

Encouraging the innovators

Dr Andrea Rappagliosi, Chair of EuropaBio's Healthcare Council, calls for a supportive European policy framework to encourage the development of innovative new medicines...

Today, healthcare biotechnology represents a great part of the development of new medicinal products: 20% of today's drugs and 50% of drugs under development are derived from biotechnology. Over 400 biotech medicines and vaccines are being tested for more than 100 diseases and more than 325 million patients have already benefited from approved biotech medicines.¹ Biotechnology healthcare is providing new strategies against unmet medical needs, through improved diagnostic tests, new hope for patients, and new knowledge on the cause of diseases.

In the last decade, European institutions have shown an increasing interest in biotechnology as a solid contributor to the innovation capacity and economic growth of Europe. The basis for recent European R&D policy lies in the blueprint of the 2000 Lisbon Summit, which stated the challenging goal 'to make Europe the most dynamic and competitive knowledge-based economy in the world by 2010', better known as the 'Lisbon Agenda' or the 'Lisbon Strategy'. This has led to the creation of the European Research Area, including the Framework Programmes for Research. The 7th Framework Programme, which covers the period 2007-2013, has dedicated €8bn to life sciences and biotechnology, of which €6bn will support health research.

Following on from the Lisbon Summit, the conclusions of the Stockholm Summit in 2001 called for the use of the full potential of biotechnology and a strengthening of the competitiveness of European biotechnology. Both summits have led to the EU Commission Communication 'Life sciences and biotechnology: A Strategy for Europe'.² This strategy consists of policy orientations and a concrete action plan to transform policy into action and foster continuing dialogue. It provides a framework for action and for co-operation between stakeholders. The strategy has a clear vision: 'Biotechnology is behind the paradigm shift in disease management towards both personalised and preventive medicine based on genetic predisposition, targeted screening, diagnosis, and innovative drug treatment'.³ Moreover, the Communication is pragmatic, proposes concrete actions, is based on facts, respects societal values, seeks coherence, is based on dialogue, respects regulatory oversight and principles, and addresses world challenges, including in the developing world. The

strategy affects the whole of Europe, across all policies, sectors and stakeholders.

The G10 recommendations of 2002 also called for the development of a biotechnology strategy in Europe, including the implementation of the directive on biotechnology inventions. However, in March 2005, the European Council stated that 'five years after the Lisbon strategy, the results are mixed...there is a high price to be paid for delayed or incomplete reforms...urgent action is therefore called for'.⁴ The G10 Group set out a framework of 14 wide-ranging recommendations. In its final report of May 2002, a set of actions to strengthen the Europe-based pharmaceutical industry was presented.⁵

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Building on these recommendations, the Commission created the Pharmaceutical Forum in 2005 to take the process forward around three key themes: information to patients on pharmaceuticals; pricing and reimbursement policy; and relative effectiveness. The forum was a multi-stakeholder platform and was composed of the European Commission DG Sanco and DG Enterprise; members of the European Parliament; health ministers of the 27 member states; the European biotech healthcare industry (EuropaBio); the innovative pharmaceutical industry (EFPIA); the generics industry (EGA); OTC medicines industry (AESGP); insurers (ESIP); sick funds (AIM); patients (EPF); wholesalers (GIRP); pharmacists (PGEU); and doctors (CPME). The aim of the forum is to 'improve the performance of the pharmaceutical industry in terms of its competitiveness and contribution to social and public health objectives'⁶, and to find new mechanisms to improve patient access to innovative medicines, taking into account the need to control the 27 national healthcare budgets.

In April 2007, European Commission President Barroso presented a new communication on the mid-term review of the Biotechnology Strategy.⁷ The review proposes to refocus actions on the promotion of research and market development for life sciences and biotechnology applications, and the 'Knowledge Based Bio-Economy'; on fostering competitiveness, knowledge transfer and innovation from the science base to industry; on encouraging societal debates on the benefits and risks of biotechnology; and on an improved implementation of legislation to ensure competitiveness.

Finally, on 2nd October 2008, the High Level Pharmaceutical Forum presented their final conclusions and recommendations.

Information to patients

The report stressed the need to provide citizens with more information in effective communication formats (electronic and non-electronic means), 'taking account of local traditions, healthcare systems and languages'⁸. While acknowledging that doctors are the most appropriate people to provide information to their patients, the forum recommended that 'member states, the Commission and health actors...consider new collaborations in the field of information to patients. Such collaborations should respect transparency, disclosure of financial and other support, as well as definition of responsibilities.'⁹

Relative effectiveness

The forum's report addressed the need to improve information flow, in terms of both patient access to information and data sharing in research and development. Specifically, it spoke of the need to improve data availability and transferability. The report encouraged member states and stakeholders to regularly exchange information in order to consolidate scientific evidence at national level, to provide this evidence to pricing and reimbursement authorities, and to inform healthcare professionals and patients on the most effective drugs.

The exchange of effectiveness data should, according to the report, 'aim to identify any barriers, whether scientific, technical or legal, that prevent all the parties involved from circulating the information easily'.¹⁰ It adds that: 'national authorities and companies should also consider ways of having early dialogue during product development to improve the generation of appropriate data as far as possible'.¹¹

Pricing and reimbursement

Member states were called upon to agree on a clear set of expectations on innovations they consider to be valuable. Communication of these common expectations 'will give companies a clear direction on healthcare priorities and indications on the evidence needed by authorities, while bringing authorities clarity on the mid- to long-term budget needs,' according to the report. Companies were urged to 'deliver the innovative medicines that society needs'¹², and to co-operate with patient organisations.

Member state authorities, stakeholders and the Commission were encouraged to 'strengthen their efforts to ensure access to orphan medicines in all EU member states',¹³ and to pursue early dialogue on research and development, improve exchange of 'knowledge on the scientific assessment of the clinical added value,' establish specific pricing and reimbursement mechanisms, and increase awareness of orphan diseases.

The report concluded that: 'Member states and the Commission, in co-operation with relevant stakeholders, should within the next two years undertake a first review of progress following the recommendations from the Pharmaceutical Forum in the field of pricing and reimbursement.... Further co-operation and exchange of experiences at EU level is needed'.¹⁴

'Focusing on cost, rather than the value of medicines for patients and society, has a direct impact on the strategic decisions about new research facilities or other investments.'

European Commission Vice-President Verheugen said: "The forum's recommendations can lead to important savings and permit a better reward for pharmaceutical innovation. I call upon industry and national authorities to help implement these recommendations to the benefit of the patients and healthcare budget."¹⁵

Policy considerations for healthcare biotechnology

EuropaBio strongly supports the continuation of the multi-stakeholder discussion, especially in the field of relative effectiveness and pricing, in order to ensure that patient-centred healthcare systems are established in Europe. To avoid short-sighted and budget-driven healthcare, we must work with all stakeholders so that decisions on healthcare do not result in inhibiting patient access to the best treatments, and hindering the development of the outstanding potential that the biotech sector can bring to patients, to disease management and to healthcare systems.

The healthcare biotech industry needs a predictable environment in order to take advantage of the great benefits of new treatments for diseases that present today's biggest medical challenges. Biotech medicines are already opening new avenues in cancer treatment, and are fostering research into – and developments of – treatments for rare diseases. Moreover, pharmacogenomics and predictive biomarkers lead the way to develop personalised medicines targeted to individuals' specific biology in many clinical conditions, such as neurodegenerative and autoimmune diseases.

Ensure an appropriate system to reward innovation

Society is on the brink of a new era in which pharmaceutical research, thanks to the use of genomics

and pharmacogenomics, will yield an entirely new class of medical interventions with respect to prevention, detection, treatment and cure. This will throw a radically new light on the concept of effectiveness (tailored medicines with higher rates of success) and cost (tailored medicines instead of one size fits all). This potential progress must be incentivised and rewarded.

Innovation in healthcare can be radical or incremental, and the benefit of the latter should be acknowledged with adequate reward in terms of pricing levels and reimbursement conditions. While radical innovation is widely accepted, it is essential to recognise that innovation in healthcare is often a cumulative activity, and small incremental improvements are fundamental aspects of the research and development process. Products that deliver incremental innovation provide alternatives for patients that do not respond well to the first product in class and create competition, thereby driving pricing and value optimisation.

In this field, we need greater creativity in finding solutions that can accelerate and facilitate patient access to the best treatments available. Rewarding innovation also comes in different forms: through price setting or readjustment, unrestricted access for the patient population defined as needing new therapy, therapeutic guidelines recognising new therapy, and speed of access.

For these reasons, it is crucial to reward innovation, both radical and incremental, in order to attain leadership in the research, development, manufacturing and commercialisation of bio-pharmaceuticals.

‘Policy-makers need to work with each other to create a seamless policy framework that industry can rely on when making business decisions that affect Europe’s scientists, patients and industry.’

The development of a series of shared European standards for Health Technology Assessment (HTA) would ensure the highest level of transparency, dialogue and trust amongst stakeholders and public authorities, including social values that are part of our solidarity-based healthcare systems and are the foundation of our European society. Namely:

A broader perspective: HTA should seek to determine the value of a technology whilst considering a wide range of perspectives that include economic, medical, technological, social, ethical, psychological and anthropological aspects. This evaluation should also take place in the context of the whole budget for healthcare, and be equally made for processes, procedures, service costs and products. HTA should take account of issues such as the policy context, community need, alternative treatment, uniqueness,

disease rarity, utilisation patterns, cost-effectiveness, budget impact, organisation and staffing impact, future evidence that will become available, ethics, access and healthcare funding mechanisms within this framework. HTA may reach different conclusions on the same technology in different countries or regions.

An improved early stage dialogue: HTA should be based on an in-depth interaction to discuss specific data required in order to decide on the reimbursement of a product, looking at its specific field and patient need. This would create predictability by clarifying payer expectations and the ability of industry to meet these expectations. At present, dialogue generally starts once a medicine has been approved and the data generated. What constitutes therapeutic progress, and whether and at what level to fund this progress, should be much clearer, and the rarity of the disease should be taken into account.

Flexibility: HTA is a flexible process that should be used collaboratively. The use of HTA to make reimbursement decisions should have as its key objective the rapid uptake of innovative health interventions and technologies, and improve health outcomes for patients without losing sight of our social values. A proper use of clinical and/or cost-effectiveness assessment of a medicine should be aimed at increasing the understanding of its different benefits, relevance to patients and public health, and impact on the healthcare system.

The ‘right’ timing: A ‘one size fits all’ approach to the timing and procedure of appraisals fails to take account of the complexity of conducting assessments, and ignores differences in treatments and therapeutic areas and patient populations. Often the sort of data needed to confirm cost-effectiveness and clinical effectiveness is data on the real life clinical use of a medicine. This can only be collected once a medicine has been on the market for a period of time, which again may differ depending on prevalence. Manufacturers should therefore be able to submit health outcomes information to the relevant government bodies throughout a product’s life-cycle. This evidence should receive appropriate attention and reward from payers.

Managing uncertainty: Uncertainty in economic evaluation of innovative therapies is often managed by a call for more evidence. However, the willingness to invest in research to obtain additional evidence may be limited by the number of patients available, the heterogeneity and natural history of the disease, the mechanism of action of the therapy, and the ethical burdens surrounding the choice of specific patient population. If the region of uncertainty is wide but includes the possibility of significant benefits, interim funding may be considered, provided that data is collected during use of the therapy in order to improve the informed decision.

Patient and public involvement: Both patients and the public have to be regularly informed about what the use and procedures of HTA are, and how they fit in making the

healthcare system more efficient for them. They should have the same rights as other stakeholders and actors in the process of evaluating new therapies, which should not just be based on economics. Also, patient and public involvement needs to be clear, transparent and become a key structural element of any evaluation of innovative therapies.

Maintain a political and legislative environment that is favourable for innovation

Focusing on cost, rather than the value of medicines for patients and society, has a direct impact on the strategic decisions about new research facilities or other investments. Maintaining an industrial climate that is favourable for innovation also implies creating a framework that supports our investment in R&D. Developing new drugs is a lengthy and costly process. One of the most recent estimates by Adams and Brantner (2005) is a cost of €803m per new chemical entity.¹⁶

The output of pharmaceutical industry research in terms of new treatments made available to patients is a widely discussed topic, but it is a fact that over the last 30 years, disease mortality rates in Europe have declined by almost 40%. The average life expectancy at the beginning of the 1900s was only 55 years, whereas a child born in Europe today can expect to live beyond 80. These are two impressive public health outcomes to which our industry has undoubtedly been a major contributor.¹⁷

Therefore, a well-structured industrial policy environment that is able to promote innovation in healthcare for the benefit of patients is crucial to ensure that the massive investments realised by the pharma-biotech industry lead to new, more effective and safer treatments.

Ensure strong and coherent political support and leadership to realise long-term investments in healthcare

Nowadays, not only in Europe, but governments all over the world are looking for ways to balance a number of competing policy goals. These include: economic growth; industrial development; attraction of foreign direct investment; advancement in education, science and technology; overall budgetary controls; and complex and evolving needs. Balancing these options is particularly difficult in the area of healthcare.

At EuropaBio, we share the concerns that the European authorities have in ensuring effective management of the limited resources allocated to healthcare. However, this pressure in European countries has frequently resulted in short-term, often punitive, pharmaceutical cost-containment measures, which bring only temporary relief, while undermining patient benefits. This is why we think that focusing the European political healthcare debate solely on pricing systems and cost containment initiatives fails to capture the broader contribution to society of our industry.

European healthcare biotechnology companies are led by some of the most innovative and brightest scientists in

Europe who need a predictable long-term framework in which to anchor their research and to attract investment to Europe.

Europe's policy-makers can do more to support the healthcare biotech sector. Policy-makers need to work with each other to create a seamless policy framework that industry can rely on when making business decisions that affect Europe's scientists, patients and industry.

Patients across Europe will benefit from the promotion of innovation. In order to achieve this, we need the support of European policy-makers to ensure an appropriate system that rewards innovation; to maintain an industrial climate that is favourable for innovation; and to ensure strong political support and leadership to realise long-term investments in healthcare. Especially in times of economic crisis, support for innovation and its value will preserve our future.

¹ <http://www.phrma.org/files/Biotech%202006.pdf>

² COM(2002)27

³ COM(2002)27

⁴ 7619/1/05 REV 1

⁵ European Commission: High Level Group on Innovation and Provision of Medicines. Recommendations for action. Brussels, European Communities, 2002, p30

⁶ http://ec.europa.eu/enterprise/phabiocom/comp_pf_en.htm

⁷ COM(2007)441

⁸ European Commission High Level Pharmaceutical Forum 2005-2008 Final Report. Recommendation 2.1, p. 7

⁹ European Commission High Level Pharmaceutical Forum 2005-2008 Final Report. Ethical guidance as to collaborations and public-private partnerships among partnering organisations for generating and delivering information to patients on diseases and treatment options. p. 46

¹⁰ European Commission High Level Pharmaceutical Forum 2005-2008 Final Report. Recommendation 6.1, p. 10

¹¹ European Commission High Level Pharmaceutical Forum 2005-2008 Final Report. Recommendation 6.3, p. 11

¹² European Commission High Level Pharmaceutical Forum 2005-2008 Final Report. Recommendation 8.1, p. 12

¹³ European Commission High Level Pharmaceutical Forum 2005-2008 Final Report. Recommendation 7.3, p. 12

¹⁴ European Commission High Level Pharmaceutical Forum 2005-2008 Final Report. Recommendation 10, p. 13

¹⁵ <http://europa.eu/rapid/pressReleasesAction.do?reference=IP/08/1451&format=HTML&aged=0&language=EN&guiLanguage=en>

¹⁶ Adams C, Brantner Van V (2005) 'New Drug Development: Estimating Entry from Human Clinical Trials', (7th July 2003), Washington DC: FTC Bureau of Economics

¹⁷ <http://www.efpia.org/Content/Default.asp>



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