IT'S TIME TO POWER UP HEALTH SYSTEMS

A VISION FOR FUTURE-PROOF HEALTH SYSTEMS

efpia
European Federation of Pharmaceutical Industries and Associations

SUMMARY

European health systems face unprecedented challenges, especially from the spiralling demand for complex and costly long-term health and social care for age-related chronic diseases, but also from health threats from antimicrobial resistance and climate change.

Nevertheless, despite these challenges, EFPIA sees many reasons to be optimistic about a healthier future for Europe. Now is the time to use the lessons learned from COVID-19 pandemic, and the renewed political and public prioritisation of health, to ambitiously **POWER up** our future health systems.

This starts with investment: governments must no longer regard health spending as a cost or burden, or a target for short-term cost-containment. Rather, it represents a societal investment both in the wellbeing of citizens and in our economic future.

This investment should focus on five POWER up priorities: **People**-centricity, **Outcomes**, **Workforce** empowerment, **Efficiency** and **Resilience**. POWERing up also means embracing the transformational tools we need – namely **digitalisation** and **innovation** that generates value for patients, health systems and society as a whole.

In this paper we explain our positions on each of these POWER up priorities and highlight the contributions of the research-based pharmaceutical industry as a vital and committed partner within European Union health systems.

TABLE OF CONTENTS

INTRODUCTION: THE POWER UP AGENDA

1

It's time to power up health systems

European Union (EU) countries provide some of the best healthcare in the world. Europe has long been a centre of excellence for high-quality healthcare, education, research and innovation. Solidarity is prioritised – with most countries providing universal health coverage for core services.

Nevertheless, the COVID-19 pandemic has pushed health systems to their limits, exposing and exacerbating serious underlying flaws. As well as the enormous direct impact of COVID-19 itself, the pandemic has indirectly caused immense and lasting damage via disruption to routine healthcare services. COVID-19 also amplified pre-existing gaps and variations in disease burden, healthcare access, and outcomes between and within EU countries.

The impact of COVID-19 was exacerbated by health system weaknesses related to decades of under-investment, short-term savings, and misplaced spending on inefficient healthcare models that reward the quantity of care delivered, rather than its quality and the outcomes achieved.

Looking to the future, COVID-19 has propelled health to the top of the political agenda for years to come, and proven how inseparable it is from our prosperity. More than ever before, European citizens rightfully expect strong health systems that meet their needs and those of society at large. The pandemic has also shown the remarkable achievements that are possible when health stakeholders collaborate toward shared goals – for example in delivering innovative technologies and care delivery models.

Now is the time to use the lessons learned from this crisis – positive and negative – and the renewed political and public prioritisation of health, to ambitiously reform our health systems.

Now is the time to 'power up' our health systems to address the challenges of the 21st century.

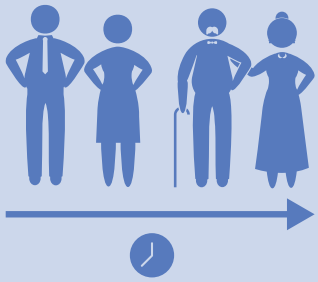
UNPRECEDENTED CHALLENGES WARRANT AMBITIOUS REFORM

Europeans are living longer, in part because of medical progress and innovation. However, the chronic diseases that account for most ill health, deaths and healthcare spending in Europe all become more common with age. These chronic, non-communicable diseases include cardiovascular disease, cancer, diabetes, respiratory diseases, and Alzheimer's disease. Mental illness is also an increasing problem in our societies. Societal ageing is therefore driving ever greater demand for complex and costly long-term health and social care.

Future health expenditure across the EU, dominated by chronic disease care costs, will continue to grow rapidly unless there is a shift to disease prevention and more sustainable models of care.

At the same time, societal ageing threatens the financing of health with a shrinking working-age fraction of the population having to support rising levels of health spending.

Added to this, health systems face long-term threats from antimicrobial resistance, environmental risks, and climate change.



Ageing challenge in numbers

- By 2100, the proportion of the EU-27 population aged 65 years or over will increase by around 50%, **from 20.2% in 2019 to 31.3%**.
- The proportion of the very old is growing even faster. For example, the proportion of people aged 80 and over will more than double **between 2019 and 2100, from 5.8% to 14.6%**.
- The shrinking working age population will have to generate greater tax revenues to support rising societal spending needed to meet the needs of the ageing population. The EU's old-age dependency ratio is projected to double **from 31.4% in 2019 to 57.1% by 2100.** This means that by 2100 there would be fewer than two (1.75) persons of working age for every person aged 65 or over, as compared to around three currently.

Chronic disease challenge in numbers

- Almost one third of people aged over 15 years report living with two or more chronic non-communicable diseases (NCDs).
- 37% of Europeans aged 65 and over have multiple chronic conditions, but this figure varies almost three-fold between EU member states.
- Among 10 European countries, the prevalence of multi-morbidity among people aged 50 and above rose from 38% in 2007 to 42% in 2015.
- NCDs account for 9 out of 10 deaths in the EU. Cardiovascular disease and cancers alone account for almost two thirds of deaths in the EU.
- NCDs account for up to 80% of all healthcare spending in the EU. Cardiovascular disease, cancer, type 2 diabetes and chronic respiratory disease alone account for at least 25% of health spending.
- Around 550,000 people of working-age die prematurely every year across the EU due to NCDs, amounting to 3.4 million life-years and €115 billion in economic potential lost annually.
- NCDs also result in hundreds of billions more in informal care, as well as societal costs such as lost production and social welfare.
- Four major NCDs alone result in a 2% loss in gross domestic product across the EU.
- The total health and economic cost of NCDs in the EU is projected to increase by over 70% by 2050.

However, these challenges need not inevitably lead to health system and economic unsustainability. These risks can be avoided or offset by maintaining better health and providing better, more efficient healthcare throughout life.

Inefficiency is holding health systems back, with one in every five Euros spent on health being wasted. Smart spending on pharmaceuticals will be essential to help improve efficiency, but pharmaceuticals account for only one fifth of health expenditure. True efficiency gains will require organisational reform to integrate services and budgets in people-centred ways, and align them toward common goals of improving health outcomes. Only this way can investments in one area generate long-term or system-wide value. Only this way can we secure a sustainable future for our healthcare systems while benefitting patients, healthcare professionals, providers and payers alike.

HEALTH SPENDING IS AN INVESTMENT, NOT A COST

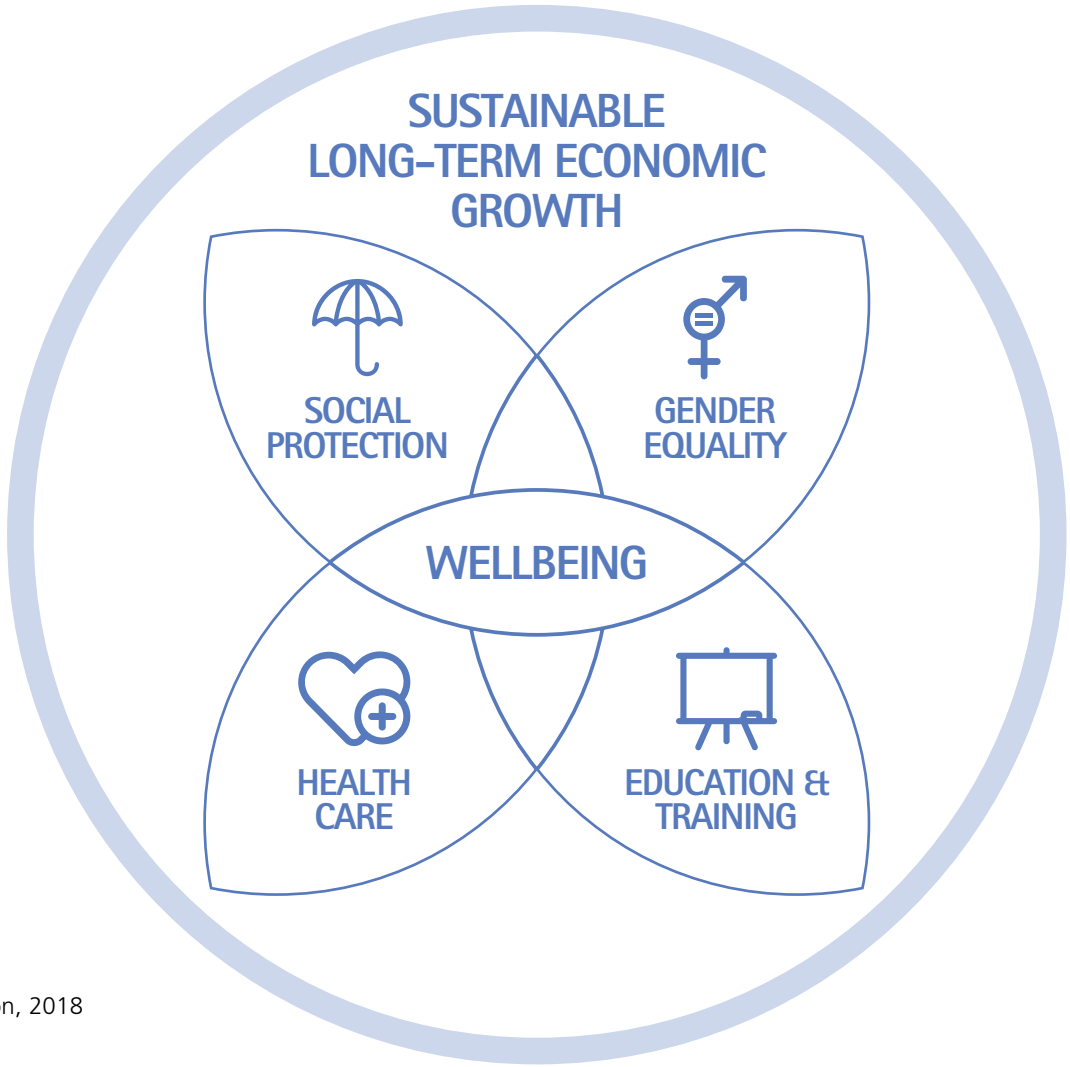
Securing the future of health systems starts with financing them according to their true value to societies and economies.

Governments must no longer regard health spending as a cost or burden, or a target for short-term cost-containment, but rather as a societal investment both in the wellbeing of citizens and in our economic future.

Better health drives prosperity. Healthier people enjoy longer and more productive working lives – contributing to the economy while consuming less healthcare.

The health sector is essential to any countries' economic performance and stability, having a positive impact across other sectors through the jobs it generates and from the purchase of goods and services. It also reduces social exclusion through benefits on employment, working conditions and household income.

The health sector (both public and private) is also in itself a driver of growth and innovation, and one of the largest employers in Europe with a highly skilled workforce.



Source: European Commission, 2018

"The EU should encourage governments to act according to these principles, and to level up health spending across the EU"

Overall, every Euro spent on public health in high-income countries is estimated to return 14 Euros to the economy. This benefit is doubled for national-level spending.

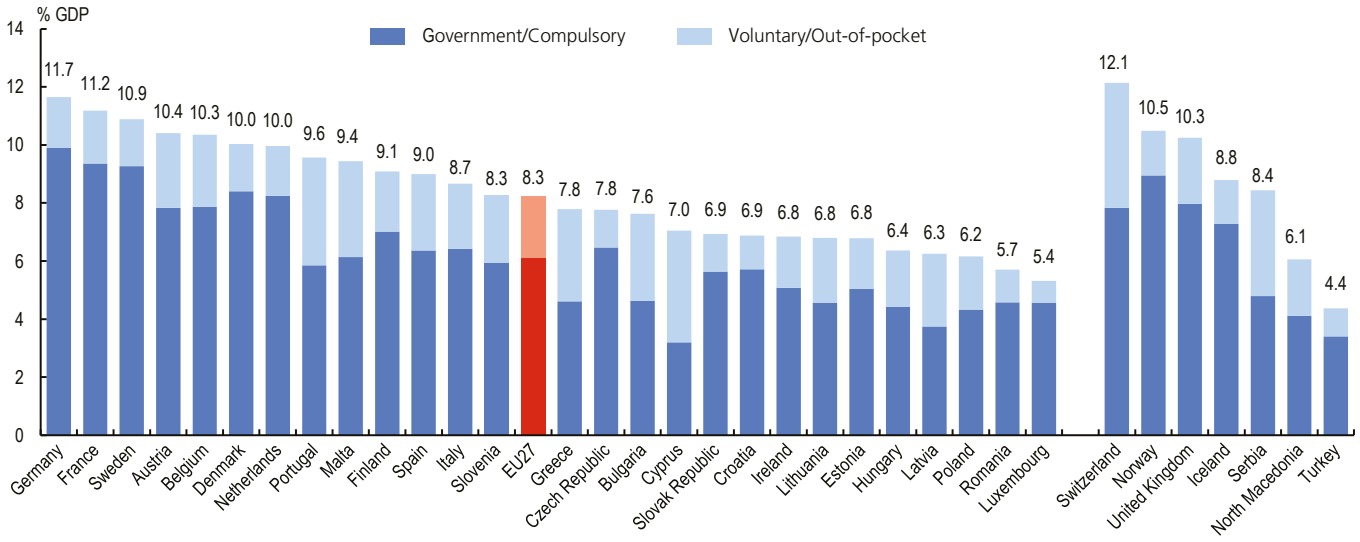
Health system spending is therefore a societal investment in our economic future, as well as our health. It builds the foundations for stronger and more sustainable economic growth, which in turn supports better healthcare provision. This 'virtuous circle' will help to solve the challenge posed by societal ageing and other long-term threats, and better prepare health systems for future shocks.

This principle already enjoys wide policy support.

- The Tallin Charter of 2008 enshrined the principle of 'health systems for health and wealth'.
- The 'economy of wellbeing' principle, championed by the Finnish Presidency of the EU in 2019, is supported by the Organisation for Economic Co-operation and Development (OECD) and the World Health Organization. The World Bank Group and the International Monetary Fund also recognise the importance of investing in health.
- Organisations representing over 40 million health professionals from 90 countries have also urged G20 leaders to put health at the centre of their economic recovery packages post-COVID-19.

The EU should encourage governments to act according to these principles, and to level up health spending across the EU. The EU will never achieve equity in healthcare access and health outcomes while some countries spend twice as much on health as others, as a percentage of gross domestic product.

Figure 5.3. **Health expenditure as a share of GDP, 2019 (or nearest year)**



Note: The EU average is unweighted.
Source: OECD Health Statistics 2020; Eurostat Database; WHO Global Health Expenditure Database.
StatLink: https://stat.link/urm8qw

DIGITALISATION IS NOW THE DIFFERENCE

We and others have advocated and worked for years to modernise health systems according to these principles. So what's going to make the difference now?

The answer is digitalisation.

Digitalisation is already revolutionising healthcare, but we are only beginning to feel its power. Digital health tools proved their worth during COVID-19, providing adaptable new ways for people and health systems to interact. More profoundly, system-wide digitalisation will help unleash the full power of data – allowing better and faster decisions to be made throughout our healthcare and innovation systems. In a sense, all healthcare will be digital, with benefits at every level.

INDUSTRY – A VITAL PARTNER IN POWERING UP HEALTH SYSTEMS

The European research-based pharmaceutical industry is a vital and committed partner within EU health systems. Research-based companies are critical:

- providers of innovation and technologies that improve and extend patients' lives and provide efficiency gains for health systems
- pioneers in people-centred, outcomes-based and data-driven healthcare
- partners in intersectoral EU-level and global collaborations, including initiatives to tackle the COVID-19 pandemic and to address longer-term challenges
- as a geostrategic asset for the EU, as a knowledge-based economy, in terms of its research and development, intellectual property production, growth and employment footprint.

Despite the challenges that health systems face, we see many reasons to be optimistic about a healthier future for Europe.

However to realise this future, we must **POWER** up our future health systems. This means investing smartly in five **POWER** priorities: People-centricity, Outcomes, Workforce empowerment, Efficiency and Resilience. It also means embracing the transformational tools we need – namely digitalisation and innovation that generates value to patients, health systems and society as a whole.

We recognise that national health systems differ in important ways. Nevertheless, we believe that all can and must POWER up to better meet the needs of the citizens they serve, and that these priorities can guide policymakers and healthcare leaders to achieve this transformation.

System-wide change is never easy. However, collaborative actions against COVID-19 have shown what can be achieved when sectors come together towards a common goal.

We cannot afford to fail, and we can only succeed together.

REFERENCES

Boyce T, Brown C. Economic and social impacts and benefits of health systems. WHO Regional Office for Europe, 2019. Available at https://apps.who.int/iris/handle/10665/329683

Council of the European Union. Council conclusions on the Economy of Wellbeing. (2019/C 400/09), 2019. Available at https://eur-lex.europa.eu/legal-content/EN/TXT/PDF/?uri=CELEX:52019XG1126(06)&rid=5

European Parliament Special Committee on Beating Cancer. Public consultation Synopsis Report - The impact of the COVID-19 pandemic on cancer prevention, health services, cancer patients and research: lessons from a public health crisis. European Parliament Special Committee on Beating Cancer, 19 April 2021. Available at https://www.europarl.europa.eu/cmsdata/232900/Synopsis%20report%20BECA%20COVID-19%20Consultation%20.pdf

Eurostat. Population structure and ageing. 2020. Available at https://ec.europa.eu/eurostat/statistics-explained/index.php?title=Population_structure_and_ageing#The_share_of_elderly_people_continues_to_increase

In support of a #HealthyRecovery https://healthyrecovery.net

International Monetary Fund. A strategy for IMF engagement on social spending. 2019. Available at https://www.imf.org/en/Publications/Policy-Papers/Issues/2019/06/10/A-Strategy-for-IMF-Engagement-on-Social-Spending-46975

IQVIA. EFPIA Patients wait indicator survey. 2020. Available at https://www.efpia.eu/media/554526/patients-wait-indicator-2019.pdf

Jagric T, et al. The impact of the health-care sector on national economies in selected European countries. Health Policy 2021;125:90–7

Jansen C, Amesz B. Every day counts – the impact of COVID-19 on patient access to cancer care in Europe. EFPIA, 2021. Available at https://efpia.eu/media/602636/every-day-counts-covid19-addendum.pdf

Kyriakides S. Keynote speech by Commissioner Kyriakides Value-based healthcare conference', 4 March 2021. Available at https://ec.europa.eu/commission/commissioners/2019-2024/kyriakides/announcements/keynote-speech-commissioner-kyriakides-value-based-healthcare-conference_en

Masters R, et al. Return on investment of public health interventions: a systematic review. J Epidemiol Community Health 2017;71:827–34

Nozal AL, et al. The economy of well-being: creating opportunities for people's well-being and economic growth. OECD, 2019. Available at https://www.oecd.org/officialdocuments/publicdisplaydocumentpdf/?cote=SDD/DOC(2019)2&docLanguage=En

OECD. Health at a glance 2019: OECD indicators. OECD, 2019. Available at https://www.oecd-ilibrary.org/social-issues-migration-health/health-at-a-glance-2019_4dd50c09-en

OECD/European Union. Health at a Glance: Europe 2020: State of Health in the EU Cycle. OECD, 2020. Available at https://www.oecd-ilibrary.org/social-issues-migration-health/health-at-a-glance-europe-2020_82129230-en?_ga=2.52510440.1357533625.1626362665-1150508910.1626362665

OECD. Tackling wasteful spending on health. 2017. Available at https://www.oecd.org/health/tackling-wasteful-spending-on-health-9789264266414-en.htm

OECD. The economy of wellbeing – creating opportunities for people's well-being and economic growth, 2019. Available at https://www.oecd.org/officialdocuments/publicdisplaydocumentpdf/?cote=SDD/DOC(2019)2&docLanguage=En

Palladino R, et al. Multimorbidity and health outcomes In older adults In ten European health systems, 2006–15. Health Affairs 2019;38:613–23

Palm W, et al. Gaps in coverage and access in the European union. Health Policy 2021;125:341–50

Rechel B, Jagger C, McKee M. The Economics of healthy and active ageing. European Observatory on Health Systems and Policies / WHO, 2020. Available at https://eurohealthobservatory.who.int/publications/i/living-longer-but-in-better-or-worse-health

Tallinn Charter. Health systems for health and wealth. WHO European Ministerial Conference on Health Systems, Tallinn, Estonia; 27 June 2008. Available at https://www.euro.who.int/__data/assets/pdf_file/0008/88613/E91438.pdf

United Nations, Department of Economic and Social Affairs, Population Division. World Population Ageing 2019: Highlights (ST/ESA/SER.A/430). 2019. Available at https://www.un.org/en/development/desa/population/publications/pdf/ageing/WorldPopulationAgeing2019-Highlights.pdf

Vandenberghe D, Albrecht J. The cost of non-communicable diseases in the European Union; A future projection. Proceedings of International Academic Conferences 9412102, International Institute of Social and Economic Sciences, 2019. Available at https://ideas.repec.org/p/sek/iacpro/9412102.html

Vandenberghe D, Albrecht J. The financial burden of non-communicable diseases in the European Union: a systematic review. Eur J Public Health 2020;30:833–9

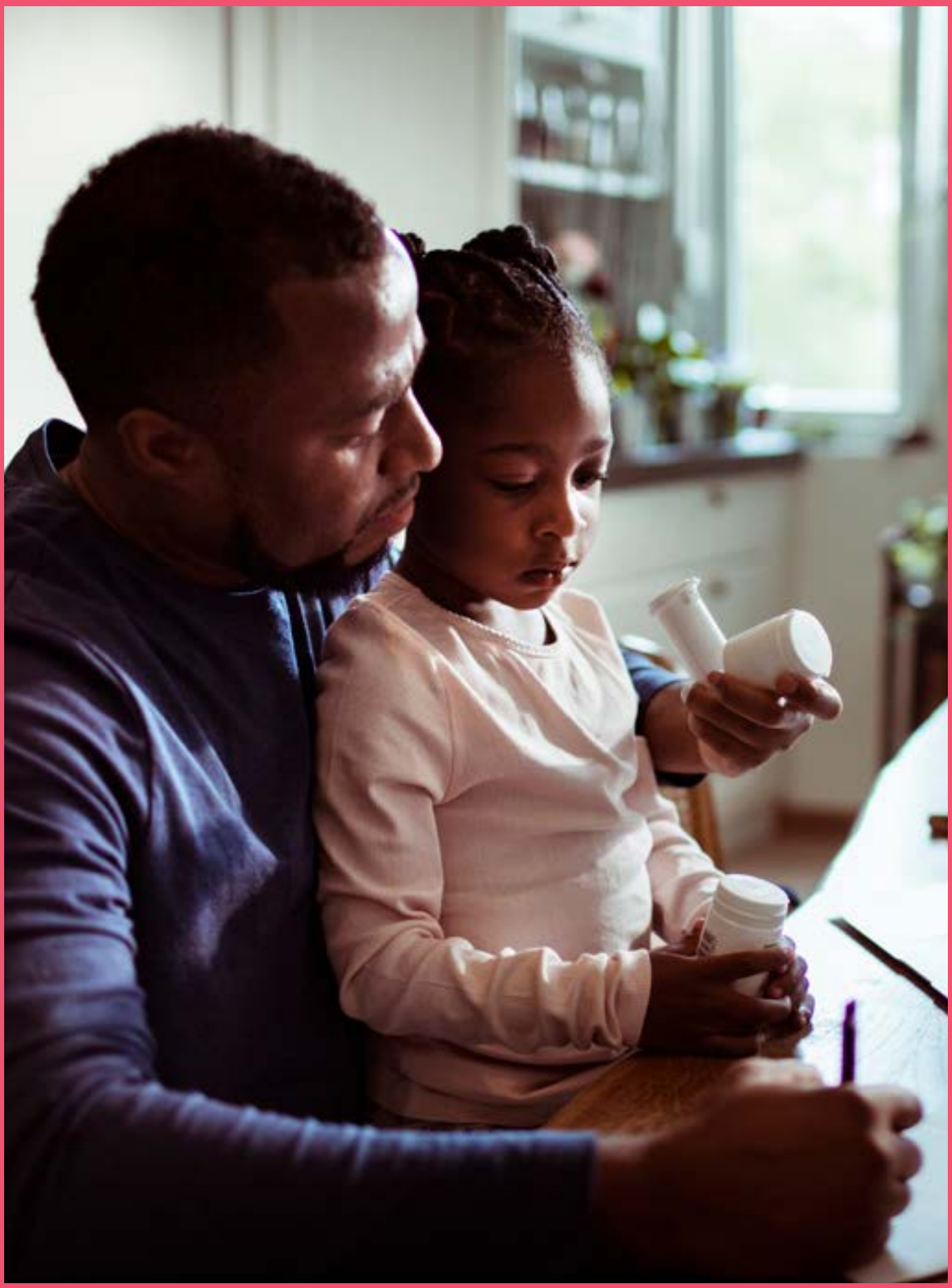
Williams G, Cylus J, Roubal T, et al. Sustainable health financing with an ageing population: Will population ageing lead to uncontrolled health expenditure growth? WHO/European Observatory on Health Systems and Policies, 2020. Available at https://apps.who.int/iris/bitstream/handle/10665/329382/Policy-brief-1997-8073-2019-3-eng.pdf

World Bank. Cause of death, by non-communicable diseases (% of total) – European Union, 2019. Available at https://data.worldbank.org/indicator/SH.DTH.NCOM.ZS

World Bank group. Health. Available at https://www.worldbank.org/en/topic/health/overview

WHO Regional Office for Europe. European programme of work 2020–2025. 2020. Available at https://apps.who.int/iris/bitstream/handle/10665/339209/WHO-EURO-2021-1919-41670-56993-eng.pdf?sequence=1&isAllowed=y

WHO Regional Office for Europe. Health inequity and the effects of COVID 19: assessing, responding to and mitigating the socioeconomic impact on health to build a better future. 2020. Available at https://apps.who.int/iris/bitstream/handle/10665/338199/WHO-EURO-2020-1744-41495-56594-eng.pdf



PEOPLE-CENTRICITY: THE DRIVING FORCE

2

Future health systems should have people-centricity as their driving force, delivering an integrated continuum of care and empowering patients as active partners.

The principle that healthcare should be patient- or people-centred sounds simple and intuitive, and it enjoys high-level policy support. However, it has yet to be applied to health systems as a whole in a comprehensive manner.

We believe that health systems can only work at full power if all their components are orientated toward meeting people's needs, improving their health and experiences of healthcare according to what matters most to them. Every service, technology, system, policy and payment should pull in the same people-centric direction.

In short, people-centricity should be the driving force throughout the entire healthcare system.

INTEGRATED PEOPLE-CENTRIC SYSTEMS

Integration is the key to achieving people-centricity.

Present health systems are not people-centred primarily because health services and budgets are fragmented according to disease specialties and provider sectors (e.g. primary, hospital and long-term care).

This is particularly problematic for patients with multiple chronic diseases, who typically need care from many different healthcare providers. Often these services do not co-ordinate well with each other, leaving patients to navigate a complicated, discontinuous system. Fragmentation also impairs **efficiency**, because of duplication, delays and unnecessary care. It can also lead to under-treatment when patients "fall through the gaps" between providers.

Separated, siloed budgets for different health services also prevent health systems from investing in one area to reap benefits and cost savings in other areas (see Section7).

We support a holistic, fully people-centric approach in which all health system services and budgets are integrated across professional, institutional and sector boundaries to provide a seamless continuum of care. This way, patients can more easily access the care they need throughout their lives – spanning disease prevention and health promotion, early detection and screening, diagnosis, treatment, rehabilitation and end-of-life care.

These models are increasingly enabled by advances in genomic tests and biomarkers that allow healthcare to be personalised, and by **digital** health systems that facilitate a free flow of real-time information between patients and all the healthcare professionals involved in their care.

"Various marginalised groups are particularly underserved by health systems, including the homeless and migrants."

This approach is expected to improve:

- **efficiency and value** – through the optimal use of the right resources at the right time and in the right place. Holistic **payment models** and health technology assessments (HTA) that break down existing budget siloes would better incentivise and reap benefit from **innovations** that deliver longer term value and across different components of the system. For example, earlier diagnosis and management of chronic diseases can realise 'downstream' savings by avoiding the complications that cause costly hospital-based care, long-term care and early death. This in turn supports the economic and **environmental sustainability** of health systems.
- **equity** – because people-centricity means all people. At present, gaps and variations persist between and within European countries in disease burden, access to healthcare **innovation**, and outcomes – as COVID-19 highlighted. Various marginalised groups are particularly underserved by health systems, including the homeless and migrants. People-centricity means that healthcare is tailored to people's needs throughout life and that nobody is left behind, or outside of, health systems.
- **resilience** – through greater responsiveness, adaptability and flexibility, as well as play its full role in strengthening the EU's strategic autonomy.

EMPOWERING PATIENTS AS PARTNERS

Peoples' experiences of living with a condition and using health services and technologies, and their preferences and priorities, must inform how we organise and deliver healthcare.

Patients, caregivers and the public must be enabled to participate as fully informed and active partners in decision-making at all levels of the health system, including

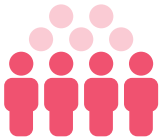
- the care they receive as individuals
- in the planning, design and evaluation of healthcare services and policies
- throughout the lifecycle of healthcare innovations, including research and development and HTA – for example via patient-reported outcomes (PROs)
- in the allocation of resources.

The EU could play an important role in creating a common framework and driving alignment in

- how to make health systems more people-centric across all these levels
- how authorities should collaborate with patients and patient organisations
- standardised measurement of PROs and the use of these data for benchmarking within and between member states.

Knowledge is power, and citizen empowerment builds better health. The COVID-19 crisis reinforced the importance of reliable health information. Improving health literacy is vital to disease prevention, early disease detection, and better disease management, for example through self-care and adherence to medication. Therefore future health systems must also take full advantage of **digital** health tools and platforms that empower patients, carers and the public as partners in their own healthcare.

INDUSTRY: PARTNERING FOR PEOPLE-CENTRICITY



"We believe that delivering the right treatment at the right dose to the right person at the right time can reduce inappropriate or low-value care"

EFPIA and its members are committed to driving the transition to people-centred healthcare.

Enhancing patients' lives

Industry is committed to developing treatments that meet the needs of patients and caregivers – enhancing their lives and their experience of healthcare. For example, we aim to develop treatments that:

- are simpler and easier to use, for example by reducing how often they need to be taken or moving from injectable to oral administration
- are better tolerated
- improve outcomes that matter most to each individual.

Industry is also pioneering the use of digital health tools to complement its medicines in delivering these benefits.
Such innovation can enhance the value of care for healthcare providers, health systems and society, as well as for patients and carers.

Personalised medicine

Personalised healthcare is revolutionising many fields, using new diagnostics and biomarkers to individualise patients' treatment and to direct the use of innovative treatments. EFPIA members are actively contributing to this field as pioneers in personalised medicines. We believe that delivering the right treatment at the right dose to the right person at the right time can reduce inappropriate or low-value care, benefitting patients and giving efficiency gains for healthcare systems and society.

EFPIA is collaborating with other stakeholders to identify and overcome the obstacles preventing Europe from taking full advantage of personalised medicine. An EFPIA-commissioned report by the London School of Economics focusing on personalised cancer medicine has recommended solutions in three key areas:

- stronger collaboration to deliver transparent and well-informed decision-making processes
- investing in appropriate infrastructure
- improvements in institutional structures, for example in regulation, HTA and reimbursement.

CONNECTING HEALTHCARE awards

Patient support programmes

Beyond providing innovative treatments, many pharmaceutical companies provide services and support to help patients manage their disease and improve the effect of treatment, often called Patient Support Programmes.

Some of these activities are delivered through partnership with patient organisations, and several of them are recognised each year by the EFPIA Connecting Healthcare Awards. For example, recent award winners include:

International Map of Axial Spondyloarthritis (IMAS)	This project has captured real-life experiences and insights from over 4,000 participants with axial Spondyloarthritis, triggering discussions with patients and rheumatologists worldwide to inform clinical decision-making, shorten diagnostic delay, and ensure patients receive optimal care.
The Dreamcatcher	This innovative digital app helps patients capture and share their ambitions, reframing the relationship between patients and healthcare providers by putting the patients' wishes at the heart of conversations.
Conectados	This project supports people undergoing treatment with chronic lymphocytic leukaemia (CLL), connecting patients with psycho-oncologists to provide support on issues such as motivation, nutrition, exercise and wellbeing.
Breaking Depression	This project communicates the experience of people affected by major depressive disorder (MDD). It uses the ancient Japanese art of kintsugi, which involves repairing objects, incorporating cracks and the signs of repair into their history. This powerful metaphor reminds society that, with time, care and patience, people with MDD can begin to heal.

An example of another initiative is Nobody Left Outside (NLO), supported by MSD. NLO is a collective of organisations representing people in some of the most marginalised communities in Europe, including homeless people, LGBTI people, people who use drugs, prisoners, sex workers and undocumented migrants. People in these groups are often at risk of poor health, but face many barriers in accessing healthcare. The NLO initiative helps organisations collaborate toward improving healthcare access for the communities they represent – on the basis that nobody should be left outside our healthcare systems.

Supporting carers

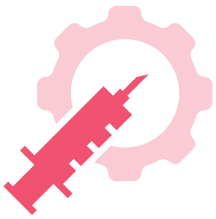
Caregivers are a cornerstone of the healthcare system, improving patient care while reducing pressure on parts of the health system – yet they are often overlooked. Estimates suggest that the economic value of unpaid care, as a percentage of the overall cost of formal long-term care provision in EU Member States, ranges from 50–90%.

EFPIA believes that governments, public institutions, the public and industry must collaborate to better support carers in the vital role they play, and in addressing their own needs.

We applaud the work of Eurocarers in supporting and empowering informal carers across Europe. Two EFPIA members, Merck and Pfizer Oncology, recently supported the development of the Essential care and cancer toolkit, by Eurocarers in collaboration with the European Cancer Patient Coalition, MacMillan Cancer Support and Nurses and the European Association for Palliative Care.

Another example is the international Embracing Carers™ Initiative, funded and co-ordinated by Merck. This initiative is dedicated to improving carers' health and wellness, while increasing awareness and support for them within healthcare systems around the globe. It does this by collaborating with organisations to support carer initiatives, driving greater visibility and awareness of carer challenges, supporting increased policy attention and action, and creating innovative opportunities for healthcare system integration.

Most recently, Embracing Carers® created the Carer Well-Being Index to determine the current and residual impacts of COVID-19 on unpaid carers, including its impact on their economic, physical and psychological well-being.



Involving patients in research and development

To deliver people-centric treatments we need to engage with patients throughout the lifecycle of a medicine, from early research and development to understand patients' needs, all the way to evaluating the real-world effects in clinical practice.

To this end, we collaborate widely with patient organisations and patient communities, as equal and valued partners. We believe in open and transparent dialogue between patients, industry and decision-makers throughout the lifecycle of medicines.

EFPIA has advanced the field of patient engagement through the EFPIA Patient Think Tank, having co-created clear principles around working together, and through various multistakeholder public-private partnerships, such as PARADIGM and GravitateHealth.

EFPIA co-led the PARADIGM initiative with the European Patients' Forum (EPF). PARADIGM has produced a unique set of recommendations and tools to enable effective, meaningful, ethical, innovative, and sustainable patient engagement during medicines research and development. The many PARADIGM resources include a Patient Engagement Monitoring and Evaluation Framework to help partnerships between patients and patient organisations, bio-pharmaceutical companies, regulators and HTA bodies to self-evaluate the progress and impact of patient engagement in the medicines development lifecycle.

The European Patients' Academy on Therapeutic Innovation (EUPATI) is a multi-stakeholder public-private partnership. It provides education and training to increase the capacity and capability of patients and patient representatives to understand and meaningfully contribute to medicines research and development, and to improve the availability of medical information for patients and other stakeholders. Many individual EFPIA member companies have contributed funding to EUPATI.

Gravitate Health is an ambitious partnership involving 39 members spanning academia, patient organizations, medicines regulators, healthcare providers and payers, digital innovators, EFPIA members and other stakeholders. It aims to equip and empower citizens with digital information tools that help them to be confident, active, and responsive in their patient journey, specifically encouraging safe use of medicines for better health outcomes and quality of life. Specifically, the Gravitate Lens (G-Lens) will focus on approved electronic product information content, offering a route for patients to access trustworthy, up-to-date information that better meet their individual needs.

EU-PEARL
EU PATIENT-CENTRIC CLINICAL TRIAL PLATFORMS

In the EU Patient-Centric Clinical Trial Platforms (EU-PEARL) project, leading research hospitals, regulators, patient advocates and industry are working together to help shape the future of clinical trials. Specifically, this project aims to support a wider use of patient-centric 'platform' trials, which allow novel techniques and treatments developed by multiple companies and organizations to be tested efficiently to help address unmet needs. Patients and carers are integrally involved to ensure their perspectives are consistently incorporated in trial designs and outcome measures.

REFERENCES

EFPIA. Strengthening health systems through smart spending. 2020. Available at https://www.efpia.eu/news-events/the-efpia-view/statements-press-releases/strengthening-health-systems-through-smart-spending/

EFPIA. Working together with patient groups. 2017. Available at https://efpia.eu/media/288492/working-together-with-patient-groups-23102017.pdf

EFPIA. Working together with patients: principles for remunerating patients, patient organisation representatives and carers for work undertaken with the pharmaceutical industry. 2019. Available at https://www.efpia.eu/media/413114/workingtogetherwithpatients_patient-remuneration-principles.pdf

EFPIA. Connecting Healthcare guide. 2021. Available at https://efpia.eu/media/602853/connecting-healthcare-awards-2021.pdf

Eurocarers website: About carers. Available at https://eurocarers.org/about-carers

European Observatory on Health Systems and Policies. Achieving person-centred health systems: evidence, strategies and challenges. Edwards N, et al. (eds). Cambridge University Press, 2020. Available at https://www.euro.who.int/en/about-us/partners/observatory-old/publications/studies/achieving-person-centred-health-systems-evidence,-strategies-and-challenges-2020

G7 Health Ministers. Ensuring access to health for all, 2019. Available at https://www.elysee.fr/admin/upload/default/0001/05/59c3454ab28edc451be46e846b70dc38a532db95.pdf

Gill J, et al. Access to personalised oncology in Europe. London School of Economics, 2020 https://www.efpia.eu/media/580518/access-to-personalised-oncology-in-europe.pdf

IQVIA. Assessing person-centred therapeutic innovations. 2018. Available at https://www.efpia.eu/media/413282/assessing-person-centered-therapeutic-innovations-wpa4_web.pdf

IQVIA. EFPIA pipeline review 2021 update. Available at https://www.efpia.eu/media/602563/iqvia_efpia_pipeline-review_final.pdf

IQVIA. Patients wait indicator survey. 2020. Available at https://www.efpia.eu/media/554526/patients-wait-indicator-2019.pdf (Accessed 27 Feb 2021)

OECD Health Ministerial Meeting. The next generation of health reforms. 2017 Available at https://www.oecd.org/health/ministerial/ministerial-statement-2017.pdf

OECD/European Union. Health at a Glance: Europe 2020: State of Health in the EU Cycle. OECD, 2020. Available at https://www.oecd-ilibrary.org/social-issues-migration-health/health-at-a-glance-europe-2020_82129230-en?_ga=2.52510440.1357533625.1626362665-1150508910.1626362665

Palm W, et al. Gaps in coverage and access in the European union. Health Policy 2021;125:341–50

Wilsdon T, et al. The benefits of personalised medicine to patient, society and healthcare systems. 2018. Available at https://www.efpia.eu/media/362040/cra-ebe-efpia-benefits-of-pm-final-report-6-july-2018.pdf

World Health Organization Regional Office for Europe. European programme of work 2020–2025. 2020. Available at https://apps.who.int/iris/bitstream/handle/10665/339209/WHO-EURO-2021-1919-41670-56993-eng.pdf?sequence=1&isAllowed=y

World Health Organization. Framework on integrated, people-centred health services, 2016. Available at https://www.who.int/servicedeliverysafety/areas/people-centred-care/framework/en/

World Health Organization. Out-of-pocket payments for health, particularly for medicines, are unaffordable for many in Europe, 2019. Available at https://www.euro.who.int/en/health-topics/Health-systems/pages/news/news/2019/4/out-of-pocket-payments-for-health,-particularly-for-medicines,-are-unaffordable-for-many-in-europe

3 OUTCOMES MATTER MOST

Future health systems should focus on delivering and paying for what matters most – better health and longer lives.

People at heart, outcomes in mind

Current healthcare systems tend to focus on the amount of healthcare delivered, not the value it actually brings to patients, health systems and society.

For example, healthcare funders mostly measure and pay for healthcare according to the number of tests, services, procedures and medicines provided. Inevitably, resources are focused on meeting these targets rather than on delivering the health outcomes that matter most to patients and society.

This can lead to:

- poor efficiency – by incentivising the overuse of inappropriate healthcare
- underprovision of care or cherry-picking of patients with lesser care needs – especially for simple capitation models where providers are remunerated on the basis of a fixed number of registered patients regardless of how much care they need
- under-investment in interventions and activities that will improve outcomes in the long term.

We believe that future health systems should orientate all their resources toward delivering better outcomes – thereby maximising value (i.e. outcomes achieved by the resources used).

This is not relevant only to certain services, therapy areas, patient groups and medicines: it should apply as an organisational principle across the whole health system.

Focusing on outcomes allows health systems to be more:

- **people-centric** – through the complementary use of patient-reported outcomes and clinical outcomes (see panel). Clinical outcomes (such as blood-sugar levels, tumour growth and mortality) are important but do not give the full picture of the end result of care. Patient-reported outcomes (such as quality of life, psychological wellbeing and physical functioning) allow patients to communicate and engage better with health systems and care to be assessed according to outcomes that matter most to patients themselves. Making aggregate outcomes data publicly available also helps patients make better-informed choices about the healthcare they receive
- **integrated:** because different services are incentivised to work together toward the same ultimate aim – to measurably achieve the best result for patients
- **efficient:** by allowing resources to be allocated toward achieving the best health outcomes relative to cost (i.e. from low-value to high-value care) and potentially to realise savings elsewhere or in the longer term; this in turn can improve sustainability
- **quality-driven:** by creating **digital** 'learning systems' that systematically, consistently and continuously measure standardised outcomes – helping physicians and health services to get real-time feedback on the quality of care and benchmark their performance against their peers and to rapidly share best practices, provided there is transparent reporting
- **equitable:** by identifying and thereby helping to overcome the existing unwarranted variations in outcomes within and between providers, regions and countries
- **innovative:** by incentivising investment in **innovations** in service delivery and technologies that generate better value.

What are outcomes?

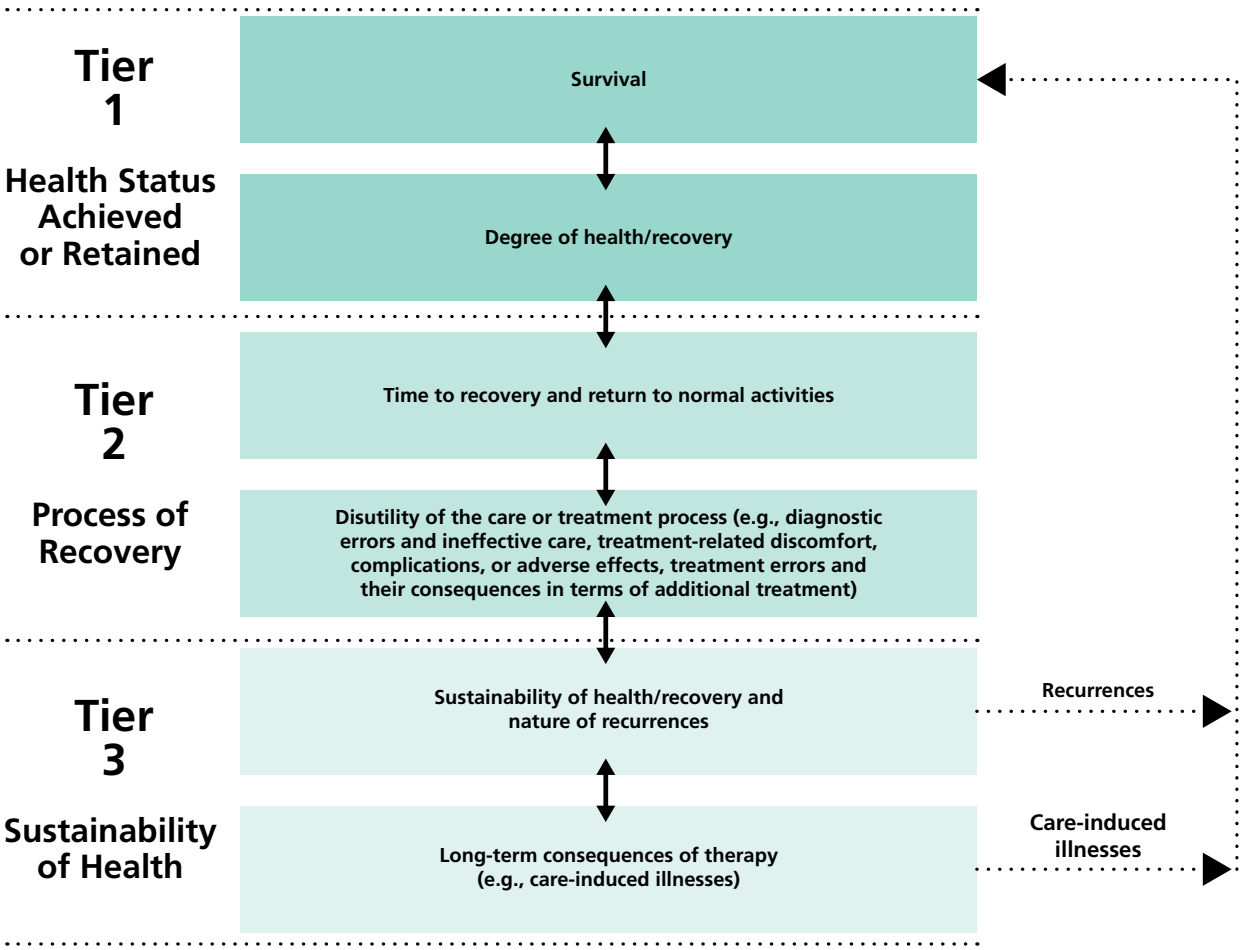
Porter defined outcomes as 'the results of care in terms of patients' health over time'. Outcomes are distinct from care processes or interventions, and from biological indicators that merely predict results.

Key principles of outcomes measurement include:

- Outcomes should involve the health circumstances most relevant to patients.
- Outcomes should cover both near-term and longer-term patient health to encompass the ultimate results of care. This includes the full cycle of care, including acute care, related complications, rehabilitation, and reoccurrences.
- Outcomes should cover the full range of services (and providers) that jointly determine the patient's results.

Outcome measurement should include sufficient measurement of risk factors or initial conditions to allow risk adjustment.

Each medical condition (or population of primary care patients) will have its own unique set of outcome measures. However, Porter defined the following general hierarchy for outcomes (reproduced with permission from Porter 2010).



TRANSITIONING TO OUTCOMES-BASED SYSTEMS: STEP-BY-STEP

The transition to outcomes-based healthcare will include several key steps.

Defining standardised and people-centred outcomes

Health systems should measure standardised outcomes metrics to allow comparison between healthcare providers, regions and even countries. This will allow benchmarking, identification of unwarranted variation, and the sharing of best and worst practices to improve patient outcomes, care quality and value, leading to more informed patients' choices and better health.

These outcomes are best defined through a multidisciplinary approach with a strong patient involvement. Important initiatives include the International Consortium for Health Outcomes Measurement (ICHOM), the OECD Patient-reported Indicator Surveys (PaRIS) and the Health Outcomes Observatory project.

Measuring outcomes: harnessing digital power

EU health systems need integrated, interoperable digital systems to collect and integrate high-quality outcomes data from many sources under clear governance (see Section 8).

Transparent reporting of outcomes data, following the model of countries like Sweden, is also important to allow benchmarking of health outcomes within and across countries, and to inform decision-making by policymakers, service providers, payers and patients.

Paying for outcomes – incentivisation through new models

The transition to outcomes-based healthcare is held back not by technical barriers, but by organisational and cultural barriers, including a lack of incentives. Health systems do not systematically incentivise healthcare providers to measure or report outcomes.

To unblock this, future financing and payment models should shift toward rewarding health outcomes, for example learning from:

- bundled payments that include the provision of rehabilitation and any necessary reoperation after surgery, which incentivise high-quality care
- funding of integrated care models through shared savings contracts
- introduction of pay-for-performance schemes in primary care
- value-based reimbursement agreements for innovative medicines (see Section 7).

These models have yet to be applied at scale across integrated health systems, and there is no one-size-fits-all approach that works for all types of providers, services and products. More work is therefore required to agree on the outcomes to be measured, how to design fit-for-purpose contractual models and the way in which to implement the necessary data collection systems and how best to overcome legal, logistical and administrative barriers.

When used for the purpose of payment models, it is also important that outcomes measures are 'risk-adjusted' to account for factors that may affect health outcomes besides the intervention or service to be reimbursed, including patient characteristics (such as age and comorbidities) and whether the treatment was administered properly.

INDUSTRY: PARTNERING FOR OUTCOMES-BASED HEALTHCARE

The industry is committed to maximizing the value that our innovations bring to patients, health systems and society and strongly support a transition to outcomes-based care.

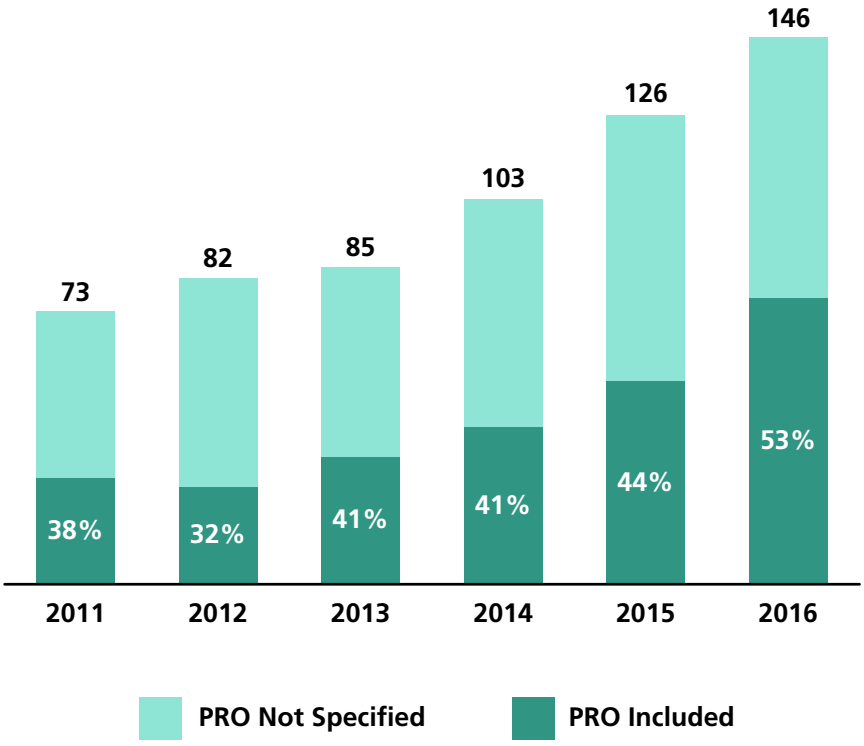
We believe in empowering people to have control over their care, outcomes and health data – and we are taking a lead by investing and collaborating with patients, professionals and other health systems partners toward this end.

The industry is increasingly incorporating patient-reported outcomes in clinical trials and in regulatory and HTA submissions.

- Between 2012 and 2016, 70% of oncology indications for 49 medicines approved by the European Medicines Agency and Food and Drugs Administration included PRO data in their regulatory submissions.
- In 2016, just over half of HTA submissions made to eight agencies included PROs.

Increasing use of PROs in oncology HTA submissions

(CADTH, G-BA, HAS, NICE, PBAC, SMC, TLV and ZIN*)



The industry is also engaged in several public-private partnerships to support the use of PROs in healthcare decision-making, including through the Innovative Medicines Initiative.

For example, the Health Outcomes Observatory project (H2O) brings together the public and private sectors to create an unprecedented, standardised data governance and infrastructure system across Europe to incorporate patients' experiences and preferences in decisions affecting their own healthcare and that of the entire patient community.

H2O will allow patients to measure their outcomes in a standardised way, whilst keeping full control of their data. It will enable ethical, secure analysis of the data when patients consent to this, in the interest of society, science and patient care. Ultimately, it aims to foster innovation in healthcare across Europe and beyond to deliver better outcomes for all.

Other relevant projects include the European Health Data and Evidence Network (EHDEN; see Section 8), HARMONY, ROADMAP, BigData@Heart and PIONEER.

REFERENCES

EFPIA. Healthier future. The case for outcomes-based sustainable healthcare. 2020. Available at https://www.efpia.eu/media/412313/the-case-for-outcomes-document-17102018.pdf

EFPIA. Addressing healthcare challenges. Novel pricing and payment models: new solutions to improve patient access. 2020. Available at https://efpia.eu/media/554543/novel-pricing-and-payment-models-new-solutions-to-improve-patient-access-300630.pdf

EFPIA. Strengthening health systems through smart spending. 2020 https://www.efpia.eu/news-events/the-efpia-view/statements-press-releases/strengthening-health-systems-through-smart-spending/

IQVIA. EFPIA pipeline review 2021 update. Available at https://www.efpia.eu/media/589818/iqvia_efpia_pipeline-review_final-report-2021-public-19apr21v2.pdf

OECD. Better ways to pay for healthcare. OECD, 2016 https://www.oecd.org/publications/better-ways-to-pay-for-health-care-9789264258211-en.htm

Porter ME. What is value in health care? N Engl J Med 2010;363:2477–81

Smith PC, et al. Building on value-based healthcare. Towards a health system perspective. European Observatory on Health Systems and Policies, 2020 https://eurohealthobservatory.who.int/publications/i/building-on-value-based-health-care-towards-a-health-system-perspective-2020-brief

The value of health: improving outcomes – final report. 2016. Available at https://www.europeanallianceforvalueinhealth.eu/wp-content/uploads/2020/11/The-Value-of-Health-Improving-Outcomes.pdf

Porter ME. Measuring Health Outcomes: The Outcome Hierarchy, Supplementary Appendix 2 to Porter ME. What is value in health care? N Engl J Med 2010;363:2477–81. Available at: https://ichom.org/files/articles/nejmp1011024_appendix2.pdf

WORKFORCE: EMPOWERING THE POWER BASE

4

The future health workforce must be empowered with skills, tools and innovations that help them deliver maximal value to patients and the health system.

No healthcare without health workers

There is no healthcare without a health workforce. The healthcare workforce is a fundamental building block of health systems and a key determinant of how well they perform.

The COVID-19 pandemic has emphasised the vital contribution that healthcare workers make to society and has boosted their standing in the eyes of the general public.

However, COVID-19 has had, and continues to have, a major negative impact on healthcare staff. In addition to the risk of COVID-19 infection itself, many frontline staff are exhausted and under severe mental strain owing to prolonged periods of excessive pressure and high excess death rates, in some cases with low levels of staff and equipment. Burnout, distress and mental health problems are widespread.

This pressure on health workers was exacerbated by the existing shortages of doctors and nurses that exist in many EU countries. COVID-19 has shown how the depletion of the workforce and implicit attrition in service delivery left some countries ill-equipped to deal with a spike in demand for healthcare.

More broadly, a lack of skilled workforce also contributes to variations in patients' access to innovative services and technologies, and to their health outcomes. Health is already the single largest employment sector in Europe and the EU will need 11 million newly trained or imported health and long-term workers to satisfy the rising demand in the health and long-term care sectors between 2018 and 2030.

Now is the time for healthcare systems to invest in and empower healthcare workers to deliver the ideal health systems for the future.

EMPOWERING THE FUTURE HEALTH WORKFORCE

Rather than thinking about the workforce working for healthcare systems, we should ask: how can future health systems better work for the health workforce?

We believe that innovation in organisational models, digital solutions, medicines, diagnostics and other technologies should be part of the solution – helping the workforce to achieve the best possible and personalised care for their patients.

Empowering the workforce within future health systems will involve:

- Fostering and enabling the levels of collaboration and co-ordination that underpin people-centric integrated care models
- Enabling task-shifting for a more resilient and flexible workforce
- Expanding the specialist expertise and capacity needed to deliver innovative treatments, e.g. through expert centres and European reference networks
- Upskilling staff to use the digital health and genomic technologies
- Supporting healthcare organisations to adopt innovation that facilitates and enhances the work of healthcare professionals, including digital tools that enable efficient data collection, allowing healthcare professionals to focus on the interaction with their patients
- Implementing organisational, financial, governance and regulatory environments that support transformational change.

INDUSTRY: PARTNERING IN POWER

"EFPIA supports the public-private partnership approaches and collaboration across the professions to leverage the opportunities and tackle the challenges in our health ecosystem"

We salute the work already underway to help empower the workforce of the future, for example by the OECD, UK Topol Review, Expert Panel on effective ways in investing in health, WHO Europe, the European Commission and the Partnership for Health System Sustainability and Resilience.

EFPIA supports the public-private partnership approaches and collaboration across the professions to leverage the opportunities and tackle the challenges in our health ecosystem and is ready to contribute, including through initiatives such as the European 'Pact for Skills'.

As part of the EU Health Coalition, we also support calls for major investments in training the next generation of researchers, with greater coordination and harmonisation of training programmes across Europe to strengthen the research ecosystem and careers within it.

The industry's substantial investment in research and development in Europe contributes to the continuous professional development of healthcare professionals through participation in clinical trials and other research activities. Within our own industry, EFPIA members will continue to build the best possible workforce to serve patients and health systems. To this end, we are committed to life-long learning and professional development.

In another example, one EFPIA member, AstraZeneca, is partnering with public entities in the Reskilling for Employment (R4E) initiative launched by the European Roundtable for Industry. This will help enable unemployed and 'at-risk' workers to reskill at a time when the job landscape in Europe is undergoing significant change. The ultimate goal is to reskill 1 million adults of all ages in Europe by 2025 – empowering the human capital in Europe's recovery, promoting social inclusion and smoothening the transition to a green and digital economy.

In the future, EFPIA members intend to support workforce enablement and empowerment in collaboration with all parties by:

- Delivering innovation that enhances the work of healthcare professionals
- Ensuring real-world data collection does not unnecessarily burden healthcare professionals
- Contributing our expertise to Lifelong Learning in Healthcare. The framework for such actions includes ethical, transparent and responsible engagement, quality content and robust processes such as educational needs assessment, learning design and outcomes assessment.

REFERENCES

EU Health Coalition. A shared vision for the future of health in Europe: lessons learnt from the COVID-19 pandemic. 2020. Available at https://www.euhealthcoalition.eu/recommendations/

European Commission. Building a European Health Union: Reinforcing the EU's resilience for cross-border health threats (COM(2020) 724 final). 2020. Available at https://ec.europa.eu/info/sites/info/files/communication-european-health-union-resilience_en.pdf

Expert Panel on Effective Ways of Investing in Health. The organisation of resilient health and social care following the COVID-19 pandemic. European Union, 2020. Available at https://ec.europa.eu/health/sites/health/files/expert_panel/docs/026_health_socialcare_covid19_en.pdf

Greenberg N. Mental health of health-care workers in the COVID-19 era. Nat Rev Nephrol 2020;16:425–6

Grubanov Boskovic S, et al. Health and long-term care workforce: demographic challenges and the potential contribution of migration and digital technology (EUR 30593 EN). European Union, 2021. Available at https://ec.europa.eu/jrc/en/publication/eur-scientific-and-technical-research-reports/healthcare-and-long-term-care-workforce-demographic-challenges-and-potential-contribution

Khan Burki T. Burnout among cancer professionals during COVID-19. Lancet 2020;21:P1402

OECD/European Union. Health at a Glance: Europe 2020: State of Health in the EU Cycle. OECD, 2020 https://www.oecd-ilibrary.org/social-issues-migration-health/health-at-a-glance-europe-2020_82129230-en?_ga=2.52510440.1357533625.1626362665-1150508910.1626362665

OECD. Empowering the health workforce – strategies to make the most of the digital revolution. OECD, 2020. Available at https://www.oecd.org/health/health-systems/Empowering-Health-Workforce-Digital-Revolution.pdf

Socha-Dietrich K. Engaging and transforming the health workforce. In: Health in the 21st century: putting data to work for stronger health systems. OECD, 2017 Available at https://www.oecd-ilibrary.org/sites/e3b23f8e-en/1/2/4/index.html?itemId=/content/publication/e3b23f8e-en&_csp_=c23ceb96c5ce2feb951a759a657e4b9f&itemIGO=oecd&itemContentType=book

Topol E, et al. The Topol Review: Preparing the workforce to deliver the digital future. NHS England, 2019. Available at https://topol.hee.nhs.uk/wp-content/uploads/HEE-Topol-Review-2019.pdf

Wharton G, et al. The Partnership for Health Sustainability and Resilience. Interim report of the pilot phase. 2021. Available at http://www3.weforum.org/docs/WEF_PHSSR_Interim_Report_of_the_Pilot_Phase.pdf

World Health Organization Regional Office for Europe. Health workforce website. Available at https://www.euro.who.int/en/health-topics/Health-systems/health-workforce

World Health Organization. A universal truth: no health without a workforce. WHO, 2013 https://www.who.int/workforcealliance/knowledge/resources/hrhreport2013/en/

World Health Organization Regional Office for Europe. European programme of work 2020–2025. WHO, 2020 https://apps.who.int/iris/bitstream/handle/10665/339209/WHO-EURO-2021-1919-41670-56993-eng.pdf?sequence=1&isAllowed=y

EFFICIENCY IS IMPERATIVE

5

Future health systems must become more efficient through a system-wide transition to value-based healthcare.

Defining efficiency and value

Health systems are estimated to waste up to 20% of spending on interventions that do not improve health outcomes.

While we support greater investment in health, according to the economy of wellbeing (**Section 1**), increased funding should be combined with action to reduce inefficiencies.

Future health systems must become more efficient, but it is important to define efficiency carefully. We believe that improving efficiency means maximising the patient- and population-level health **outcomes** achieved through a certain health investment, or maintaining the same outcomes while achieving long-term savings for health systems or society. This is closely related to the concept of value-based healthcare.

EFPIA believes that efficiency and value should be defined in **people-centred** ways, i.e. based on what matters most to patients themselves. This is one of six key principles for value-based healthcare that we share with fellow members of the European Alliance for Value in Health – representing patients, healthcare practitioners, health authorities, healthcare management and the medical technology industry (see Panel). More traditional ways of defining efficiency in relation to output (e.g. number of procedures performed), ignores the appropriateness and quality of care and the health outcomes achieved for patients.

We need to identify inefficient, low-value spending and redirect these resources to services and technologies known to generate better value. This way, we can improve patients' health outcomes and quality of care without increasing overall expenditure and realise the full return on investment from health spending.

European Alliance for Value in Health

Six key principles of value-based, sustainable, and people-centred health systems:

1. Outcomes that matter to people and patients, as well as benefits valued by health systems and societies, are at the centre of decision-making

2. Interventions and services addressing prevention, social care and healthcare are organised in an integrated way around people and patients

3. Resources are allocated towards high value care and prevention, with outcomes and costs of care measured holistically

4. Continuous learning, education and healthcare improvement is based on evidence, and supported by data and insights

5. Innovative ways of care delivery are fostered

6. Financing models and payments reward value and outcomes.

EFFICIENCY GAINS ACROSS HEALTH SYSTEMS

"A significant share of health spending in OECD countries is at best ineffective and at worst, wasteful. Solutions exist."

Inefficiency and wasteful in healthcare can occur in different forms, for example:

- Some patients receive repeated diagnostic tests or services, simply because information is not shared across providers.
- Some patients receive 'low-value care', i.e. care that is ineffective, or that works for only some groups of patients.
- Patients sometimes receive care that causes serious complications that could have been avoided. It is estimated that around 10% of hospital expenditure goes to correcting preventable medical mistakes or hospital-acquired infections.

Improving efficiency and reducing waste is not about cutting costs, but about investing smartly in health systems, as we pointed out in the EFPIA report 'Strengthening health systems through smart spending'.

Integration

Poor co-ordination between healthcare providers within fragmented health systems is a key source of inefficiency – causing duplication and inconsistent care or poor follow-up. Integrated models that align all resources and incentives toward measurably improving **outcomes** that matter most to **people** will be more efficient (Sections 2 and 3). More integrated budgets spanning the health and social care sectors would encourage strategic investments into services and technologies that free up resources in the longer-term or elsewhere in the system for reinvestment.

Digital health technologies will support these efficiency gains, by helping to measure performance and optimise resource allocation.

Disease prevention

Future health systems must invest more in primary disease prevention and public health. This is one of the most efficient ways to protect people, health systems and economies.

Each year over 1 million premature deaths in the EU – two thirds of the total – could be avoided through better disease prevention and healthcare. Almost two thirds of these avoidable deaths are preventable through effective primary prevention and other public health measures.

Vaccination offers a prime and timely example of disease prevention and high-value innovation can have enormous benefits to health systems and society. Vaccination has been a cornerstone of European public health for decades, preventing disease, reducing the burden on health services and supporting healthy populations. Vaccines are a 'best buy' in health, providing a substantial return on investment. Now, COVID-19 has underscored their unparalleled value in protecting people, health services and broader economies.

However, at present only 3% of health spending is devoted to disease prevention and public health across the EU and less than 1% (median 0.3%) to immunisation funding.

Many medicines also play an important role for secondary prevention, especially in chronic diseases such as diabetes and cardiovascular disease, to prevent serious events and complications.

Timely and effective treatment

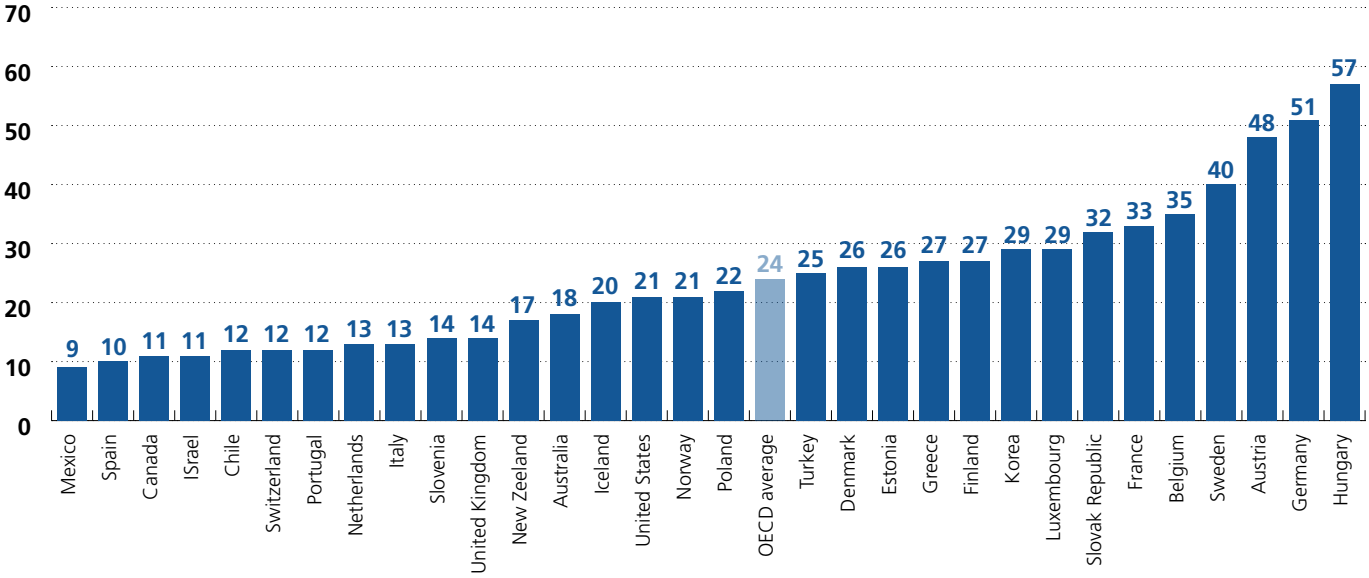
Investing in better screening and treatment can avoid downstream costs. The remaining one-third of the 1 million avoidable deaths in Europe each year could be avoided through earlier and better healthcare.

Most of the healthcare costs of chronic diseases are spent on hospital care for advanced disease and complications. Earlier diagnosis and effective, timely treatment gives far better patient outcomes, avoids unnecessary downstream costs, and reduces the societal life-time burden of ill health. In fact, poor adherence to prescribed medicines is thought to cost as much as EUR 125 billion in avoidable hospitalisations, outpatient visits and emergency care.

For example, diabetes absorbs 9% of the total health expenditure in the EU and is rising. An estimated 75% of this expenditure is for complications that could be significantly reduced by innovative medicines, such as diabetic retinopathy, heart and kidney failure. However, only 6.2% of diabetes expenditure is used for medicines.

On average, 30% of health systems expenditure is spent on inpatient care. However, hospital admissions for chronic conditions are often avoidable, as illustrated by the wide variation between countries in the frequency of hospital discharges among patients with diabetes.

Number of hospital discharges for diabetes per 1000 diabetics



Source: OECD (2015), Cardiovasculat Disease and Diabetes: Policies for Better Health and Quality of Care

Further opportunities to improve efficiency are explored in the EFPIA publication 'Strengthening health systems through smart spending' and other publications.

INDUSTRY: PARTNERING FOR GREATER EFFICIENCY

EFPIA champions efficiency and value-based healthcare, together with patients and other health stakeholders – including the European Alliance for Value in Health.

The pharmaceutical industry is committed to increasing the value of its products and services by focusing on the patient and patient outcomes and to demonstrating how innovative therapies can often decrease costs and thereby improve efficiency elsewhere in the system.

EFPIA and its members are also actively involved in various public-private partnerships that aim to improve efficiency, for example through health literacy and adherence to medicines in GravitateHealth.

Many EFPIA members have also united with other key stakeholders to create All.Can – a platform dedicated to improving the efficiency of cancer care by focusing on what matters to patients.

All.Can
Changing cancer care together

EFFICIENCY BENEFITS THE PLANET

In the process of extending and improving patients' lives, health systems contribute to environmental damage and the climate crisis, which in turn impair health.

Furthermore, it has been well documented that climate change can adversely impact human health. Its influence on health may be directly or indirectly from changing infectious disease patterns; increasing extreme weather events and the risk of drought, floods and subsequent food insecurity; and increasing respiratory disease from poor air quality. The recently published Commission Zero Pollution Action Plan, highlights the persistent threats of climate change, environmental pollution, biodiversity loss and an unsustainable use of natural resources which pose multiple risks to human, animal and ecosystem health. They include infectious and non-communicable diseases, antimicrobial resistance and water scarcity.

Europe is at the forefront of global efforts to reduce greenhouse gas emissions, with increasingly ambitious decarbonisation targets and various policy initiatives underway at EU and national level. In this context there is increasing attention on reducing the environmental footprint of health systems.

Efficiency is key to sustainability

We support the World Health Organization's vision for an environmentally sustainable health system that improves, maintains or restores health while minimising negative impacts on the environment and leveraging opportunities to restore and improve it, to the benefit of the health and wellbeing of current and future generations. Furthermore, we welcomed and embraced the Commission's focus on the Green Deal Strategy and a more sustainable Europe.

Like others, we believe that efforts to reduce the environmental impact of healthcare must not occur at the expense of health quality or equity. Rather, it will be best achieved by reducing unnecessary healthcare demand and consumption – as well as the impact of healthcare supply itself.
Therefore we agree with WHO and others that prioritising and incentivising disease prevention, improving chronic disease management, strengthening primary care, promoting innovative care models (including telemedicine) are key to improving the environmental sustainability of health systems, as well as their economic sustainability. In this respect, what's good for patients and health systems is also good for the planet.

Pharmaceuticals in the environment

One of the unintended but inevitable results of delivering life-changing medicines to patients is that trace amounts of active pharmaceutical ingredients can find their way into the environment at all stages of the product's lifecycle.

The European pharmaceutical industry, represented by Association of the European Self-Care Industry (AESGP), EFPIA and Medicines for Europe is committed to continue playing an active role in addressing concerns around risks associated with pharmaceuticals in the environment. Minimising the impact of medicines on the environment while safeguarding access to effective treatments for patients is a critical issue across all sectors of healthcare.

OUR COMMITMENT AND ACTION

EFPIA members are committed to making a positive impact on patients' lives while operating sustainably and addressing environmental concerns. We believe that our industry can and should contribute significantly to decarbonising health systems, in collaboration with our partners. We recognise the importance of reducing waste and pollution, including greenhouse gas emissions throughout healthcare systems.

We strive to contribute to a healthy environment and to lead efforts to mitigate climate change and the move towards circularity, in alignment with the aims of EU Green Deal and climate policy and circular economy vehicles. To this end we aim to deliver innovation that improves health outcomes while optimising resource consumption, supporting climate action.

In 2020 we published a White Paper on Climate Change, in which EFPIA members committed to:

- Establish climate change policies and strategies based on materiality and impact for individual companies, addressing their entire value chains (where the majority of emissions occur)
- Pursue science-based CO2 reduction targets and to annually and publicly disclose CO2 performance calculated according to recognised methodologies (e.g. the Greenhouse Gas Protocol)
- Contribute to reduced energy consumption and increased energy efficiency, and seek opportunities to use energy from renewable sources throughout the value chain.

Our Climate Change White Paper highlighted many examples of how EFPIA members are already reducing CO2 emissions and switching to alternative energy sources at company sites, and improving sustainability throughout their global supply chains. Furthermore, our Circular Economy White Paper provides examples of how companies are designing out waste and pollution, keeping products and materials in use and regenerating natural systems.

Multiple initiatives under the Eco-Pharmaco-Stewardship (EPS) have contributed over the last couple of years to improving scientific understanding, finding new ways to detect the trace amounts of pharmaceuticals in the environment, understanding their impact, prioritising active pharmaceutical ingredients posing a potential risk to the environment and also further reducing discharges from manufacturing plants. As an industry, we are striving to continually enhance our processes to deliver life-saving treatments in ways that are also protective of the environment.

These include the AMR Industry Alliance, which has developed a framework for assessing and promoting safe discharge targets for antibiotic manufacturing and the #medsdisposal campaign established to improve the collection of unused or expired medicines. Moreover, innovative risk-based frameworks such as our extended Environmental Risk Assessment (eERA) model and research initiatives such as IMI iPiE and IMI PREMIER recognise the importance of bringing different stakeholders together to address the ongoing concerns around pharmaceuticals in the environment.

REFERENCES

European Alliance for Value in Health. Transforming towards value-based, sustainable, and people-centred health systems in Europe. 2020.
Available at https://www.europeanallianceforvalueinhealth.eu/wp-content/uploads/2020/11/EAVH-Transforming-European-Health-Systems-Feb-2021.pdf
https://www.europeanallianceforvalueinhealth.eu/value-based-health-systems/our-principles/

European Commission. Commission Green Deal Strategy December. European Commission, 2019.
Available at https://ec.europa.eu/info/strategy/priorities-2019-2024/european-green-deal_en

European Commission. Zero Pollution Action Plan May 2021. European Commission, 2021.
Available at https://ec.europa.eu/environment/strategy/zero-pollution-action-plan_en

EFPIA. Value-based healthcare – an industry perspective. 2019.
Available at https://www.efpia.eu/media/412971/value-based-healthcare-an-industry-perspective.pdf

EFPIA. Strengthening health systems through smart spending. 2020.
Available at https://www.efpia.eu/news-events/the-efpia-view/statements-press-releases/strengthening-health-systems-through-smart-spending/

EFPIA. White paper on climate change. 2020.
Available at https://www.efpia.eu/media/554662/white-paper-climate-change.pdf

EFPIA Webpage on Environmental, Health, Safety and Sustainability. 2021.
Available at https://efpia.eu/about-medicines/development-of-medicines/regulations-safety-supply/environment-health-safety-and-sustainability/

EFPIA. White paper on Circular Economy. 2020.
Available at https://efpia.eu/media/554663/circular-economy.pdf

Errea M, et al. Opportunities to increase efficiency in healthcare. Office of Health Economics, 2020.
Available at https://www.ohe.org/publications/opportunities-increase-efficiency-healthcare

European Academies Science Advisory Council; Federation of European Academies of Medicine. Decarbonisaiton of the health sector: a commentary by EASAC and FEAM. 2021.
Available at https://easac.eu/fileadmin/PDF_s/reports_statements/Health_Decarb/EASAC-FEAM_Decarbonisation_Health_Sector_Web_publication_FINAL.pdf

Eurostat. Preventable and treatable mortality statistics. 2020
https://ec.europa.eu/eurostat/statistics-explained/index.php/Preventable_and_treatable_mortality_statistics#Number_and_rate_of_avoidable_deaths

Fairve P, et al. Immunization funding across 28 European Countries. Expert Rev Vaccines 2021. Epub Mar 24

GAVI. New evidence shows investments in vaccination produce even greater returns than previously thought. 2020. Available at https://www.gavi.org/vaccineswork/new-evidence-shows-investments-vaccination-produce-even-greater-returns-previously

Lenzen M, et al. The environmental footprint of health care: a global assessment.
Lancet Planet Health 2020;4:e271–79

OECD. Cardiovascular disease and diabetes: policies for better health and quality of care. OECD, 2015
Available at https://read.oecd-ilibrary.org/social-issues-migration-health/cardiovascular-disease-and-diabetes-policies-for-better-health-and-quality-of-care_9789264233010-en#page72

OECD. Tackling wasteful spending on health. OECD, 2017.
Available at https://www.oecd.org/health/tackling-wasteful-spending-on-health-9789264266414-en.htm

OECD. Health at a glance: Europe 2018. OECD, 2018.
Available at OECD. https://www.oecd-ilibrary.org/social-issues-migration-health/health-at-a-glance-europe-2018_health_glance_eur-2018-en

OECD. Health at a glance 2019: OECD indicators. OECD, 2019.
Available at https://www.oecd-ilibrary.org/social-issues-migration-health/health-at-a-glance-2019_4dd50c09-en

OECD/European Union. Health at a Glance: Europe 2020: State of Health in the EU Cycle. OECD, 2020
https://www.oecd-ilibrary.org/social-issues-migration-health/health-at-a-glance-europe-2020_82129230-en?_ga=2.52510440.1357533625.1626362665-1150508910.1626362665

Ozawa S, et al. Return On Investment from childhood immunization in low- and middle-income countries, 2011-20. Health Aff (Millwood) 2016;35:199–20

WHO Regional Office for Europe. Environmentally sustainable health systems: a strategic document. WHO Regional Office for Europe, 2017. Available at https://apps.who.int/iris/handle/10665/340375

RESILIENCE: FUTURE PROOFING OUR HEALTH SYSTEMS

6

Europe must support innovation to strengthen future health systems.

Resilience encompasses all challenges

COVID-19 has been a rude awakening to the need for EU member states to consider – singly and collaboratively – how to make their health systems more resilient.

Resilience has been defined by some based on the ability to withstand and respond to health shocks such as COVID-19 while maintaining routine functions. The COVID-19 pandemic has certainly underscored the need to better prepare health systems to manage both the direct impact pandemics, and to avoid the collateral damage caused by the disruption to critical health services for other diseases.

Beyond COVID-19 we believe that resilience must encompass the health system's capacity to manage all types of public health challenges – including the rising pressure from age-related chronic diseases (Section 1), antimicrobial resistance, and the health threats posed by environmental risks and climate change. We must concertedly strengthen our health systems against all these long-term stresses, as well as unpredictable shocks.

POWER BUILDS RESILIENCE

POWERing up health systems – focusing on **people-centricity, outcomes, efficiency** and the **workforce** – will inherently strengthen them.

Why? Because healthier people are critical to build more resilient societies. The far higher rates of severe disease and death from COVID-19 in people with underlying conditions should once and for all teach us the societal value of investing smartly in better disease prevention and management throughout life.

In addition:

- Strengthened, digitally enabled public health systems will be better prepared to identify and address acute threats
- Optimised primary, community and home care services will help protect hospital capacity for people who need it – particularly during pandemics
- Building health literacy and patient empowerment will help combat disinformation that can undermine public health responses, as shown during the COVID-19 pandemic, and better enable patients to manage their own conditions.

The POWER priorities therefore align with many expert recommendations on improving health system resilience post COVID-19.

ENSURING MEDICINES SUPPLIES: RISING TO THE CHALLENGE

The COVID-19 pandemic has tested Europe's medicine supply chains as never before, dramatically increasing demand of certain critical products while ad-hoc and unilateral measures by Member States threatened supply processes during the first phase of the pandemic.

Medicines supply chains are complex, international, long-term enterprises involving many stakeholders. The average medicine has around 350 components whose supply must be carefully co-ordinated and timed to provide a finished product; the whole process typically takes around 2 years. For biological products, including vaccines, the process of production and distribution can be even more complicated.

For the most part, Europe's research-based pharmaceutical industry actively rose to the challenge posed by COVID-19 and maintained continuous supplies of medicines. Pandemic preparedness plans were triggered late 2019 allowing manufacturers to enhance both manufacturing and supply capacity. In addition, diversification of supply channels, demand forecasting, and in some cases close cooperation with health authorities and EU-level coordination of national measures (e.g. large orders for stockpiling purposes), were key elements of our collective response to the pandemic.

Pharmaceutical companies are constantly investing in strengthening their supply systems, for example using digital technologies (predictive analytics and artificial intelligence) to improve demand forecasting, agile supply chains and industry workforce support. These endeavours will need co-operation with, and support from, the EU and Member States to help overcome bureaucratic obstacles and ensure success. However, there is significant room for improvement, necessitating collaborative EU-wide action.

STRENGTH THROUGH EUROPEAN UNITY

Since diseases know no borders, EU Member States need to work together to strengthen their health systems and public health capacities. This should aim not only for all to be better prepared against cross-border threats, but toward equal access to healthcare and comparable health outcomes for patients.

The EU also needs reinforced capabilities to monitor national and regional healthcare demands and capacities in order to better inform the supply of essential medicines, medical equipment and other healthcare resources to better coordinate the allocation of supplies based on actual patient needs at Member State level, both in normal times and during emergencies.

Specific measures that would help strengthen supply chains and build resilience to future shocks include:

- Greater cooperation and transparency between EU agencies (especially the European Medicines Agency and European Centre for Disease Prevention and Control), national authorities, industry and other supply chain actors to provide better real-time data for demand forecasting and monitoring, and a common definition and reporting framework for shortages
- A solidarity-based allocation mechanism, with political backing from Member States and EU institutions to be triggered in case supplies are temporarily limited by external shocks and may not cover demand for medicines, as well as a joint understanding and visibility regarding the demand signals (either via patient need, or stockpiling, or economic demand by operators)
- The establishment of the proposed Health Emergency Preparedness and Response Authority (HERA) dedicated to improving Europe's capacity and readiness to respond to cross-border health threats and emergencies
- Regulatory harmonisation among relevant authorities to help make medicines distribution more agile
- Practical measures to reduce the logistical and administrative burden of medicines supply, such as flexible labelling requirements.

OPTIMISING CLINICAL TRIALS

Clinical trials are an essential step in bringing new treatments and vaccines to patients.

The COVID-19 pandemic has caused thousands of trials to be suspended or stopped globally. At the same time, the pandemic mobilised an unprecedented, collaborative research and development effort that has delivered regulator-approved COVID-19 vaccines and treatments of enormous value to society in record time.

All partners in the EU innovation environment must take this opportunity to urgently reflect on how we can optimise clinical trials to better serve patients and health systems.

These learnings may include:

- adaptations to improve resilience, e.g. through tele-health and remote monitoring to reduce the need for some clinic visits
- novel trial approaches, including 'adaptive' designs that can be updated as data accumulates, platform trials that allow multiple interventions to be tested simultaneously, and the wider use of real-world data to complement data from clinical trials
- frameworks that facilitate public-private collaboration
- harmonised EU-wide legislation that facilitates clinical trials with advanced therapy medicinal products consisting of or containing genetically modified organisms (GMO)
- streamlining regulatory approval processes.

INDUSTRY: PARTNERING FOR MORE RESILIENT HEALTH SYSTEMS

EFPIA is an active advocate and partner in strengthening the resilience of EU health systems. We fully support the goals of the European Commission on preparedness against cross-border threats and will work with all parties in the coming years to support their work in this crucial area, including via the Structured dialogue on security of medicines supply convened by the Commission.

In May 2021, an EFPIA-commissioned report assessed the impact of the COVID-19 pandemic on cancer care, integrating contributions from numerous stakeholder organisations and industry partners. This report made six specific recommendations to European health systems to help them recover optimally from the COVID-19 disruption, while adapting to be able to absorb future shocks more effectively.

- Clear the cancer backlog now, using innovative practices which emerged during the pandemic
- Maintain the proven agility of R&D and marketing authorisation processes
- Continue the intensified European collaboration in clinical assessment to use scarce HTA resources more efficiently after the pandemic

- Continue the adoption of digital health to increase remote care and use healthcare resources more efficiently after the pandemic
- Maintain and build adaptive surge capacity to be ready for future disruptions to cancer care
- Safeguard cancer budgets as a critical enabler for improving continuity, efficiency, and sustainability of cancer care.

EFPIA will continue to work collaboratively with all partners on other relevant initiatives with respect to broader aspects of improving health systems resilience, including on access to innovation to address unmet medical needs, and tackling the health system impacts of demographic ageing, antimicrobial resistance and climate change.

EFPIA is the founding member of the European Medicines Verification System, data from which could provide additional intelligence regarding the quantities supplied by manufacturers on the various markets as well as the quantities of medicines dispensed to patients at national level.

Individual EFPIA members are also working on specific programmes. For example, AstraZeneca has co-initiated the Partnership for Health System Sustainability and Resilience (PHSSR) together with the London School of Economics and the World Economic Forum, based on a shared commitment to improving population health through and beyond the COVID-19 pandemic. From new models of care, to innovative financing mechanisms and breakthrough technologies, the PHSSR aims to make change happen, by identifying transferrable solutions with the greatest potential, and supporting their adoption to deliver better health and better care for all. In March 2021, the initiative published interim findings and recommendations with respect to governance, financing, workforce, medicines and technology, and health service delivery, together with a series of country level and disease area-specific reports.

EFPIA members are also collaborating with research hospitals, regulators and patient organisations in IMI projects supporting health system resilience, such as:

In the EU Patient-Centric Clinical Trial Platforms (EU-PEARL) project, leading research hospitals, regulators, patient advocates and industry are working together to help shape the future of clinical trials. Specifically, this project aims to support a wider use of patient-centric 'platform' trials, which allow novel techniques and treatments developed by multiple companies and organizations to be tested efficiently to help address unmet needs. Patients and carers are integrally involved to ensure their perspectives are consistently incorporated in trial designs and outcome measures.

The Trials@Home Consortium, which aims to help move trials from the traditional clinic setting to the participant's immediate surroundings. These so-called Remote Decentralised Clinical Trials (RDCTs) use new digital innovations and enable participants to visit a clinical trial centre less frequently, if at all. This will help larger, more diverse and remote populations to participate in clinical trials. These trials are expected to be faster and more efficient to run, and to provide results that are more representative of the real world because the data are collected in the daily context of participants' lives.

REFERENCES

Bouvy F, Rotaru M. Medicine shortages: from assumption to evidence to action - a proposal for using the FMD data repositories for shortages monitoring. Front Med 2021;8:579822

Bakker A. Learnings from COVID-19: Partnerships leading the way for clinical trial modernisation. EFPIA website. Available at https://www.efpia.eu/news-events/the-efpia-view/blog-articles/learnings-from-covid-19-partnerships-leading-the-way-for-clinical-trial-modernisation-guest-blog/

European Commission. A European One Health Action Plan against Antimicrobial Resistance (AMR). European Commission, 2017 https://ec.europa.eu/health/sites/health/files/antimicrobial_resistance/docs/amr_2017_action-plan.pdf

EFPIA. Call for more effective EU regulation of clinical trials with advanced therapy medicinal products consisting of or containing genetically modified organisms. 2020. Available at https://www.efpia.eu/media/580554/call-for-more-effective-eu-regulation-of-clinical-trials-with-advanced-therapy-medicinal-products-consisting-of-or-containing-genetically-modified-organisms.pdf

Expert Panel on Effective Ways of Investing in Health (EXPH). The organisation of resilient health and social care following the COVID-19 pandemic. European Union, 2020. Available at https://ec.europa.eu/health/sites/health/files/expert_panel/docs/026_health_socialcare_covid19_en.pdf

Haldane V, et al. Health systems resilience: meaningful construct or catchphrase? Lancet 2017;389:1513

Jansen C, Amesz B. Every day counts – the impact of COVID-19 on patient access to cancer care in Europe. EFPIA 2021. Available at https://efpia.eu/media/602636/every-day-counts-covid19-addendum.pdf

Kruk ME, et al. What is a resilient health system? Lessons from Ebola. Lancet 2015;385:1910–2

Lamont EB, et al. Trends in oncology clinical trials launched before and during the COVID-19 pandemic. JAMA Netw Open 2021;4:e2036353

Lal A, et al. Fragmented health systems in COVID-19: rectifying the misalignment between global health security and universal health coverage. Lancet 2021;397:61–7

Thomas S, et al. Strengthening health systems resilience – key concepts and strategies. European Observatory on Health Systems and Policies, 2020

Van Dorn A. COVID-19 and readjusting clinical trials. Lancet 2020;396:23–4

Watts N, et al. The 2020 report of The Lancet Countdown on health and climate change: responding to converging crises. Lancet 2021;397:129–170

Wharton G, et al. The Partnership for Health Sustainability and Resilience. Interim report of the pilot phase. 2021. Available at http://www3.weforum.org/docs/WEF_PHSSR_Interim_Report_of_the_Pilot_Phase.pdf

INNOVATION: PUTTING POWER TOOLS TO WORK

7

Future health systems must embrace innovations that generate value.

The innovation imperative

The COVID-19 pandemic has underscored the critical importance of research and innovation for public health, economies and society as a whole.

It has triggered an unprecedented mobilisation involving public research agencies, academia, private foundations and charities, and industry. These efforts have delivered astonishing progress in record time, both in terms of understanding this new disease, devising public health strategies, providing treatments and delivering an array of highly effective vaccines (see **Section 6**).

The European research-based pharmaceutical industry sector was well placed to play a leading role in the global response to COVID-19 because of its long-standing commitment and investment in research and innovation in Europe. In particular, we have:

- led the search for vaccines, diagnostics and treatments to find a permanent route out of COVID-19: currently worldwide more than 288 vaccine candidates are in development – 104 of these are in clinical trials. Four vaccines have been approved by the European Medicines Agency.
- ensured the supply of medicines, linking manufacturers and the European Medicines Agency in order to anticipate and proactively address any potential disruptions.
- supported governments in strengthening their health systems by ramping up the production of successful vaccines, and to ensuring that approved treatments and vaccines are available and affordable.

We believe in the power of innovation to help address the challenges facing health systems – sustainably improving and extending lives while increasing value for money, and tackling emergency threats. Innovation in this context means not only medicines and vaccines, but diagnostics, imaging, medical devices and surgical techniques, together with integrated models for health service design, delivery, management and financing.

To help POWER up health systems, Europe must put innovation to work holistically across all parts of the health system, where it generates value for patients and the system as a whole. Together we need to ensure that European health systems have the power tools they need.

INCENTIVISING INNOVATION THAT GENERATES VALUE

During the COVID-19 pandemic, the research-based pharmaceutical industry has joined forces with other sectors to develop diagnostics, treatments and vaccines with an unprecedented speed and success. Regulators and policymakers have streamlined regulatory processes and employed emergency measures to facilitate the search for potential treatments and vaccines.

As well as reflecting on how we can use these learnings to optimise clinical trials (see Section 6), we should use this momentum to harness innovation to POWER up healthcare systems. This can only be done by co-ordinated EU and national policies and ecosystems that embrace innovations that bring value to patients and health systems.

Value and affordability: taking a holistic approach

The current fragmentation and siloed nature of budgets within and across health and social systems means that the value and affordability of pharmaceuticals and other health innovations tends to be considered only within the limited context of a specific budget. As a result, the value that innovative treatments can provide across healthcare budgets cannot be properly assessed, leveraged or incentivised to allow patients, health systems and society to reap the full benefit.

In addition to bringing the promise of longer and healthier lives for patients, many innovations can help to reduce costs in other parts of the health or social care systems, for example by reducing the need for specialist medical or surgical care, acute hospitalisations, palliative care or long-term nursing care.

Discussions about 'affordability' should therefore not be focussed on pharmaceutical spending in isolation, but according to the value that innovation generates for patients and health systems throughout the continuum of care and across all components of the health, social care and welfare system. To this end, regulation and HTA will need to embrace patient perspectives (including patient-reported outcomes) and robust real-world data.

To illustrate the importance of this holistic approach:

- According to the OECD, on average, pharmaceuticals (both retail and hospital) account for around one fifth of total health systems expenditure across the EU. Pharmaceuticals spending varies considerably between countries, both as a percentage of health spending and GDP. Pharmaceutical spending was relatively stable, rising by an average of 1.4% per year between 2013 and 2018, after falling by 1.2% per year between 2008 and 2013 owing to cost-containment policies. By contrast, average spending on long-term care related to ill health rose three-fold more quickly, at 4.3–4.4% per year, throughout 2008–2018. Health-related long-term care already accounts for a quarter of health spending in the Netherlands, Sweden and Denmark, compared with an EU average of 12%. The OECD projects that expenditure on long-term care as a percentage of GDP could double by 2060. Innovative treatments and services that reduce demands for long-term care therefore have enormous potential value to society.
- Innovative treatments may also allow patients to return to work, thereby paying taxes and contributing to economic productivity and cutting costs of welfare support. One recent study estimated that comprehensive investment in care for 11 cancers in high-income countries – representing a 6% increase in costs – would provide a 3.7-fold return on investment by 2030 owing to productivity gains in the economy. The highest return was estimated from imaging (129-fold) and treatment (17-fold).
- The panel below provides further examples of how specific types of innovative medicines can bring value across sectors.

Affordability should also be interpreted in light of the estimate that health systems waste approximately as much money (up to 20% of resources) as they spend on all pharmaceuticals combined, and yet often have limited ability to redirect spending to improve efficiency.

Value gains from innovation:

Examples of potential value gains from specific innovations include:

- Checkpoint inhibitor combinations used in non-small cell lung cancer could extend the lives of patients and offer potential for a cure in patients where prognosis is very poor – saving costs of hospitalisation and palliative, care and allowing patients to return to work, where an estimated €717 million could be generated in GDP each year.

- Chimeric antigen receptor (CAR T) cell therapies offer the potential to save the lives of children and adults with certain cancers who otherwise have a very poor prognosis. Estimates suggest that curing cases of acute lymphoblastic leukaemia in 2020 alone could contribute up to €5.0 billion to GDP across the EU.

- Gene therapies offer the potential to cure various life-long genetic diseases, thereby avoiding the costs of life-long specialist care and support for these disorders and their complications, and instead allowing patients to have normal, productive lives.

- Other examples with important potential include mRNA vaccines in certain cancers, curative therapies for HIV and hepatitis B virus infection and disease-modifying therapies in Alzheimer's disease.

Novel payment models

Our industry believes that, when used appropriately, novel pricing and payment models can accelerate patient access, allowing payers to manage clinical uncertainty, budget impact and sustainability of the healthcare system, whilst providing sufficient incentives for innovation.

In contrast to conventional pricing models (such as simple discounts, rebates, price-volume agreements or caps), novel pricing approaches relate the price of a product to its value to patients and health systems. Novel payment approaches condition payment according to the value of the product (in terms of observable outcomes), spread payment over time, or decouple payment from the number of patients treated (see Panel).

Based on the experience of using these models today, there is already evidence to show how they can accelerate and broaden patient access, and contribute to improving the sustainability of healthcare systems.

Key examples of novel pricing and payment models

Indication-based pricing

This approach allows the pricing of a product to reflect its observed value in specific therapeutic indications. It is based on the notion that a medicine may offer different benefits to different groups of patients, and hence its value may differ by indication.

Combination-based pricing

Combination-based pricing addresses the challenge that the value of products used in combination is not simply the added value of the medicines used separately. It seeks to resolve the additional complexities in assigning value and negotiating prices for individual products used in combinations.

Outcomes-based payment

This approach means that payment for a medicine is conditional on its real-world performance, based on observable outcomes. This can be useful when the magnitude and durability of the clinical benefits, or the medicine's real-world performance may not be fully clear at launch. Various outcomes-based payment models have been developed, tailored to specific needs. For example, 'coverage with evidence development' allows access to the product on the condition that additional evidence is provided at a defined point in time.

Over-time (or staggered) payment

These models allow payers to make payments to manufacturers over fixed periods for each patient that receives therapy. Structuring payments this way helps mitigate high upfront costs associated with some one-off therapies. When the over-time payment is linked to a particular outcome being observed, necessitating the collection of real-world outcomes data, these can also be a way for payers to address uncertainty regarding clinical benefit at launch.

Subscription payment

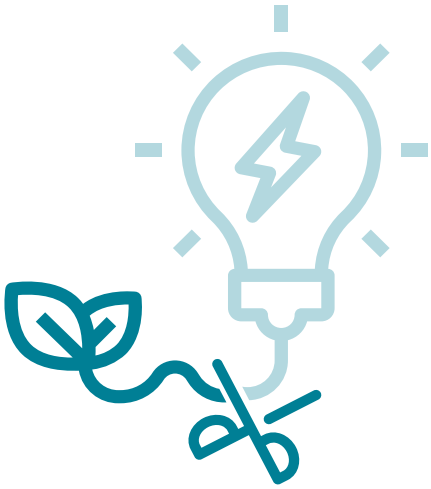
This approach may be used in some circumstances to decouple payment for a medicine from the number of patients treated. Such models could help payers to anticipate the budget impact associated with treating patients in a given disease area and ensure its sustainability in the long term.

However, the lack of appropriate data infrastructure, legal barriers and an unwillingness to adapt current systems often prevent the use of these models.

The EFPIA report Novel pricing and payment models: new solutions to improve patient access proposed the following principles to guide the use of novel payment models and to help overcome these barriers:

- **Access Principle:** Novel pricing and payment models should facilitate broad and timely patient access whilst balancing the sustainability of the healthcare system and incentives for innovation.
- **Value Principle:** A high quality, methodologically robust and mutually agreed value-based framework is the foundation for novel pricing and payment models.
- **Collaboration Principle:** Payers and companies should work together to anticipate where these models are needed and ensure they are fit for purpose.
- **Transparency Principle:** The existence of novel agreements should be transparent and disclosed, and information collected should be shared according to specifically defined principles, including mutual agreement and patient confidentiality. The industry is committed to working constructively with the European Commission, national policymakers and other stakeholders to advance the much-needed cross-stakeholder dialogue on transparency. Open collaboration and a shared commitment among payers, policymakers and the industry are required, together with the active engagement of healthcare professionals and patient organisations.
- **Infrastructure Principle:** Stakeholders should work together to ensure the required data infrastructure is fit for purpose and appropriate legal frameworks are in place.

BLOCKING INNOVATION IMPEDES POWER



European health systems are not exploiting the full value of innovation. The Patients Waiting to Access Innovative Therapies (Patients WAIT) survey showed the variations that exist between EU member states in the range of new medicines available to patients, and the delays in their availability (Panel). The OECD has also drawn attention to wide variations across Europe in the approval and reimbursement of medicines, specifically for cancer.

Patients WAIT survey

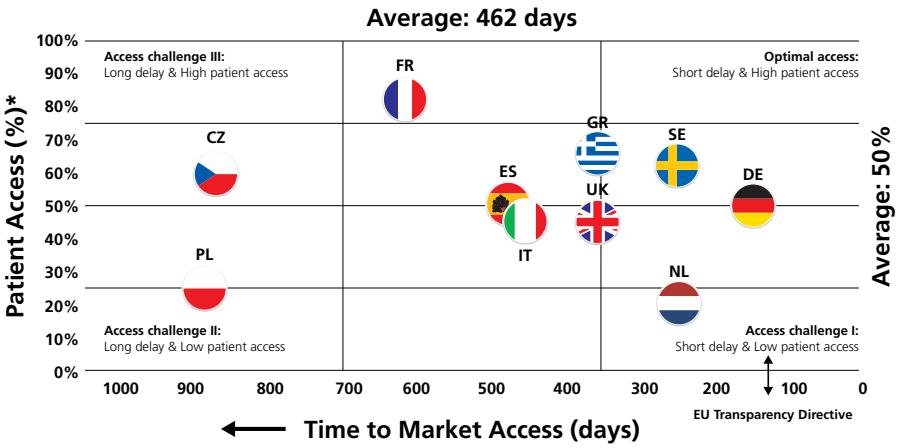
Overall, in 2019, the average delay between market authorisation and patient access varied by more than six-fold across Europe. Patients in Northern/ Western Europe could access new products 100–350 days after market authorisation, compared with 600-850 days in Southern/Eastern Europe. For some products, variations within some countries were even larger than variations between countries.

To exemplify the variation, patients in Estonia and Latvia on average had to wait over 900 days until novel cancer treatments were publicly reimbursed. That is 28 times longer than patients in Germany. Patients in France had to wait 560 days, 15 times longer than those in Germany. In this regard, Germany has demonstrated how any similarly high-income country can accelerate access to innovation that adds value. We acknowledge that countries in other parts of Europe face particular resource pressures.

For orphan medicines, the average delay between market authorisation and patient access for Orphan drugs varied from 3.7 months to 3.2 years. Moreover, 15% of countries had no access to any of the orphan medicines approved between 2015 and 2018.

The following figure also illustrates the wide inequities among EU health systems in the market access and patient access to new cancer therapies

Within Europe, huge inequalities exist in time to Market Access and Patient Access to new oncology therapies.



* Cumulative use after 12 months of reimbursement, relative to the country with the highest cumulative use

Sources: QVIA, 2020 and Vintura, 2020

Everyone involved in healthcare – from patients to service providers, researchers to clinicians, pharmaceutical companies to payers – wants patients to be able access new treatment options equitably across Europe.

EFPIA has identified 10 interrelated factors that explain unavailability and delays. These are rooted in the medicines access systems and processes in the EU Member States and the corresponding impact on commercial decision-making. They range from a slow regulatory process to late initiation of market access assessment, to duplicative evidence requirements, to reimbursement delays, and local formulary decisions, together with readiness of the health system to deliver innovative therapies (e.g. infrastructure, workforce enablement and suitable care pathways and guidelines). As the root causes are multifactorial, they can only be solved by different stakeholders working together.

The industry is a key partner to governments, regulatory authorities, and patients in ensuring that we all have an effective innovation ecosystem that drives new treatments to patients as fast as possible. We therefore welcome collaborative EU-level dialogue to identify the root causes of access disparities, delays and barriers and to design solutions.

Access solutions will also require a close collaboration of national healthcare systems (including HTA and pricing and reimbursement bodies) on issues such as pan-European evidence generation, meaningful joint clinical assessments, and novel pricing and payment models.

Together with our partners in the EU Health Coalition, we call for a permanent, high-level, multi-stakeholder Forum for better access to innovation. This would provide a space where all stakeholders – Member States, national and regional authorities, patients, civil society, healthcare professionals, policymakers, and industry can discuss the factors driving and impeding patients' access to all types of health innovation.

INDUSTRY: PARTNERING FOR A THRIVING INNOVATION ECOSYSTEM

The EU should be a global hub for research and innovation in health. We need to build a long-term, collaborative ecosystem that supports and incentivises research and development, including through interdisciplinary networks, cross-border initiatives, health data infrastructures, public-private and public-public partnerships, as called for by the EU Health Coalition.

To this end, EFPIA is partnering with other industry associations in the Innovative Health Initiative (IHI), a new proposed Joint Undertaking (public-private partnership) co-funded by the European Union under Horizon Europe. The IHI aims to enable the cross-sectoral integration of technologies, know-how, products, services and workflows for people-centred healthcare. Its ambition is to support the delivery of timely and well-substantiated prevention, diagnosis and treatment. The partnership aims to help keep EU citizens in good health, decrease disease burden for patients, care givers and health care professionals. It will also contribute to the sustainability and resilience of health care systems, to the competitiveness of health industries and to the EU technological strategic autonomy.

To be fit for purpose, such partnerships need to include:

- Transparent, open, and efficient processes that engage all relevant stakeholders in defining priorities and developing calls
- A solid intellectual property framework
- Suitable conditions that allow industry to contribute valuable assets to the Partnership that would otherwise not be accessible to the research community
- Possibility to enable global collaboration by bringing expertise and assets into EU projects from third countries and research hubs abroad.

Industry is already working on numerous public-private partnerships via the Innovative Medicines Initiative, many of which are featured throughout this paper.

REFERENCES

De la Maisonneuve C, Martins. Public spending on health and long-term care: a new set of projections. OECD, 2013 https://www.oecd.org/economy/growth/Health%20FINAL.pdf

EU Health Coalition. A shared vision for the future of health in Europe: lessons learnt from the COVID-19 pandemic. 2020. Available at https://www.euhealthcoalition.eu/recommendations/

EFPIA. Addressing healthcare challenges. Novel pricing and payment models: new solutions to improve patient access. 2020. Available at https://efpia.eu/media/554543/novel-pricing-and-payment-models-new-solutions-to-improve-patient-access-300630.pdf

Innovative Health Initiative. Strategic research and innovation agenda, 2021. Available at https://www.euhealthppp.org/strategic-agenda

IQVIA. EFPIA Patients wait indicator survey. 2020. Available at https://www.efpia.eu/media/554526/patients-wait-indicator-2019.pdf

EFPIA. The root cause of unavailability and delay to innovative medicines: reducing the time before patients have access to innovative medicines. 2021. Available at https://www.efpia.eu/media/602653/root-cause-unavailability-delays-cra-report-may-2021-final.pdf

EFPIA. Strengthening health systems through smart spending. 2020 https://www.efpia.eu/news-events/the-efpia-view/statements-press-releases/strengthening-health-systems-through-smart-spending/

EFPIA. Healthier future. The case for outcomes-based sustainable healthcare. 2020. Available at https://www.efpia.eu/media/412313/the-case-for-outcomes-document-17102018.pdf

IQVIA. EFPIA pipeline review 2021 update. https://www.efpia.eu/media/589818/iqvia_efpia_pipeline-review_final-report-2021-public-19apr21v2.pdf

OECD/European Union. Health at a Glance: Europe 2020: State of Health in the EU Cycle. OECD, 2020. Available at https://www.oecd-ilibrary.org/social-issues-migration-health/health-at-a-glance-europe-2020_82129230-en?_ga=2.52510440.1357533625.1626362665-1150508910.1626362665

OECD. Tackling wasteful spending on health. 2017. Available at https://www.oecd.org/health/tackling-wasteful-spending-on-health-9789264266414-en.htm

OECD. Addressing challenges in access to oncology medicines. OECD, 2020. Available at https://www.oecd.org/health/health-systems/Addressing-Challenges-in-Access-to-Oncology-Medicines-Analytical-Report.pdf

OECD. Pharmaceutical spending (indicator). 2021 https://data.oecd.org/healthres/pharmaceutical-spending.htm

Vintura. Every day counts – improving the time to patient access to innovative oncology therapies in Europe. 2020 https://www.vintura.com/wp-content/uploads/2020/08/White-paper-every-day-counts-improving-time-to-patient-access-to-innovative-oncology-therapies-in-europe_from-EFPIA_and_Vintura.pdf

Ward ZJ, et al. Global costs, health benefits, and economic benefits of scaling up treatment and imaging modalities for survival of 11 cancers: a simulation-based analysis. Lancet Oncol 2021;22:341–50

World Health Organization. COVID-19 vaccine tracker and landscape. https://www.who.int/publications/m/item/draft-landscape-of-covid-19-candidate-vaccines (13 July 2021)

DIGITAL HEALTH: THE POWER BEHIND POWER

8

Digitalisation is the future of healthcare

The term digital health encompasses e-health, mhealth, telehealth, electronic health records, remote monitoring, connected devices, digital therapeutics and more. It means embracing data, artificial intelligence (AI) and machine learning to improve healthcare.

Many forms of digital health proved their worth during the COVID-19 crisis, for example telehealth measures allowing health services to be maintained, disease surveillance and mechanisms supporting medicines supply chains.

However, these represent only a fraction of the potential of digital health. System-wide digitalisation will revolutionise all future healthcare – powering the integrated, **people-centric**, **outcomes-based** health systems we need.

It will help

- patients, carers and the public to engage better with health services and researchers, and to manage their own health better
- the workforce to make better and faster decisions about patient care
- payers to allocate resources more efficiently
- innovators deliver treatments that meet people's needs and generate value across the system
- health systems monitor, learn and adapt – making them more resilient.

We strongly support digitalisation within our industry and the health sector as a whole. Together we must lay the right foundations now and we believe certain overarching principles should underpin all forms of digital health:

- It should enhance (not merely replace) healthcare. 'Digital where possible – human when needed' is one way to look at this
- It must be trusted by all stakeholders
- It must not widen inequities via any form of digital divide, and hence ensuring digital health literacy and enablement is vital.

"It is clear: digital tools can significantly improve the capacity and efficiency of health systems, as well as citizens' health and well-being. They can also empower the patients. The digital transformation is essential to help European countries recover from the pandemic – to build stronger and more resilient health systems, to and support long-term competitiveness and innovation within the EU's medical industry."

Stella Kyriakides, European Commissioner for Health and Food Safety 2020

KEY CHALLENGES: FRAGMENTATION AND INCENTIVES

Digitalisation in the health sector is held back not by a lack of data, but by its fragmentation and lack of interoperability.

In fact, health systems produce huge amounts of data from various sources, such as electronic health records (where these exist), disease registries, post-authorisation medicines surveillance systems, reimbursement systems, mobile health apps and other wearable technologies. However, most health data is not put to work – around 80% remains untapped. Where it can be used, its use is often too limited to a single purpose, hence its potential is not realised.

This is partly because of well-recognised technical issues that can be overcome, namely:

- data collection systems are often not interoperable, meaning they cannot 'talk' to each other. The COVID-19 pandemic starkly highlighted this, since in many cases it was not even possible to compare the number of deaths between EU countries because of differences in data collection and classification
- they vary in quality, and/or
- there is a lack of suitable infrastructure and legislative framework for sharing, especially cross-border and on an EU level.

More fundamentally, the bodies that own or control data are currently not incentivised to share these, and the relevant data-sharing regulations are ambiguous or inhibitory. For example, variable interpretations of the EU General Data Protection Regulation (GDPR) result in different regulatory environments, some that foster digital healthcare (e.g. Estonia) and others present greater barriers (e.g. Germany).

Moreover, while many systems record data on healthcare consumption and activities, there is a lack of comprehensive measurement of health outcomes that can be compared across health systems (Section 3).

EU-WIDE PRIORITIES

The digital transformation needs EU-wide political leadership and collaboration between patients, the public, the **health workforce** and technical specialists.

EFPIA applauds the European Commission's efforts to create an EU policy environment that unlocks the value of the digital and data economy across all sectors.

The two priorities are clear: health data has a huge potential to improve patient care, health systems and research and provides an opportunity we cannot afford to miss, but suitable infrastructures and clear governance with accountability are needed to ensure its availability for different purposes, ensuring privacy and security, as well as quality.

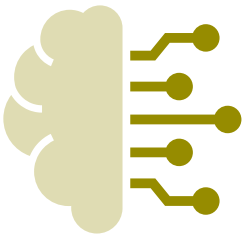
Governance

Healthcare decisions can be critical and European citizens must feel that their data is secure and being used with a positive healthcare intent. Indeed, EU citizens generally support digital health, with 80% agreeing to share their health data if privacy and security are ensured.

Therefore, Europe needs a legal and ethical governance framework for digital health that is fit-for-purpose and trusted by all parties. It should balance safety and data protection in context with the benefits for the individual and the health system. EFPIA is committed to working with all stakeholders to develop this, and have published specific recommendations to this end.

- The overarching principles should be people-centricity and societal benefit: if citizens own and control their own data, and health actors are incentivised to improve health outcomes, then data sharing will be normalised. The GDPR legislation already provides the governance framework, but implementation mechanisms are lacking.
- Citizens must have access to their data and remain in control of it. They must be free to choose the purposes for which their data are used; potential recipients in healthcare and research should be authorised subject to ongoing review. Data-sharing consent needs to be harmonised across the EU, based on a broad consent for reuse for scientific research purposes, with the option to opt-out.
- The governance framework should support the use of data for research and policymaking purposes (secondary uses), as well as for healthcare (primary use). Clarification and harmonisation of the rules in this regard is necessary. We also believe that data governance requirements should be proportionate to the risk associated with the intended use of the data.
- Overall we support the guiding principles underpinning the European Open Science Cloud, namely that data should be 'as open as possible and as closed as necessary', and be 'FAIR', i.e. Findable, Accessible, Interoperable, and Re-usable.

ARTIFICIAL INTELLIGENCE

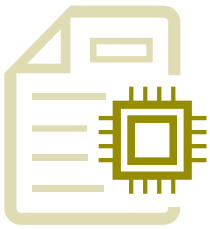


AI can bring significant opportunities for keeping people healthy, improving care, saving lives and saving money for healthcare systems.

EFPIA fully embraces the benefits that AI-powered solutions could bring to patients, healthcare professionals and health systems. Our vision is to maximise its potential to develop novel therapies and approaches to identify, treat and care for patients more efficiently, while preserving patient safety and privacy. However, further collaboration is needed to identify and address potential or unforeseen gaps in the current existing regulations, frameworks, and best practices.

We have published principles and recommendations to guide the designing of an AI legislative framework in the EFPIA Position Paper on AI.

INDUSTRY: PARTNERING FOR DIGITAL TRANSFORMATION



EFPIA and its members are committed expert partners in digital transformation, including via the following multi-stakeholder initiatives.

Data Saves Lives

Europe needs a health data coalition to build public understanding of the value of health data, and enhance confidence in how data are collected and used. The European Patients Forum is leading a vital initiative called Data Saves Lives. This platform will enable dialogue between key stakeholders to promote responsible use of health data and to facilitate societal understanding and will contribute to a change in attitudes to data-sharing

EHDEN
EUROPEAN HEALTH DATA & EVIDENCE NETWORK

The European Health and Evidence Data Network (EHDEN) project was launched to unlock the potential of real-world clinical data across Europe, overcoming the challenges that currently limit its use. It will develop a federated, harmonised EU-wide network for real-world data, with over 100 millon health records standardised according to a common data model. It will establish a self-sustaining open science collaboration supporting academia, industry, regulators, payers, governments and NGOs and the EHDEN Academy to train all stakeholders.

DRAGON

Dragon (RapiD and SecuRe AI enhAnced DiaGnosis, Precision Medicine and Patient EmpOwerment Centered Decision Support System for Coronavirus PaNdemics) will use AI and machine learning to deliver a decision support system for precise coronavirus diagnosis using computed tomography (CT) scanning. It also aims to be able to better predict the outcomes of patients, and to empower patients and citizens as partners in helping to better diagnose and treat COVID-19.

Mobilise-D

A slow walking speed is associated with greater mortality, morbidity, cognitive decline, dementia, and fall risk. As the population ages, the number of people experiencing mobility challenges is expected to rise. Mobilise-D will develop a comprehensive system to monitor and evaluate people's gait based on digital technologies, including sensors worn on the body. The project focuses on conditions which often affect mobility, namely chronic obstructive pulmonary disease, Parkinson's disease, multiple sclerosis, hip fracture recovery, and congestive heart failure. The results will help to improve the accurate assessment of daily life mobility, thereby contributing to improved and more personalised care.

IDEA FAST

IDEA-FAST aims to identify digital endpoints that provide reliable, objective and sensitive evaluation of fatigue, sleep, activities of daily life, disability and health-related-quality of life for people with neurodegenerative diseases (Parkinson's disease and Huntington's disease) or immune-mediated inflammatory diseases (rheumatoid arthritis, systemic lupus erythematosus, primary Sjögren's syndrome or Inflammatory bowel disease).

REFERENCES

Digital Europe. DIGITALEUROPE recommendations on health data-processing, 2020.
Available at https://www.digitaleurope.org/resources/digitaleurope-recommendations-on-health-data-processing/

European Commission. The EU's open science policy.
Available at https://ec.europa.eu/info/research-and-innovation/strategy/strategy-2020-2024/our-digital-future/open-science_en

European Commission. Digital Health and Care in the EU.
Available at: https://digital-strategy.ec.europa.eu/en/library/infographic-digital-health-and-care-eu

EFPIA. Position on a European Health Data Space. EFPIA 2020.
Available at https://www.efpia.eu/media/554841/efpia-ehds-position_final.pdf

EFPIA. Enabling self-efficacy through digital technologies and innovative therapies. 2019.
Available at https://www.efpia.eu/media/413161/enabling-self-efficacy-through-digital-technologies-and-innovative-therapies.pdf

EFPIA. Position paper on artificial intelligence. 2020.
Available at 2020 https://www.efpia.eu/media/580528/efpia-position-on-ai.pdf

EU Health Coalition. A shared vision for the future of health in Europe: lessons learnt from the COVID-19 pandemic. 2020. Available at https://www.euhealthcoalition.eu/recommendations/

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