

Executive summary

The 2013 Report *Priority Medicines for Europe and the World* provides a public-health-based medicines development agenda, based on a systematic methodology for this priority setting. It is an update to the original 2004 Report *Priority Medicines for Europe and the World* and takes into account changes in global health and pharmaceutical innovation since 2004 in order to better address current and future patient needs.

This latest updated report analyses pharmaceutical innovation from a global public health perspective for Europe and the world, based on the principles of equity and efficiency. For this analysis, four inter-related criteria have been applied to determine priority disease areas of research:

- Criterion 1: The estimated European and global burdens of disease;
- Criterion 2: The common risk factors amenable to pharmacological intervention that have an impact on many high-burden diseases;
- Criterion 3: The prediction of disease burden trends, based on epidemiological and demographic changes in Europe and the world;
- Criterion 4: The principle of “social solidarity” applied to diseases for which there are currently no market incentives to develop treatments.

Pharmaceutical “gaps” have been established for the diseases and risk factors identified. A gap exists for a disease or condition when: pharmaceutical treatments for that condition will soon become ineffective (e.g. due to resistance); the delivery mechanism or formulation is not appropriate for the target patient group; or when an effective medicine either does not exist or is not sufficiently effective (e.g. lack of basic scientific knowledge or lack of financial incentive due to market failure).

Within the context of identifying the pharmaceutical gaps which have an impact on the health of people in Europe, particular emphasis has been placed on identifying those research needs which are also relevant for the rest of the world. This “commonality of interest” is an important bridging aspect of the project between Europe and the world for both the 2004 Report and the updated 2013 Report.

In identifying priorities for pharmaceutical research for 2014 to 2020, data from the World Health Organization (WHO) Global Burden of Disease Database in Geneva and the Institute of Health Metrics in the United States were used to identify the most relevant diseases with the highest burden of disease, as well as the most relevant risk factors in Europe and the world. Information on predicted public health threats was obtained from the WHO, the EU and other official sources.

Pharmaceutical gaps were identified based on in-depth studies of the identified diseases and risk factors. This involved the use of data on the effectiveness of existing

treatments from multiple sources, including the Cochrane and other databases, the National Institute for Health and Care Excellence (NICE), WHO reports and industry sources. In addition, cross-cutting issues and enablers and barriers to innovation are addressed in Chapters 7 and 8.

Although substantial progress has been made since the original 2004 Priority Medicines Report in the development of diagnostics and medicines for some disease areas, a number of the pharmaceutical gaps persist and new gaps have been identified. In order to close these gaps, suggestions are made for updated research agendas and a supportive policy environment. Key findings of the updated 2013 Report are:

- **The population of Europe and the world is ageing**, with more people — especially women — living beyond the age of 80. Since 2004, for the first time in Europe, there are now more people over the age of 65 than under 15 years. With this ageing there is a marked increase in diseases of the elderly such as osteoarthritis, low back pain, hearing loss and Alzheimer disease.
- **Many chronic noncommunicable diseases (NCDs)** contribute substantially to the disease burden (disability and mortality) in both Europe and the world. While prevention remains important, more research into the development of new medicines and the improvement of existing medicines will benefit all.
- **Ischaemic heart disease and stroke** are the largest contributors to the European burden of disease and among the leading contributors to the global burden of disease. Effective medicines exist to treat CVD, which reduce the incidence of recurrent heart attacks or strokes. However, these medicines are not adequately utilized for secondary prevention. Research is needed on how to optimize secondary prevention treatment through the use of existing medicines.
- **Depression** is also a large and increasing contributor to the current and future global burden of disease. Priority research areas remain the treatment of depression among adolescents and the elderly, reducing side-effects and identifying the best treatment strategy for different populations and age groups.
- **Stroke, osteoarthritis, Alzheimer disease, hearing loss, low back pain, chronic obstructive pulmonary disease (COPD) and alcoholic liver disease** are seven high-burden conditions, in Europe particularly, for which the currently available treatment is inadequate in reversing or halting the progression of disease. Hearing loss and low back pain were not identified as priorities in 2004 but have now been added, based on new data on the burden of disease. A major challenge for all of these diseases is the **absence of specific biomarkers** which could be used to identify potential pharmaceutical products, diagnose and monitor the progression of disease, or assess the effect of treatment. Continued support is therefore needed for basic research for these conditions.
- **Antibacterial resistance and pandemic influenza** remain major threats to global public health which require a coordinated international effort. Research priorities are the development of new rapid diagnostic tests, new business models for research and development (R&D) for new medicines and vaccines, and prevention of infections through vaccination, infection control and other environmental measures.

- **Malaria and tuberculosis (TB)** represent major threats, especially in low- and middle-income countries; TB is also an important disease in some European countries. For both diseases, rapid diagnostic tests have been developed and funding is needed for additional investment in R&D for diagnostics, medicines and vaccines. Antimicrobial resistance will remain a threat until primary prevention with vaccines occurs.
- **Diarrhoea, pneumonia, neonatal conditions and maternal mortality** are major contributors to the global burden of disease. Some existing therapies are often not available in low- and middle-income countries due to health system limitations such as health care management and affordability and other barriers. Meanwhile, the lack of point-of-care diagnostics creates problems in case management. Research is needed to improve diagnosis and treatment, including reducing the cost of existing treatments and diagnostic devices.
- For **neglected tropical diseases and rare diseases**, new mechanisms to promote the translation of basic research into clinically important products remain a priority. While progress has occurred since 2004 in the treatment of Buruli ulcer, other diseases such as leishmaniasis, trypanosomiasis and dengue still require substantial research.
- **Tobacco use, alcohol abuse and obesity** are risk factors that underlie many of the most common serious NCDs affecting both Europe and the world. While prevention efforts must take precedence, research is needed on pharmaceutical methods to address these risk factors and the pathologies exacerbated by these risk factors (e.g. COPD, various cancers, alcoholic liver disease, osteoarthritis and diabetes).
- Pharmaceutical innovation should also encompass special groups of patients such as the **elderly, women and children**, who have particular needs in relation to dosage forms and products. Research is needed on the use of electronic health records (EHRs) to deliver the much-needed information on safety and effectiveness of medicine use in these populations. Development of appropriate formulations for children and the elderly needs to be supported. Progress has been made in some oral forms but more is needed.
- **Stratified medicine**, in which specific patient groups are identified who would benefit most from particular therapies, will need to be carefully researched over the next decade.
- The systems for **market authorization** and for **pricing and reimbursement decision-making** have different roles for the EU and for the Member States and involve various institutions, but the systems are closely interlinked. In combination, these systems have to function in such a way that they balance the need for new “safe,” “effective” and “affordable” medicines. Innovation in these related areas is needed. Instead of a single market authorization or pricing and reimbursement decisions, multiple decisions over time may be required to respond to new knowledge that is being produced (e.g. using the real-life data in EHRs). Each of these decisions will have an impact on all the parties involved, and can involve both regulatory authorities and Health Technology Assessment (HTA) bodies that provide input to pricing and reimbursement decision-making. In addition, there are many cross-links for research agendas. For example, new methods for evidence

generation, benefit risk assessment and regulatory dialogue will be needed to support policy tools such as adaptive licensing as well as value-based pricing.

- Developments in the field of **real-life data utilizing EHRs** have created innovative methods to compare and evaluate the performance of new medicines after market approval. Optimal use of EHRs would build on European strengths and could shorten the time needed to bring a product to market, while ensuring safety through active post-marketing surveillance. EHRs could also deliver the much-needed information on the effectiveness and safety of medicines in special patient groups at lower cost than other data collection methods.
- A major change since 2004 has been the growth, both in Europe and worldwide, of **Public-Private Partnerships** undertaking early, translational and product development research. Notable successes have been achieved by the Innovative Medicines Initiative with enabling research activities but for Product Development Partnerships a tension exists between short-term funding commitments and the long-term development periods that their products require. This tension will need to be addressed if these partnerships are to fulfil their potential.
- The optimal role of **patients and citizens** in contributing to priority setting and to regulatory and pricing decisions needs to be further developed. Their exact role and the best mechanisms for their involvement remain to be defined.

Much has been achieved in Europe since 2004 but many research needs and opportunities remain. In this updated report several existing and some new gaps in pharmaceutical development have been identified. These will need public funding in order to further improve the health of the people of Europe and the world.