



European Federation of Pharmaceutical Industries and Associations

EFPIA Annual Report 2015

From innovation to outcomes





The industry is committed to working with all stakeholders in healthcare to underscore the importance of relationships and to ensure that the benefits of greater transparency are understood ”

Introduction to Annual Report 2015

Dialogue and partnership have never been so important.



**RICHARD
BERGSTRÖM**
Director General,
EFPIA

As European healthcare systems continue to grapple with rising demand, driven by an ageing population and the increased prevalence of chronic disease, I have witnessed a growing recognition that no single part of the healthcare eco-system in isolation can provide all the solutions. To address these significant challenges requires new approaches to and higher levels of dialogue, partnership and collaboration. EFPIA and its member companies have been committed to and engaged in these processes throughout 2015.

Our commitment is no better illustrated than by the Innovative Medicines Initiative (IMI), Europe's successful partnership for health. Tackling issues such as anti-microbial resistance, better use of healthcare data and the evolution of the European regulatory environment – in line with advances in science and a range of therapy areas – IMI continues to bring together academia, healthcare systems, regulators and industry, to tackle some of the most significant challenges in speeding the path of innovative medicines to patients.

2015 witnessed the celebration of the 50th Anniversary of pharmaceutical legislation in Europe, which has made a significant contribution to science, medicine and patients. As its member companies forge ahead with creating an exciting, new wave of pharmaceutical innovation, EFPIA continues to work with stakeholders to ensure that the regulatory and policy environment keeps pace with the rapidly-advancing science, supporting projects that include the EMA-led ADAPT SMART, and programmes that foster access to life-changing treatments for the right patient groups, at the earliest appropriate time in the product life-span, in a sustainable fashion.

New innovation is only meaningful, though, if patients across all European Member States are able to access it. During 2015, EFPIA and its member companies have been working with governments and healthcare systems to find solutions to improve access to medicines and the sustainability of healthcare, at the same time securing future medical innovation. We have witnessed the advent of new approaches, such as outcomes-based reimbursement and managed entry agreements, as well as the development of national stability pacts that balance budget stability and access to medicines, with support for new innovation.

New partnerships in 2015, extended to the creation of the European Medicines Verification Organisation, a landmark step in the fight against counterfeit medicines. Set up on 13 February 2015, EMVO is a not-for-profit stakeholder organisation that represents a key tool in combatting the emergence of falsified medicines in the EU legitimate supply chain and improving patient safety. It represents the culmination of four years of intensive work towards a dependable and secure pharmaceutical verification system.

Meaningful partnerships mean finding new, open and transparent ways of working together. During 2015, EFPIA members companies have been collecting data on collaborations with health professionals and healthcare organisations, which will be disclosed publicly in June 2016. It will be the first time ever that these transfers of value have been made public across Europe, and the industry is committed to working with all stakeholders in healthcare to underscore the importance of these relationships and to ensure that the benefits of greater transparency are understood.

In addition to the comprehensive coverage of EFPIA's activities throughout the year, the 2015 Annual report focuses on a range of key issues across European healthcare, scrutinising and appraising the impact of medicines, the role of data in the future of healthcare and the adoption of outcomes-based, sustainable models of healthcare delivery. As I look forward to 2016, continuing the dialogue, developing partnerships and effective collaboration will be key to meeting the many challenges faced by healthcare in Europe. EFPIA and its member companies are committed to playing their part.

Yours faithfully,

Richard Bergström



During 2015, EFPIA and its member companies have been working with governments and healthcare systems to find solutions to improve access to medicines and the sustainability of healthcare, at the same time securing future medical innovation ”

Foreword by the President

2015 was an important year for the European pharmaceutical industry. It marked the 50 year anniversary of the EU Pharmaceutical Legislation, 20 years since the foundation of the European Medicines Agency, and 15 years since the EU Orphan Medicines Regulation came into effect.



JOE JIMENEZ
EFPIA President
CEO Novartis

Looking back, what has been achieved is impressive. We now have **medicines for many conditions** we were unable to treat before. EMA has approved a total of 975 medicines¹ since its foundation, many of which first in class. 140 therapies alone have received a marketing authorisation for diseases so rare they affect no more than 5 in 10,000 Europeans². **Medicines are also safer** than ever before. We have a rigorous regulatory framework and a world-class medicines agency in Europe. High standards for clinical trials ensure strict safety standards for products coming to market. Robust pharmacovigilance systems are used to monitor the safety of products in clinical practice. Today's **medicines are also more effective** than ever before. HIV can now be treated like a chronic condition, with death rates down 75% since 2004³. We can cure Hepatitis C with a 12 week course of medicine. New therapies for Rheumatoid Arthritis target the causes of inflammation rather than treating the symptoms alone.

This tremendous progress is the result of continued research investments of our companies, combined with an effective intellectual property and regulatory framework. In the EU alone, we invested on average 25 billion Euros every year in the last decade⁴. These investments have also laid the foundation for **additional breakthroughs** in the next years. Cell therapy may allow the re-programming of a patient's own T-cells so that they attack the cancer cells in the patient's body. This would be a true step-change in the fight against cancer. The promise of gene therapy is to replace a defective gene that causes a disease with a healthy copy of that gene and ultimately help us cure diseases like Cystic Fibrosis. Advances in disease-modifying therapy could also provide genuine solutions for Alzheimer's disease.

However, our industry must help healthcare systems in Europe become sustainable as they face a rapidly ageing population. In order for Europe to deal with demographic change ahead, and to ensure sustainable access to innovation, the industry and the systems we operate in must continue to evolve. Building on our work in 2015, I see three areas of focus going forward:

In 2015, we have initiated several projects to accelerate the transition towards an **outcomes-based approach** to healthcare. Systematically tracking health interventions and their impact on outcomes allows systems to allocate resources where they deliver the best possible outcomes. Eliminating interventions that don't work frees up the needed resources to address the healthcare needs of an ageing population. In 2016, the IMI "Big Data 4 Better Outcomes" programme will see the formation of public-private consortia. These will agree common outcomes definitions, build the data needed to measure outcomes, and identify options to improve outcomes in key disease areas. Our "Value of Health" initiative that seeks to build political will to move towards an outcomes-focused approach will also continue into 2016. With "Healthier Futures" we will launch EFPIA's vision for an outcomes-driven sustainable future.

Sustainable access was another priority in 2015. We engaged with healthcare decision makers across Europe to explore if a pan-European system for Relative Efficacy Assessments (REA) could be established. These clinical assessments of new medicines are currently done separately in each Member State, based on national methodologies. This not only creates access delays for patients; it also leads to unequal access across Europe, which we aim to solve in collaboration with Member States. While REA should be done

scientifically at the European level, pricing and reimbursement must be kept at the country level, due to the different cultures and values that determine what a country is willing to pay for an innovation. We will also build on our work with EMA to co-develop systems for adaptive approval and access, so that innovation reaches patients as early as possible without compromising safety. Through the Innovative Medicines Initiative, we will continue to coordinate collaborative responses to global health threats like antimicrobial resistance.

2015 also showed that healthcare systems were unprepared for some of the innovations coming to market. Going forward, we need to help our healthcare system partners understand better **what type of innovations are coming** and their impact on healthcare systems. With enough time to plan ahead, systems can make relevant adjustments to allow new therapies to deliver their full potential. The way forward may be novel financing models, structural changes, or new capabilities. Partnering with healthcare systems in a solution-oriented way will enable us to jointly realise the promise of new medicines, while ensuring the right incentives are in place for future innovation.

I look forward to continuing to work with our stakeholders on this journey.

Joe Jimenez

¹ European Medicines Agency opinions on human and veterinary medicines, http://www.ema.europa.eu/docs/en_GB/document_library/Other/2015/01/WC500180920.pdf.

² European Medicines Agency, 1995-2015 – Celebrating 20 years, http://www.ema.europa.eu/docs/en_GB/document_library/Brochure/2015/03/WC500184383.pdf.

³ HIV/AIDS surveillance in Europe 2013, WHO Regional Office for Europe & European Centre for Disease Prevention and Control (ECDC), November 2014 cited in EFPIA, the pharmaceutical industry in figures (2015).

⁴ EU Industrial R&D Investment Scoreboards 2005-2015, <http://iri.jrc.ec.europa.eu/scoreboard.html>.

About EFPIA

The European Federation of Pharmaceutical Industries and Associations (EFPIA) represents the pharmaceutical industry operating in Europe. Through its direct membership of 33 national associations and 42 leading pharmaceutical companies, EFPIA is the voice in Brussels of 1,900 companies committed to researching, developing and bringing to patients new medicines that will improve health and the quality of life around the world.



We want systems that provide patients with equal and early access to the best and safest medicines...that empowers citizens to make informed decisions about their health and ensure the highest security of the medicines supply chain.



Our Vision

We support a vision of outcomes-driven, sustainable healthcare systems in Europe. We want systems that provide patients with equal and early access to the best and safest medicines; that support innovation while balancing realistically benefit and risk; that empowers citizens to make informed decisions about their health and ensure the highest security of the medicines supply chain. Such a vision will also assist policymakers in sustaining Europe's economic growth and competitiveness, by balancing healthcare budgets and helping to provide for a healthy and productive workforce. It also offers the most effective approach to deliver the innovative medicines needed to tackle current and potential health threats.

Our Commitment

Improving patient outcomes, developing sustainable models of healthcare, reducing inequalities in health, accelerating patients' access to innovative medicines and improving patient safety – these are our primary commitments. By working in partnership with all healthcare stakeholders, we seek to develop practical solutions to make these goals a reality.

We believe that these commitments are in the best interest of Europe's citizens. We will engage with all partners in healthcare delivery to discuss, design and implement policies that help us achieve these goals, while improving public health and economic wealth, as well as enhancing Europe's industrial and science base.

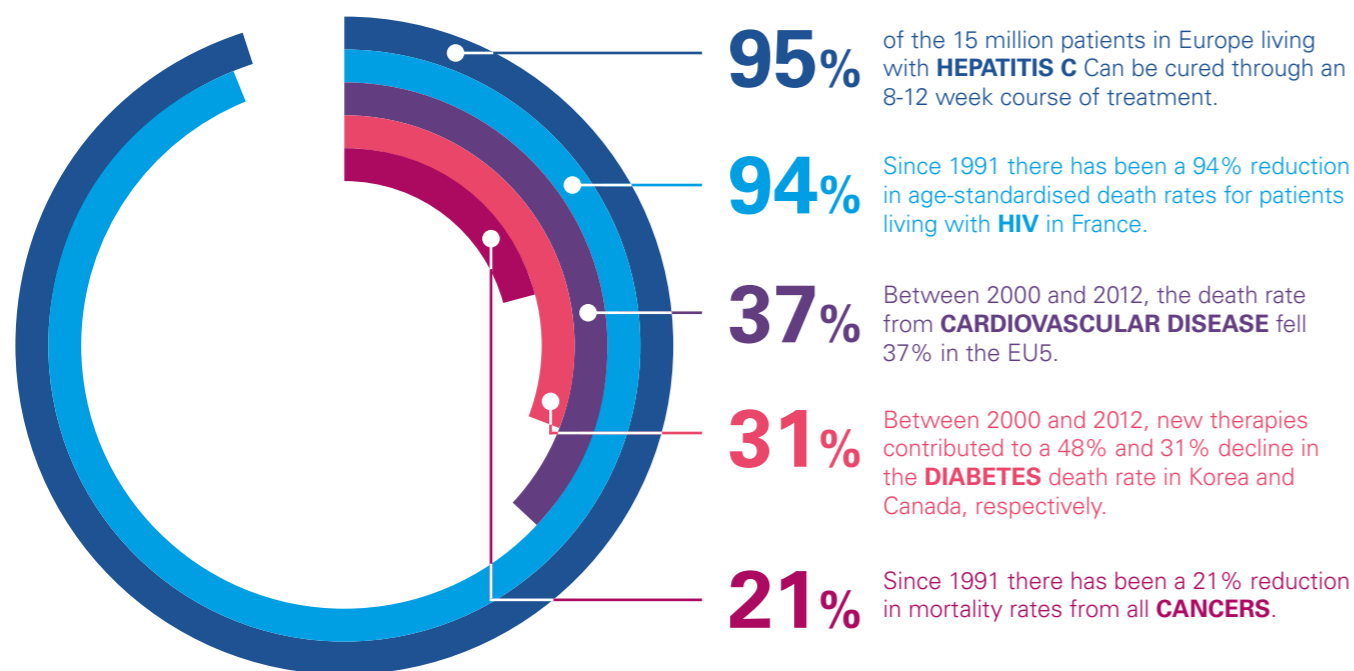




Impact of medicines in Europe

Medicines and vaccines are some of the most powerful tools that help people all over Europe to live longer, healthier and more productive lives. Over recent years, there has been dramatic, tangible progress in tackling major diseases. There are numerous examples.

MEDICINES ARE SOME OF THE MOST POWERFUL TOOLS IN TREATING AND CURING DISEASES



Source: Health Advances analysis

Cancer has touched the lives of many citizens in Europe. For many patients and their families, new medicines have significantly increased chances of survival. Since their peak in 1991, death rates have fallen by a 21% in the EU5. Today, 2 out of 3 people diagnosed with cancer survive at least 5 years, with life expectancy for cancer patients increasing by 1.74 years between 2000 and 2009, across 30 OECD countries.

Since the US Centre for Disease Control first used the term Acquired Immune Deficiency Syndrome (AIDS) in September 1982, the world witnessed the catastrophic unfolding of the AIDS epidemic. Out of the tragedy, though, a story of innovation, collaborative research and ultimately hope emerged. Beginning with the introduction of the first antiretroviral medicines in 1987, effective new treatments have transformed HIV/AIDS from a death sentence to a manageable disease.

Previously, the only hope for hepatitis C patients was liver transplantation. Those lucky to receive a donor organ had endure life-long use of immunosuppressants. New hope has been given to the 15 million people in Europe living with hepatitis C and, thanks to pharmaceutical innovation, 95% of patients can be cured through a 12-week course of medicine.

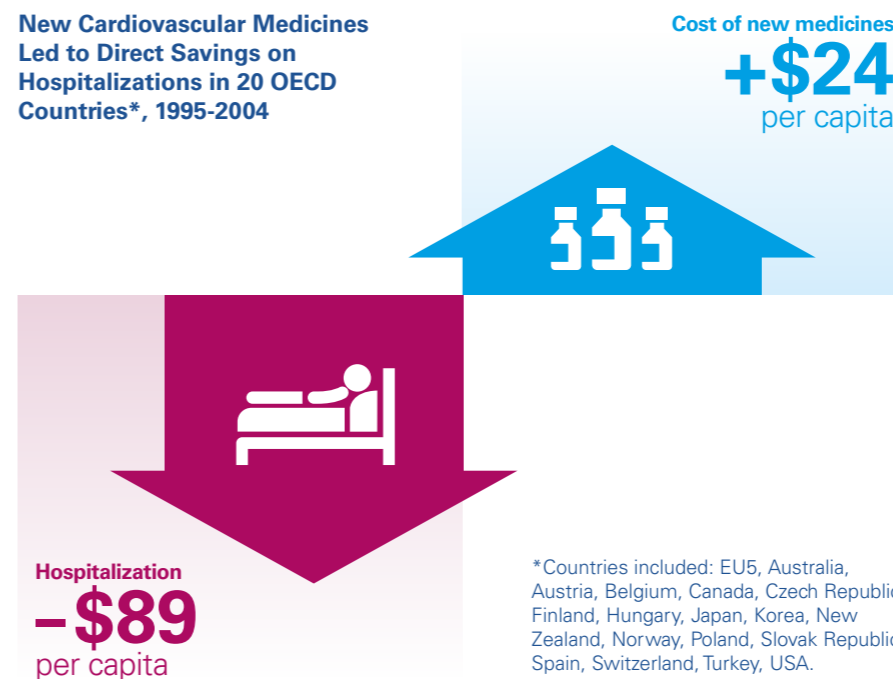
New medicines are also helping to put healthcare systems on a more sustainable path by reducing costs in other parts of the system, such as hospitalisations. New medicines have transformed the outlook for patients living with cardio-vascular disease; death rates have fallen by 37% in the EU5 between 2000 and 2012 thanks, in part, to new treatments that have revolutionised the way we manage high cholesterol. Per capita, expenditure on cardiovascular hospitalisations

would have been 70% higher in 2003, had new cardiovascular medicines not been introduced in the period 1995–2003. Ongoing innovation is expected to reduce the future need for hospitalisation further.

The value and impact of medicines goes beyond the individual and healthcare systems to benefit wider society in Europe. Critical to Europe's life science eco-system, the biopharmaceutical industry invests more of its revenue in generating new knowledge through research and development than any other sector and is the second largest funder of R&D in Europe. The pharmaceutical industry continues to generate essential economic value, with a European trade surplus of €75 billion in 2013, employing around 700,000 people, investing over €30 billion in European R&D and developing medicines that improve patient productivity.

MEDICINE USE YIELDS: SIGNIFICANT HEALTH GAINS AND SAVINGS IN OTHER PARTS OF HEALTHCARE SYSTEMS

New Cardiovascular Medicines Led to Direct Savings on Hospitalizations in 20 OECD Countries*, 1995-2004



*Countries included: EU5, Australia, Austria, Belgium, Canada, Czech Republic, Finland, Hungary, Japan, Korea, New Zealand, Norway, Poland, Slovak Republic, Spain, Switzerland, Turkey, USA.



Per capita expenditure on cardiovascular hospitalizations would have been \$89 (70%) higher in 2003 had new cardiovascular medicines not been introduced in the period 1995–2003.

Source: Health Advances analysis

Medicines costs in context

During 2015, there has been intense debate around the affordability challenges faced by healthcare systems, under pressure from rising healthcare demand. We recognise these concerns about the affordability of innovative medicines.

Spending on medicines should be considered in the context of the outcomes they deliver and wider healthcare spending. The reality is that since 2009, spending on medicines in OECD countries has actually fallen by an average of 1.8% per year. Of course, today's innovative medicines are tomorrow's generics and bio-similars, and that is why we have lower cost options for treating conditions such as heart disease and depression today. This will extend to conditions like cancer, rheumatoid arthritis, and other diseases in the future. This is the long-term, true value of innovation.

Although medicines account for less than one fifth of total healthcare spends in Europe, they are often the principle focus of cost containment policies, rather than governments undertaking an analysis of the entire healthcare spend, understanding total disease costs and identifying and reducing waste in the system.

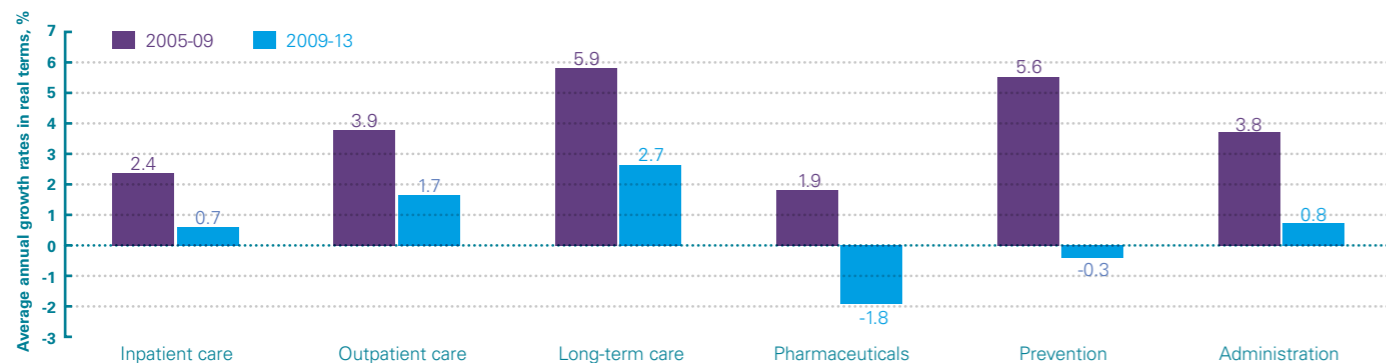
We share a common goal with all partners in healthcare: to ensure that patients across Europe get rapid access to the latest, effective and life-saving medicines. That is why we are working with governments and healthcare systems to find solutions to make medicines accessible and healthcare more sustainable.

There are many practical examples of this happening already through outcomes-based reimbursement contracts, managed entry agreements and other types of access and funding models for new medicines. In the future, we believe we can build sustainable healthcare systems by developing new pricing models, such as outcomes-based, or value-based contracts. This is in its infancy and will require partnering with patients, healthcare providers, payers and industry to create a real breakthrough and overcome some of the initial challenges, including gaps in legal systems, data, or capabilities.

At national level, the industry, and an increasing number of European governments have created national stability agreements to ensure affordable access to medicines. But much more effort is required to achieve system-wide solutions, especially in light of increasingly aged populations, which will put further strain on our healthcare systems. Our industry is committed to supporting the creation of outcomes-based healthcare systems that are fit for the challenges of the future.

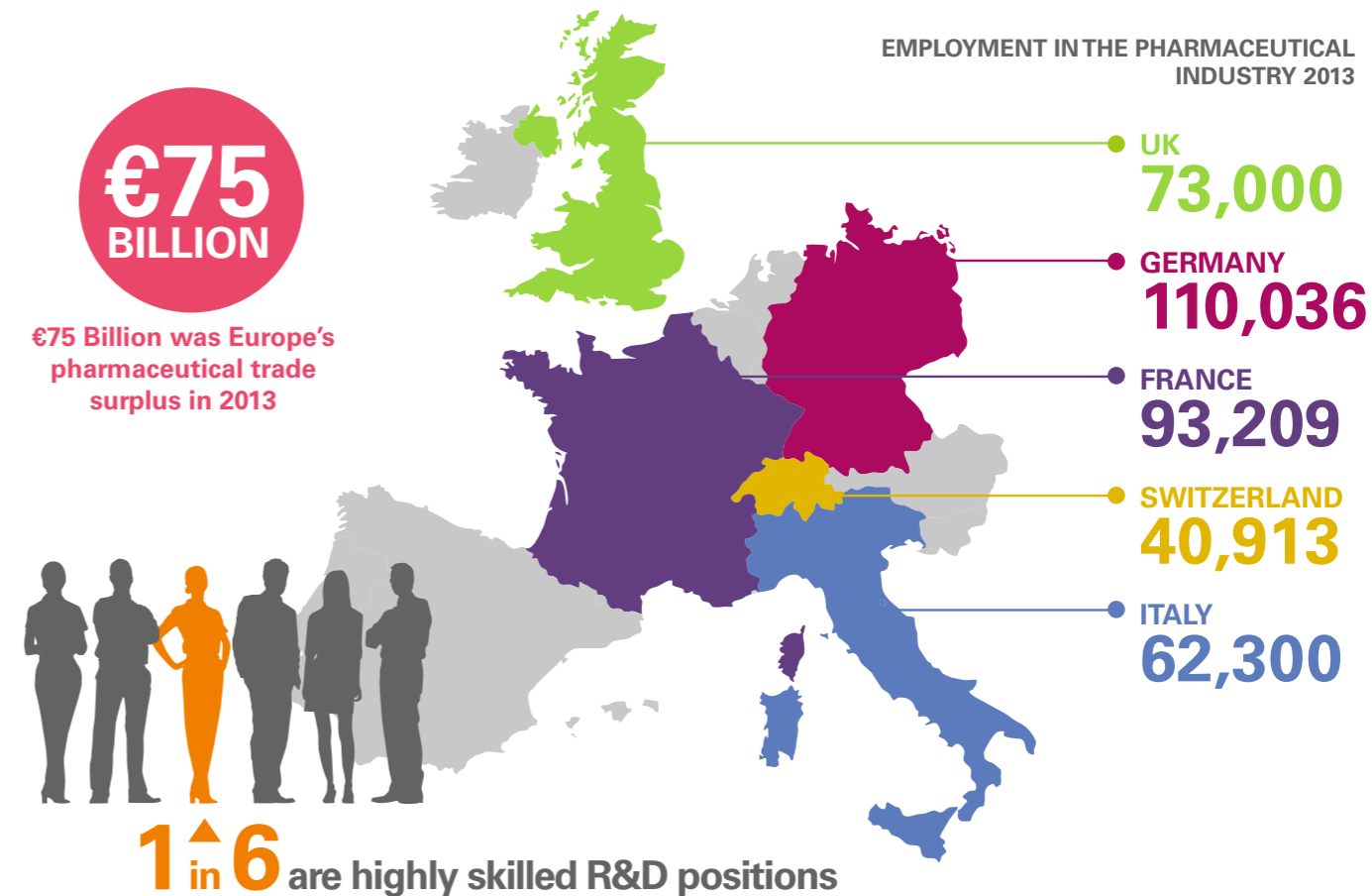
We would welcome taking an active role in this important transition, based on our commitment to the long-term sustainability of healthcare in Europe.

TOTAL HEALTHCARE EXPENDITURE HAS SEEN CONSTANT GROWTH WHILE SPENDING ON MEDICINES HAS DECREASED BY 1.8% PER ANNUM IN RECENT YEARS



Source: OECD Health at a Glance (2015)

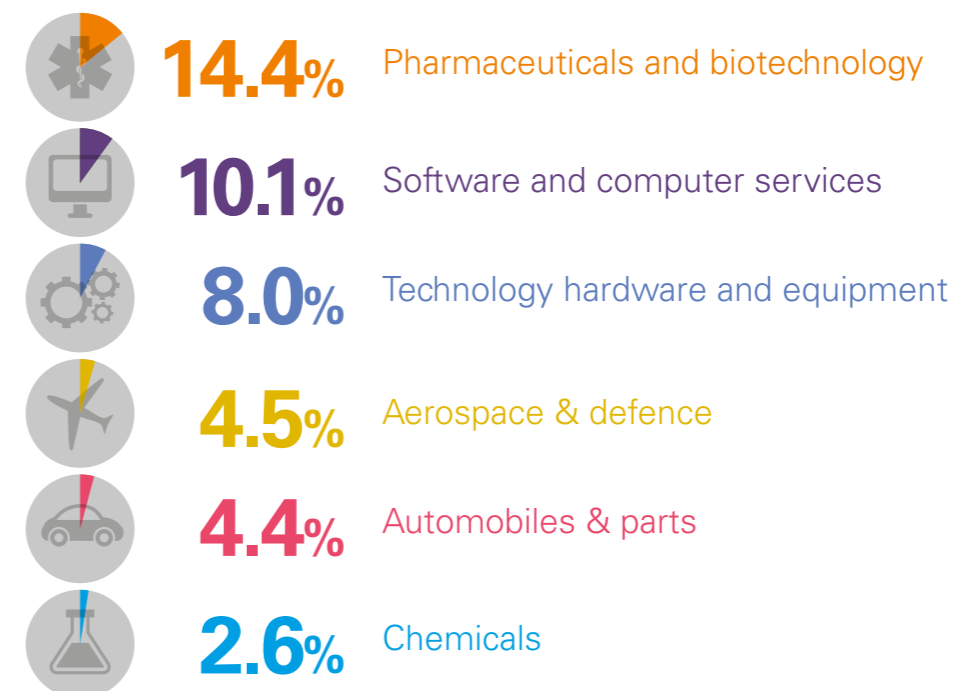
THE BIOPHARMACEUTICAL INDUSTRY MAKES A SIGNIFICANT CONTRIBUTION TO THE EUROPEAN ECONOMY



Source: Pharmaceutical Industry in Figures, EFPIA 2015

THE BIOPHARMACEUTICAL INDUSTRY INVESTS MORE OF ITS REVENUE IN GENERATING NEW KNOWLEDGE THROUGH RESEARCH AND DEVELOPMENT THAN OTHER SECTORS

R&D SPENDING AS A PERCENTAGE OF NET SALES, 2014



Source: The 2015 EU industrial R&D investment scoreboard, European Commission, JRC, DG RTD.

EFPIA Activity 2015

TRANSPARENCY

EFPIA member companies began collating data on transfers of value made to health professionals and healthcare organisations. Transfers of value made in 2015, will be publicly disclosed by the end of June 2016 - part of industry's wider commitment to transparency.

ETHICS

At the invitation of the Biomed Alliance, EFPIA provided industry comment as they developed their new code of ethics.

TRADE

EFPIA President Joseph Jimenez together with EFPIA Director General Richard Bergström, in partnership with PhRMA, conducted a series of high-level meetings with Chinese authorities and key stakeholders.

ACCESS TO MEDICINES

EFPIA and EuropaBio hosted an event on *15 years after the Orphan Medicines Regulation, are rare disease patients getting the innovation and treatments they need?* to review the opportunities made possible by the OMP regulation and underline challenges ahead.

GROWTH

EFPIA launched the *Access to finance and barriers to growth in the innovative biopharmaceutical sector* at the European Business Summit.

REGULATION

EFPIA is a key partner in the launch of the ADAPT SMART consortium, an enabling platform for the coordination of Medicines Adaptive Pathways to Patients (MAPPs) activities. MAPPs seeks to foster access to beneficial treatments for the right patient groups at the earliest appropriate time in the product life-span, in a sustainable fashion.

TRADE

EFPIA voiced the industry input in to TTIP through a meeting with the Chief negotiators during the 10th round of negotiations and publication of resources showcasing the benefits of TTIP and separating the fact from fiction.

INDUSTRY IMPACT

EFPIA published updated evidence on Health & Growth.

EFPIA launches study on the economic footprint of the pharmaceutical industry in Europe.

TRADE

EFPIA hosted a live Q&A on TTIP. The session reached over 350,000 Twitter accounts via 180 participants.

EFPIA and AIPM organised a high-level roundtable in Moscow, with the participation of regulators and Ministers from the five countries (Russia, Belarus, Kazakhstan, Kyrgyzstan and Armenia) of the Eurasian Economic Union (EAEU). EU Member State regulators and Director-General Richard Bergström shared the EU experience in creating a common regulatory system, and provided advice on the setup of a common medicines market, ahead of the planned entry into force of a regional EEU medicines market on 1 January 2016.

TRANSPARENCY

EFPIA attended ECCO congress in Vienna, presenting on HCP-HCO disclosure and hosting a stand to answer delegates' questions on transparency.

OUTCOMES BASED HEALTHCARE

EFPIA hosted the Health Collaboration Summit, bringing together over 200 senior figures from health policy and patient organisations to discuss outcomes-driven, sustainable healthcare.

In partnership with member associations EFPIA in France, Italy, Portugal, Spain, Switzerland, Latvia, Slovakia and the UK, EFPIA ran a series of workshops on outcomes-based healthcare.

HEALTH POLICY

EFPIA responded to the *Public Consultation on the preliminary opinion on access to health services in the European Union* by the Expert Panel on effective ways of investing in health, in November 2015.

RESEARCH & DEVELOPMENT

Quintiles and Somalogic joined EFPIA as Partners in Research.

TRANSPARENCY

EFPIA launched the #pharmadisclosure social media campaign to raise awareness of the importance of the relationship between industry, health professionals and healthcare organisations, as well as to provide information and resources on the public disclosure of transfers of value to health professionals.



JAN 15

FALSIFIED MEDICINES

In partnership with major stakeholders, EFPIA announces the creation of the European Medicines Verification Organisation, a landmark step in combating the emergence of falsified medicines in the EU legitimate supply chain.

RESEARCH & DEVELOPMENT

Illumina joins EFPIA as a member of the *Partners in Research* programme.

FEB 15

REGULATION

EFPIA hosted the *50 Year Anniversary of EU Pharmaceuticals Legislation: Are expectations for access to innovative medicines and sciences being met today?* examining the continuing role, challenges and opportunities for pharmaceutical legislation in Europe.

TRANSPARENCY

EFPIA published guidance, aimed at ensuring consistent and transparent reporting across the membership on the European Commission – European Parliament transparency register.

MAR 15

APR 15

NATIONAL STABILITY AGREEMENTS

In partnership with SIFFA, EFPIA signed an agreement with the government of Latvia that led to the creation of a working group on new access models for oncology products. Across Europe, governments and the biopharmaceutical industry are developing stability agreements that balance access to medicines with support for innovation. EFPIA is supporting these agreements in 5 countries of the Central and Eastern European Region.

HEALTH POLICY

The Universal Health Conference (Riga), organised by EFPIA, EGA, EPHA and EPF, gathered stakeholders from across the health community, culminating in an agreement on the Riga Roadmap – a concerted call for action, asking EU institutions and national governments to apply specified measures in order to maximize health and wellbeing and ensure the long-term sustainability of Europe's health systems.

RESEARCH & DEVELOPMENT

InterSystems joins EFPIA as a member of Partners in Research.

EFPIA published the *Putting animal welfare and the 3Rs into practice* report.

In partnership with key European stakeholders, EFPIA was a partner in the launch of the *Medsdisposal* campaign to support best practice in the safe disposal of medicines by consumers.

JUN 15

MAY 15

JUL 15

TRADE

Finalized in August and officially concluded in December 2015, EFPIA played an active role in the EU-Vietnam FTA negotiations, a benchmark for future FTA agreements in Asia.

AUG 15

SEP 15

HEALTH TECHNOLOGY ASSESSMENT

EFPIA and EUnetHTA co-hosted a meeting on Relative Efficacy Assessment, in particular the production of European joint assessments.

TRADE

An EFPIA Delegation, led by vice-President Stefan Oschmann and Director-General Richard Bergström, in partnership with EFPIA Japan, held a first exchange with Japanese officials and an event for Japanese media.

OCT 15

HEALTH POLICY

EFPIA supported and participated in a plenary session entitled *Health in all Policies* at the European Health Forum, Gastein.

NOV 15

NATIONAL STABILITY AGREEMENTS

To support stability, innovation and access to medicines, supported by EFPIA, the industry in Bulgaria signed a Memorandum of Understanding with the Minister of Health.

HEALTHCARE DATA

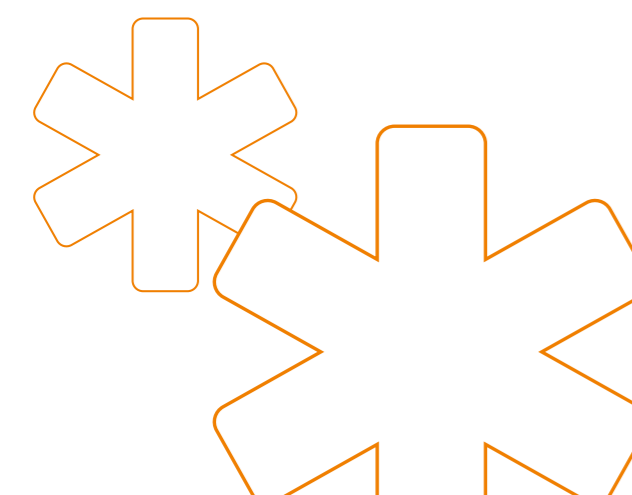
General Data Protection Regulation negotiations were concluded with the inclusion of specific provisions to safeguard health research.

ACCESS TO MEDICINES

EFPIA Director General, Richard Bergström hosted the EFPIA DG Lecture, entitled *Innovators or Speculators? Price, value and access to medicines in Europe*. In the format of a conversation between EFPIA DG Richard Bergström and Menno Aarnout, Executive Director of the International Association of Mutual Benefit Societies (AIM), the conversation focused on price, value and access to medicines in Europe.

INNOVATION

EFPIA launched the Pipeline project to highlight future pharmaceutical innovation and its impact on health outcomes, societies and healthcare systems.





Innovation

With over 7000 medicines in development, this exciting, new wave of medical innovation will play a key role in addressing the challenges faced by patients and healthcare systems.

Innovation is the lifeblood of our industry, bringing with it hope for patients in Europe and the wider world, while offering the prospect of long-term sustainability to health systems.

This means new treatments for diseases such as cancer, diabetes, hepatitis C, cardio-vascular disease and neurological conditions, but also encompasses a determined effort to address unmet medical need and rare diseases.

The potential of this exciting new wave of innovation is enormous. We already stand on the brink of a revolution in cancer treatment by exploiting the ability of the body's immune system to locate and eradicate cancer cells. Collaboration between sectors is driving innovative approaches to treating diabetes such as a contact lens that can measure glucose levels in tears, to deliver the optimum insulin dose.

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

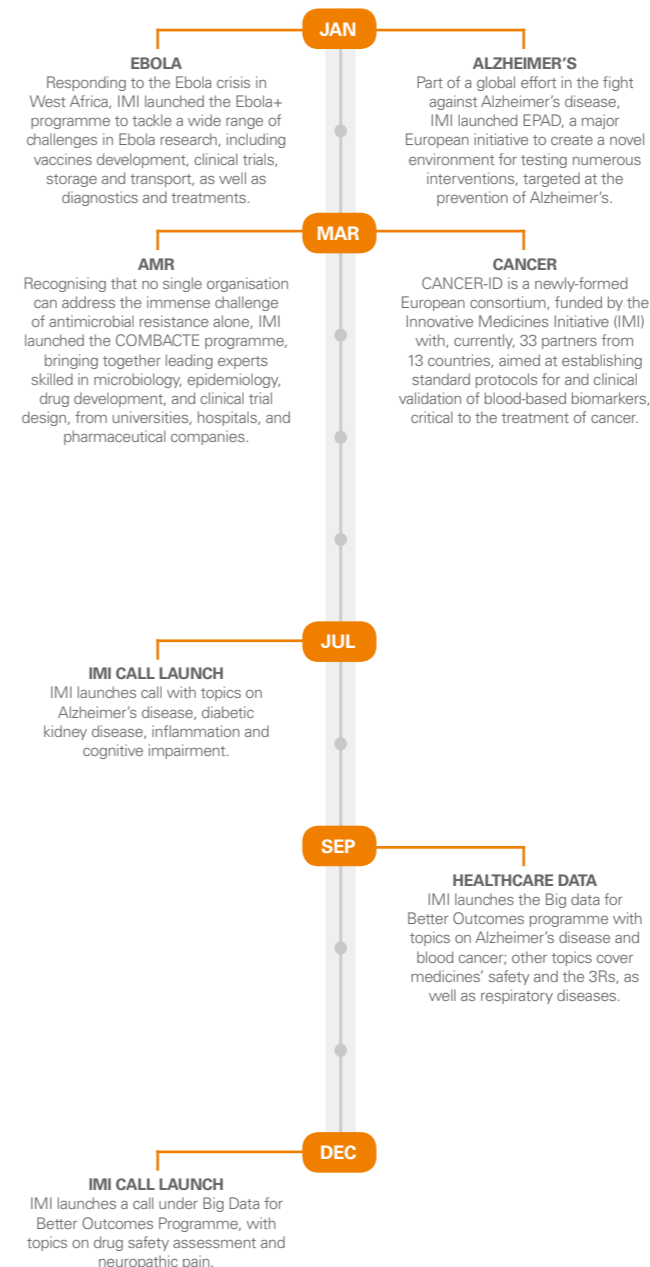
Significant advances in the vaccines sector are targeting global health issues such as malaria, Ebola, HIV, Cancer even Alzheimer's disease.

EFPIA is working with partners across the research community to help create an environment that sparks new ideas, drives innovation and delivers new treatments that patients, healthcare systems and society all need.

Faced with the exciting potential to advance treatment options for patients – but complex science – high attrition rates in developing new medicines and a long and costly process from discovery to delivery for patients, the European Commission and EFPIA took action to create the world's largest public-private partnership in the life sciences area: the Innovative Medicines Initiative (IMI). IMI's second phase, IMI 2, has a total budget of €3.3 billion, with €1.638 billion (half the budget) coming from the European Commission's 'Horizon 2020' Programme, €1.425 billion from EFPIA companies, and up to €213 million from other life science industries or organisations.

IMI has established a collaborative framework for research, backed by the European Commission and involving key players in healthcare research, including universities, the pharmaceutical and other industries, small and medium-sized enterprises (SMEs), patient organisations, and medicines regulators.

IMI TIMELINE 2015



IMI IN ACTION

Launched in September 2015, IMI's EPAD, a consortium dedicated to the European Prevention of Alzheimer's Dementia, employs an 'adaptive' trial design, capable potentially of delivering better results, faster and at lower cost. Harnessing the expertise of more than 36 organisations across Europe, including universities, regulatory agencies, pharmaceutical companies and patient organisations, EPAD is a prime example of what open research can achieve, aiming to tackle a disease for which there is still no cure and limited treatment possibilities exist.

Traditional clinical trials, which are costly and may last years, subject half of the participants to the drug under investigation, and half to placebo. In adaptive clinical trials, several candidate drugs are compared simultaneously to each other and to a placebo, meaning that a greater proportion of patients benefit from a potentially active treatment.

The EPAD project does not operate in isolation, linking with the IMI's EMIF-AD and AETIONOMY projects to form the IMI Alzheimer's disease platform. It is also working closely with other, similar initiatives worldwide, including the US-based Global Alzheimer's Platform.



...closer collaboration will result in reduced attrition, faster patient access to new treatments and improved outcomes for patients.

Getting innovative medicines to patients faster

Biomarker-driven research, genome sequencing and exceptional progress in many fields of basic science have opened the door to an increased understanding of many diseases. Leading to more accurate disease classification, this progress is transforming rapidly the way we generate data and conduct clinical trials. Advances in informatics are simultaneously revolutionising the way we collect data and produce evidence.

Medicine's Adaptive Pathways to Patients (MAPPs) builds on these advances in medical science, helping to design an approvals process that adapts quickly to an individual patient's response to therapies. In short, MAPPs aims to balance rapid access to novel therapies for patients who need them, with the need to monitor continuously benefits and risks throughout the medicine's use.

Within this context, the Innovative Medicines Initiative has given rise to ADAPT SMART to explore new concepts, align the various stakeholders and create a consensus across healthcare identifying the evidence required, how multiple sources of available data can be best used to facilitate adaptive pathways and which scientific challenges need to be addressed.

Bringing sectors together to advance research

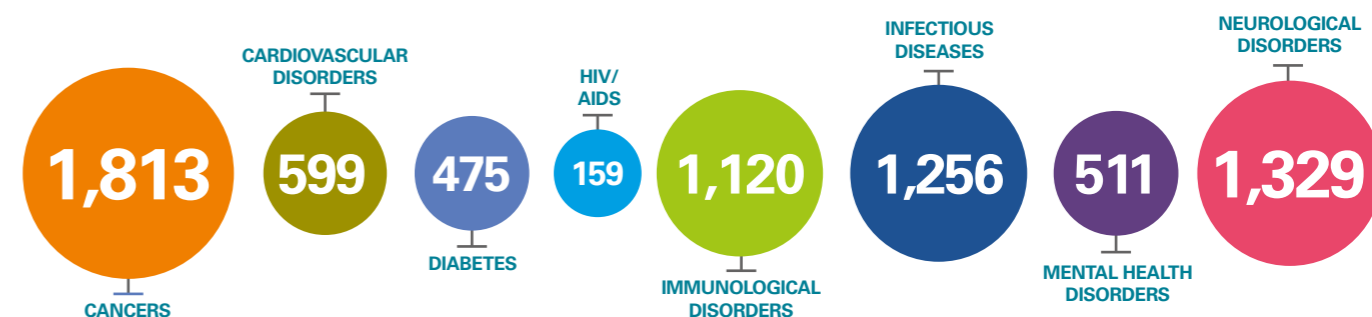
By breaking out of siloed approaches and working together, we can speed up the development of an impressive range of resources to deploy against the many diseases and medical conditions that we have not been able to address until now. In fact, closer collaboration will result in reduced attrition, faster patient access to new treatments and improved outcomes for patients. Integrated approaches such as these serve as important models of collaboration among sectors – including the imaging, medical devices, IT, diagnostic and animal health industries.

This is the message that EFPIA has conveyed with the establishment of "Partners in Research," a constituent entity open to companies operating in research-associated sectors. It seeks to support the evolution of the life science environment in Europe, helping to facilitate the integration of a range of technologies and sciences that are needed to address unmet medical needs and to exploit the vast health potential offered by science.

Looking to the future

Investing a greater percentage of its revenue in research and development than any other sector, EFPIA and its member companies are committed to driving and advancing this exciting new science. Advances in genomics, data analytics, immune-oncology and biologics are transforming the outlook for European patients living with a wide range of diseases.

WITH OVER 7000 MEDICINES IN DEVELOPMENT, THE EXCITING NEW WAVE OF MEDICAL INNOVATION WILL PLAY A KEY ROLE IN ADDRESSING THE CHALLENGES FACED BY PATIENTS AND HEALTHCARE SYSTEMS



THE EFPIA INNOVATION PIPELINE PROJECT

Pharmaceutical R&D productivity currently is booming, with thousands of compounds in development. Once on the market, these products could help Europe tackle unmet health needs in various disease areas, and bring great benefit to patients and societies. Despite new technologies, Europe still faces unmet needs in some disease areas. However, over the next 5-10 years, 6 major innovations address potentially some of those unmet needs and bring great benefits to both patients and society.

True innovation often has a disruptive effect. In order to make some of these innovations available, accessible and affordable, European health systems must be prepared by adapting in areas, such as financing models, delivery systems and value assessment. Undertaking reforms in collaboration with pharmaceutical industry to incorporate suitable changes in their policies.

Providing the appropriate tools and resources, the Innovation Pipeline Project aims to support an informed dialogue between EFPIA and key stakeholders across the healthcare systems about the value

of innovation and the sustainability of healthcare systems in terms of accessibility and affordability of these cutting-edge therapies. The result: a treatment paradigm shift; high health system impact; and substantial budget impact. This will help to identify game-changing therapies that have already given rise to an HPV vaccine for cervical cancer, anti-tumour necrosis factor [anti-TNF] therapies for rheumatoid arthritis [RA], an HCV polymerase inhibitor for hepatitis C, and a tyrosine kinase inhibitor used in cancer treatment. The results of the Innovation Pipeline Review Project will be launched at the EFPIA Annual Conference in June 2016.

Outcomes-based healthcare – beyond the hype curve

Rising demand for healthcare, driven by an ageing population and increased prevalence of chronic disease, is placing unprecedented pressure on our healthcare systems. By focusing on an outcomes-based approach, EFPIA believes we can put our healthcare systems on a more sustainable path.

Many have embraced the concept of value-based healthcare since Michael Porter and Elisabeth Teisberg published their book “Redefining Healthcare” in 2006. Their theories originally were developed to respond to problems in U.S. healthcare, but the central principles – that healthcare systems should be focused on delivering value – measured as health outcomes divided by costs – has taken hold across the globe, even in systems with traditionally much less market competition, such as the European-style, single-payer model. The starting points are different but the central problem remains the same. The actors in the system do not have the necessary incentives to deliver what actually matters: better health for patients.

This is not surprising, since the central tenets of the model instinctively are sound – and hard to argue against. Instead of paying for hospital beds, visits to the doctor, pills, screenings and surgical interventions, we should be paying for better health and longer lives. If we only could get perfect knowledge about exactly what type of intervention would bring the best health outcome for each patient, and direct our resources to those specific measures, then we would achieve simultaneously several goals that otherwise would seem difficult to reconcile: better health outcomes and quality of life for patients; more value for money; sustainable healthcare budgets, despite an ageing population; reduced waste and inefficiency in healthcare spending; and reward for true innovation.

So why isn't this being done everywhere?

When a number of national payers were interviewed for an EFPIA study on Managed Entry Agreements, one message was quite uniform: don't make it more complicated than absolutely necessary. What's more, though outcomes-based agreements are very attractive in principle – who doesn't want to pay only for what works?

However, they can also be complicated to implement. Indeed, several countries that have formed the avant-garde in concluding outcomes-

based risk-sharing agreements, such as Italy, the UK, the Netherlands and Sweden, have rediscovered recently the simple elegance of more straightforward, financial-type deals.

And stand-alone arrangements based on clinical performance might have unintended drawbacks if they do not form part of a larger “outcomes-friendly” environment. For example, a pharmaceutical company realised that one of their drugs that was covered by an outcomes-based agreement in a European country was sometimes avoided by doctors because of all the administration that comes with collecting real world data on outcomes. Instead, they prescribed alternative drugs that did not come with the added paperwork.

The picture is similar if you broaden the perspective from medicines to the entire healthcare systems. The latest decade has seen plenty of experimentation with alternative payment models in healthcare, both in the U.S. and in Europe. The common denominator has been to move away from transaction-based, fee-for-service type arrangements – in which a provider is paid for the number of visits or the number of screenings or tests – to payment models designed to reward quality of care and health outcomes, or contain costs, or both.

Since many of these schemes were introduced quite recently, and many have not been evaluated properly, there is still an ongoing debate about the actual, long-term effects of these payment models. The evidence is rather positive on some capitation based models, when the provider is paid a fixed amount per patient and therefore should be incentivised to invest in prevention, in order to avoid more costly treatment, or bundled payments that are fixed per episode of care, including post-treatment complications. These schemes, which certainly also have their draw-backs, all have in common that they don't peg payments to the outcomes per se, but that they nonetheless incentivise providers to keep their patients as healthy as possible (for the lowest possible cost). Looking at the experience of pure pay-for-performance models, though, where providers receive



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extra payment when certain quality indicators are met, the evidence so far is mixed at best.

Why is that? One obvious problem is that health systems often don't have the capacity to measure the actual health outcome, but instead must rely on surrogate metrics. So instead of rewarding the long-term improvement of a patient's health, they reward: adherence to clinical guidelines; or the number of times a doctor talks to his or her patients about prevention and healthy lifestyles; or the number of patients of a certain category that are referred to a specialist or prescribed a certain medication; or something else related to process, rather than actual outcome.

This can sometimes lead to perverse incentives, similar to fee-for-service systems, if providers only focus on producing whatever process that is rewarded, regardless of the effect on the patient's health. It is also inherently more complicated to tag payments to health outcomes than to procedures or interventions. For example, if the data is not risk-adjusted for different patient groups, providers that treat many high-risk patients might lose out, compared with providers that have mostly healthy patients on their lists.

So where to go from here? Before throwing out the baby with the bathwater, it is important to realise that true, value-based healthcare is still in its infancy, and that healthcare systems all over the world are still grappling with how it works and what kind of tools are necessary to make it work. Like other fashionable concepts such as big data or mobile health, the initial hype is almost always followed by a temporary slump, when reality sets in with all its boring details. But after this, perhaps forceful, awakening, the real, step-by-step progress can start.

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

registries and Electronic Health Records as key components – and standardised outcomes metrics that will make it possible to compare health outcomes across providers and even countries. Patient involvement is key when agreeing on these outcomes metrics, but equally so is the involvement of healthcare professionals, since their backing is absolutely critical to making the systems work.

EFPIA is ready to engage with all stakeholders to make this important transformation become a reality all over Europe. Through the Innovative Medicines Initiative, the world's largest public-private partnership, EFPIA companies are collaborating with public partners in the programme “Big Data for Better Outcomes”, with the objective of defining health outcomes in different disease areas, identifying the data sources that are needed to track these outcomes, and using the data to improve healthcare delivery. EFPIA is also engaging with policymakers and stakeholders, both at European and national level, in discussions on how best to facilitate the change to a more outcomes-focused healthcare model. The new EFPIA communications toolkit “Healthier Future” will enable EFPIA members and partners to have that important conversation.

The good news is that we don't have to wait for the perfect system to be in place, but this change can be implemented step-by-step. The key is to learn from each other and spread the use of models that have been proven to work - and there are a lot of truly inspiring examples out there already! What's more, even though reduced waste and better value for money will be one of the rewards, the main driver for change must be better health outcomes and putting the patient at the centre of healthcare management. That is an objective against which it is hard to argue. Just as no one would pay simply for the TV repair guy showing up, healthcare budgets shouldn't be spent just on patients getting a pill or seeing a doctor. They should be spent on better health. That is common sense, and common sense sooner or later wins out.

Data: driving discovery, delivering better patient care

As healthcare begins to embrace the digital revolution, the potential of data to change the way we deliver healthcare, improve patient outcomes and shape future research is the new frontier. It's a future towards which EFPIA and its members are committed to working, with all partners in healthcare.

The rise in chronic disease, driven by Europe's ageing population, poses a major threat to our health systems. While chronic disease devastates individual patient lives, it simultaneously places an unrelenting economic burden on national health services.

The capture and analysis of healthcare data is becoming an increasingly vital tool in addressing these challenges. Data can be obtained from a variety of important sources.

For several decades, the clinical trial has been the gold standard of measuring the safety and efficacy of medicines. Through the 20th century the rulebook for conducting trials grew thicker and more complex. Regulatory agencies on both sides of the Atlantic have demanded increasing amounts of documentation demonstrating the performance of new medicines, from the relatively small scale phase 1 trials testing the safety profile of new molecules, to the large phase 3, randomised controlled trials (RCT), often encompassing thousands of patients. Clinical trials continue to play an important role in generating data and evidence to shape healthcare. With the advent of the digital revolution, though, encompassing electronic health records, disease registries, patient reported outcome measures, mobile health apps and advances in data analytics, our healthcare eco-systems are generating unprecedented amounts of "real world data".

Real World Data is all health data that is generated and collected in real clinical practice right across our healthcare systems. Combining pre-clinical and clinical data generated by industry with real world data collected in clinical settings and beyond, we can drive medical innovation and improve patient care. It allows clinicians to better target interventions to the patients where they will have the most impact, thereby improving patient outcomes but also increasing efficiency. Data collection and analysis can help us better understand the side effects of medicines and improve patient safety. Genomic science is facilitating a revolution in personalised medicines and, crucially, all of this data can inform and shape tomorrow's breakthrough innovations.

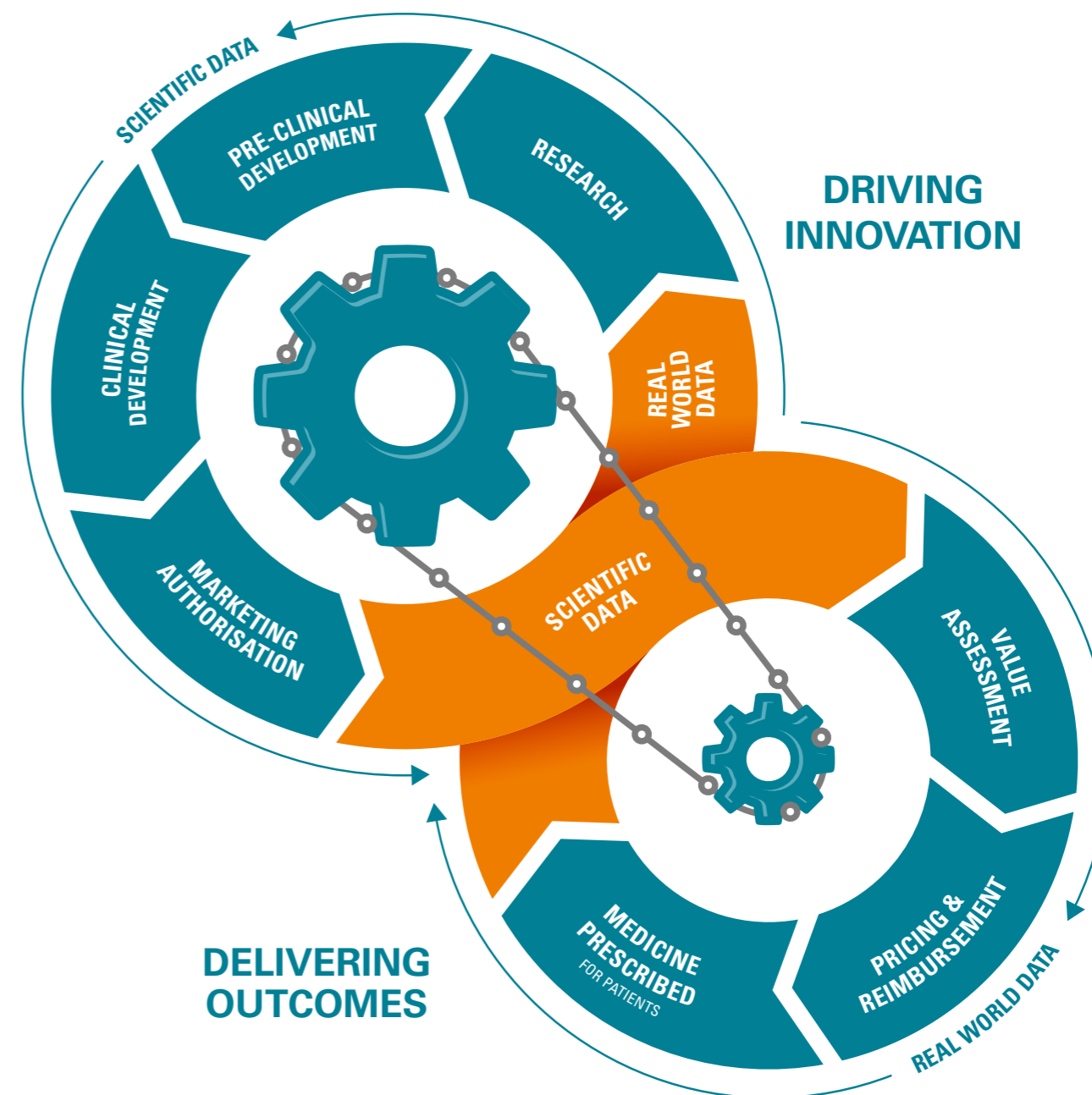
But maximising the potential of all this data for patients requires a new level of collaboration to address a number of challenges.

In reality, much of our RWD is scattered across the healthcare system without any possibility of bringing this data together. They are noted down in individual patient's records, recorded in registries kept by small groups of specialists for tracking a specific disease in a selected number of patients, or collected for reimbursement purposes and then discarded. Data should be generated according to the same standards in order to be comparable. E-health systems should be made compatible between hospitals or countries, with a single, electronic health record for individual patients, with all health data collected in one place. There should be a means of linking all data collected for a variety of purposes.

The situation, though, is changing rapidly. Considerable efforts are now being made both by public and private stakeholders to capture, consolidate and most importantly utilise this data to benefit patients, science and our healthcare systems. Regulation needs to evolve in order to allow data to be transferred between systems and used for research or quality improvement.

Trust is critical to ensuring a new health data ecosystem can thrive. It is vital to be cognizant of the sensitive nature of healthcare data. Patients need clarity on how and why their data will be used. Through its experience in conducting clinical research, the pharmaceutical industry has developed very high standards of data protection, recognising that as we move into a world of many different data sources and opportunities, these standards will need to evolve. Without patients, there can be no research and we are ready to adapt our standards as needed.

THE MEDICINES LIFE-CYCLE: FROM INNOVATION TO OUTCOMES



An expansion in data resources should be paralleled by a further evolution in patient focus. Patients are not outside the ecosystem – they are inside and driving it. This necessitates finding new ways to work with patients and keeping them informed about the results of research, while encouraging participation.

The future is exciting and it is within reach. At EFPIA, we are playing an active part in creating the future. In partnership with the European Commission through the Innovative Medicines Initiative, we are addressing four major areas:

- The creation of a technical and governance infrastructure for the research ecosystem (example – EHR4CR)
- Using new and existing data sources to improve the efficiency of the R&D process and medicines safety (WEB-RADAR)

- Supporting the evolution towards health system decision-making, based on patient outcomes (BD4BO)
- Building patient competences to engage in the R&D process (EUPATI)

With more information about how different interventions actually compare in terms of health outcomes for patients, healthcare managers and policymakers will be able to take better informed decisions on implementing clinical practice and resource allocation, creating not only better health outcomes for patients but also getting more value from every euro spent on healthcare. Over the next few years, we will be working with our partners in using data to deliver more innovation and better care for patients in Europe.

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Joining EFPIA as a Partner in Research was an easy decision for us. SomaLogic is a proteomics platform technology company dedicated to uncovering new biology and useful biomarkers for drug development and patient management. We are able to make common cause through EFPIA with the world's leading drug developers, and the academic consortia with whom they collaborate, to shape the future of medicine.

BYRON HEWETT, CEO



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EFPIA represents the pharmaceutical industry in Europe. Through its direct membership of 33 national associations and 42 leading pharmaceutical companies, EFPIA provides the voice of 1,900 companies committed to researching, developing and bringing new medicines to improve health and quality of life around the world.



EFPIA Brussels Office
 Leopold Plaza Building, Rue du Trône 108,
 B-1050 Brussels, Belgium
 Tel: + 32 (0)2 626 25 66
www.efpia.eu * info@efpia.eu

* At time of publication