

Life Sciences for Europe

An integrated strategy for healthcare innovation (2019-2024)



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About this study

This study was commissioned by the American Chamber of Commerce to the European Union (AmCham EU) and conducted independently by Charles River Associates (CRA).

More on the report and for a full list of references, see www.amchameu.eu/lifesciences4eu.

AmCham EU

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CRA

CRA is a leading global consulting firm with a dedicated Life Sciences Practice. CRA's policy team provides analysis and insights into issues affecting the life sciences industry and healthcare systems. We work across global, regional and national policy areas to help navigate the evolving healthcare regulatory landscape and anticipating changes in the policy environment.

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List of abbreviations

ATMP	Advanced Therapy Medicinal Products	IP	Intellectual Property
BRCA	BReast CAncer gene	IT	Information Technology
CAR-T	Chimeric Antigen Receptor T-cell	IVD	In Vitro Diagnostic
CE	Conformité Européenne	IVDR	In Vitro Diagnostic Regulation
DGs	Directorates-General	MA	Meta-Analysis
DRG	Diagnosis Related Group	MD	Medical Device
EC	European Commission	MDR	Medical Device Regulation
EHR	Electronic Health Records	MDx	Molecular Diagnostics
EMA	European Medicines Agency	MEP	Member of the European Parliament
ERN	European Reference Networks	MES	Managed Equipment Service
EU	European Union	MI	Molecular Imaging
FFS	Fee-for-service	NCD	Non-communicable Disease
GDP	Gross Domestic Product	NGS	Next Generation Sequencing
GDPR	General Data Protection Regulation	OECD	Organisation for Economic Cooperation and Development
GMP	Good Manufacturing Practice	PD-1	Programmed Cell Death-1
HCP	Health Care Professional	PCSK9	Proprotein convertase subtilisin/kexin type 9
HER2	Human Epidermal Growth Factor Receptor 2	P&R	Pricing and Reimbursement
HCV	Hepatitis C Virus	PIE	Pharmaceuticals in the Environment
HTA	Health Technology Assessment	PPP	Public & Private Partnerships
ICT	Information and Communication Technology	QALY	Quality-adjusted Life Year
IMI	Innovative Medicines Initiative	RCT	Randomized Clinical Trial
IoT	Internet of Things	RWE	Real World Evidence
		SPC	Supplementary Protection Certificate

Executive summary

Today, the healthcare industry looks very different to how it did ten years ago. New technologies have revolutionised healthcare – delivering benefits to patients and reducing healthcare costs, allowing patients to contribute to the labour market and the economy. Innovation in pharmaceuticals, medical devices, diagnostic technologies and increasingly digital health has transformed the way we deliver and manage treatments and organise healthcare systems. Although each type of health technology has its own distinct challenges, the increasing use of integrated, combined treatment options (that combine pharmaceuticals, medical devices, diagnostics and digital health solutions) are posing new challenges for the healthcare system.

As Europe moves into the new legislative cycle (2019-2024), the time is ripe to examine the challenges and opportunities facing the healthcare life sciences sector in Europe over the next five to ten years, and to identify some of the common challenges arising across the wider life sciences sector as well as those that are due to the combined use of health technologies.

The objective of this report is to set out novel policy solutions to improve the policy environment and foster the wider life sciences sector in Europe. This goes hand in hand with the European Commission's objectives to 'ensure that Europe maintains its global leadership in strategic sectors with high-value jobs'.¹ In addition, in March 2018, the Council of the EU stressed 'the urgent need for a comprehensive and long-term EU industrial strategy which should be in place at the latest by the beginning of the next EU institutional cycle'.²

Identifying key trends and challenges across the life sciences sector

The report first reviews key trends and challenges across the life sciences sector, including those that prevent the development of new medical innovation, and patient access to innovative products and services in the EU. We have grouped the trends into three categories: socio-economic (including trends such as healthcare expenditure and the development of new funding models); technology (such as the movement towards multi-indication medicines and combination therapies in oncology as well as medicine/device combinations); and policy (covering changes to the regulatory framework; changes in procurement or the evolution of health technology assessment (HTA) and the intellectual property (IP) and incentive regime).

We then explore the extent to which each segment (ie, our four types of technology: medicines, medical devices, diagnostic technologies and digital health) shares some common challenges, and if the use of these technologies in combination introduces additional challenges.

Given the objective of this project is to focus on novel policy solutions for the entire life sciences sector, we have prioritised issues most affected by the integrated, combined use of these technologies (see **Figure 1**).

1 Juncker, J-C. (2014) 'A New Start for Europe: My Agenda for Jobs, Growth, Fairness and Democratic Change'. Political Guidelines for the next European Commission.

2 Council of the European Union (12 March 2018) EU industrial policy strategy: Council adopts conclusions. <http://www.consilium.europa.eu/en/press/press-releases/2018/03/12/eu-industrial-policy-strategy-council-adopts-conclusions/pdf>.

Figure 1: Identified common issues affecting the life sciences sector



Source: CRA analysis

Table 1: Summary of policy proposals for European policy-makers in areas where evolving technologies are introducing new challenges or where technologies are converging



Overcoming limited funding and budgeting issues

1. Integrated funding models

Action: Collect evidence/share best practices from Member States on bundled payment schemes and assess the feasibility of outsourcing care to third party or ‘pooled’ budgets to avoid silo-based decision-making.

2. Budgeting for long-term spending (horizon scanning)

Action: The European Commission should continue to foster long-term planning around the adoption of innovation, eg, by developing joint horizon scanning for pharmaceuticals across EU Member States and identifying technology/solutions of value for medical devices.

3. Monitoring and benchmarking the performance of budget holders

Action: EU Member States should continue to share best practices on how to improve budget management, but also how to reward innovation, such as introducing innovative payment models and value-based arrangements as part of multi-annual budgeting or coupling to value by anticipating long-term spending through national horizon scanning.

4. Conducting holistic value assessment

Action: Encourage Member States to focus on outcomes and value and adopt a clear definition of ‘value’ in the context of a value assessment framework that takes into consideration wider patient, health care systems as well as the wider social and economic benefits to society.



Tailored approaches to value assessment across different technologies

1. Early dialogue and horizon scanning

Action: The application and value of horizon scanning varies by technology and there is no one-size-fits-all approach. But for integrated, combined products a joined up approach to early dialogue procedure and horizon scanning is required.

2. Appropriate value assessment mechanisms and methodologies

Action: Member States should consider the correct instruments to conduct value assessment and the occasions where a hybridised process is relevant for co-dependent technologies.



Introducing robust data protection and security

1. Building horizontal interoperability

Action: Develop clear and practicable interoperability working with industry, for health information sharing amongst health professionals across Europe via a consistent digital health strategy and the promotion of European Reference Networks (ERN) to foster collaboration.

2. Regulatory clarity and legal certainty

Action: Ensure the smooth implementation of the General Data Protection Regulation (GDPR) and promote HCP's awareness of the legal aspects around data sharing, ensuring an appropriate balance between security of data and HCP understanding of what can be shared.

The European Commission should oversee the development of certification mechanisms that can enhance citizens' trust in digital health services.

3. Education and training

Action: Promote education and appropriate training programmes to improve electronic (eHealth) and mobile (mHealth) skills and enhance Information and Communication Technologies (ICT) literacy.



Adapting to new healthcare business models

1. An industrial strategy incorporating the whole life sciences sector

Action: Call on the next European Commission to learn from Member State initiatives (eg, Denmark, France, UK) and build on the March 2018 industrial strategy Council conclusions to introduce an EU life sciences sector strategy in 2020, which underpins innovation through strong IP incentives.

2. Requirements for evidence development need to better incorporate digital capabilities

Action: Call on the next European Commission to follow through on the April 2018 Communication to implement a Digital Health Action Plan across the EU27 by March 2024, and to deliver a European harmonised health data network.

3. The role of partnerships

Action: Foster the development of partnerships between private healthcare providers and healthcare systems such as 'Managed Equipment Services' – a consortium of private healthcare providers and healthcare systems to build infrastructure, services and capabilities.

4. A flexible and adaptable regulatory regime fit for future technologies

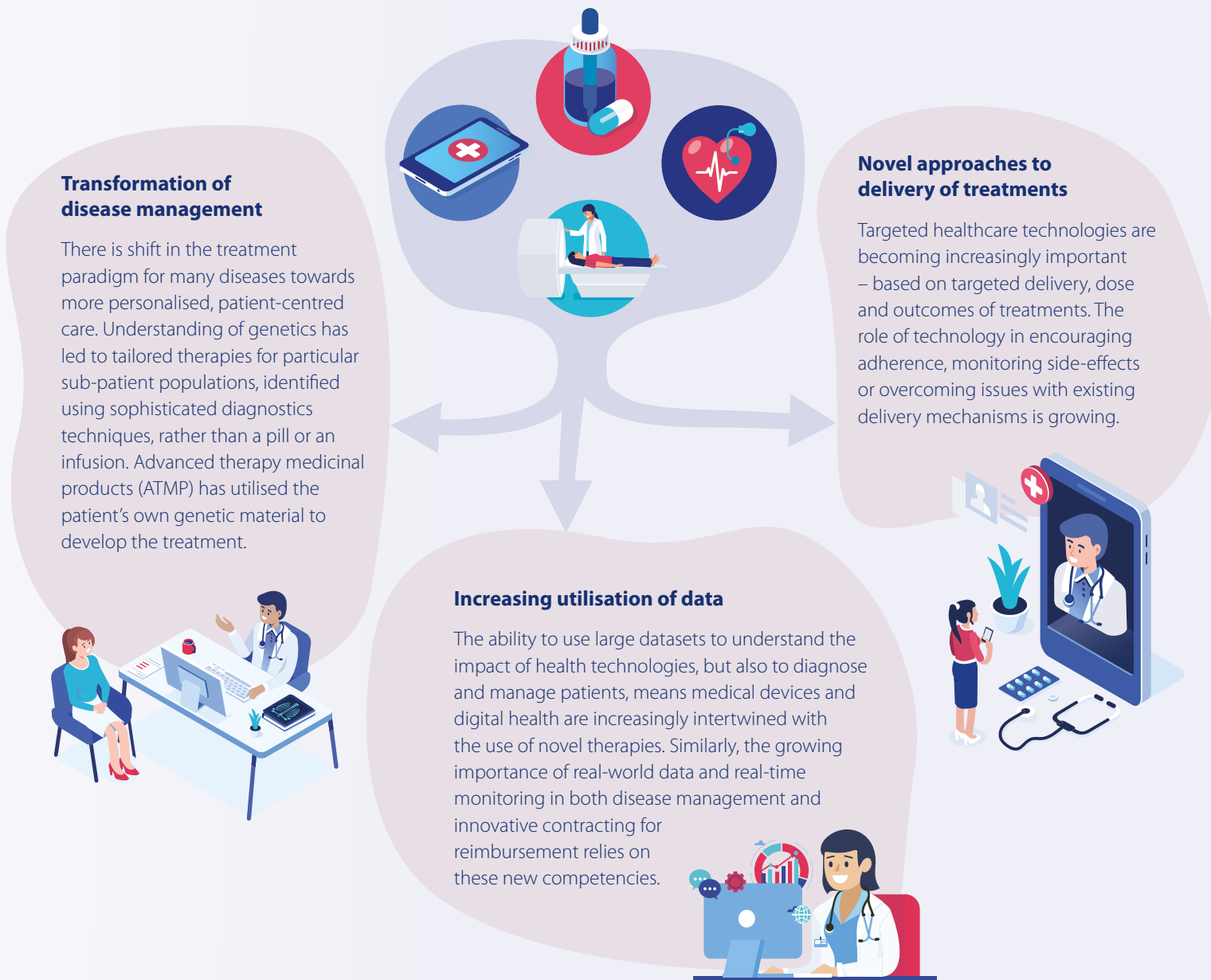
Action: Ensure that the European Medicines Agency (EMA) retains a global regulatory leadership role through the development of alternative regulatory pathways and constant interaction with scientific discovery, and that they are upgrading their understanding and assessment protocols accordingly.

1. Introduction

The innovative life sciences sector is delivering new solutions with the potential to deliver groundbreaking benefits to patients and the healthcare system. We can observe this across pharmaceuticals, medical devices, diagnostic technologies and new digital-based solutions as well as increasingly in the combined use

of these technologies (see definition in Appendix). The transformation of disease management, increasing utilisation of data and novel approaches to the delivery of treatment are shaping the sector as a whole. It is also predicted that the use of combined, integrated technologies will only accelerate.

Figure 2: Trends across the life sciences sector stemming from the convergence of technologies



1.1 Common challenges

There is increasing interest from healthcare purchasers in buying value-based health solutions (that may incorporate a range of health technologies), but the healthcare systems in many countries are still structured as if these technologies worked in isolation.³ They have distinct requirements for value assessment and pricing, and their funding is often through separate budgets and involving different mechanisms. As set out by the Organisation for Economic Co-operation and Development (OECD), the convergence of different technologies offers immense opportunities but also raises novel challenges for all healthcare stakeholders, including policymakers, regulatory authorities, payers, physicians and patients.⁴

The intensity of competition and the speed of technical obsolescence is increasing. The evolution of technology-orientated companies is changing the market structure (consolidation in some areas, fragmentation in others) and shifting to provide new value propositions, with implications across the value chain. Indeed, we can observe an increasing number of partnerships between pharmaceutical companies developing new technologies, with growing integration across the healthcare and information technology sectors.⁵

However, it is also important to recognise that there are significant differences between technologies in terms of nature of innovation, the role of competition, the product life cycle and the potential risks to patients and the healthcare system. This places limitations on the extent

to which different policy frameworks should be aligned across the sector and where different rules and processes are appropriate.

The policy debate, to date, has not focused on the shared challenges and opportunities facing different technologies, nor on the implications for policy reform that should be incorporated into a life sciences strategy. Such a strategy should account for shared challenges posed by integrated, combined use of technologies but also consider the differences in sectoral needs. This is consistent with the Council stressing 'the urgent need for a comprehensive and long-term EU industrial strategy which should be in place at the latest by the beginning of the next EU institutional cycle.'⁶ As we look towards the election of a new European Parliament this should be a policy priority, as well as for the European Commission.

The objective of this report is to set out distinctive policy solutions that will improve the policy environment for the life sciences sector. It also develops novel recommendations that would help address the common healthcare challenges and combined integrated use of different health technologies, whilst recognising the need for distinct regulatory landscapes, value assessment frameworks, as well as reimbursement and funding models appropriate for individual health technologies. AmCham EU – representing biopharmaceutical, medical devices, diagnostics and technology companies – has a unique perspective to contribute to this debate.

3 Interview with policy-maker.

4 OECD (2017) 'New Health Technologies: Managing Access, Value and Sustainability'. OECD Publishing, Paris. DOI: <http://dx.doi.org/10.1787/9789264266438-en>.

5 KPMG (2017) 'Pharma outlook 2030: From evolution to revolution'.

6 Council of the European Union (12 March 2018) EU industrial policy strategy: Council adopts conclusions. <http://www.consilium.europa.eu/en/press/press-releases/2018/03/12/eu-industrial-policy-strategy-council-adopts-conclusions/pdf>

1.2 Approach

In line with the objectives of the report, AmCham EU asked CRA to review the challenges and opportunities facing the entire healthcare life sciences sector in Europe over the next five to ten years and consider policy areas to prioritise. The project aims to:

1

Identify the **common** challenges facing all four types of healthcare technology: medicines, medical devices, diagnostic technologies, and new developments in digital health (including eHealth and mHealth).



2

Set out **recommendations** for how the policy environment needs to change if the sector as a whole is to deliver for European patients and the economy.



3

Test these with external stakeholders and integrate their advice into the recommendations.



In order to develop this report, CRA has carried out its research across three key steps as described in **Figure 3** below.

Figure 3: Project steps

Literature review and industry validation

An initial literature review of public policy documents, academic articles, press articles and trade association and market intelligence reports was carried out to identify overlapping issues affecting all areas of the life sciences sector (listed in Appendix). This was done using search terms or keywords such as 'trends', 'barriers', 'challenges' for 'life sciences', 'medicines', 'diagnostics', 'medical devices' and 'digital health'

CRA also captured the perspective from industry experts representing each area of the life sciences sector (listed in Appendix) through a series of six structured interviews. The objective of these interviews was to validate the current trends and issues identified in the literature review and the impact on the convergence of technologies across all four areas.



Development of novel policy proposals

As part of step two, CRA considered novel policy proposals that seek to address the challenges affecting the sector. CRA focused on a shortlist of common challenges by narrowing down the overlapping issues to those where it was identified most valuable to develop novel policy proposals, using two criteria:

- Technologies have different policy frameworks today, but common policy challenges across segments suggest convergent policy debate; or
- Novel challenges are emerging due to convergence of technologies across the life sciences sector.



External interview programme

As part of step three, after developing these policy proposals, CRA tested these solutions first internally with AmCham EU members, and then externally through a series of nine interviews with European policy-makers, patient representatives and stakeholders across the sector (listed in Appendix).

Source: CRA analysis

2. Key trends and challenges across the life sciences sector

A number of issues affect the development of innovation and patient access to innovative products and services in the EU. These are largely the result of socio-economic, technology or policy-related developments. In this section we review key trends

in the life sciences sector across these three areas, and then explore the extent to which these represent common challenges for the different types of technology (medicines, medical devices, diagnostic technologies and digital health).

2.1 Socio-economic trends

The first socio-economic trend identified by different stakeholders relates to the ageing population in Europe and increased prevalence of chronic illnesses and non-communicable diseases (NCDs).

In OECD countries, health spending has in many cases risen faster than economic growth over the past 20 years. Public expenditure on health and long-term care in OECD countries is set to increase from around 6% of gross domestic product (GDP) in 2015 to almost 9% of GDP in 2030 and as much as 14% by 2060.⁷ This is unless governments can contain costs, according to an OECD projection. Some experts estimate that the cost of healthcare is expected to double by 2050 if reforms are not undertaken, although – as the most recent OECD figures show – the gap between healthcare expenditure and economic growth has become smaller over time since 2001 and the current trend is unclear. Nevertheless, it is indisputable that healthcare expenditure will remain one of the most important issues for governments.⁸

While healthcare expenditure has continued to grow, spending on medicines and other medical technologies (as a share of total healthcare spending) has remained the same or even fallen in absolute terms in recent years. The growth in spending on pharmaceuticals has remained below total health

spending growth over the last decade, with average annual growth rates in the 2009–2014 period much lower compared to pre-financial crisis years.⁹ Between 2010 and 2016, the average EU expenditure on pharmaceuticals as a percentage of GDP fell from 1.58% to 1.39% – mainly triggered by cuts in public spending.¹⁰ It is therefore difficult to say whether healthcare expenditure would become unsustainable, however it is likely that there will be continued pressure on healthcare budgets. Many of these socio-economic trends have associated challenges to funding innovation and a focus on spending in all four types of technology (see **Table 2**).

The second socio-economic trend is linked to the development of digital health and the opportunities this offers to patients. It is increasingly important to involve patients in the decision-making regarding their treatment. This could be through involvement in the process for assessing medicines, information about their treatment and the data collected on them. However, it is also about encouraging a strong focus on prevention and wellness via both clinical and social care provision.

Finally, there is also increasing interconnectedness across European economies with shared expectations. This could result in increased mobility of patients

7 OECD (2015) 'Healthcare costs unsustainable in advanced economies without reform'.

8 Wilson, K. (2016) 'Innovation and sustainability of European healthcare systems'.

9 OECD (2015) OECD Health Statistics 2015: Focus on Health Spending, July 2015. <https://www.oecd.org/health/health-systems/Focus-Health-Spending-2015.pdf>.

10 OECD (2017) 'OECD Data – Pharmaceutical Spending'.

and HCPs across the EU.¹¹ While it is recognised that the impact of the EU Cross-Border Healthcare Directive (2011/24/EU) remains small, its role in the efficient treatment of patients will grow.¹² With an increase in patient data and the technological trend

of digitalisation, this creates a need for cross-border sharing of data and medical health records in order to treat patients across Europe more effectively, and to maintain the EU's global competitiveness.

Table 2: Socio-economic trends across the life sciences sector and associated challenges

	Key trends	Associated challenges
	<ul style="list-style-type: none"> Despite the moderate growth in pharmaceutical expenditure, there is greater focus on managing pharmaceutical spending via cost containment policies, ultimately leading to limitations and inequalities in access. Patients are more integrated in the decision-making and management of their health (patient-centred models). 	<ul style="list-style-type: none"> Pressure to reduce costs, leading to limited funding and strategies for cost containment. This includes increased application of tendering and cross-country collaboration in medicines policy and procurement. Discrepancies in access to medicines across Europe.
	<ul style="list-style-type: none"> There is a focus on prevention to avoid future costs. As diagnostics become more integrated into the healthcare system, a lack of skilled personnel to handle complex technology. 	<ul style="list-style-type: none"> Lack of resources to manage diagnosis and the need for continuous education of different stakeholders (HCPs, payers, policy-makers etc.).
	<ul style="list-style-type: none"> Focus is increasing on areas of spending on medical devices whose use is growing and there is limited recognition of the need to reward innovation. 	<ul style="list-style-type: none"> Pressure to reduce costs and limited patient access to medical devices that can increase healthcare system efficiency.
	<ul style="list-style-type: none"> The rise of mHealth across Europe has been driven by an increase in chronic diseases and patients taking a more active role in disease management. Technological innovation is moving faster than people, limiting digital literacy.¹³ 	<ul style="list-style-type: none"> Limited funding opportunities and uptake of digital health by patients and HCPs.

Source: CRA analysis

11 Interview with policy-maker.

12 Azzopardi-Muscat, N., Baeten, R., Clemens, T., Habicht, T., Keskimäki, I., Kowalska-Bobko, I, and van Ginneken, E. (2018) 'The role of the 2011 patients'rights in cross-border health care directive in shaping seven national health systems'. *Health Policy*, 122(3): 279–283. See also, aforementioned review covers Belgium, Estonia, Finland, Germany, Malta, Poland and The Netherlands.

13 Interview with industry expert.



“With our commitment to rare disease patients, we look to gene therapy as an opportunity to improve the lives of people who have complex diseases with significant unmet needs. Our aim is to address the root cause of the genetic disease, rather than treating the symptoms indefinitely. We believe it is important to work in collaboration with a variety of stakeholders, including healthcare professionals, patient advocates, policy-makers and payers, to ensure patients who may benefit from gene therapies will have access.”

Nolan R. Townsend
Regional President - International Developed Markets (IDM)
Pfizer Rare Disease

2.2 Technological trends

The second group of trends relates to technological developments affecting the whole of the life sciences sector. As explained in **Table 3**, the pace of innovation is accelerating across the entire sector. Digital health (including eHealth and mHealth) is an emerging and rapidly developing field which has the potential to transform healthcare, increasing its quality and efficiency.¹⁴ The emergence of personalised medicine is paving the way towards chimeric antigen receptor (CAR) T-cell therapy (CAR-T) and gene therapies, increasing the curative potential across many diseases. There are also trends towards multi-indication medicines and combination therapies in oncology, as well as medicine/device combinations, which are emerging and pushing the boundaries of available treatment options. This is also leading to the potential for 'digital medicines', and can be observed in medical technology with the rise of bioelectronics.¹⁵

While technologies are increasingly used together, the different individual technologies still need to navigate different rules. New targeted medicines often have a diagnostic technology that needs to be assessed and reimbursed, and may be delivered through an implantable device. Further, digital technology may be used to track the performance of patients and ensure they adhere to protocols.

This convergence in the use of these technologies is causing the structure of companies involved in the healthcare sector to change. Across the industry, smaller companies are increasingly important drivers of innovation, often working with academia and partnering with larger players. New players that were not previously active in life sciences are beginning to invest in healthcare technologies, particularly those with technological and data analytics capabilities.¹⁶

14 European Commission. Green Paper on mobile health (mHealth).

15 OECD (2016) 'New Health Technologies: Managing access, value and sustainability'. See also, Investing News Network, 4 January 2017. 'Top Medical Device Trends for 2017'. See also, Intland Software. 'Medical Technology Trends in 2018'.

16 KPMG. 'Collaboration – the future of innovation for the medical device industry'.

Table 3: Technology trends across the life sciences sector and associated challenges

Key trends



- Innovation in speciality medicines and the growth in personalised medicine has led to the transformation of disease management.¹⁷
- Use of multi-indication medicines and combinations of treatments is growing. As well as the emergence of CAR-T and gene therapies.



- Stratifying patients in different groups based on biomarkers has increased clinical use of companion diagnostics.
- Emerging technologies such as high-throughput screening and next-generation sequencing are driving the market to make molecular diagnostic tests faster, more accurate and cheaper.¹⁸



- Emerging technologies such as artificial intelligence (AI), robotics and 3D printing are likely to have a significant and disruptive impact on healthcare systems.¹⁹
- The automation of medical imaging technology is simplifying surgical procedures and improving affordability.
- Streamlining medical device life cycles into healthcare has led to collecting, managing and analysing 'Big Data' in order to improve healthcare efficiencies, placing significant emphasis on software and information technology (IT) infrastructure.²⁰



- Patient access to technology and utilisation of data has meant that numerous devices and apps have been developed to track different conditions, communicate with healthcare providers, educate patients and assist doctors.²¹

17 Outlook for Global Medicines through 2021. Report by the QuintilesIMS Institute. See also, TaylorWessing. 'Personalised medicine – challenges of authorisation and reimbursement'.

18 Frost and Sullivan, 11 August 2017. 'Molecular Diagnostics Vendors in Western Europe Leverage Innovation and Need for Point-of-care Testing'.

19 MedCityNews, 15 December 2016. '8 technologies that will transform healthcare in 2017 and beyond'.

20 McKinsey. 'The age of analytics: Competing in a data-driven world'.

21 KPMG. 'Pharma outlook 2030: From evolution to revolution'.

Associated challenges

- Need to adapt and evolve existing regulatory and pricing and reimbursement (P&R) pathways, ensuring speed of access is not compromised.²²
 - Concerns about justification of prices and the need for new pricing models to deal with innovative therapies (eg, combination therapies, multi-indication medicines).
 - Complexity of treating complex diseases (eg, cancer or rare diseases) for HCPs; lack of general understanding of the future innovation trends/landscape.
- Different development, authorisation and P&R pathways for medicines/companion diagnostics, creating challenges for access and uptake.²³
 - Increased use of genomic medicine in the clinical setting creating challenges in funding novel infrastructure required for national diagnostic platforms.²⁴
 - Ability for HCPs to interpret and use diagnostic results from automated processes.
 - New In Vitro Diagnostic Regulation (IVDR) including high-risk classification for in vivo diagnostics.
- Continue to ensure that regulations provide standards to assess the efficacy and safety of emerging technologies but do not create obstacles for patient access.²⁵
 - Interoperability with legacy systems and data exchange, alongside the incentive structure to make this a reality.²⁶
 - Sustainable disposal of medical devices in line with new standards on environmental protection.²⁷
- Immature data infrastructure across healthcare systems and connectivity (access and speed of the internet) is limiting the adoption of these technologies. Thus there is an uneven adoption of eHealth solutions across Europe.²⁸
 - Interoperability of new digital solutions within existing healthcare systems.
 - Established companies in the life sciences sector will be disrupted by the digital transformation in healthcare. New entrants and disruptive business models are already challenging incumbent companies.²⁹

Source: CRA analysis

- 22 Corbett, M. S., Webster, A., Hawkins, R., and Woolacott, N. (2017) 'Innovative regenerative medicines in the EU: a better future in evidence?' *BMC medicine*, 15(1): 49.
- 23 The new IVDR makes special provisions for companion diagnostics. The revision means IVD CDx will be classified as high individual risk or moderate public risk (category C) requiring a conformity assessment not only by their manufacturer but also by Notified Bodies, emphasising the importance of clinical evidence. Yet there are different development and authorisation pathways for these products, creating challenges for access and uptake. The EMA has responded to this challenge with intentions of developing a guideline on the co-development of predictive biomarker-based assays, in the context of the lifecycle of a medicine.
- 24 Gaff, C. L., Winship, I., Forrest, S., Hansen, D., Clark, J., Waring, P., and Sinclair, A. (2017) 'Preparing for genomic medicine: a real world demonstration of health system change'. *NPJ genomic medicine*, 2(1): 16.
- 25 Interview with trade association.
- 26 Jiang, F., Jiang, Y., Zhi, H., Dong, Y., Li, H., Ma, S., and Wang, Y. (2017) 'Artificial intelligence in healthcare: past, present and future'. *Stroke and Vascular Neurology*, svn-2017.
- 27 Interview with industry expert.
- 28 World Health Organization (WHO) (2016) 'From innovation to implementation: eHealth in the WHO European Region'. See also, Interview with trade association.
- 29 Roland Berger. 'Digital health market to average 21 percent growth per year through 2020'.

2.3 Policy trends

The final group of trends reflects changes to the legislative and policy environment. The life sciences sector is going through a period of unprecedented regulatory change. At the European level this is primarily due to new regulations impacting medical devices and in-vitro diagnostics, the EU Medical Device Regulation (MDR) and IVDR. The new framework is intended to strengthen the current approval system for medical devices and in vitro diagnostics, introducing a new risk-rule classification system. Additionally, there are new classification rules for high-risk products that have to undergo more rigorous assessments – eg, high-risk software, nano-products and reusable surgical instruments. Overall, there is a general perception that the regulatory framework needs to adapt and evolve with new technologies, ensuring the speed of access is not compromised.³⁰ This view is also shared on the mechanism for value assessment. Some argue that the established HTA decision frameworks across countries are no longer fit-for-purpose to evaluate the true value delivered by more patient-centric innovation.³¹ It has also been argued that there is a need for more appropriate and consistent value assessment frameworks for medical devices that are distinct and adapted to the nature of that technology.

There has been a long-standing discussion on the need for Member States to collaborate on value assessment and more specifically on clinical assessment, which is typically based on global evidence (eg, worldwide clinical trials in the case of pharmaceutical products). Consequently, the European Commission published a proposal on EU cooperation on HTA, aiming to address three key issues:

1. Impeded and distorted market access;
2. Duplication of work for national HTA bodies; and
3. Sustainability of HTA cooperation.³²

Given the timelines for full implementation of the proposal, EU HTA will remain a priority issue for the incoming 2019-2024 European Parliament and European Commission. There is also a debate regarding the roles that intellectual property rights (IPR), as well as the different incentives play in encouraging innovation. Regulatory data protection (RDP), supplementary protection certificates (SPCs), and other incentives to develop paediatrics and orphan medicinal products have been central to the debate as to whether the overall incentive framework provides the right balance between rewarding innovators and ensuring that society benefits from innovative products at affordable prices. In light of the ongoing review of IP incentives, initiated by the June 2017 Council conclusions, this key issue for the fostering of innovation will also require the attention of both incoming Members of the European Parliament (MEPs) and the new Commission.

Countries are using different mechanisms to deal with transformative therapies entering the market at high upfront costs. Some are sticking to traditional price-cutting mechanisms (mandatory discounts, price cuts) while others are supporting innovative pricing models (value-based, indication-based, combination pricing).³³ Additionally, the proportion of healthcare products acquired through public procurement has gradually increased over the past decade in Europe. At the same time, national authorities are showing interest in European cross-border collaboration on pricing for pharmaceuticals as a means to manage expenditure.³⁴ **Table 4** provides an overview of these key policy trends and associated challenges.

30 Corbett, M. S., Webster, A., Hawkins, R., and Woolacott, N. (2017) 'Innovative regenerative medicines in the EU: a better future in evidence?'. *BMC medicine*, 15(1): 49.

31 Leyens, L., and Brand, A. (2016) 'Early patient access to medicines: health technology assessment bodies need to catch up with new marketing authorization methods'. *Public health genomics*, 19(3): 187–191. See also, Medicines for Europe (2017) 'Value added medicines: time to adjust HTA decision frameworks'.

32 Proposal for a Regulation of the European Parliament and of the council on health technology assessment and amending Directive 2011/24/EU.

33 Deloitte. '2018 Global life sciences outlook Innovating life sciences in the fourth industrial revolution'.

34 AmCham EU, 14 June 2016. 'Improving access to medicines in the European Union'.

Table 4: Policy trends across the life sciences sector and associated challenges

Key trends	Associated challenges
 <ul style="list-style-type: none"> The proliferation of transformative therapies is putting into question current pricing models. Debates on medicine pricing mechanisms are flourishing on the international scene.³⁵ The European Commission has launched several public consultations on key policy issues which will have an impact on the life sciences industries: for example, SPCs; the paediatric regulation, pharmaceuticals in the environment (PIE) and the latest REFIT initiative concerning the REACH Regulation (Registration, Evaluation, Authorisation and Restriction of Chemicals). 	<ul style="list-style-type: none"> There are challenges in planning, budgeting and paying for these medicines when the cost-effectiveness and savings generated from treatments are realised over a long period of time.³⁶ Despite efforts by regulators to approve products early (eg, adaptive licensing), there is no associated conditional reimbursement mechanism and payers have been asking for more value evidence. Countries use RWE differently to facilitate early access and innovative contracting. The review of the IP incentives framework for medicines reflects policy-makers' efforts to control future budget impact. However, there is a clear need for a solution that sustainably finances new medicines without damaging patient access to future innovation. Potential restrictive regulations due to reluctance to adopt risk-based approaches, or recognise the long lifecycles of biopharmaceuticals, can have implications for manufacturing and good manufacturing practices (GMP) issued by regulatory agencies. Increased restrictions on the use of authorised chemicals in the manufacturing process for innovative medicines.³⁷
 <ul style="list-style-type: none"> There is heterogeneity in the approach to value assessment for diagnostics across Europe and the degree to which it is integrated with the assessment of associated therapies. Heterogeneity in approach to testing. European laboratories have a significant degree of freedom in choosing how to implement biomarker testing, and the market is constrained by the lack of skilled personnel to handle complex technology. 	<ul style="list-style-type: none"> There have been challenges with the reimbursement of diagnostics, and there is variation in how countries approach assessment. Mechanisms to ensure quality of diagnostic testing are evolving but vary significantly across countries.
 <ul style="list-style-type: none"> Funding medical devices has been a long-standing issue for the sector. There are no specific budgets or funding for medical devices, and these are linked to spending on pharmaceuticals and other areas of healthcare.³⁸ 	<ul style="list-style-type: none"> Limited coherent funding and reimbursement pathways for the vast range of devices, plus different reimbursement procedures for inpatient and outpatient devices, continues to challenge access and uptake.
 <ul style="list-style-type: none"> While there is recognition of benefits from digitalisation, there has not been an active approach to setting up new access pathways so that patients can benefit from these technologies. Currently there are no requirements or standards for the development, authorisation or value assessment of – or P&R for – digital health solutions.³⁹ 	<ul style="list-style-type: none"> Enabling digital health technologies requires robust cybersecurity and clear guidelines on the ownership and privacy of data.

Source: CRA analysis

35 OECD (2016) 'New Health Technologies: Managing access, value and sustainability'.

36 Interview with industry expert.

37 Interview with industry expert.

38 Campling, N. C., Pitts, D. G., Knight, P.V. and Aspinall, R. (2017) 'A qualitative analysis of the effectiveness of telehealthcare devices (ii) barriers to uptake of telehealthcare devices'. BMC health services research, 17(1): 466.

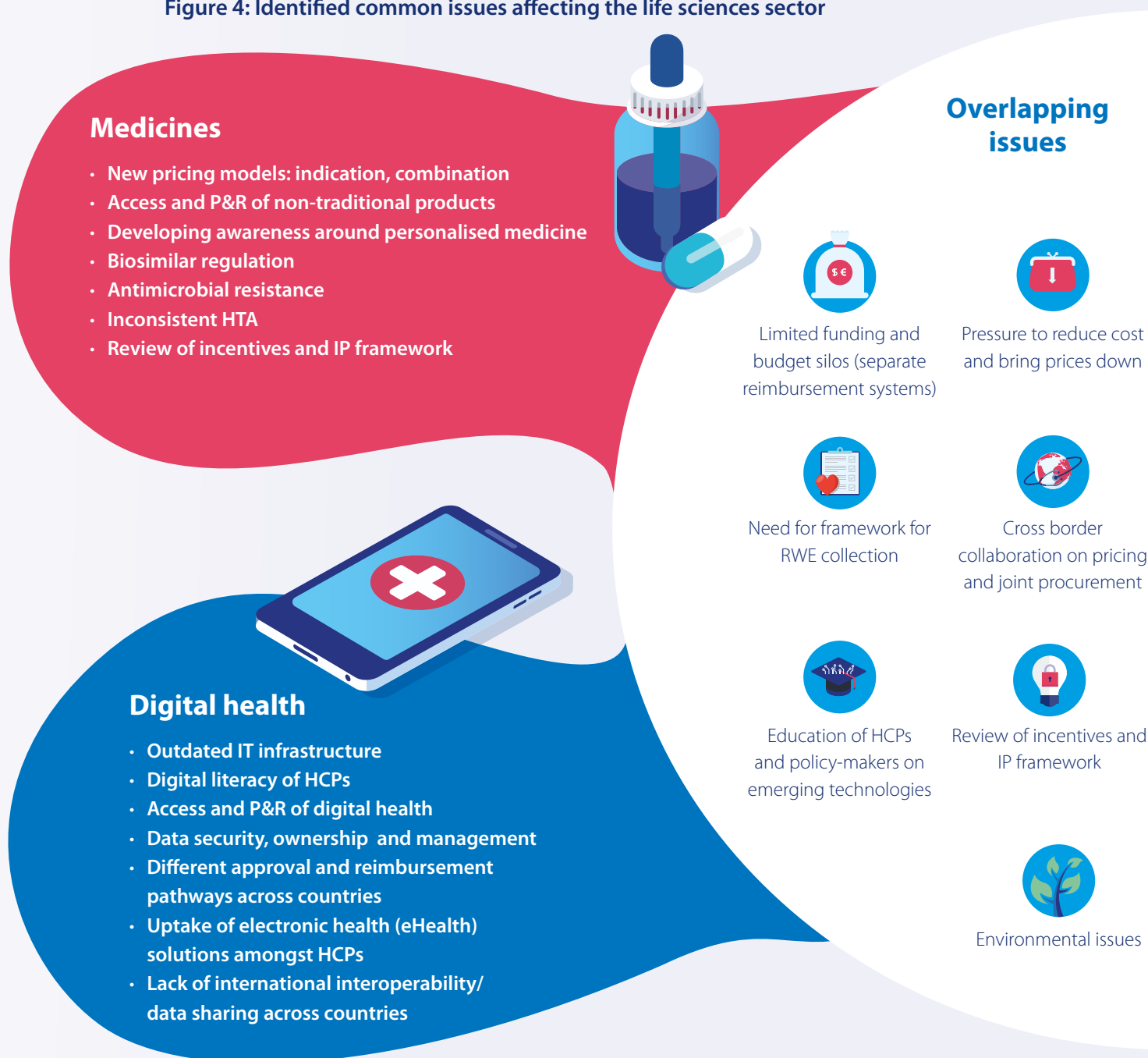
39 Interview with industry expert.

2.4 Overlapping issues and common challenges

As set out in the previous three sections, socio-economic, technology and policy trends affect all medical technologies and there are many common issues.

Figure 4 provides an overview of the issues affecting each area, establishing a set of common issues relevant across the life sciences sector.⁴⁰

Figure 4: Identified common issues affecting the life sciences sector



⁴⁰ Internal and external interviews confirmed that this list of issues for the four areas was a good reflection of sectoral priorities impacting life sciences in Europe.



Diagnostic technologies

- New IVDR
- New MDR – high-risk classification for in vivo diagnostics
- Limited provision of biomarkers
- Lack of resources to manage diagnosis
- Interpretation of diagnostics results



Inconsistent regulatory regime for integrated/combined technologies



Inappropriate and inconsistent value assessment frameworks



Interoperability of technologies



Immature data and health infrastructure/digital connectivity



Limitations in utilising 'Big Data' (privacy/ownership)



Impact of market consolidation



Medical devices

- Diagnostic classification within medical devices
- New set of EU regulations for medical devices
- Separate systems for reimbursement for hospital and community care sectors
- Innovative contracting mechanisms
- Clear approval/P&R pathways across medical device categories

The objective of this project is to focus on novel policy solutions for issues of importance to the entire life sciences sector that are not addressed by existing industry positions or policy initiatives. We have prioritised issues where:

- Technologies have different policy frameworks today, but common policy challenges across segments suggest convergent policy debate; and

- Novel challenges are emerging due to the integrated, combined use of some healthcare technologies across the life sciences sector.

Table 5 provides a more detailed description of these overlapping issues and the extent to which they represent common policy challenges across segments or a novel challenge that is emerging due to a combination of these technologies.

Table 5: Common life sciences sector issues and need for novel policy solutions

Source: CRA analysis.



Limited funding and budget silos (separate reimbursement systems)

- There are discrepancies between how diagnostics are funded across markets, which can impede access (eg, budget silos between diagnostics and medicines in the diagnosis-related group (DRG)).
- Funding for digital health is under transition as there is no established reimbursement mechanism for this technology.
- Current payment models exclude many innovative medicines (particularly where curative) from broader payment models.



Need framework for RWE collection

- RWE collection is a necessary enabler for many innovative pricing models.
- Future pricing may relate more to tracking utilisation across different indicators to relate the RWE value to the price.



Education of HCPs and policy-makers on emerging technologies

- There is a lack of education of HCPs and policy-makers on emerging technologies, which can lead to resistance that impedes access.



Inappropriate and inconsistent value assessment frameworks

- Assessment procedures are not well developed to account for targeted treatments which are high cost but low volume.
- Managing market access for more innovative medical devices is getting more challenging. To date, the value assessment process for medical devices has not helped facilitate access.
- It is more challenging to calculate the value of the diagnostics, which in turn are important for determining treatment pathways.
- A shift towards value-based care is a key enabler of personalised medicine and digital technologies.
- Emerging technologies will need new approaches to value assessment as currently there is weak consensus of what defines value and a lack of clarity on evidence requirements.



Pressure to reduce cost and bring prices down

- Healthcare systems are under enormous pressure because funding has not kept up with the increase in societal demand and the innovations entering the market.
- Increasing use of tendering for innovative medicines (eg, HCV).
- The price of technology is getting cheaper and the costs of diagnostics are decreasing (eg, next generation sequencing (NGS)). Consequently, this is resulting in a new wave of personalised therapies that are more expensive.



Inconsistent regulatory regime for integrated/combined technologies

- There is a lack of clear regulatory guidance for new technologies and uncertainties on responsibility (eg, should the EMA do more on eHealth).
- Regulatory framework is more reactive rather than proactive in keeping up with new technology developments or changes (eg, biosimilars).





Limitations in utilising 'Big Data' (privacy/ownership)

- Data privacy issues create barriers for innovative payment models that take into account patient outcomes and the use of RWE.
- Restrictions on data can limit the potential for digital health platforms to provide value added services.



Interoperability of technologies

- The separate development of technologies and lack of co-development guidelines for medicines in combination with other technologies can limit interoperability.



Immature data and health infrastructure/ digital connectivity

- Technology is moving faster than people. There are delays in adopting new technologies, as healthcare providers have low awareness or limited skills to use them in practise.
- Many innovative products entering the market have requirements for specialised healthcare infrastructure and diagnostic testing.
- Some markets within Europe lag behind because they do not have the necessary platforms to use this new technology.



Review of incentives and IP framework

- Robust IP protection is important for bringing new innovation to the market, as the time between first-in-class and fast followers is becoming shorter due to increasing disruptive technologies.



Impact of market consolidation

- Technology and data are being leveraged to digitalise treatments by new players entering the sector (eg, IBM, Google) either in collaboration, or independently, leading to changes in business models.
- Future technology combinations may require legal changes in P&R systems in order to negotiate joint value propositions.
- The development of new technologies that shift the treatment paradigm may change roles and responsibilities across the value chain.



Cross border collaboration on pricing and joint procurement

- Governance around cross-border pricing collaboration (eg, joint price negotiations) is challenging, with different healthcare structures, budgets, priorities and purchasing power.



Environmental issues

- Debates surrounding sustainable disposal of medicines and medical devices – environment practice should be in line with new standards on environmental protection but some argue that this may affect the development process and slow down access to innovation.
- Environmental proposals should be risk-based and take the life cycles of healthcare products and processes into account.



Issues related to:

- Medicines
- Diagnostic Technologies
- Medical Devices
- Digital Health

Scores regarding:

- Novel challenges due to convergence of technologies
- Converging policy debate across the sector

Strength of score:

- None
- Low
- Moderate
- High

As a result of this exercise, we identified four main types of issues shared across four types of technology (sectors) and where novel policy solutions are lacking:



Limited funding and budget silos



Inappropriate and inconsistent value assessment frameworks



Inconsistencies across restrictions of data usage, interoperability and privacy issues



Impact of market consolidation

3. Developing novel policy proposals

For each of the shared challenges facing innovators across the life sciences sector, we first developed strawmen policy solutions and then tested these with industry and external stakeholders.

The objective in each case was to consider the merits of policy recommendations that encourage an integrated approach to the health life sciences sector, and to determine whether an integrated or a differentiated approach was more appropriate.



Overcoming limited funding and budgeting issues



Introducing robust data protection and security



Tailored approaches to value assessment across different technologies



Adapting to new healthcare business models

3.1 Overcoming limited funding and budgeting issues

While healthcare expenditure continues to grow (section 2.1), spending on pharmaceutical technologies has remained moderately stable. Despite this, the impact of effectively combining medicines and other medical technologies may play a huge role in containing healthcare expenditure in the future. Health systems and health providers often tend to categorise spending in groups or identifiable 'silos' for budgetary control, often based on the organisation providing the care or the financing arrangements.⁴¹

The problem of silo-based budgeting can be seen in the example of oncology, which incorporates all the life science technologies. Identifying the right cancer treatment involves diagnostics (human epidermal growth factor receptor 2 (HER2), breast cancer gene (BRCA), programmed cell death protein 1 (PD-1) testing), and patients are treated with innovative medicines administered through medical devices and use e/mHealth solutions to track their outcomes. The budget within each type of technology also varies. Equally, treating a patient successfully can mitigate future costs but also present savings beyond the healthcare budget.⁴² This will only become more complex with next generation sequencing, gene and cell therapies and use of RWE.

All these technologies are subject to silos – within hospital departments and organisations, between government departments (central and local), health sectors and social care, the private sector and public and voluntary sector. However, this issue is not just illustrated across different technologies. In addition to budget silos, traditional P&R processes for medicines are challenged by innovative products that can be used across and in combination with multiple indications.

Pharmaceutical innovation should always start with the indication that can deliver the best value to patients and where there is the greatest level of unmet need. However, these traditional processes disincentive companies to research and market medicines that could provide clinical value in a broader range of indications. Given the trends in the personalisation of treatments and greater knowledge of the underlying genetic mechanisms of diseases, medicines that target these mechanisms may provide value across several diseases, particularly in oncology. Therefore, P&R systems should not act as a barrier to access given the trends in scientific progress and innovation.



In particular, medicines coverage is subject to a rather complicated budgeting system as Member States apply various measures to influence pharmaceutical expenditure in order to contain costs linked to medicines budgets. This is exacerbated as Member States struggle to pay for high-value curative therapies, such as CAR-T and gene therapies.



There are also discrepancies in how diagnostic services and tests are funded across markets, as budgets for packaged tests can be different from the funding in state-owned laboratories.



Silo budgeting has a detrimental impact on the funding and reimbursement process for medical devices, and it diminishes incentives for manufacturers to develop innovative technologies for both community and hospital settings.



There is a lack of funding for digital health, and suppliers within healthcare providers must often battle with a fragmented marketplace with no clear route to market. Digital health funding flows are driven by projects and programmes in silos.

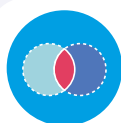
41 Hubert, M. M., Karellis, A., Sherman, M., Gill, S., Beecroft, R., and Sampalis, J. S. (2016) 'Beyond Budget Silos: Budget impact analysis of transarterial radioembolization with yttrium-90 glass microspheres for hepatocellular carcinoma from a hospital perspective'. *Value in Health*, 19(3): A308.

42 McClellan, M. and A. Thoumi (2015) 'Oncology payment reform to achieve real health care reform'. *Journal of oncology practice*, 11(3): 22330–230. <http://dx.doi.org/10.1200/JOP.2015.004655>.

The key challenges that need addressing are:

1. Silos reduce the incentive to bring technologies to market and reduce benefits to society as technologies are never launched.
2. In applying budget restrictions, there is a tendency to consider the expenditure on medicine use separately, rather than consider resource use overall. This may lead to increased consumption of other healthcare resources and prevent health systems from effectively allocating resources across the whole spectrum of care.

Innovative policy solutions to overcome limited funding and budget issues



Integrated funding models

A solution to address silos resulting from fragmented budgets is the integration of funds through pooled budgets or bundled payments. These new models disburse single payments across groups of provider entities with the aim of paying for health outcomes, which incentivises improving coordination, efficiency and effectiveness of care.

Firstly, bundled payments seek to align the interests of providers by supplying a fixed payment for all services provided for a patient with a given condition during a single episode of care, especially when multiple providers are involved in the delivery. Bundled payments for care improvement reimburses hospitals and providers based on episodes of care over time rather than individual fee-for-service (FFS) billing determined by DRG classification. This bundled payment is then distributed among all providers in a healthcare system that are involved with that patient, including hospitals and other facilities.⁴³ This payment model is already in use in certain therapy areas. In the United States, cardiovascular care is one therapeutic area where bundled payments have been most impactful as patients receive care in multiple healthcare settings (eg, hospitals, outpatient primary care, subspecialty clinics, skilled nursing facilities), and using bundled

payment mechanisms has the potential to substantially improve care coordination and generate savings.⁴⁴ Under this model, a participating provider is incentivised to provide efficient care, reducing the number and cost of services contained in the bundle. A similar model has been adopted in the Netherlands, where insurers pay a bundled payment to a principal contracting entity – the care group – to cover a full range of diabetes-care services for a fixed period of 365 days. In order for bundled payments to cover providers of different technologies in primary care,⁴⁵ to help support the application of bundled payments, one option could be to link the bundled payment to outcomes rather than treatments.⁴⁶ This would be merging outcome-based agreements with bundle payments to the healthcare provider and is technology agnostic, meaning it actually incentivises the use of innovative technology combinations that deliver better value and health outcomes.⁴⁷

Second, outsourcing care to a third party or allowing payments between healthcare providers is one way of conducting bundled payments. This allows the upfront cost to be paid by a third party provider and can allow a project to be completed more efficiently or even make it a possibility in the first place. In health, this has largely taken the form of outsourcing treatment for certain chronic diseases – such as diabetes – to private ‘integrated care’ groups which manage the entire care

43 Sutherland, J., and Hellsten, E. (2017) ‘Integrated Funding: Connecting the Silos for the Healthcare We Need’. & Deloitte (2016). ‘The evolution of oncology payment models: What can we learn from early experiments’. <https://www2.deloitte.com/content/dam/Deloitte/us/Documents/life-sciences-health-care/us-lshc-evolution-of-oncology-payment-models.pdf>.

44 Shih, T., Chen, L. M., and Nallamothu, B. K. (2015) ‘Will bundled payments change health care?: Examining the evidence thus far in cardiovascular care’. *Circulation*, 131(24): 2151–2158.

45 Struijs, J. (2015) How bundled health care payments are working in the Netherlands. *Harvard Business Review*.

46 Spinks, T. et al. (2017) ‘Development and Feasibility of Bundled Payments for the Multidisciplinary Treatment of Head and Neck Cancer: A Pilot Program’. <http://dx.doi.org/10.1200/JOP>. See also, Interview with academic expert.

47 Interview with academic expert.

pathway at a lower cost.⁴⁸ However, there are a number of elements that should be considered before potential application of this solution. For example, in a disease area with a limited number of players and a relatively small patient population, this mechanism could lead to increased costs because the sunk costs of using a third party may be too high. Therefore, in the case of rare diseases, this may not be an appropriate mechanism to overcoming limited funding and budget issues.

Third, and finally, another model consists of combining funds from different organisations through ‘pooled’ budgets, allowing payments between healthcare providers to purchase integrated support to achieve shared outcomes. This will enable organisations to build on previous joint working experience in order to fund truly integrated care services.⁴⁹

There are multiple challenges associated with these approaches, because budget holders and policy-makers are often more concerned about cost containment and saving healthcare budgets today, rather than taking a long-term sustainable view.



Multi-annual budgeting

Policy-makers and decision-makers typically organise healthcare funding for a single twelve-month period. A different approach to enabling access to novel therapies would be multi-annual budgeting that takes into account a longer time horizon, eg, in a broader context of health targets. Many novel therapies lead to accrual of patient value incrementally over the long term (eg, oral hepatitis C regimens or proprotein convertase subtilisin/kexin type 9 (PCSK9) inhibitors in cardiovascular care).⁵⁰ This approach to budgeting could allow for innovative payment models whereby the cost of treatment is not provided through a full upfront payment, but rather paid

for in instalments over time. This would be particularly beneficial for enabling patient access to transformative and gene therapies for rare diseases. This payment process could also be linked to outcomes and the collection of RWE to ensure payments are based on clinically observed results.



Budgeting for long-term spending

Sound budgeting practices are an important component in maintaining fiscal sustainability and efficiency of long-term care spending. There is room to improve and widen the scope of the projection methodologies used (while recognising the limitations posed by the uncertainty involved in such long-term projections).⁵¹ For example, budgeting for long-term spending could be coupled with national horizon scanning to allow the identification of new and emerging healthcare technologies that are likely to have significant impact, in addition to early assessments of their likely impact on provision of healthcare and healthcare outcomes. This budgeting competency, however, is only applicable at Member State level.



Monitoring and benchmarking the performance of budget holders

Beyond funding issues and budget silos, there is also a need to ensure adequate monitoring and benchmarking of the performance of health systems and how budgets are being utilised. Measuring comparative performance of health budgets (and how they lead to better health outcomes) should set the scope for improvement and guide policy-making.⁵² In addition, indicators on outcomes in terms of population health should be further developed. Monitoring and benchmarking can be

48 Brigid Pike and Deirdre Morgan (2014) ‘The integration of health and social care services’, Health Research Board. http://www.hrb.ie/uploads/tx_hrbpublications/The_integration_of_health_and_social_care_services_2014.pdf.

49 Lillie Wenzel, Laura Bennett, Simon Bottery, Richard Murray, Bilal Sahib. ‘Approaches to social care funding options’. The King’s Fund, https://www.health.org.uk/sites/health/files/Approaches-social-care-funding_1.pdf.

50 Licking E, Garfield S (2018) ‘A Road Map to Strategic Drug Pricing’. In Vivo – *The business and medicine report*, March 2016. (34/No 3), retrieved July 2018 at [https://www.ey.com/Publication/vwLUAssets/ey-in-vivo-a-road-map-to-strategic-drug-prices-subheader/\\$FILE/ey-in-vivo-a-road-map-to-strategic-drug-prices-subheader.pdf](https://www.ey.com/Publication/vwLUAssets/ey-in-vivo-a-road-map-to-strategic-drug-prices-subheader/$FILE/ey-in-vivo-a-road-map-to-strategic-drug-prices-subheader.pdf).

51 Interview with a representative from the European Commission (DG ECFIN).

52 European Commission (2016) ‘Joint Report on Health Care and Long-Term Care Systems and Fiscal Sustainability’, Volume 1. Economic and Financial Affairs Economic Policy Committee ISSN 2443-8014 – Institutional paper 037. October 2016.

used to identify more specific issues and see if there are areas with recurrent overruns and leftover budget, which can help the central commissioner allocate funds more effectively.⁵³

Monitoring and controlling expenditure with specific budgetary tools; using performance-based budgeting and spending reviews to a wider extent to improve the quality of spending, introducing spending targets and spending ceilings, as well as budget buffers and early-warning mechanisms, can give fiscal and health authorities more tools to prevent arbitrary cost-cutting that does not serve patients and health system objectives.⁵⁴ Beyond budgets, measuring performance means monitoring progress towards system goals – often defined in abstract terms as health, responsiveness and equitable financial protection.⁵⁵ Monitoring performance implies identifying and measuring concrete outcomes that reflect actual progress in their direction, as assessment frameworks aim to do. Performance assessments of services and specific providers can help sustainably develop budgets accordingly. This can also help inform the quality of the budget.⁵⁶



Conducting holistic value assessment

A major impediment to developing policies is evidenced by the relative value of new healthcare technologies. This further emphasises the need for national policies to promote value assessment frameworks such as HTA.

However, even well-established HTA systems such as those in France, the UK and Germany do not conduct holistic value assessments.⁵⁷ Many stakeholders have argued that there is a need to think about the broader definition of 'value' in the context of value assessment frameworks, and to what extent this value should take into consideration wider social and economic benefits.⁵⁸ HTA bodies should be encouraged to adopt a societal perspective considering the impact of treatments on broader societal costs, such as productivity and social care costs.⁵⁹ For example, too little weight is currently given to the evidence from different stakeholders, particularly patients. As many systems still focus on healthcare costs rather than societal costs, the focus tends to be on short-term costs and outcomes rather than longer-term benefits. Even where these are incorporated, evidence on gains in productivity are rarely given much weight. In systems where a wider assessment of costs and benefits is undertaken, the assessment of budget impact often significantly impacts decision-making. Access can be challenged where systems have disproportionate focus on cost-effectiveness and quality-adjusted life year (QALY) thresholds, leading to very rigid willingness-to-pay thresholds as a means of controlling budgets. This has implications for the role of value assessment, the methodology applied and the process.

53 Interview with European Commission (DG ECFIN).

54 European Commission (2016) 'Joint Report on Health Care and Long-Term Care Systems and Fiscal Sustainability', Volume 1. Economic and Financial Affairs Economic Policy Committee ISSN 2443-8014 – Institutional paper 037. October 2016.

55 Kutzin, J. (2013) 'Health financing for universal coverage and health system performance: concepts and implications for policy'. Bulletin of the World Health Organization. 91:602-611. <http://www.who.int/bulletin/volumes/91/8/12-113985/en>.

56 Interview with a representative from the European Commission (DG ECFIN).

57 Charles River Associates (2014) 'A comparative analysis of the role and impact of Health Technology Assessment: 2013 Final report'.

58 Interview with European Cancer Patient Coalition (ECPC).

59 Interview with industry association.

3.2 Tailored approaches to value assessment for different technologies



For medicines, efforts are ongoing to integrate different approaches to value assessment and to improve methodological processes and evidence generation for HTA. However, evolution of methodologies typically lags behind the pace of innovation (eg, value assessment frameworks for gene therapies).



Value assessment for diagnostics is not always integrated with the assessment of associated therapies, and there is no uniform approach to the role of HTAs to inform coverage, access and utilisation of molecular diagnostics (MDx). This varies significantly across countries.



For medical devices, HTA organisational structures, processes and scientific methods vary considerably across countries.⁶⁰ The access model is decentralised and Member States' decisions are made at differing times and for differing purposes. For many medical devices, evidence from robust, randomised controlled trials (RCT) is often limited or unavailable at the time of launch. Adopting anything like a pharmaceutical paradigm, based on an expectation of multiple RCT being available at the time of launch, could have significantly negative consequences for access to many new medical devices.



Reimbursement of eHealth solutions is predicated on achieving quality outcomes against evidence-based standards, but HTA in this domain is embryonic.

The use of HTA for different healthcare technologies has been developing over the last thirty years. That the use of HTA varies by type of technology is consistent with fundamental differences in evidence and life cycle. Medical devices have a shorter life cycle, with value heavily dependent on associated procedures. Procurement and hospital assessment have played large roles, with assessment often ex-post. Equally, diagnostic technologies are sometimes not formally assessed but in the case in which they are, this can be integrated with associated medicines in some markets. The assessment regime therefore depends on whether they are a companion to complementary diagnostics and the significance of the investment. The application of HTA to digital health is only now developing as this sector progresses. Therefore, maintaining different approaches that are appropriate for the different technologies should be the overriding principle.

Although it is possible to assess clinical added value and cost effectiveness at marketing approval for medicines (at least for some products), for medical devices, the appropriate time to conduct a clinical assessment for medical technologies is often significantly after CE marking (a certification mark that meets high safety, health and environmental protection requirements), when the effectiveness data is available to demonstrate the full value of the technology. Given these important differences and distinct roles, HTA and CE regulations should remain disentangled for medical devices and hence not harmonised with pharmaceutical processes. However, there are also other situations, eg, companion diagnostics for personalised medicine, where assessing the benefits and cost associated to the diagnostics needs to occur while the medicine is being assessed (as otherwise this will result in inconsistency and delays).

60 'Health technology assessment of medical devices: a survey of non-European union agencies'. *International Journal of Technology Assessment in Health Care*, 31:3 (2015), 154–165.

Innovative policy solutions to tailor approach to value assessment for different technologies

A number of specific innovative policy solutions can be considered in order to appropriately tailor the approach to value assessment for different technologies across countries.



Continued development of early dialogue and horizon scanning

The importance of an 'early' HTA process has been identified as important for medicines and diagnostic technologies such as predictive biomarkers and even eHealth. It is argued that when HTA analyses are performed earlier, during research and development, they may prevent the development of technologies 'unlikely to ever provide sufficient added value to society, and rather facilitate translation of the promising ones.'⁶¹ Similarly, linking HTA more closely to a process of horizon scanning could support the prioritisation of future assessment pathways, a process in which stakeholders need to be involved. Horizon scanning should be about informing the dialogue with the manufacturer. This could be useful when thinking of specific companion diagnostics.⁶²



Developing appropriate value assessment mechanisms and methodologies

The use of HTA must continue to evolve to reflect novel technologies. Value assessment and HTA serve different purposes in the context of different technologies and this often requires different ways to assess value. Different approaches to value assessment include:

1. HTA, which often adopt a strict adherence to the hierarchy of evidence, demanding that technologies

are supported by evidence from robust, randomised controlled trials;

2. Value-based procurement enabling value-based healthcare which takes into account the wider patient and societal outcomes together with the life cycle cost of healthcare delivery and services; and
3. Value-based arrangements including the use of managed entry agreements and risk sharing schemes which are based on patient outcomes.

Identifying the correct instruments to conduct the value assessment is critical. In utilising multi-decision criteria, the value new technologies can bring to patients can be established, as well as supporting different departments within healthcare systems and the wider society. The value assessment process itself could then be made more robust by utilising real world evidence.

This could also involve developing an approach for cases where a number of technologies work more closely together. This should be based on flexibility and pragmatism in evidence development, streamlined timelines and inclusion of a broad scope of benefits. This would allow for the relevant stakeholders given the technology to be involved in implementation support. In the cases where technologies are 'co-dependent', meaning that their use needs to be combined (either sequentially or simultaneously) to achieve or enhance the intended clinical effect of either technology. Consideration should be given to how the HTA process can be hybridised. This would involve adapting the timing and HTA methodology depending on the different technologies included.⁶³ Such an approach would allow greater consistency and improved timeliness, whilst allowing the standalone approach applied to each technology to continue (reflecting their own specific requirements). It would be important to link this to integrate horizon-scanning.

61 Miquel-Cases, A., Schouten, P. C., Steuten, L. M., Retèl, V. P., Linn, S. C., and van Harten, W. H. (2017) '(Very) Early technology assessment and translation of predictive biomarkers in breast cancer'. *Cancer treatment reviews*, 52: 117–127.

62 Interview with academic expert.

63 'How to assess co-dependent technologies'. Dr. Mira Pavlovic-Ganascia, Deputy Director for HTA, Haute Autorité de Santé, France.



“Thanks to scientific progress fewer patients die of cancer than ever before. A strong and thriving life sciences sector in Europe is good not only for the future of innovation in Europe generally, but also because it directly benefits the lives of Europeans themselves. The next Commission should focus on re-launching the EU’s life sciences strategy so that it targets diseases, such as cancer, where it can make a real difference to the lives of Europeans.”

Deepak Khanna
Senior Vice President and Regional President (EMEAC)
MSD Oncology

3.3 Introducing robust data protection and security

There will be dramatic changes in the way healthcare is delivered over the coming decades. Looking at digital health from a broader perspective, one of the most promising developments for future work will lie in the emergence of the Internet of Things (IoT) paradigm, of which health services and applications including mHealth will be a key component.⁶⁴

From this standpoint, it will be increasingly difficult to consider eHealth-specific requirements individually and separately from other similar IoT applications that will require robust security, privacy, authentication, coverage, bandwidth, quality of service and horizontal interoperability.⁶⁵ Data privacy issues create barriers for innovative payment models for new technologies that take into account patient outcomes and the use of RWE.



The last two decades have seen a surge in the amount of data generated and collected throughout the healthcare value chain, as well as the introduction of new platforms, tools and methodologies in storing, structuring and analysing ‘Big Data’. The healthcare sector handles and stores sensitive personal data in the form of medical records or research data (clinical trials, clinical investigations, epidemiological research, patient registries, etc.).



Intelligently designed algorithms and microelectronics found in sensors and wearables are expanding the mHealth applications of smartphones by effectively turning them into point-of-care diagnostic tools – security and privacy of personal data will become a critical concern.⁶⁶



Many innovative technologies rely on the use of patient data. A range of stakeholders, including patient organisations, gather and use patients’ data.



Equally, the patient’s fundamental right to the protection of their health data is an important issue in diverse healthcare settings, including care given through eHealth or across borders.

64 Hassanalieragh, M., et al (2015) ‘Health monitoring and management using Internet-of-Things (IoT) sensing with cloud-based processing: Opportunities and challenges’. In *Services Computing (SCC)*, 2015 IEEE International Conference on (pp. 285–292). IEEE.

65 AmCham EU (2016) AmCham EU’s response on the consultation on next phase of EU–US cooperation in eHealth/Health IT Roadmap – Workstream: International Interoperability.

66 Vashist, S. K. (2017) Point-of-Care Diagnostics: Recent Advances and Trends.

Innovative policy solutions to introduce robust data protection and security

The General Data Protection Regulation (GDPR) (EU 2016/679) sets out to harmonise data protection legislation in the EU.⁶⁷ For example, patients can request data from their pacemakers or electronic health records (EHR) and even request that such data be transferred to another provider. While the GDPR does not impose an obligation on controllers to maintain technically compatible systems, companies will have to explicitly inform users about the right of data portability. Where technically feasible, the data subject has the right to have personal data transmitted directly from one controller to another.⁶⁸

The GDPR aims primarily to give control to citizens and residents over their personal data and to simplify the regulatory environment for international business by unifying the regulation within the EU.⁶⁹ A stakeholder we interviewed considered that the GDPR provides strict standards on the use of data and this is being transposed into every Member State's legislation. It has therefore been argued that there are more than enough policies in place at EU level to ensure robust security measures and privacy of patient data.⁷⁰ Beyond data protection and security, there remain other issues and policy solutions, which we introduce below.



Building horizontal interoperability

Market fragmentation in eHealth is aggravated by the lack of technical and interoperability across Member States. The health information and communication systems and standards currently used in Member States are often incompatible and do not facilitate access to

vital information for provision of safe and good quality healthcare across different Member States.⁷¹ When it comes to data ownership and sharing of data – in most cases, it is the Member States who own all the data.⁷² It is in the interests of patients that healthcare systems serve, that their data is appropriately securely shared amongst stakeholders (eg, to determine patient populations and measure outcomes).

Developing clear and practicable interoperability for information shared among different health professionals and among different healthcare settings (countries and systems) is needed, provided that these professionals guarantee a sufficient level of data protection.

In fact, the Commission has recently (April 2018) adopted a communication setting out a plan for EU action on digital health.⁷³ This covers three objectives:

- Enabling citizens' secure cross-border access to their electronic health records and the possibility of sharing their records across borders;
- Facilitating the use of larger data sets through a shared European data infrastructure; and
- Providing digital tools that enable citizens to manage their health more actively within integrated care systems.

For the plan to be most effective, the Commission should promote the adoption of internationally recognised standards for interoperable electronic health records across Member States. This approach will provide the basis for the exchange of e-prescriptions and electronic patient summaries, enabling the cross-border exchange of patient information. Voluntary coordination in sharing data and resources for disease prevention and research

67 The GDPR has a twofold aim of affording citizens increased protection and empowerment over personal data, while also enhancing the circulation of those data within the EU. The GDPR is intended to provide a modern regulatory framework for the collection and processing of personal data, and therefore will profoundly impact the medical technology industries, including eHealth.

68 Vollebregt, E. (2016) 'The new General Data Protection Regulation impact on medical devices industry, ehealth-law-and-policy'. Volume: 3 Issue: 7 (July 2016).

69 Presidency of the Council: Compromise text. Several partial general approaches have been instrumental in converging views in Council on the proposal for a General Data Protection Regulation in its entirety. The text on the Regulation which the Presidency submits for approval as a General Approach appears in annex'. 201 pages, 11 June 2015. <http://data.consilium.europa.eu/doc/document/ST-9565-2015-INIT/en/pdf>.

70 Interview with trade association.

71 Official Journal of the European Union (2008) Commission recommendation of 2 July 2008 on cross-border interoperability of electronic health record systems (notified under document number C(2008) 3282) (2008/594/EC).

72 Interview with trade association.

73 European Commission (2018) Press releases: database – European Commission – Fact Sheet. The digital transformation of healthcare – Brussels, 25 April 2018.

can support further personalisation of treatment and better anticipate epidemics.

One area where there is a strong need for greater harmonisation and cooperation across Member States is in rare diseases and rare cancers.⁷⁴ Many of those affected by rare or complex conditions do not have access to diagnosis and high-quality treatment. Expertise and specialist knowledge may be scarce because patient numbers are low.⁷⁵ European Reference Networks (ERNs) provide a good example of a solution to help overcome interoperability across Member States, particularly around diagnosis. ERNs are virtual networks involving healthcare providers across Europe which aim to tackle complex or rare diseases and conditions that require highly specialised treatment and a concentration of knowledge and resources. While there has been some work on developing ERNs, patients and HCPs should be able to more readily share patient information with other HCPs across borders to get a second opinion on diagnosis and ultimately help improve diagnostic efficiency.⁷⁶



Regulatory clarity and legal certainty

There is a need to develop regulatory clarity and legal certainty for the digital health environment, promoting proportionate and risk-based approaches that help health innovation and interoperability to evolve. The EU digital agenda will be important in addressing these issues. There are also EU initiatives to develop industry-led, EU-wide codes of conduct, such as the ongoing work to create a privacy code of conduct on mHealth apps, and the recently launched working group to develop guidelines for health apps data to be reliably linked to EHRs. The development of certification mechanisms that can enhance citizens' trust in eHealth services and applications, can aim to facilitate their effective uptake in clinical practice and across borders.



Education and training

There is general consensus from our interviews that upgrading the level of awareness and education of health professionals towards the use of new healthcare technologies is vital to ensure its adoption and proper implementation.⁷⁷

There are two elements to this. Firstly, many patients and health professionals are unaware of the benefits of the electronic health record and other eHealth and mHealth applications. This lack of adequate information hinders the acceptance of eHealth solutions by both patients and health professionals and can create disparities across Member States where this awareness is lower.

Secondly, there is a growing need for education and appropriate training programmes to improve eHealth and mHealth skills, and ultimately enhance literacy in ICT. As digital technologies continue to introduce fundamental ways that healthcare is delivered, education programmes need to target general healthcare professionals and constantly be evolving. Good ICT education is a critical success factor for building 'Big Data' infrastructure. However, this is much broader than just data: the entire healthcare system needs an upgrade in terms of knowledge and understanding of new scientific and technological trends, including the use of AI.⁷⁸ As digital innovation continues, this will support future uptake of real-time patient monitoring and data transfer.

Similarly, there is also a need to promote greater understanding of the legal aspects around data sharing, so it is clear to HCPs what the restrictions are, ensuring that stakeholders are not undertaking unnecessary precautions in protecting patient data. HCPs have a tendency to restrict data sharing and cross institution collaboration because they think there are legal barriers in doing so. There should be education and training on ensuring there is an appropriate balance between security of data and HCPs understanding of what can

74 Interview with ECPC.

75 European Commission (2018).

76 Interview with ECPC.

77 Interview with trade association, interview with European Commission, interview with academic experts, interview with patient association.

78 Interview with representative from the European Commission (Health systems, medical products and innovation).

be shared.⁷⁹ It was suggested in an interview that one of the biggest issues facing many healthcare systems is the process for determining the value of a new technology before it has actually been implemented in the real

world. While some technologies are expensive, many are seeing a reduction in cost, in parallel with the creation of massive efficiencies.



“*The life sciences industry is committed to turning research into innovation, contributing to Europe’s knowledge-based economy and competitiveness while improving the quality of life for European citizens. The current scientific breakthrough we are witnessing will have the potential to redefine the treatment paradigm and cure disease, ensure a more personalised and individualised approach as well as regenerate organs. The extensive range of new medicines available and in the pipeline would not be here without world class IP protection in Europe. Innovation lies at the heart of our sector and we call upon the next Commission to ensure a strong EU Life Sciences and Industrial strategy that guarantees the key conditions, including strong IP protection, are in place to continue to develop and deliver innovation to improve people’s lives and our economies.”*

Rich Buckley
Vice-President Global Corporate Affairs
AstraZeneca

79 Interview with patient association.

3.4 Adapting to new healthcare business models

The types of companies involved in the provision of health life sciences and the way they organise themselves is changing. This can be seen in the medical device industry, which is evolving due to advances in technology and convergence with eHealth, resulting

in new partnerships between medical and information technology companies.⁸⁰ Changes in the treatment paradigm towards more personalised, patient-centred care is reshaping the relationships between providers of medicines and those of diagnostic technologies.



In pharmaceuticals, scientific and medical breakthroughs are changing the process by which medicines are delivered (eg, the use of combination therapies in oncology or the delivery of CAR-T treatment) and the manufacturing process, with personalisation leading to a dynamic process where laboratories use a patient's own genetic material to develop treatment. As a whole, the sector is increasingly focusing on outcomes and real-world data – the 'Beyond the Pill' approach.⁸¹



Personalised medicines increase the importance of diagnostics. Whether these companion diagnostics should be specific to a particular medicine or used by a class affects the relationship between diagnostic technologies and medicine producers. Future combinations of pharmaceuticals or combinations of different technologies (eg, diagnostics) may require legal changes in order to negotiate joint value propositions through the pricing and reimbursement process.



Emerging healthcare technologies have converged and changed the treatment paradigm in different diseases. The utilisation of real-time data monitoring to assist patients, caregivers and healthcare professionals is likely to be increasingly important. There are also some examples of new types of collaboration between medical device and pharmaceutical companies to enable more comprehensive healthcare approaches for patient treatments and to accelerate the introduction of precision medicine for diseases like cancer.⁸²



Technology and data are being leveraged to digitalise treatments, provision of care, and disease management. Similarly, the growing importance of real-world data and real-time monitoring in both disease management and innovative contracting for reimbursement relies on new competencies within the sector. Tech giants such as Google, Apple and Microsoft are investing heavily in health technologies, developing technology aimed at saving lives, from collaborations with health system to diagnostic devices and programmable biological cells.⁸³

The development of these new technologies has the potential to shift the treatment paradigm for many diseases, but this also changes the roles and responsibilities through the value chain. In particular,

the role of data analytics capabilities is increasing.⁸⁴ It is likely that we will see new business models emerging as the roles of industry and healthcare providers change.

80 EY. Medical technology report 2016: Pulse of the industry.

81 Deloitte. 2016 Global life sciences outlook - Moving forward with cautious optimism.

82 Kubala M (2018) 'How pharma and medtech collaborations fuel progress', prescouter.com, accessible at <https://prescouter.com/2018/04/pharma-medtech-collaborations/>.

83 Daniel B (2018) Here's what Google, Apple and Microsoft are up to in healthcare, UK Tech News, accessible at <https://www.uktech.news/news/google-microsoft-apple-samsung-tech-giants-healthcare-20170323>.

84 KPMG (2016) Collaboration – The future of innovation for the medical device industry.

Innovative policy solutions to adapt to new healthcare business models

Europe needs to remain globally competitive. If Europe is to deliver the benefits of converging technologies for patients and the healthcare system, and if life sciences are to remain an important engine for growth and employment in the European economy, new innovative policy solutions will be required. The pharmaceutical sector alone employs some 745,000 people in Europe and generates three to four times more employment indirectly (upstream and downstream) than it does directly.⁸⁵ These converging technologies may require changes to laws and regulations to encourage innovative activity, allow new partnerships to flourish and be flexible enough so that new technologies are available to patients as rapidly as possible. In order for Europe to remain a competitive location for life sciences, new solutions should address the challenges surrounding the development and testing of new technologies as well as their commercialisation.



A joined-up approach incorporating the entire life sciences sector

As set out in other policy areas, the convergence of technologies offers large benefits to patients but there are challenges in terms of how they are funded (and the problem of budget siloes), how they are assessed (and the process for HTA) and how they operate together to take advantage of the advances in information technology (data security).

As noted above, scientific and medical breakthroughs are changing the process by which medicines are delivered. These commercial challenges are mirrored by challenges during product development: namely issues associated with support for basic research, evidence collection, patient participation and the regulatory process.

To create an environment that encourages innovation, a joined-up approach is needed that focuses on integration of R&D, healthcare system sustainability and incentivising innovation through robust IP protection in the European life sciences industry. It was argued that whilst Europe has a robust and competitive research environment to ensure the discovery of new products, there remain challenges in trying to secure capital investment in the EU compared to the US in order to develop the product and bring it to market.

The Innovative Medicines Initiative (IMI), the world's largest public-private partnership (PPP) in life sciences, has been working to improve health and wellbeing – by speeding up the development of, and patients' access to, next-generation vaccines, medicines and therapies, especially for areas of unmet need. Ten years from the start of IMI, greater efforts have been made to create a new life sciences ecosystem, integrating more stakeholders, but also addressing the wider set of health technologies and to foster inter-sectoral collaboration.⁸⁶ For example, IMI is integrating even more digital technology into its projects. One of them, PROACTIVE, has sought to develop a tool that would remotely measure patient-reported outcomes. That gives rise to ideas around decentralised clinical trials, with new technologies and mobile devices that could track patient outcomes in clinical trials and assessments without people having to go into clinical research centres.⁸⁷

To date, industrial strategy has been left to Member States.⁸⁸ This has to change. The most recent European Commission pharmaceutical industrial strategy proposal was developed over five years ago, and more recently Directorate-General for Internal Market, Industry, Entrepreneurship and SMEs (DG GROW's) Communication on a renewed EU industrial policy strategy makes no reference to the life sciences sector.⁸⁹

85 EFPIA (2017) The Pharmaceutical Industry in Figures Key Data 2017.

86 Celebrating ten years of the Innovative Medicines Initiative.

87 Innovative medicines initiative – Project Factsheets: PRO-active – Physical Activity as a Crucial Patient Reported Outcome in COPD – <https://www.imi.europa.eu/projects-results/project-factsheets/pro-active>.

88 For example, the Danish life sciences growth plan, March 2018 <https://em.dk/english/news/2018/03-06-life-science>; UK government 'Industrial strategy: Life Sciences' August 2017, <https://www.gov.uk/government/publications/life-sciences-industrial-strategy>; French CSIS agreement, July 2018 <https://www.leem.org/sites/default/files/2018-07/CSIS-leem-Press-release.pdf>.

89 European Commission (2014) 'Pharmaceutical Industry: A Strategic Sector for the European Economy'. http://ec.europa.eu/growth/content/pharmaceutical-industry-strategic-sector-european-economy-0_en. See also, European Commission (2017) Communication from the European Commission to the European Parliament, The European Council, The Council, The European Economic and social committee, The committee of the Regions and the European Investment Bank. Investing in a smart, innovative and sustainable Industry A renewed EU Industrial Policy Strategy – COM/2017/0479 final.

This should be renewed, taking into account all the challenges facing medicine, medical devices, diagnostic technologies and digital health.



Industry, partnerships and patient relationships

The life sciences industry has made significant progress in delivering treatments to the healthcare system, but it faces significant limitations on the relationship it can have with patients and the information that can be shared between different stakeholders. This has led to complex distribution models to facilitate efficient and convenient delivery to patients, even while there is inefficient reliance on particular types of evidence generation. As technologies develop and care is increasingly personalised, collecting patient information is critical, not only for determining the best treatment, but also in terms of tracking the value delivered. Therefore, partnerships between different types of companies and healthcare systems will be ever more important, while preserving data privacy rules. This has implications for the relationships between companies and patients and between companies.

One example of this is the new model of managed equipment service (MES) which creates a partnership model to achieve improved quality of care. This consists of setting up PPP in the form of a consortium of private healthcare providers and healthcare systems to build infrastructure, services and capabilities. An MES arrangement ensures that public hospitals have access to modern health infrastructure, equipment and/or services over an agreed period, with the government

making regular, pre-arranged payments based on agreed performance parameters. Instead of huge capital outlays that would otherwise be required for building or equipping hospitals, MES arrangements offer public entities an opportunity to spread costs over the contract period, thereby allowing for long-term, sustainable budgeting.⁹⁰



A flexible and adaptable regulatory regime fit for future technologies

Regulation needs to keep up with the growth and development of technology, rather than reacting to the identification of barriers after they have developed. The need for an adaptable regulatory regime that keeps up with ongoing changes in technology is evidenced by the adoption of new legislation such as the IVDR and the GDPR. Alternative regulatory pathways need to be developed to fit the changing needs in scientific progress. In order to achieve this, regulatory systems should have constant interaction with scientific discovery, ensuring they are upgrading their understanding and assessment protocols accordingly.⁹¹ Technologies differ in terms of the nature of innovation, competition and the resulting product life cycle. A coherent approach is to look for consistency where the technologies are integrated or face the same challenges, but otherwise to allow different regulatory approaches tailored to their requirements.⁹² A joined-up approach to regulation delivers the benefit of integration while allowing the different technologies to be regulated appropriately for the benefit of patients, the healthcare system and society.

90 The World Bank (2018) How Managed Equipment Services in Kenya help the private sector contribute to healthcare – accessible at <http://blogs.worldbank.org/ppps/how-managed-equipment-services-kenya-help-private-sector-contribute-healthcare>.

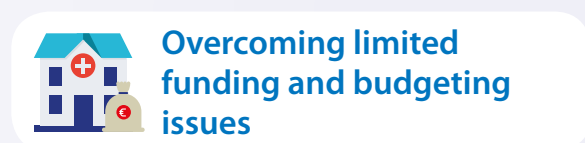
91 Interview with representative from the European Commission (Health systems, medical products and innovation).

92 Interview with a representative from the European Commission (DG GROW).

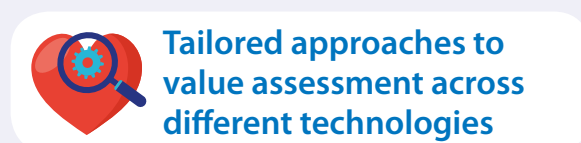
4. Conclusion and policy recommendations

Across the life sciences sector the development of new technologies has revolutionised healthcare, creating new solutions based on more integrated, combined healthcare technologies. Trends such as the personalisation of treatments and the shift towards patient-centred care have resulted in medical devices and e- and mHealth being increasingly intertwined with the use of novel therapies. The ability to use large data sets has grown, as has the development of novel approaches to deliver new treatments. Additionally, we see common challenges emerging across the different components of the life sciences industry.

We found four main types of issues shared across all sectors where novel policy solutions are lacking:



Overcoming limited funding and budgeting issues will become a key issue, exacerbated by the convergence in healthcare technologies. In addition to addressing the overall level of spending on healthcare technologies, the introduction of new financing arrangements to address 'silo' budgeting issues and/or budget predictability can help address the issue of limited funding or better planning for innovation. Directing the financing away from pay-per-service to policy objectives – eg, improving patient outcomes as an overall objective in a therapy area, such as diabetes care – requires the integration of funds through pooled budgets. Bundled payments can be an effective mechanism to deal with this issue. These new models disburse single payments across groups of provider entities with the aim of paying for health outcomes, which incentivises improving coordination, efficiency and effectiveness of care. Other solutions – such as multi-annual budgeting, budgeting for long-term spending, or monitoring and benchmarking the performance of budget holders – also need to be explored.



Tailoring approaches to value assessment for different healthcare technologies, including the emergence of non-traditional players, will be instrumental in ensuring equitable access to all novel healthcare technologies, especially as these are used in combination. This will require more appropriate and consistent value assessment frameworks tailored to the needs of different technologies (eg, HTA for medicines, value procurement for medical technologies), and to developments in scientific research processes (eg, ADAPT SMART).⁹³ In the cases where technologies are 'co-dependent' (used in combination, eg, personalised medicine and companion diagnostics), considerations should be given to how the HTA process can be hybridised (ie, adapt the timing and HTA methodology). This allows for greater consistency and improved timeliness whilst simultaneously allowing the standalone approach applied to each technology to continue (reflecting their own specific requirements). This will require the continued development of early dialogue and horizon scanning as well as the opportunity to use integrated processes that remove duplication and speed up patient access.

⁹³ See eg, the IMI project ADAPT SMART: ADAPT SMART is an enabling platform for the coordination of Medicines Adaptive Pathways to Patients (MAPPs) activities. MAPPs seeks to foster access to beneficial treatments for the right patient groups at the earliest appropriate time in the product life-span in a sustainable fashion. <http://adaptsmart.eu/home/>.



Introducing robust data protection and security

Introducing robust data protection and security for new healthcare technologies, and ensuring these measures are understood by healthcare providers will be key to the smooth development of digital health. Building horizontal interoperability across technology and data collection platforms will allow for greater data sharing and integration in healthcare systems. Interoperability of IT systems across Member States will also allow sharing of vital patient care data across borders, as well as greater regulatory clarity and legal certainty. The next European Commission clearly has a key enabling role to play here upgrading the general level of awareness and education of health professionals regarding the use of new healthcare technologies, but also towards data sharing protocols that will also be essential prerequisites to ensuring the adoption and proper implementation of these disruptive and patient empowering technologies.

As the life sciences sector continues to innovate, responding to today's policy challenges will be essential to ensure patients are able to access tomorrow's cures. By establishing a more integrated approach, or differentiated approach where appropriate to policy-making, this will encourage innovation throughout the sector while balancing future healthcare systems' sustainability.





Adapting to new healthcare business models

Lastly, the emergence of new healthcare business models is changing the role of the existing innovators and how they interact with healthcare providers. This will require an environment that encourages innovation, adopting a joined-up approach that focuses on integration of R&D, IP protection, life cycle manufacturing, healthcare system sustainability and fostering innovation in the European life sciences industry. It is essential for the EU to retain its global competitiveness, especially vis-à-vis the US. Building on the March 2018 Council conclusions and renewing efforts over the next legislative cycle to develop an industrial strategy that takes into account all the challenges facing medicines, medical devices, diagnostic technologies and digital health would help to foster a policy environment that can adapt to the changing needs of a new industrial health sector.

Appendix: definitions and interviews

Table 6: Medicines, medical devices, diagnostic technologies and digital health based on EU definitions

	<p>Medicines</p> <p>Any substance or combination of substances presented as having properties for treating or preventing disease in human beings; or</p> <p>Any substance or combination of substances which may be used in or administered to human beings either with a view to restoring, correcting or modifying physiological functions by exerting a pharmacological, immunological or metabolic action, or to making a medical diagnosis.⁹⁴</p>
	<p>Diagnostic technologies</p> <p>An in vitro diagnostic medical device 'is any medical device which is [...] intended by the manufacturer to be used in vitro for the examination of specimens [...] derived from the human body, solely or principally for the purpose of providing information: concerning a physiological or pathological state'.⁹⁵</p> <p>Diagnostics also refers to a process by which genetic information is used to evaluate patients at risk of developing particular diseases, or who have mutations which can be targeted by specific medicines. This includes NGS, assays for specific mutations, and gene expression profiles which characterise sections of an individual's genome.</p> <p>An in vivo diagnostic refers to molecular imaging (MI) which is a discipline at the intersection of molecular biology and in vivo imaging. It enables the visualisation of the cellular function and the follow-up of the molecular process in living organisms without perturbing them. MI is used in the field of cancer, neurological and cardiovascular diseases. This technique also contributes to improving the treatment of these disorders by optimising the pre-clinical and clinical tests of new medication.⁹⁶</p>

94 Article 1(2) of Directive 2001/83/EC – Directive 2001/83/EC of the European Parliament and of the Council of 6 November 2001 on the Community code relating to medicinal products for human use.

95 Directive 98/79/EC of the European Parliament and of the Council of 27 October 1998 on in vitro diagnostic medical devices OJ L 331 of 7 December 1998.

96 COCIR (2015) eHealth toolkit: Integrated Care: Breaking the silos - Fifth Edition May 2015.



Medical devices

Any instrument, apparatus, appliance, software, material or other article, whether used alone or in combination, including the software intended by its manufacturer to be used specifically for diagnostic and/or therapeutic purposes and necessary for its proper application, intended by the manufacturer to be used for human beings for the purpose of:

- diagnosis, prevention, monitoring, treatment, or alleviation of disease or alleviation of or compensation for an injury or handicap; and
- investigation, replacement or modification of the anatomy or of a physiological process, or control of conception [...] and which does not achieve its principal intended action in or on the human body by pharmacological, immunological, or metabolic means, but which may be assisted in its function by such means.⁹⁷



Digital health

eHealth

Refers to tools and services using ICTs that can improve prevention, diagnosis, treatment, monitoring and management. So far the digital service infrastructure for eHealth is planned to support several services:

- Cross-border patient summary service;
- ePrescriptions and eDispensations.⁹⁸

mHealth

Mobile health, or mHealth, is the provision of eHealth services and information that relies on mobile and wireless technologies. Similarly to eHealth, of which it is part, mHealth describes a broad set of technologies that can support a variety of health-related services, and is not a separate category of services in itself. Mobile technologies are utilised across the range of healthcare, social care, wellness and prevention, and form an integral part of telemedicine, telehealth and telecare.⁹⁹

97 Directive 2007/47/EC of the European Parliament and of the Council of 5 September 2007 amending Council Directive 90/385/EEC on the approximation of the laws of the Member States relating to active implantable medical devices.

98 European Commission – CFF Digital.

99 COCIR (2015) eHealth toolkit: Integrated Care: Breaking the silos - Fifth Edition May 2015.

Table 7: Literature review summary

Type	Number	Examples
Public policy documents from stakeholders	15	<ul style="list-style-type: none"> • European Commission: The Future of Health Care: deep data, smart sensors, virtual patients and the Internet-of-Humans • European Commission: Joint Report on Health Care and Long-Term Care Systems and Fiscal Sustainability • European Commission: Green Paper on mobile Health ('mHealth') • Council of the European Union: EU industrial policy strategy
Academic articles	25	<ul style="list-style-type: none"> • 'The new EU regulation on in vitro diagnostics: potential issues at the interface of medicines and companion diagnostics' (Enzmann, 2016) • 'Implementing genome-driven oncology' (Hyman, 2017) • 'Artificial intelligence in healthcare: past, present and future' (Jang, 2017)
Press articles and insight pieces	15	<ul style="list-style-type: none"> • BCG: Moving Public Procurement of Medtech Beyond Purchase Price-to Patient Outcomes • FT: Technology is the tool to spur a healthcare revolution
Trade association and market intelligence reports	23	<ul style="list-style-type: none"> • Reviewed positions of EFPIA, Medicines for Europe, MedTech Europe, COCIR and Digital Europe • AmCham EU: Improving access to medicines in the European Union • OECD: New Health Technologies: Managing Access, Value and Sustainability

Source: CRA analysis

For a full list of references, see www.amchameu.eu/lifesciences4eu

Table 8: List of internal industry interviews

Sector	Company
Medicines	Lilly; Celgene; Gilead Sciences
Diagnostic technologies	Abbott Diagnostics
Medical devices	Abbott
Digital health	IBM Watson Health

Source: CRA analysis

Table 9: List of external interviews

CRA conducted nine external interviews with European policy-makers, patient representatives, trade associations and other experts across the life sciences sector. Below is the list of individuals/organisations who agreed to be named.

The views expressed in this document are those of the authors and do not necessarily reflect the position of the organisations who took part in these interviews.

Stakeholder type	Organisations
European policy-makers	European Commission: Directorate-General Health and Food Safety (DG SANTE) Directorate B - Health Systems, Medical Products and Innovation (Andrzej Rys) European Commission: Directorate-General for Internal Market, Industry, Entrepreneurship and SMEs (DG GROW), Biotechnology and Food Supply Chain European Commission: Directorate-General for Economic and Financial Affairs (ECFIN), Sustainability of Public Finances (Santiago Calvo Ramos)
Pharmaceutical and medical technology trade associations	Digital Europe European Coordination Committee of the Radiological, Electromedical and Healthcare IT Industry (COCIR), (Nicole Denjoy, Secretary General)
Patient association	European Cancer Patient Coalition, (Lydia Makaroff, Director and Alex Filicevas, Head of EU Affairs)
Academic experts	Dr. Chris Henshall, Visiting Fellow at the Office of Health Economics

Source: CRA analysis

Today, the healthcare industry looks very different to how it did ten years ago. New technologies are transforming the way we deliver treatment and organise our healthcare systems, bringing groundbreaking benefits to patients. The growing use of integrated, combined treatment options is increasingly driving convergence across the sector: offering immense opportunities but also posing novel challenges.

As Europe moves into the start of a new legislative cycle, the time is ripe to examine the challenges and opportunities facing the healthcare sector in Europe over the next five to ten years. In 'Life Sciences for Europe: an integrated strategy for healthcare innovation (2019-2024)', we identify the common trends and challenges arising across the industry as well as those that are due to the combined use of health technologies. The report sets out a framework of innovative policy solutions for European policy-makers to consider across these key areas.

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