

Pharmaceutical Strategy - Timely patient access to affordable medicines

A statement by the Helmholtz Association

The Helmholtz Association welcomes the European Commission's approach to develop a European Pharmaceutical Strategy. Ensuring equitable access to affordable healthcare, including but not limited to medicines, is obviously of crucial importance in maintaining and improving citizens' health and well-being. A successful European pharmaceutical strategy should place EU citizens' well-being at its center and will require coordinated, concerted action by many stakeholders working in partnership: from government policymakers to researchers, clinicians, patient organizations, industry, regulatory agencies and insurance carriers.

In this statement, Helmholtz will focus on three of the challenges listed in the Roadmap document and the European Commission's approach to overcome them.

4. Innovation efforts are not always aligned to public health and health systems' needs

Helmholtz agrees with this assessment and the approaches to find new ways for pharmaceutical innovations to reach and benefit patients. It is central to note that these "public health needs" are sometimes difficult to prepare for, as the most recent example of the need for vaccines and treatment options for SARS-coronavirus infections demonstrates. The EU Pharmaceutical Strategy should therefore provide for foresight studies based on epidemiological, demographic and socioeconomic data in order to anticipate future needs and envisage possible solutions. Aside from infectious diseases, there are many other urgent public health needs, such as cancer, cardiovascular or neurodegenerative diseases, diabetes or other chronic diseases that place a huge burden on healthcare systems and citizens. These must also be considered adequately.

Importantly, health innovations cannot be generated "on demand" as soon as a public health need arises. Today, the most innovative technologies, diagnostic tests and therapies originate in academia, spin-off companies or small and medium enterprises (SMEs), from where they are further developed on a larger scale by industry. Understanding basic molecular mechanisms, elucidating disease mechanisms and developing innovative technologies with which to explore them are thus the prerequisites to finding suitable drug targets and developing safe and effective early-detection diagnostic tests and therapies. Crucial to a productive pharmaceutical pipeline is therefore an ecosystem of partnerships in which academia works together with industry and other stakeholders as equal partners in a smoothly functioning continuum.

It is therefore imperative to promote sustainable fundamental and translational biomedical research over the long term in all fields related to human health because we simply cannot know all urgent public health needs of the future. This is true for "classic" research on drugs and vaccines, where innovative approaches and therapies result from basic research, as well as for research on novel technologies, biomarkers for diagnostic tests and innovative therapeutic interventions. Examples include biological therapies such as RNA vaccines; gene, cell or immune therapies; radiotherapy and nuclear medicine; as well as environmental medicine including exposome-based preventive approaches to human health.

5. Challenges for the EU pharmaceuticals innovation ecosystem

While there is financial support for newly founded and well-established companies and their research and innovation activities, efforts to build a European pipeline that seamlessly connects fundamental and translational research with later technology readiness levels suffers from fragmentation and lack of funding.

In particular, the proof-of-concept phase, often referred to as the “valley of death” because of a lack of funding and collaboration opportunities, is a key obstacle hindering a successful pharmaceutical strategy in Europe.

One potential way to overcome this “valley of death” is to support networks of research institutions in facilitating comprehensive clinical trials to test therapies for a variety of diseases, including rare diseases.

To this end, a number of difficulties must be overcome. For example, there is a need for a consistent, unified regulatory landscape in Europe to make it feasible to carry out clinical trials involving different countries. This is especially important when patient numbers are small, or when rare diseases or molecularly stratified patient groups are involved, making international multi-centric studies a necessity to achieve adequate patient cohorts. An example of this approach is the IMI-funded project COMBACTE-NET, in which partners all over Europe jointly conduct clinical trials, aiming at the discovery of novel anti-infective drugs. This strategy could be adapted to other public health needs.

Secondly, access to innovative clinical studies for patients in Europe must be improved, since now many pharmaceutical companies carry out their trials in countries outside of Europe to avoid overly complicated regulatory requirements. Increased support and simplified regulations for innovative approaches to precision medicine, such as basket and umbrella studies based on molecular diagnostic methods, are also needed.

Finally, new ways of funding investigator-initiated trials that are better aligned to the necessary funding volume, timeframe or specifically designed milestone models, must be established.

In general, clinical trials should also include previously neglected population groups, e.g. children, women, the elderly, and orphan diseases in order to ensure that drugs are safe and effective for all potential users. For example, many cancer entities defined according to molecular criteria qualify as orphan diseases because the patient groups are correspondingly small. The adoption of precision medicine approaches will increasingly result in diagnostic tests and medicines tailored to small, highly stratified patient groups, rather than blockbuster drugs suitable for large populations.

Pharmaceutical research and development can be streamlined and made more efficient by integrating datasets from different sources (-omics analyses, clinical data and other real-world evidence) as well as artificial intelligence approaches to data analysis. In past years, numerous large-scale databanks at the international, European and national level have been developed and integrated: ICGC, BBMRI, EATRIS, EGA and the newly launched German Human Genome-Phenome Archive (GHGA) are only a few examples of such databases. Additional support for these databanks could contribute not only to drug discovery and development, but also to outcomes research that would benefit healthcare systems and make pharmaceutical research and development more efficient.

To ensure the worldwide competitiveness of the EU pharmaceutical sector, it is essential to guarantee adequate protection for intellectual property, in particular patent protection, for all parties involved in pharmaceutical innovations. Robust patent protection is the prerequisite for the investments necessary to carry out clinical trials and to underwrite the extremely long (more than 10 years) and costly (billions of Euros per product) pharmaceutical research and development process.

In addition, a new investment fund is conceivable, e.g. administered by the European Investment Bank, specifically for pharmaceutical product development and related activities. Further, generating a European value chain is not only a financial issue, but also involves administrative and regulatory affairs, which are not always compatible with a high-risk start-up culture. Thus, we suggest specific support of biotech start-up hubs, which will not only facilitate access to finances possibly through funding by public-private partnerships, but also cover all other aspects of biomedical entrepreneurship.

6. Technological and scientific developments may challenge the regulatory framework and consequently lead to unintended barriers to needs-driven innovation

Regulatory procedures must of course be adhered to before any health innovation can enter the market and benefit patients. While the role of the regulatory framework is ultimately to protect patients and other end users, at the same time it will be necessary to re-evaluate the entire complex of regulations in Europe so that they support and facilitate rather than hamstring the pharmaceutical research and development process.

As just one example, the especially stringent regulations with respect to testing in minors or the difficulty of testing medications in elderly subjects with cognitive issues or co-morbidities often lead to their exclusion from clinical trials, with the negative consequence that fewer medications targeting these groups end up being approved. It will be necessary to find a workable balance so that such groups are no longer underserved. Moreover, the EU regulatory framework must be consistently applied throughout all European countries so that large-scale, cross-border clinical trials are feasible.

Big data and artificial intelligence are becoming increasingly important in biomedical research and development and they will be indispensable in a forward-looking EU pharmaceutical strategy. It will be essential to provide an adequate, patient-centric legal and ethical framework at both the European and national levels that balances respect for individuals' privacy and informed consent issues, while ensuring that biomedical research and development and cross-border data sharing have sufficient latitude.

In summary, Helmholtz welcomes and confirms the European Commission's Roadmap towards a Pharmaceutical Strategy. While such a strategy must clearly be guided by public health needs, it is vital to note that these needs often arise unexpectedly, underlining the essential need for long-term fundamental and translational research on human health and disease mechanisms. Challenges and bottlenecks in the European pharmaceuticals innovation ecosystem and regulatory framework must be addressed appropriately, while maintaining the safety and privacy of citizens, research organizations, and enterprises alike.

Brief portrait of the Helmholtz Association

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