As a first step towards priority setting and prudent allocation of scarce health resources, many countries are pro-actively thinking about medical technologies that are not yet on the market. Over half of OECD countries now deploy some degree of horizon scanning, most often to focus their immediate priorities for HTA. These early awareness and alert systems consider technologies in a two- to three-year horizon and some of them exhibit good practice by considering the broader governance impact of new technologies along the following dimensions: patient benefits, impact on process of care, regulatory considerations, purchasing and reimbursement considerations, utilisation/budget impact, legal and ethical considerations, and additional factors affecting appropriate dissemination of a new technology. International co-operation is common and developing in horizon scanning activities but opportunities exist to improve collaboration and shared work in this area to avoid duplication of effort.

Foreseeing technological changes in the medium to long term and assessing their potential impact on health care systems are more challenging tasks. The future of technologies considered at an early stage of their development is hard to predict and few countries actually conduct foresight studies in the health sector. Such studies, however, might be useful to envisage the impact of potentially disruptive technologies through scenarios, so as to envisage needed changes in regulatory frameworks and workforce planning and education.

Another area for improvement is the identification of unmet medical needs and priority for research. Such initiatives have recently taken place for Alzheimer's disease (OECD, 2015b) and AMR (Cecchini et al., 2015) – areas where a combination of scientific challenges and market failures led to failures in innovation (Box 1.4). It might be worth further identifying unmet medical needs to encourage research in neglected areas.

Box 1.4. **Why are we not getting the technology we need? The case of AMR and dementia**

Failure of the existing innovation model to produce health technology in areas of unmet need is illustrated by the emerging problems of antimicrobial resistance (AMR) and dementia.

AMR is now recognised as a top-order global health problem. Worldwide, AMR results in 700 000 deaths each year and if not addressed could escalate into a full-blown global health and economic crisis (Cecchini et al., 2015). While indiscriminate use of antibiotics is responsible for creating the problem, development of antibiotics to combat resistant

Box 1.4. **Why are we not getting the technology we need? The case of AMR and dementia** *(cont.)*

bacteria has slowed – the last major new class was discovered in 1987 (Butler et al., 2013). Given other policies to combat AMR (prevention; limiting antibiotic use), investment in this area has become unattractive. Incentives for private capital to develop new antibiotics are currently insufficient as the expected profitability is much lower than for other therapeutic categories, such as chronic diseases. In addition, cheap and effective diagnostic devices at the point of care are desperately needed, yet no such product has been developed. The same can be said for effective vaccines. The market is clearly not delivering in this important area.

Recent proposals suggest policy options to address this innovation failure (AMR Review UK, 2016; WHO, 2015; Cecchini et al., 2015). They aim to "delink" incentives from volume and comprise two categories:

- *Upstream interventions* target the early phases including basic research, which typically requires public funding due to the uncertainty of success, the time lags involved and the difficulties to appropriate returns. Examples include partnerships, grants and seed funding. While more financial risk is taken on by sponsors, enterprise participation is encouraged and it may be cheaper than downstream rewards (Spellberg et al., 2012).
- *Downstream mechanisms* – e.g. prizes or tax concessions – aim to boost the reward at the end of the development process. These reduce the risk to sponsors but they inflate the required amount because they essentially aim to replace returns through global product sales.

An ideal approach should combine up- and downstream mechanisms to encourage global innovation by lowering early development costs and boost the reward at the end of the development process. While countries have invested in the former, effective and large-scale action on the latter is still insufficient. Global research platforms may make research spending more cost-effective (Cecchini et al., 2015).

Dementia is emerging as another leading health priority across the world. Here the innovation problem is largely due to the complexity of the disease. This complexity results in high rates of research failure, necessitating alternative innovation models that reduce these risks. These include shared public-private funding, and a higher public investment in basic, upstream research (dementia makes up less than 0.5% of R&D budgets). Permitting early-phase clinical studies involving people with pre-symptomatic dementia must also be examined. As with AMR, global sharing of research data is crucial (OECD, 2015b).

Regulatory and reimbursement reform is another way to stimulate investment. Costs can be reduced by simplifying processes and harmonising them across countries. Clear reimbursement policies that ensure sufferers have access to effective interventions can reduce investor uncertainly. Industry, academia, regulators, payers and patient organisations each play important roles at various stages, and stronger collaboration between these groups is needed (OECD, 2015b).

AMR and dementia illustrate the problems with the current innovation system, which does not always deliver technology in the areas of greatest need. As global health burden patterns evolve and budgets tighten, governments and policy makers must become more pro-active and engage with industry throughout the development process to ensure that truly innovative products – in areas of health need – are developed to add value to patients, populations and the global community.

3.2. Quick access to promising technologies for unmet needs can be provided while still protecting patients

Market entry regulation needs to adapt to speed access to promising treatments for unmet medical needs, to improve safety and performances of medical devices and to address the specificities of new technologies.

Provide quicker access to promising medicines for unmet needs while mitigating patient risk

In the pharmaceutical sector, regulation of market entry is simultaneously perceived as costly and too stringent by pharmaceutical and biotech companies and some patients' associations, and as insufficient by public health experts. Both parties are right. On one hand, new drug approvals rely on demanding standards for producing evidence on safety and efficacy based on RCTs, which take several years to conduct and are costly. This sometimes delays access to promising medicines treating unmet medical needs, generating frustration for patients and clinicians.

On the other hand, current regulation is not entirely satisfactory. Several studies have shown that information communicated by companies responsible for conducting clinical trials is incomplete and biased towards good results. Too often, RCTs compare new products to placebos while in reality they will compete with existing treatments. In addition, patients recruited for RCTs are often not representative of the entire patient population, who, for example, may be affected by more than one disease, which in turn affects their response rate to the medicine.

Since the end of the 1980s and following pressure from the HIV patient community to expedite access to new treatments, regulatory agencies have implemented accelerated pathways to approve earlier and more quickly promising treatments for high unmet medical needs; i.e. severe diseases without any available treatments. Such treatments can be approved earlier in their development phase, with lower levels of evidence requirements, based on surrogate markers[9] instead of survival, for instance. In the United States and the European Union, conditional approval[10] can be granted on the condition that the company provides further evidence on the benefits of the medicine in real life.

Regulatory agencies are under pressure to do more. "Adaptive pathways" are under discussion in the United States and Canada and are being piloted in Europe. They consist of early approval based on incomplete clinical trial results, followed by post-marketing studies to be performed by companies. While it is reasonable to respond to patients with desperate needs for treatment, countries should consider several conditions to make the system work. First, patients must be adequately informed of the quasi-experimental status of products approved through such pathways. Second, regulatory agencies must be provided with the means to ensure that companies comply with their commitment to produce additional evidence within the agreed delay. The threat of withdrawal in case of non-compliance might be more effective than current systems of fines, which do not seem high enough to encourage compliance. Such an option would also clearly put the responsibility on firms in case of withdrawal. In addition, since adaptive pathways have the potential to significantly reduce the cost of producing evidence before market entry and provide companies with earlier returns on investments, payers and patients should benefit from these financial gains though lower prices and greater affordability. Finally, adaptive pathways should be reserved for exceptional circumstances and the generation of evidence before marketing authorisation should remain the standard rule.

Strengthen regulation of medical devices to improve safety and performance

The regulation of market entry for medical devices is often considered less stringent than that for pharmaceuticals. Evidence requirements for market entry vary across categories of devices according to potential risks for patients, but also across countries. Devices associated with higher risks for patients (such as those surgically implanted in a patient's body) are typically subject to higher scrutiny in all countries.

The regulation is nonetheless unsatisfactory in several respects. First, the high number of recalls after marketing authorisation suggests that evidence produced before market entry may not be sufficient. In Europe, where devices can be sold as soon as they get "CE marking"[11] from one of the dozens of notified bodies, safety problems are not uncommon. As notified bodies compete for user fees on the speed of their process and approval rates, they do not always apply the highest standards to grant approval. The fact that a vast majority of companies producing medical devices are small and medium enterprises is often invoked as a reason for not increasing approval standards, but this is not really acceptable from a risk management perspective.

Second, post-marketing surveillance systems,[12] which all primarily focus on safety issues, could do much more. The reporting of safety issues itself is incomplete, relying mainly on manufacturer reporting, with insufficient contributions from health care providers and patients. Post-marketing monitoring of performance is far from systematic. Yet national experiences of disease-specific registries have been very useful in identifying subperforming medical devices and influencing clinical practice and reimbursement policies. For instance, findings from Australia and the United Kingdom's orthopaedic registries showed that cemented hip prostheses were more performant than non-cemented ones. Similarly, a Swedish cardiac registry showed that drug-eluting stents – initially developed as a clinical improvement over bare-on-metal stents due to the slow release of a drug to prevent fibrosis – were actually less safe than bare-on-metal stents (Lagerqvist et al., 2007). Once the information becomes available, countries are more or less quick in making the best of it: while Sweden quickly adopted cemented prostheses in 98% of hip replacements, France only used them in 51% of cases in 2012. Such information is crucial to improve the quality of care and should diffuse more rapidly across borders.

Many countries have indeed acknowledged the need to more rigorously regulate medical devices. Revisions to the relevant EU legislation to strengthen the regulatory process were finally agreed upon and in the process of adoption at the time of writing (Council of the European Union, 2016). These revisions include: a more comprehensive description of risk classification and management; reinforcement of rules concerning clinical data; stricter pre-market control of high-risk devices; reinforced requirements for manufacturers to collect data on real-life performance of their device; and introduction of EU-wide standardised information for patients receiving implants (Hansson, 2016). These changes are expected to increase transparency and improve safety, notably through systematic reporting of clinical investigations, improved oversight of notified bodies by competent authorities, and how compliance of rules for clinical investigations comply with international standards to facilitate use of their results by other jurisdictions. Post-market vigilance will be improved through: an electronic system and a central database of incident reporting; requirements for manufacturers to establish a risk management system; introduction of a unique device identification (UDI) system; and better access to information for all stakeholders. The United States also introduced UDIs for devices to enhance traceability and monitoring. This

information not only allows closer monitoring of devices but also offers great opportunities, when associated with electronic health records (EHRs), to produce real-world evidence (RWE) on the safety and comparative performance of competing medical devices. Countries should seize this opportunity and imagine ways to share evidence more effectively with their counterparts.

Adapt regulation to hybrid technologies and mobile applications

Countries need to respond to regulatory challenges posed by hybrid technologies, such as PM, wearable devices and 3D bioprinting. An example of regulatory response comes from the United States. In 2002, the US FDA created a special Office of Combination Products (OCP). The OCP's role is to ensure timely and effective pre- and post-market review of combination products by overseeing the timeliness of and co-ordinating reviews involving more than one agency centre. The OCP also streamlines submission of a single investigational application for a combination product, if appropriate, determining the need for separate marketing applications on a case-by-case basis. A sponsor may also choose to submit two marketing applications for a combination product to receive some benefit that accrues only from approval under a particular type of application (e.g. new drug product exclusivity, orphan drug status, or proprietary data protection when two firms are involved).

In Australia, the Therapeutic Goods Administration (TGA) recently recognised that some therapeutic products do not fit neatly within traditional categories. The TGA now provides a list of device/medicine boundary products that have been approved and identifies whether they have been classified as a medicine or a device. The TGA is also undergoing a broader review of its current regulatory pathways, which may help in providing assistance in determining the most appropriate regulatory pathway for these new therapeutic products. Challenges will remain in those countries where medicines and medical devices are regulated by different agencies. Progress in convergent medical technologies will require reshaping existing institutional structures to allow effective and timely regulatory reviews that cut across traditional disciplinary boundaries.

OECD countries also need to respond to specific challenges raised by developments in PMs and biomarker diagnostics. In the United States and Europe, reforms are under way or in discussion to harmonise regulatory requirements for IVD tests, be they developed by commercial sponsors or in laboratories.

In a similar vein, policy makers face distinct regulatory challenges regarding ICT, specifically mHealth applications. Some applications are embedded in medical devices and thus already subject to regulatory review. However, mobile applications available directly to consumers increasingly blur the line between wellness and medical advice.

To respond to the mHealth revolution in a manner that protects patients while not hindering appropriate innovation, health care systems should create a regulatory framework that ensures safety in terms of clinical risk and risks to privacy and security, encourages high-value innovation, and prevents ineffective, unsafe and low-value products from flooding the market and crowding out the more beneficial ones. Owing to the peculiarities of this domain – its rapid evolution, the entry of new actors and stakeholders, and the extension of the risk profile to data privacy – an innovative regulatory approach is required with appropriately nuanced processes, expertise and oversight. Some jurisdictions recognize this and are moving in the right direction.

3.3. A lifecycle approach for Health Technology Assessment can be adopted to inform coverage and funding decisions

HTA is increasingly used to inform coverage and funding decisions, but payers could do more to respond to challenges raised by earlier approval of promising technologies and to improve the performance and value of medical devices.

HTA methods, use, scope and role vary widely across countries and across technologies. While some countries systematically use HTA to inform coverage decisions (e.g. Australia, France), others only assess new technologies with uncertain effectiveness or high prices (e.g. England). HTA systematically includes an economic evaluation in some countries (e.g. Australia, Canada, England, Sweden) and only occasionally in others (e.g. France). In many but not all countries, medicines are more often subject to HTA than other technologies or procedures (Auraaen et al., 2016).

In most cases, HTA is performed once, at or just after market entry, relying on evidence existing at that time. It commonly informs one-off decisions to include new technologies in the range of benefits covered by health care payers. Only a few countries perform systematic or ad hoc re-assessment of technologies to adjust the range of benefits covered. Withdrawals from the "benefit basket" happen rarely and are most often due to obsolescence of clinical interventions or budgetary cuts, without much reference to HTA. Systematic re-assessment of all technologies after a given period of time would probably cost too much for the expected benefits, but ad hoc re-assessments, triggered by the production of new evidence or where initial assessment was inconclusive, are desirable.

Better articulate approval, Health Technology Assessment, coverage and funding decisions

For pharmaceuticals, the trend towards earlier approval based on lower levels of evidence complicates HTA expected to inform coverage or pricing decisions. For a number of recently approved medicines, HTA agencies struggled to assess clinical benefits, let alone cost-effectiveness, and were not able to provide conclusive assessments to decision makers. In such cases, payers face a dilemma: they can either delay decisions to reimburse a product or base their decisions on incomplete evidence.

Coverage with evidence development (CED), which conditions positive coverage decisions on further development of evidence, is used in several countries as an option for select medicines, devices and procedures. At the end of a specified period of evidence development, payers are expected to get more information from the company on effectiveness and sometimes cost-effectiveness of the technology, and to then decide whether to continue or stop coverage or to restrict coverage to subgroups of indications or populations. The Netherlands, Sweden, and the United States (Medicare), for instance, use such approaches. Results of these experiences are mixed but enough experience has been accumulated to draw some lessons. First, it is very difficult to stop coverage on economic grounds, whatever the results of the assessment, especially when the treatment concerns severe diseases with no alternative treatments. Second, in some cases, compliance with evidence development requirements is poor, suggesting that incentives are insufficient for companies to respect their commitments.

To deal with uncertainty and lack of evidence, payers increasingly use performance-based managed entry agreements (MEAs) for pharmaceuticals, linking the final price paid for a medicine to its performance in real life. In such arrangements, the effectiveness of the

medicine observed in real life is compared with benefits claimed by the manufacturer. If observed outcomes are lower than expected, the company has to refund a share of the costs incurred. Most often, financial arrangements take the form of *ex post* rebates, but they can also consist of provision of free stocks, for instance. These agreements are widely used in Italy and England, mainly for oncology medicines. Here again, results are mixed. In Italy, the scheme was assessed as quite burdensome in terms of administration; the amount recouped by the National Health Service accounted for only 5% of total spending for the relevant indications, not reflecting high therapeutic success but rather difficulty in getting results from companies on post-marketing assessment. More generally, clinical results of performance-based MEAs – 40% of which concern oncology medicines in Europe – are usually not made available beyond involved parties. To date, the experience is that performance-based agreements do not increase knowledge on therapeutic benefits of new drugs. If decision makers and payers continue to rely on MEAs to manage uncertainty in spite of these contrasting results, their use should be limited until the associated challenges are overcome. In all cases, post-market evidence should be made available to the scientific community and international counterparts.

Finally, parallel or joint early dialogue (scientific advice) between regulatory agencies and HTA agencies could help pharmaceutical companies design and shape pivotal studies to answer (ideally) all questions; i.e. the demonstration of safety and efficacy for marketing authorisation and comparative effectiveness study by comparison to standard reference treatment for HTA. Such early dialogue is currently promoted at the European level, involving a network of HTA agencies and the European Medicines Agency. It could reduce development time and costs and accelerate access to treatment. A multistakeholder dialogue was engaged in Europe to move in this direction.

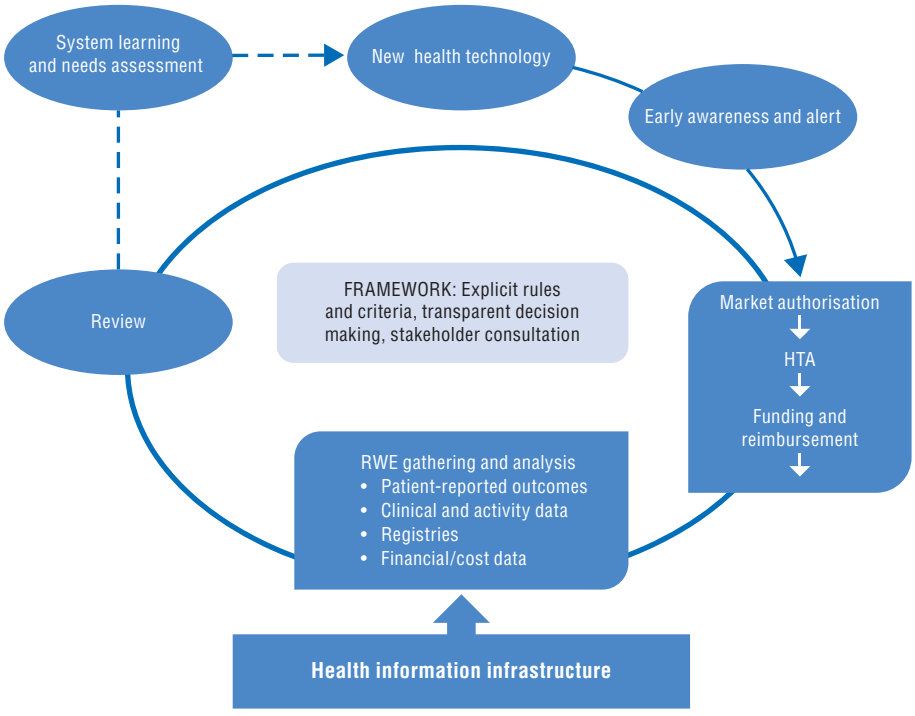
Use real-world evidence to adjust technology coverage

Collection of RWE could significantly improve the management of new technologies. Such evidence can be collected in two ways: through post-market studies designed to collect specific information on health outcomes, and potentially costs; or through routinely collected data. In both cases, assessment methods differ from that used in initial pre-market clinical trials and need to be refined. RWE cannot be expected to fill information gaps in situations where original pre-market evidence assessed a product's efficacy with a high-level of uncertainty. In addition, the effectiveness of a medicine in real life depends on a number of factors – including patient compliance – that usually do not affect clinical trials. However, RWE can be useful in helping to understand how a clinically effective product performs in different real-life circumstances. This information could, for example, be useful in revising posology, better targeting treatment (e.g. if it becomes clear that some patients with co-morbidities do not respond well), or revising cost-effectiveness estimates. These revisions could be reflected in coverage conditions.

New capacities in the generation and use of health care data offer great opportunities to fill information gaps – for both new and existing treatments. Information produced by clinicians, facilities, payers and patients themselves increasingly allows the generation of RWE; i.e. critical information on the safety and effectiveness of technologies in real life. An additional legal framework may be required to create incentives for doctors, patients and companies and to balance evidence generation with patient data protection. This will require adapting existing HTA agencies and methods. Instead of considering HTA as a one-off event, stakeholders should continuously draw upon RWE to monitor the use of medical

interventions and their outcomes and to continually update coverage conditions and clinical guidelines (Figure 1.2).

Figure 1.2. **Lifecycle framework for successful integration of health technologies in health care systems**



An open question is who will generate and fund the collection of such evidence. In some cases, the payer might be equipped and willing to bear the cost. In other cases, the promoter of the technology could be requested to do so. In any case, stakeholders should consider health data a public good and share both findings and data. International collaboration, including among experts, might be required to set high standards for the production of high-level evidence. At the EU level, several initiatives are targeted towards producing high standards for RWE generation (i.e. PARENT,[13] IMI GetReal[14]) and the European Network of HTA agencies (EUnetHTA) is working on methodologies to support post-marketing evidence generation.[15]

3.4. Solutions are needed to manage access to and budget for high-cost medicines

Countries need to find solutions to respond to the proliferation of high-cost medicines. They should first seek mechanisms to increase the negotiating powers of purchasers (payers and providers). Second, they should re-examine the incentives created by orphan drug legislation.

Seek mechanisms to increase purchasers' negotiating power

In pharmaceutical markets, the respective negotiation powers of purchasers and sellers need to be rebalanced. One option envisaged to increase purchasers' power in negotiations with global companies is joint procurement. Several countries in Europe and Latin America are working on such initiatives. This can only work if participating countries share a number

of policy goals and characteristics, such as comparable income levels and/or willingness to pay. At a minimum, countries and payers should increase transparency and exchange of information to reduce the information asymmetry between them and global companies.

Payers are also seeking opportunities to foster competition in some therapeutic areas, such as oncology. Competition could occur at the level of providers or at the level of purchasers, through calls for tender for instance, provided that several medicines have the same indication and comparable effect on patients. This is not an easy task as providers and patients generally value choice and like having access to a wide range of therapeutic options. This is complicated by the fact that treatments are increasingly tailored to patient categories (i.e. PM), reducing opportunities for competition.

Finally, more radical options are proposed, such as compulsory licensing where affordability of essential treatments is impaired by pricing strategies. OECD countries, however, have been reluctant so far to use this option, even where it could be used (Kapczynski and Kesselheim, 2016), for fear of sending too negative a signal to investors and companies investing in R&D to develop new treatments.

Re-assess the relevance of incentives created by orphan drug legislation

OECD countries should assess whether incentives based on the extension of the market exclusivity period beyond original patent protection work as intended and are still relevant. Such incentives exist for all medicines and have been implemented to compensate developers for the length of the regulatory approval. Orphan medicines benefit from a further extension of market exclusivity and from a number of financial incentives, aimed to encourage their development and address market failures, such as tax credits, earlier and easier approval, waiver of regulatory user fees and extended market exclusivity.

The costs and benefits of incentives for orphan medicines, in particular, need to be examined. Incentives to invest in the development of treatments for rare diseases have been successful: the number of orphan medicines has continuously increased. The industry now envisages the development of orphan medicines as a good business opportunity, since all incentives are now combined with exceptionally high prices (EvaluatePharma, 2015). From payers' point of view, this is becoming a bitter pill to swallow. In spite of public support, including funding of basic research in addition to incentives mentioned above, orphan medicines are not available and affordable to all patients who need them. Moreover, companies are suspected of adopting "salami-slicing strategies" by marketing new medicines with narrow indications to claim an orphan drug status and a high price and then develop other indications (orphan or non-orphan). Finally, some orphan medicines perform very well – two of them are in the 50 top-selling medicines worldwide – which suggests that they may not need additional public subsidies to be commercially viable. Policy makers should launch a global assessment of the costs of public incentives for orphan medicines and of associated benefits, in terms of access to treatment and health benefits brought to patients.

3.5. Information infrastructure and governance can be constructed to realise health technology potential

Vast amounts of digital health data are generated by health care systems, and increasingly by individuals themselves, through the digital technologies mentioned above as well as by everyday activities such as social media and web browsing. An unprecedented amount of health-related data now flows across all areas of the economy, and advances in computer science enable them to be captured, stored and processed more effectively.

Health care systems are often thought of as data-rich and information-poor but emerging techniques and technologies – and more importantly, a new mindset of data as a valuable resource as opposed to a by-product – can enable the extraction of valuable information from these mountains of data.

Putting health data to work presents many opportunities to improve population health and individual outcomes. These opportunities can be grouped into four overlapping themes:

- *Improving patient care.* Information derived from health data can help providers in all settings manage uncertainty, and can enable more accurate, timely and co-ordinated decision making. It can also help evaluate and improve the effectiveness of therapies, care models and treatment protocols, and enable better personalisation and continuity. For example, data algorithms are improving the accuracy of personalised treatments for cancer, and accurately identifying people with chronic disease at risk of hospital admission.
- *Managing the health care system.* Analysis of health data can help monitor performance and drive greater transparency, accountability and continuous quality improvement. It can inform decisions regarding resource allocation and priority setting across health care systems. In the future, an integrated information system may enable funding and contract management based on health outcomes as opposed to volumes of services.
- *Enhancing surveillance and population health.* "Big Data" analysis especially can enable more accurate surveillance of population health care needs, help predict changing needs and help model new service configurations. For example, analysis of clinical, social care, environmental, socio-economic and commercial data combined with individuals' data on daily activities and/or sentiments can be deployed to predict acute exacerbations of chronic disease.
- *Enabling health research.* Better use of data enables research that is faster, deeper and of considerably larger scale than was previously possible. This should lead to richer evaluation of clinical and public health interventions, driving more productive investment in health. It can enhance prevention and treatment of complex diseases such as dementia.

Realising these opportunities can help establish the goal of a "learning health care system", leading to better health outcomes and more effective and efficient use of scarce resources. This includes providing the infrastructure and tools to evaluate the safety and utility of health technology in a consistent and cyclical fashion (Figure 1.2). However, to build such a 21st century information infrastructure, the right institutional and governance mechanisms need to be in place.

To generate useful information from health data, routine linkage of sources containing relevant data must be enabled, as no one dataset will contain all the necessary information. Health care systems still tend to capture data in silos and analyse them separately. Standards and interoperability are key policy issues that must be addressed – for example, in implementing an EHR (Box 1.5). In practice, interoperability means common protocols and ontologies that define the basic mechanisms by which users negotiate, establish, manage and exploit data. A 2013-14 OECD survey revealed that only a minority of countries regularly link all relevant health databases (OECD, 2015c).

A 2016 OECD survey of 30 countries revealed that most countries are investing in development of EHRs, but only some are actively progressing the possibility of putting the data to work to realise the opportunities listed above (more detailed results of the survey are

Box 1.5. **The electronic health record**

A key part of health information infrastructure is the electronic health record (EHR) – a comprehensive interconnected database that can capture and share a variety of information about people's health status, their history of encounters with the health care system, the results of all diagnostic and therapeutic interventions, and (ideally) their key social and demographic characteristics.

The critical functions of the EHR are that it puts information about people's health and their disease management within easy reach and provides them with the opportunity to contribute information to their record. The latter is important. For example, patient-reported measures on outcomes of care are valuable to providers, regulators, payers and researchers as well as other consumers.

Implementing an EHR is an industry-wide transformation, and mirrors the requirements of establishing a general health information infrastructure. It includes enactment of new legislation, for example to ensure the protection of information privacy; appropriate governance mechanisms; standards for both semantics and for the interoperability of EHRs across different settings; engagement of regional authorities, insurers and health care providers in the effort; collaboration with vendors and the private sector; and training and public education (OECD, 2013).

provided in Chapter 6). Nine countries exhibit both high governance readiness and high technical and operational readiness to harness EHR data. Others still have a way to go. These nine counties are overcoming challenges ranging from garnering adequate financial and human resources, to managing culture change, to effectively engaging the public, to ensuring data usability, quality, security and privacy protection. They are well-positioned to capitalise on the opportunity to develop world-class health information systems that not only support information needs regarding health care system quality, efficiency and performance reporting, but also create a firm foundation for scientific research and discovery.

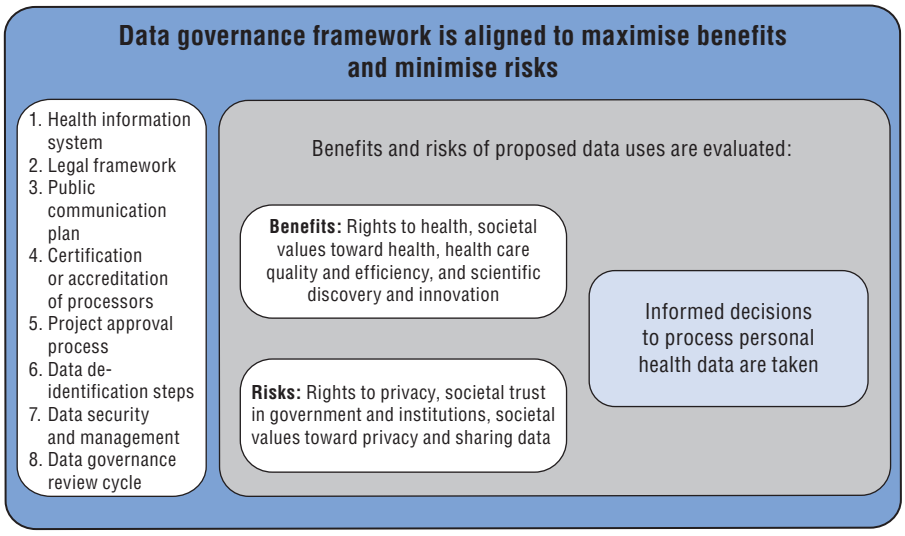
Realising the potential of data requires not only investment in technical infrastructure but also human capital and expertise. Health care systems that are successfully modernising their information systems are recruiting and training data scientists, security experts and biostatisticians. It is also important to have health professionals and managers at ease with the fundamentals of data science and computing. Providers, policy makers and managers must have the requisite knowledge and skills to work with computer processing experts and ICT and legal professionals in developing and using the tools offered by digital technology (OECD, 2015a). This can go some way to overcome their reluctance and to help them embrace the opportunities of health data at all levels of the system.

Many OECD countries report legal barriers to the use of personal health data. As mentioned above, this includes enabling data linkages and developing databases (OECD, 2015c). A key problem is that the legislative instruments governing data, privacy and security pre-date the digital era; meanwhile, the lines between the various uses of health data are blurring, as is the case in the area of dementia (OECD, 2015b). Legal mechanisms enabling the use of health data need to be updated periodically.

Collection and use of personal health data present a number of important risks to the privacy of individuals. These can contribute to a loss of public confidence in government and its institutions. Yet equally significant risks to individuals and societies arise when health information assets are not developed, are unused, or are very difficult to use. The OECD

developed a governance framework that contains technical, legal and political mechanisms to help realise the benefits and manage the risks of using health data in a transparent, explicit way (Figure 1.3) (OECD, 2015c). The OECD Council Recommendation on health data governance will assist countries with these challenges (OECD, 2017).

Figure 1.3. **OECD health data governance framework**



Source: OECD (2015), *Health Data Governance: Privacy, Monitoring and Research*, OECD Health Policy Studies, OECD Publishing, Paris, *www.oecd.org/publications/health-data-governance-9789264244566-en.htm*.

Conclusion

The sustainability of health care systems depends on intelligent adoption of technologies that enable gains in population-based outcomes. When technologies emerge that provide clear evidence of patient benefits in an affordable manner, they must be integrated into the health care system as soon as possible to improve its performance. Equally, policy makers must create the right institutions and mechanisms to ensure that technologies that do not deliver value to patients and societies are excluded from coverage and funding, and do not enter routine use across health care systems. This can be achieved by:

- Better preparing for new technologies through co-operative horizon scanning activities.
- Considering new incentives and mechanisms to address gaps in the pipeline of delivering innovations in areas with large unmet needs.
- Ensuring prompt access to treatments for severe diseases without alternative therapeutic options, without compromising safety, through conditional approval and/or coverage and assessment of products' performance in real life. This should be accompanied by clear messages to companies, patients and providers that new evidence may lead to coverage restrictions or price reductions – and by the necessary mechanism to do so.
- Adapting the regulatory framework to new types of products (hybrid technologies).
- Aligning economic incentives in health care systems to encourage take-up and diffusion of cost-effective technologies and appropriate use ("pay for value").
- Rebalancing negotiating powers of buyers and sellers in segments of the pharmaceutical market where prices are too high and re-examining the costs and benefits of incentives embedded in orphan drug legislation.

- Seeking opportunities for digital technologies and data analytics to improve care delivery, ensure secure and easy access to information by the appropriate parties, and improve population health outcomes via access to digital services.

In a context of unprecedented technological change, the overarching objective for policy makers should be, more than ever, to pay for value, thereby ensuring that new value-adding technologies are accessible to patients who need them, while discouraging or stopping to pay for innovations that do not provide value. Critically, this will require leveraging and mobilising new data and information systems at all points throughout the innovation and care process to increase ongoing generation and validation of knowledge about patient care, outcomes and efficiency.

Notes

1. This is the definition of value predominantly adopted in this report. For more detailed discussion on the use of the term value, see Box 2.1 in Chapter 2.
2. Chapter 2 provides a more detailed discussion of the past impact of health technology on health, expenditure and value.
3. These include most injectable and biologic agents used to treat complex conditions such as rheumatoid arthritis, multiple sclerosis and cancer and often require special handling or delivery mechanisms.
4. Orphan drugs refer to medicines developed for rare conditions. Countries use different thresholds to consider if a disease is rare: "rare conditions" are those that affect less than 1 in 1 500 people in the United States, less than 1 in 2 000 people in the European Union and less than 1 in 2 500 people in Japan.
5. In practice, economic evaluation most often consists of cost-utility analysis via estimation of an incremental cost-effectiveness ratio (ICER), i.e. the ratio of incremental costs to incremental benefits (measured in QALYs) of the new technology, by comparison with a reference treatment. In principle, this should go along with the definition of an ICER threshold, beyond which the assessed technology will not be funded through health coverage schemes (Culyer, 2016). Countries are often reluctant to set ICER thresholds. According to an OECD survey conducted in 2014-15, only five member countries (Hungary, Korea, Poland, the Slovak Republic and the United Kingdom) have published such a threshold.
6. Coverage in this report refers to funding by health coverage schemes, be they residence-based universal health coverage schemes or health insurance.
7. I.e. setting the price of medicine in relation to health gains.
8. Box 2.1 in Chapter 2 of this report reviews different conceptions of value in health care systems.
9. A "surrogate marker" is a laboratory measurement or physical sign that is used in therapeutic trials as a substitute for a clinically meaningful endpoint that is a direct measure of how a patient feels, functions or survives and is expected to predict the effect of the therapy.
10. Conditional approval consists of temporary approval of a medical product for a given period during which the company is required to provide further evidence of its safety and effectiveness.
11. CE stands for *Conformité Européenne*, and is a mandatory conformity marking for certain products sold in the European Economic Area. The CE marking represents the manufacturer's declaration that the product meets European standards, either via self-certification or working with an organisation called a "notified body", depending on the level of risk of the product. Medical devices are subject to such CE marking standards, as are products such as machinery, toys and radio equipment. National competent authorities in each country identify one or several "notified bodies" accredited to conduct "conformity [to EU Directive requirements] assessments". There were 59 notified bodies at the time of writing.
12. Post-marketing surveillance (PMS) is the practice of monitoring the safety of a pharmaceutical drug or medical device after it has been released on the market.
13. See *http://patientregistries.eu/*.

14. See *www.imi-getreal.eu/*.

15. See *www.eunethta.eu/activities/eunethta-joint-action-3-2016-20/work-package-5-life-cycle-approach-improve-evidence-gener*.

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