

## Impact Assessment of the Revision of the General Pharmaceutical Legislation

ERS appreciates the opportunity to contribute to this roadmap on the revision of the General Pharmaceutical legislation (namely Directive 2001/83/EC1 and Regulation (EC) No 726/20042).

### Join the dots

Legislation must foster medical advancement not hamper it. We need more joined-up legislation across the Union and Member States in this area, and we hope the evaluation and revision of the general pharmaceutical legislation will lead to increased interconnection across EU policies related to health and medical research. We need to work together to have improved access, affordability and availability of medicines for patients and Europe must remain a leading centre for medical innovation and excellence in science. This entails that Europe must be a place where researchers and clinicians are supported not impeded by the general pharmaceutical legislation.

### Learn the lessons of the pandemic

SARS-CoV2/COVID-19 has highlighted the gaps and challenges in the current pharmaceutical regulatory framework, particularly in the access and availability of medicines, vaccines and medical devices, for example:

- Major disruptions in care for lung cancer patients who suffered immensely from postponed treatments. The backlog in access to essential cancer therapies will unfortunately lead to increased mortality in the next years.
- Prior the pandemic, respiratory diseases represented an enormous and increasing healthcare and economic burden across Europe, with over 600,000 deaths a year and six million hospital admissions with total costs exceeding €380 billion per year [European Lung White book]. The pandemic has increased this burden.

The current Covid-19 crisis has also increased the need for international engagement, including harmonisation and information sharing on standards and collaboration of scientific research and regulatory frameworks. Accordingly, the pharmaceutical legislation must take into account a global approach. Therefore, it is key that the EU maintains and further increases our collaboration globally.

### **Adaptive clinical trials**

To support and accelerate product development and authorisation in areas of unmet needs, we believe it is essential to develop standardised research protocols and common platforms for rapid recruitment into adaptive clinical trials. The 'eternal' trials like Remap-cap and Recovery that have been adapted for COVID-19 must be utilised for other areas such as anti-fibrotic medicines.

The COVID-19 pandemic showed our ability to achieve remarkable results in terms of developing vaccines in 10 months rather than 10 years. **Regulators must be allowed flexibility to adapt procedures to the latest science whenever needed and not only in times of pandemic.**

### **Real world data (RWD)**

ECDC has shown during the current crisis how important real-world data is to making decisions. Real world data can complement and be a useful addition to clinical trial data, however, systems need to be able to speak to each other. Real world data needs to be better integrated into the approval system of new medicines.

### **Definition of unmet medical needs**

We agree with the statement that there is a need to stimulate innovation and breakthrough therapies, especially in areas of unmet needs that are not covered by the rare disease and medicines for children legislation. However, the current definition of unmet medical needs in the legislation is too restrictive - as it relies solely on the availability of adequate alternative treatments. We would argue for a broader definition of unmet medical needs in the legislation. One that covers other elements and a wide range of situations such as the positive/benefit risk balance or patient reported outcomes and one that moves beyond just the availability of adequate alternative treatments.

There have been very few new classes of drug therapy introduced for the treatment of respiratory disease over the past 50 years. It has proved difficult to find new classes of drugs - particularly at the small molecule level - that are even as effective as existing therapies or have a comparable safety record. For example, there is no disease modifying treatment for chronic respiratory disease (COPD) and certain severe asthma patients do not respond well to available treatments and there is currently no cure, although it is one of the most common chronic diseases.

If we are to tackle unmet medical needs, such as those described above, academic non-profit initiatives must be facilitated by the pharmaceutical legislation in conjunction with

funding from programmes such as Horizon Europe, EU4Health and national programmes and EU agencies such as HERA.

### **Access, access, access**

When medical innovation is achieved, for example in the field of asthma or the field of lung cancer treatments and diagnostics (e.g. better tumour biological profiling, advanced invasive and non-invasive diagnostics, immunotherapies and targeted therapies) they do not always reach the patient. There are remaining inequalities in financing, as well as timely access and availability of new treatments between Western and Eastern Europe. The updated pharmaceutical legislation should ensure that the exciting clinical advances in both respiratory medicine and lung cancer are translated into routine care, in a timely and equal manner. We support all policies options that will advance access in the legislation.

### **Incentives**

Incentives need to be looked at carefully in order to accelerate innovation in areas where there is market failure. We support the Commission's initiative to revise the system of incentives (p.4), with the aim to foster R&D in sectors that have been until now de-incentivised. Incentives could also be given to maintain older medicines which are often as efficient and safe as new medicines. Also, repurposing - the use of new indications for old medicines - must be advanced and incentivised by the legislation.

### **Antimicrobial Resistance (AMR)**

ERS fully supports the particular attention to Antimicrobial Resistance (AMR). Resistant strains of Tuberculosis, known as MDR-TB and XDR-TB, pose a major threat in Europe with a lack of effective therapies and the risk of transmission amongst deprived populations. Overall in the WHO European Region, every third pulmonary TB patient has a drug-resistant form of TB. Moreover, 70% of the world's XDR-TB patients live in the European Region ([WHO/ECDC report on Tuberculosis \(TB\) surveillance and monitoring in Europe 2021](#)). Although a new drug (Bedaquiline) has been approved for the treatment of MDR-TB recently, it has frequent and sometimes serious side-effects which further limits its use.

The new proposed Health Emergency Preparedness and Response Authority (HERA) must have the scope to have a key role in the fight against AMR.

### **Digital tools**

We agree that the new pharmaceutical legislation should make full use of digital tools. Sharing of data at the EU level is key to foster innovation, to have a comprehensive view of medical needs & availability of medicines across EU. ERS welcomes the mention of the need of complementarity with European Health Data space (p.1).

- It is crucial to exploit the potential of ever-growing data in a trustworthy European framework where Member States are encouraged and supported

to better align their use of health data, to stimulate research and accelerate innovations, while protecting the privacy of citizens.

- We need better co-ordination on shortages. The shortages alert system of the EMA must be linked to the European Health Data Space and electronic patient records. It must be better understood when shortages are occurring.

## **Medical devices**

In view of the fast development in science and technology, we need a more adaptive legislative framework. This work also needs to be continuously coordinated with related legislative fields, e.g. medical devices, where the rapid technological development increasingly blurs the lines between medicinal products and medical devices. We would therefore like to see a strong synergy between the medical devices' legislation and the pharmaceutical legislation. Many of the innovations in medicine will come in the device area and the cross over between medicines and medical devices will increase.