

Improving access to medicines in the European Union

Value, measured outcomes and innovation crucial to fostering sustainable healthcare systems

Ensuring the sustainability of healthcare systems requires a strategic and comprehensive policy response. Current short-term cost containment measures that exclusively target the price of medicines risk undermining, rather than improving access. The American Chamber of Commerce to the European Union (AmCham EU) believes that focusing on meaningful patient outcomes and the value of innovative medicines is part of the solution. Europe also needs to further encourage research and development and innovation, of which the patent system remains a key driver. This will deliver new life-saving treatments to patients and spur economic growth and job creation in the EU.

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AmCham EU speaks for American companies committed to Europe on trade, investment and competitiveness issues. It aims to ensure a growth-orientated business and investment climate in Europe. AmCham EU facilitates the resolution of transatlantic issues that impact business and plays a role in creating better understanding of EU and US positions on business matters. Aggregate US investment in Europe totalled more than €2 trillion in 2015, directly supports more than 4.3 million jobs in Europe, and generates billions of euros annually in income, trade and research and development.

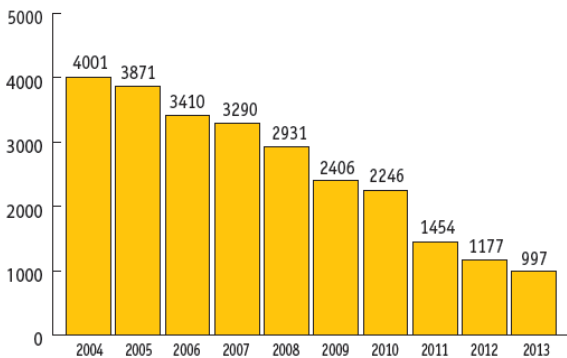
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Introduction

In the last decades, the life expectancy and quality of life of European citizens have improved dramatically. Many diseases ranging from HIV/AIDS to cancer, once seriously debilitating and often a death sentence, are now manageable.¹ Key to this success are investments made by the pharmaceutical industry, which allow for new innovative treatments to become available to patients.

TOTAL NUMBER OF DEATHS AMONG AIDS CASES IN EUROPE (TOTAL EU/EEA)



Source: HIV/AIDS surveillance in Europe 2013, WHO Regional Office for Europe & European Centre for Disease Prevention and Control (ECDC), November 2014

Despite scientific and technological progress, healthcare systems are under increasing pressure, mainly due to Europe's ageing population, skyrocketing rates of chronic disease, slow economic growth and shrinking public budgets. Policy makers seem to focus more on short-term cost containment than pursuing the goals all stakeholders share, which include **ensuring access to treatment, fostering sustainable healthcare and encouraging continued innovation.**

The American Chamber of Commerce to the European Union (AmCham EU) would like to contribute to the ongoing discussions at the EU

level regarding access to medicines. **Investing in healthcare** is needed now more than ever if society is serious about improving its population's well-being and ensuring not only the sustainability of healthcare systems, but also the prosperity and competitiveness of the European economy. Health and wealth are strongly interdependent and cannot exist without one another.² However, health can only contribute to wealth when the right policies are in place. European institutions play a key role in promoting **longer-term policies** focused on the value of medical innovation, sustainability of healthcare systems and the re-launch of economic growth.

What matters to patients

AmCham EU recognises the concerns around the affordability of innovative therapies. The industry wants to be part of the solution to the sustainability of healthcare systems and is increasingly engaging in innovative pricing and funding models. Nevertheless, the narrow focus on price as the main barrier to access overlooks the fact that medicines represent one-fifth of total healthcare costs in Organisation for Economic Co-operation and Development (OECD) countries.³ Data also show that medicines' prices have grown on average more slowly than overall health spending.^{4,5} The debate

¹ Chart: EFPIA, *The Pharmaceutical Industry in Figures*, Key data; 2015

² AmCham EU, *Explaining AmCham EU's Position on Investment in Healthcare*, December 2011.

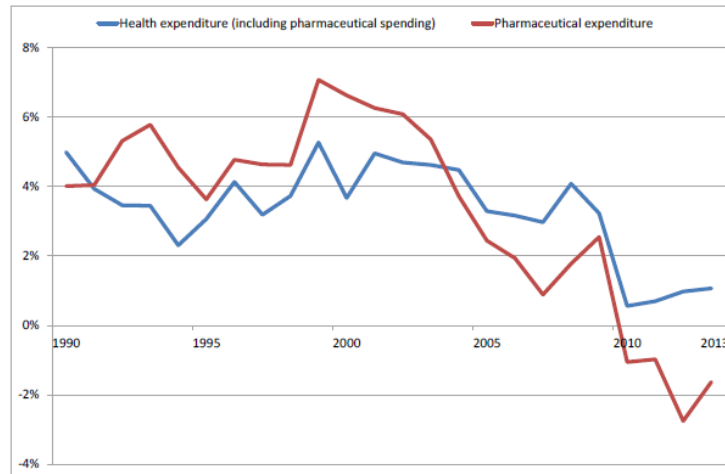
³ OECD, *Health at a Glance 2015*, p.30; OECD, 'Pharmaceutical Expenditure And Policies: Past Trends And Future Challenges', *OECD Health Working Papers*, No. 87

⁴ OECD, *Pharmaceutical expenditure and policies: Past trends and future challenges*; DELSA/HEA 6; 2015, p. 14.

⁵ Chart: Belloni, A, Morgan D, Paris V, 'Pharmaceutical Expenditure And Policies: Past Trends And Future Challenges', *OECD Health Working Papers*, No. 87, 2016;

around access to medicines needs to be seen within the broader context of improving health outcomes while ensuring the sustainability of healthcare systems.⁶

Figure 4. Average annual growth in health and retail pharmaceutical expenditure, OECD average, 1990-2013



Note: Excludes Greece, Israel, Mexico, New Zealand, Turkey and United Kingdom.
Source: OECD Health Statistics 2015

Investing in new innovative treatments is cost-effective, and brings real value to the economy and society at large. Innovative medicines help generate savings in other areas of health spending, such as hospitalisations. They increase patients' quality of life and life expectancy, allowing them to remain integrated, valued and productive members of society. Across 30 OECD countries, life expectancy at birth increased by 1.74 years between 2000 and 2009, and innovative medicines accounted for 73% of this improvement.⁷ Faced with an ageing population, Europe would strongly benefit from a healthier, more productive workforce. Innovative medicines are also crucial to preventing future health costs. This is important to consider when developing health-related policies, as the future risks proving costly. The number of Europeans over the age of 65 will increase by 75% over the next 50 years, and incidence of dementia will more than double.⁸

In this context, **society as a whole needs to invest more in prevention and early detection.** Vaccines play a key role in preventing the outbreak of diseases, reducing the need for reactive care and treatment. Appropriate treatment at the right time can also help sustain work productivity and reduce potential sick leaves, a burden for public budgets. In fact, every 1€ invested in vaccines translates into a long-term return for governments of 4€ thanks to reduced public expenditure (e.g., social insurance transfers) and increased tax revenue from healthy individuals in gainful employment.⁹

Early investment in healthcare may result in increased future health benefits and decreased costs downstream. Cost-containment measures should focus on patient needs first and the long-term sustainability of healthcare systems.

⁶ See also AmCham EU, *AmCham EU position on the Communication on effective, accessible and resilient health systems*, 18 September 2014.

⁷ F. Lichtenberg, 'Pharmaceutical innovation and longevity growth in 30 developing OECD and high-income countries, 2000 – 2009', *Health Policy and Technology* 07/2012; 3(1).

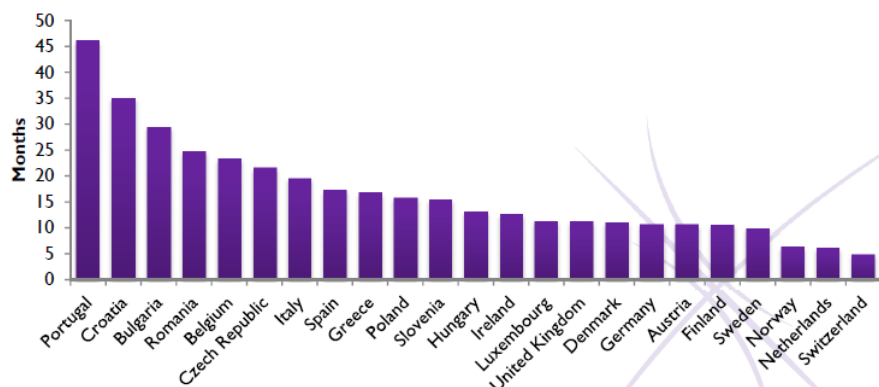
⁸ EFPIA, *Health and Growth Evidence Compendium*, 2013.

⁹ SAATI Partnership, *Adult Vaccination: A Key Component of Healthy Ageing – The benefits of life-course immunisation in Europe*, UK, International Longevity Centre (ILC), November 2013.

Pricing and transparency: how to ensure affordable access

National pricing and reimbursement (P&R) decisions focused on short-term cost containment can negatively impact access to medicines in the EU and increase health inequalities. One example is ‘external reference pricing’ (ERP), through which EU Member States determine prices of medicines based on a comparison of prices in other Member States.¹⁰ This practice does not take into account the value of the product to patients’ health and society. As noted by the OECD, ERP may well undermine the availability and affordability of medicines in some countries.¹¹ Moreover, as stated in a study commissioned by the European Commission, ‘in the current EU framework differential pricing is not possible given the wide-spread use of external price referencing in Europe and the existence of parallel trade (...)’.¹² In such a context, confidential agreements between suppliers and payers allow for the prevention or at least limitation of the scope for price comparisons and facilitating affordable supply to those in greatest need.¹³ AmCham EU believes that the ability to charge different prices in different EU countries (differential pricing), paired with targeted programs for low/middle-income countries and patient assistance programs, is essential to increase access and affordability.

Figure 3.5 Average launch delays for in-patent oncology drugs: 2001-2013



Note: Delays based on packs common to at least 15 countries in the sample.
Source: IMS Health

Tendering is another tool increasingly used by governments, local authorities, healthcare providers, and other organisations to purchase medicines – often with a sole focus on price and allowing for only one single winner.¹⁴ Lowest price-only tenders, however, exclude other key measures of value, such as comparing the total economic impact of different products. Such tenders may be appropriate for commodity procurement of non-differential products, but are inappropriate for the purchase of medicines because they do not adequately take into account all the sources of value that a particular choice of medicine or manufacturer may offer to patients, the health system, and society. **A medicine’s net unit cost is an important element of the procurement decision, but it should not**

¹⁰ Chart: Europe Economics (2013), *External Reference Pricing*; http://www.europe-economics.com/publications/external_reference_pricing_-_final_report.pdf

¹¹ OECD, *Pharmaceutical Pricing Policies in a Global Market*, 2008, p. 205

¹² Gesundheit Österreich, SOGETI, *Study of the policy mix for the reimbursement of medicinal products: Proposal for a best practice-based approach based on stakeholder assessment*; 2015, p. 31

¹³ Dermot Glynn, *The case for transparency in pricing*; Comp Law 2015; http://www.jordanpublishing.co.uk/practice-areas/competition/news_and_comment/the-case-for-transparency-in-pricing#.VwENq_197eO (accessed on 04/04/2016)

¹⁴ AmCham EU, position paper, *Implementation of Directive 2014/24/EC on public procurement in the EU healthcare sector*, November 2015

http://www.amchameu.eu/system/files/position_papers/amcham_eu_calls_for_a_more_strategic_use_of_procurement_to_stimulate_innovation_uptake_-_20.11.2015.pdf

be the predominant criterion for medicines. Lowest-price only tenders may increase the likelihood of supply disruption. Systems that lead to prices of medicines going down to or near (sometimes even below) production cost, result in only those manufacturers with the lowest cost base being able to supply. This may increase the likelihood of product recalls or shortages.¹⁵ Over time, they may also reduce the number of suppliers in the market, eliminating backup options in the event the remaining manufacturers have supply disruptions.¹⁶ Disruptions in medicines' availability have a negative impact on patients and the healthcare system, including increased costs in sourcing alternative supplies and increased vulnerability of the supply chain to suppliers who may provide substandard, contaminated, adulterated, and even counterfeit materials.¹⁷ When tenders are used, we encourage the use of Most Economically Advantageous Tender (MEAT) award criteria with best price-quality ratio assessment. Tender criteria should encompass the full value that the medicine and the company that has commercialised it bring to patients, the health system, and society as a whole.

Identifying inefficiencies in the healthcare system generates savings and shifts resources towards treatments and practices offering the most value. This 'out with the old, in with the new' approach, building on an appropriate measure of 'value', must be adopted across the entire spectrum of care, as advocated for example by the European School of Oncology (ESO)'s Innovation Taskforce for the cancer care pathway.¹⁸

Technological developments such as **e-health, m-health and innovative Information and Communications Technologies (ICTs) represent an immense opportunity to improve care delivery** and should be promoted. This should also be supported by increased health literacy, enabling individuals to take control over their health in everyday life, as healthy individuals, as patients but also as consumers, caregivers or citizens.¹⁹

AmCham EU believes that Health Technology Assessment (HTA) has the potential to reduce patient waiting time and strengthen the equity of care throughout Europe. Yet, inconsistencies in the approach and different data requirements among national authorities are a serious burden for the industry in clinical trial design and data collection, and contribute to delays in access to medicines for patients. AmCham EU **supports further collaboration on HTA and harmonisation of the European assessment of relative clinical effectiveness (REA) at time of launch**, as a first data input into national HTA. Harmonisation should focus on clinical performance, while economic criteria (affordability, price) should remain a country-specific competency to account for the differences among healthcare systems.

In addition, health technologies should be assessed in a total cost of care context, taking into account their impact on indirect costs as well. Costs of these technologies should be weighed against the increased productivity they permit, as well as other costs they enable healthcare systems to avert.

An illustrative example is Hepatitis C, for which an assessment of the disease burden should take into account its potential consequences: end-stage liver disease, liver transplants, and other interventions.²⁰

¹⁵ HIS Global Insights, *German Pharmacists criticise contracts amid mounting drug shortages concerns*, 17 October 2013

¹⁶ European Commission (2012), *Cost-containment policies in public pharmaceutical spending in the EU*, Economic Papers 461; p. 34.

¹⁷ <http://rx-360.org/AlertsNews/Alerts/DrugShortagesMay2012Update/tabid/246/Default.aspx>.

¹⁸ A. Wagstaff, 'Five Steps to Putting Innovation at the Heart of Cancer Care', *Cancer World*, January-February 2014

¹⁹ Kickbusch I and Maag D Health Literacy, Kris Heggenhougen and Stella Quah, editors *International Encyclopedia of Public Health*, Vol 3. San Diego: Academic Press; 2008. pp. 204-211

²⁰ European Association for the Study of the Liver, 'EASL Recommendations on Treatment of Hepatitis C', *J Hepatology*, vol. 63, 2015

Value assessment of new curative treatments for disease should recognise benefits brought over the long term that could potentially lead to its eradication in EU member states.

Lastly, it is widely recognised that **patients should be placed at the centre of HTAs**, by giving sufficient voice to their perspective and placing the emphasis on long-term quality of life.

Intellectual property and off-patent market competition: creating the right framework for research and development

The **research and development (R&D) conducted today will result in the new life-saving medicines of tomorrow**. Biopharmaceutical R&D has led to the development of nearly every important medicine of the last century and a half, including antibiotics, vaccines, human immunodeficiency virus (HIV) and Hepatitis C treatments, and cancer and cardiovascular medicines. Industry has developed over 550 medicines in the last 15 years for the world's emerging health needs.^{21,22} Over 7.000 medicines are currently in development around the world, focusing on areas of high, unmet medical need.²³ In Europe alone, the research-based pharmaceutical industry invests around €30 billion in R&D each year.²⁴

However, R&D is a long-term, risky endeavour. Bringing a product from discovery to patients takes more than a decade, with a lot of uncertainty (success rates are sometimes less than 10%).²⁵ In order to ensure innovative medicines continue to be developed and reach patients in need, it is **essential that the EU provides the right framework for innovation** and notably a predictable, strong intellectual property (IP) protection regime. IP protection is a proven and critical facilitator of access and brings significant long-term benefits. The goal of patent law, not only in healthcare but in all sectors, is to transfer innovation to the public by making the details of patented inventions broadly available. This promotes research by others into areas outside of the claims of the original patents, which avoids overlap of R&D, optimises inventions and accelerates further innovation.

AmCham EU contests the argument that patents are the main obstacle to access to medicines. In fact, 95% of the pharmaceutical products in the World Health Organisation (WHO)'s Essential Medicines List (EML) are 'off-patent', and for the patented ones, including patient access programs and voluntary licensing schemes, are largely in place.²⁶ Yet even these 'off-patent' drugs are often unavailable or unaffordable.²⁷ IP contributes to facilitating access to today's medicines in the short term, by creating a framework for rapid dissemination of ideas and efficient technology transfer.²⁸ This leads to a faster launch and faster access to new medicines in developing countries²⁹ and the

²¹ U.S. Food and Drug Administration, 'Summary of NDA Approvals & Receipts, 1938 to the Present' *About FDA*, January 18, 2013.

²² Pharmaceutical Research and Manufacturers of America (PhRMA), 'Medicines in Development,' *PhRMA*, 2015.

²³ Health Advances analysis; Adis R&D Insight Database

²⁴ EFPIA, *Health and Growth: 10 Facts for European Health Policy Makers*, 2014; Tufts Center for the Study of Drug Development; see Tufts, http://csdd.tufts.edu/files/uploads/Tufts_CSDD_briefing_on_RD_cost_study_-_Nov_18,_2014.pdf

²⁵ C. Adams, V. V. Brantner, 'Spending on Drug Development', *Health Economics*, 19:2, 2010, pp. 130-141.

²⁶ A. Attaran, R. Beall, *Patent-based Analysis of the World Health Organization's 2013 Model List of Essential Medicines*, WIPO Report, April 2016.

²⁷ United Nations World Intellectual Property Organisation (WIPO), *Striking a balance: The patent system and access to drugs and healthcare*, 2009

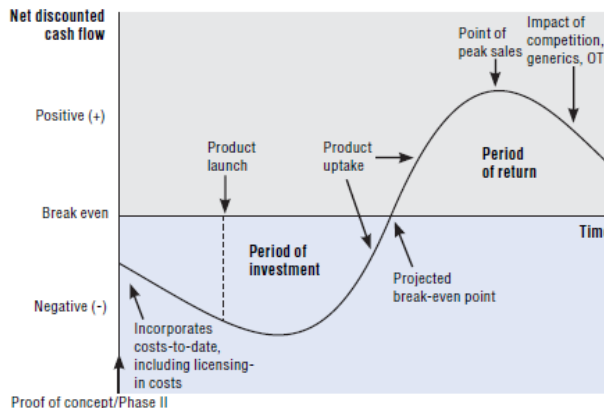
²⁸ Rozelle Laha, 'Pfizer, IIT-Delhi Join Hands for Healthcare Innovations,' *Hindustan Times*, November 30, 2015.

²⁹ Ernst R. Berndt and Iain M. Cockburn, 'The Hidden Cost of Low Prices: Limited Access to New Drugs in India,' *Health Affairs* 33, no. 9 (2014): 1567-75; Iain M. Cockburn, Jean O. Lanjouw, and Mark Schankerman, 'Patents and the Global Diffusion of New Drugs', National Bureau of Economic Research, 2014; Margaret Kyle and Yi Qian, 'Intellectual Property Rights and Access to Innovation: Evidence from TRIPS' (National Bureau of Economic Research, 2014).

introduction of many medicines that would not otherwise be available in those countries (in either brand or generic form).³⁰

Innovative medicines will be tomorrow's generics and biosimilars. The period of patent protection is already limited and after its expiration, products lose exclusivity and prices drop due to increased competition.^{31,32} AmCham EU believes that rights granted by IP protection, including the Supplementary Protection Certificate (SPC) and the data exclusivity regime, should not be eroded as they are essential incentives to bringing new medicines onto the market. Pro-innovation policies are entirely consistent with support for access to quality and affordable generics in the future, and the use of generic and biosimilar drugs may create headroom for innovative treatments. However, generic utilisations should not be made at the expense of any existing IP. The use of a generic or biosimilar drug should be supported as long as the prescribing physician and patient retain the discretion to oppose substitution if considered inappropriate for the patient, and when the same level of quality and security as the original product is ensured. Physicians should have the freedom to prescribe the best possible therapy for their patients, and patients should maintain their right to choose.

Figure 2.6. Hypothetical cash-flow profile of a pharmaceutical



Source: Adopted from Gregson et al. (2005).

It is also important that policies reflect the specificities of certain medicines. With the increasing presence of biologic medicines in pharmaceutical markets, many governments and payers are realising that policies to encourage competition in small molecule generic markets are not appropriate for biosimilars due to their specificities. **Biosimilars differ from generics** due to their molecular size and structure, as well as higher development and manufacturing costs and longer development times. Furthermore, unlike the small molecule generics market, interchangeability and/or substitutability cannot be assumed for biosimilars due to the inherent variability of biological medicine. In its scientific evaluation, the European Medicines Agency (EMA) does not make a recommendation on the interchangeability and/or substitutability of a biosimilar and its reference product.³³ As a consequence, the prescribing physician should always have the option to designate which biological product should be dispensed to the patient, based on sound and scientific evidence.³⁴ Nevertheless, **the example of biosimilars also shows that sustainable competition can lead to both price decreases** – on average up to 28% – and increased access to treatment with high quality biological medicines earlier in the treatment algorithm.³⁵

³⁰ *Ibid.*; Michael P. Ryan, 'Pharmaceutical Foreign Direct Investment, Technology Transfer, Health Competitiveness, and the Jordan-United States Free Trade Agreement,' *George Washington University Law School, Creative and Innovative Economy Center*, May 2007

³¹ IMS, *The Impact of Biosimilar Competition*, 2015

³² Chart: OECD, *Pharmaceutical Pricing Policies in a Global Market*, 2008

³³ EMA Procedural advice for users of the Centralised Procedure for Similar Biological Medicinal Products applications; EMA/940451/2011; March 2015; p. 35: 'The decisions on interchangeability and/or substitution rely on national competent authorities and are outside the remit of EMA/CHMP. Member States have access to the scientific evaluation performed by the CHMP and all submitted data in order to substantiate their decisions.'

³⁴ EFPIA, *EFPIA Policy Principles for Off-patent Biologic Medicines in Europe*; 2015

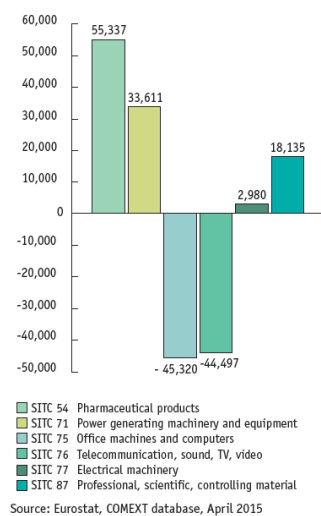
³⁵ IMS, *The impact of biosimilar competition*, 2015

Finally, **promoting pre-competitive public-private partnerships (PPPs)** such as the Innovative Medicines Initiative (IMI) **helps increase effectiveness of R&D spending**. These initiatives enhance collaborative research to tackle shared challenges that hold back medicines' development, including areas of unmet need such as rare diseases. Nonetheless, studies show that 67 percent to 97 percent of medicine development is still conducted in the private sector.³⁶ While public institutions and academia are important contributors to the advancement of basic research, translating knowledge into medicine remains the domain of the biopharmaceutical industry. **PPPs should therefore be complemented by new, market-based incentives for R&D**, notably in areas of high risk and low return on investment.

A success story in this sense is EU legislation on orphan medicinal products (OMPs).³⁷ Until 2000, rare diseases therapies were largely unexplored due to the small number of patients affected, the complexity of research and the regulatory system. Since the approval of the OMP Regulation, 118 OMPs have been approved in Europe, while only eight orphan-like products had previously been approved. This has allowed thousands of patients affected by severe and life-threatening rare diseases to access treatments that were not previously available before.

Promoting high standards internationally

EU-28 TRADE BALANCE - HIGH TECHNOLOGY SECTORS (€ MILLION) - 2014



The Commission has recognised the strategic role of the pharmaceutical sector, with it providing a unique contribution to the EU's trade surplus, investment in R&D, and creation of high-skilled jobs in Europe.^{38 39} From a trade perspective in particular, pharmaceuticals account for 3% of the EU's manufacturing base, and 6% of the EU's total exports.⁴⁰ **EU trade policy must therefore remain ambitious and contribute to protecting and promoting high standards globally**, such as through effective protection and enforcement of intellectual property rights (IPR). Moreover, **regulatory convergence and cooperation** provisions in free-trade agreements such as the Transatlantic Trade and Investment Partnership (TTIP) can raise standards levels globally. Reducing unnecessary administrative barriers and duplicative testing would also speed up the development process and result in faster access for patients worldwide.

³⁶ R. Chakravarthy, K. Cotter et al., *Public and Private Sector Contributions to the Research & Development of the Most Transformational Drugs of the Last 25 Years*, Boston, Tufts University School of Medicine, January 2015, p. 4.

³⁷ OMP Regulation EC 141/2000 and accompanying Commission Regulation EC 847/2000

³⁸ European Commission Staff Working Document *Pharmaceutical Industry: A Strategic Sector for the European Economy*, SWD (2014) 216 final/2, August 2014.

³⁹ Chart: EFPIA, *The Pharmaceutical Industry in Figures*, Key data; 2015

⁴⁰ Copenhagen Economics, "How a strong pharma chapter in TTIP will benefit the EU", prepared for EFPIA, May 2016, <http://www.efpia.eu/uploads/Modules/Documents/2188-02-efpia-ttip-study-23may2016.pdf>

Conclusion

Investing in healthcare is key to ensuring a strong European economy and the well-being of its citizens. AmCham EU believes that the issue of access to medicines should be part of a wider, strategic discussion aimed at achieving more effective and sustainable healthcare systems in the long term. Investment should focus on medicines' value and meaningful patient outcomes, and enable healthcare systems to adapt to the evolution of digital technology and other healthcare innovations. To this end, AmCham EU calls for **a broader view towards outcomes-based healthcare** that provides:

- A holistic approach covering the whole spectrum of health care provision in relation to health outcomes;
- Resources and infrastructure to measure value and outcomes;
- Flexible frameworks to ensure fast access to medicines through innovative and alternative pricing schemes, such as differential pricing combined with the increased use of value-based pricing methods; and
- Strong intellectual property protection to increase access to medicines and incentivise their development.

AmCham EU remains committed to promoting measures to improve access to medicines for patients across the EU, and looks forward to further contributing to the debate and engaging with policy-makers and key stakeholders.