ECL contribution to the European Parliament own initiative report on the Pharmaceutical Strategy for Europe

The Association of European Cancer Leagues (ECL) is a non-profit, pan-European network of 30 national and regional cancer societies. **ECL members operate across the whole cancer continuum and patient pathway. Cancer leagues are the main resource for the public for cancer control information and services**. ECL provides a unique platform for members to collaborate with their international peers, primarily in the areas of cancer prevention and early detection, access to medicines, and patient support, creating opportunities to advocate for these issues at the EU level.

On 25 November 2020, the European Commission published the Pharmaceutical Strategy for Europe and ECL welcomed the Communication and the emphasis posed on patients' needs.

Notably, the Strategy seeks to tackle the EU medicine shortages crisis, better alignment between R&D investment and patient needs, and the ever-increasing costs of new treatments.

Now that the European Parliament is working on its own-initiative report on the file, ECL has the pleasure to contribute by providing some reflections points and recommendations to successfully implement the flagship initiatives outlined in the chapters of the European Commission's Communication and in the draft report of the European Parliament:

1. MEDICINES - A STRONG ECOSYSTEM AT AN IMPORTANT CROSSROADS
2. DELIVERING FOR PATIENTS: FULFILLING UNMET MEDICAL NEEDS AND ENSURING ACCESSIBILITY AND AFFORDABILITY OF MEDICINES
3. SUPPORTING A COMPETITIVE AND INNOVATIVE EUROPEAN PHARMACEUTICAL INDUSTRY
4. ENHANCING RESILIENCE: DIVERSIFIED AND SECURE SUPPLY CHAINS
5. THE EU IS LEADING THE WORLD IN HEALTHCARE

Medicines - a strong ecosystem at an important crossroads

Great progress has been made recently on human health in the European Union, with increased life expectancy at birth. The Communication outlines the progress made thanks to new treatments coming to the market, **ECL wants to stress that that these new health technologies are very expensive, their prices continue to rise, and this widens the inequalities in access to the new therapies.**

ECL calls on the European Institutions to envisage measurable and feasible long-term, actionable, strategies to ensure a sustainable pharmaceutical ecosystem.

Delivering for patients: fulfilling unmet medical needs and ensuring accessibility and affordability of medicines

ECL welcomes with pleasure the extensive paragraph on putting patients at the center of all health policies. We note that the detailed considerations address unfair competition, want to steer innovation where this is much needed, improve accessibility, availability, and affordability of medicines.

ECL calls on the European Commission to:

- Set an unambiguous and clear definition for the term 'unmet medical needs' (UMN), based on transparent and objective criteria. The definition should include but not be limited to the following: incidence[1], survival rates, existing alternative treatments, mortality, and severity of the disease;

- Put patient-centredness and participatory decision-making at the heart of defining UMN;

- Steer public and private investments to address and meet public health and patients' needs, with particular attention to children and adolescents with cancer and rare cancers with low survival;

- Ensure that research priorities are aligned with the needs of patients and health systems by fostering collaboration between regulators, academia, healthcare professionals, patients, and payers at the early stages of R&D;

- Encourage the Member States to make use of the diagnostic tools necessary to find out the most effective treatments with positive cost-benefit ratios. This would reduce avoidable harm to patients, improve treatment outcomes, and possibly reduce costs for national healthcare systems;

- Implement an approach to medicine development driven by a mechanism of drug action, disease biology, and patient needs. This is vital in order to align the general

[1] See Gatta, G. et al (2017) 'Burden and centralised treatment in Europe of rare tumours: results of RARECAREnet - a population-based study'. Lancet Oncology, 18 (8), 1022-1039. Available here: https://doi.org/10.1016/S1470-2045(17)30445-X

pharmaceutical legislation (namely Directive 2001/83/EC and Regulation (EC) No 726/2004), and the two Regulations on Orphan Medicinal Products and paediatric medicines with science;

- Create a system of rewards/obligations to cover knowledge gaps in basic research to address neglected diseases, guarantee the supply of medicines, and steer accessibility;

- **Discourage the creation of artificial subsets of common diseases** ('salami-slicing') just to receive for each of them the orphan disease designation and the related incentives and benefits. The Directive 2001/83/EC should include the definition of 'orphan medicinal product' which should also be more specific in the Regulation (EC) No 141/2000 . Hence, **personalised medicines** should not be handled as orphan medicinal products;

- **Create targeted incentives** to support early-stage medicine development and ensure medicines with proven added value and medicines in areas where there currently are no treatments. To this end, digitalization can be of great help. Real-Word Data (RWD) and Real-World Evidence (RWE) can play a major role when it comes to clinical trials with a limited number of patients (e.g., rare cancers) and therefore little evidence of the safety, efficacy, and real added value of new health technologies. **The application of both RWD and RWE in the research & development and post-marketing phase** would be informative to identify areas where innovation is needed to meet patients' needs, better assess the effects on patients also after the marketing authorisation;

- **Assess the impact of the incentives already granted before extending the market exclusivity period.** This could be done by looking at (i) the real added value brought by the new health technology applying a European Health Technology Assessment, and (ii) the impact of the new product into the market on pharmaceutical expenditure, and on health expenditures more broadly;

- Provide incentives proportionate to the costs required to put a specific medicine on the market and to the return-on-investment. Hence, transparency and disclosure of costs to national authorities and payers are essential to **stop blindfolded negotiations on price and reimbursement.** To this end, the marketing authorization holders should report to the European Medicines Agency the annual revenues of the year and this could be implemented with the extended mandate of the Agency, and/or EURIPID database can be used to share net prices of medicines;

- Improve the Health Technology Assessment (HTA) framework to streamline the evaluation of medicines and equip the HTA bodies with adequate resources and tools, such as access to data. The HTA is of critical importance given its impact on the identification of unmet medical needs.

ECL supports the suggestions of the Committee on Industry, Research and Energy which:

- Calls on the Commission to develop a new incentive model, look into decoupling mechanisms as an alternative to exclusive protections, and attach strict conditions to public funds by the principle of a fair return-on-investment;

- Calls for public investment in R&D to be made transparent and for it to be reflected in product availability and pricing;

- Calls on the Commission to support pricing models based on real production costs;

- Calls on the Commission to promote joint procurement and apply most economically advantageous tender (MEAT) criteria more stringently.

On the contrary, we note strong industry-centric feedback coming from the Committee on Legal Affairs and we believe that the Union's competencies under the Treaties can be revised when needed or we would have never had amendments of Treating throughout the years. The Legal Affairs Committee calls also for Intellectual Property Helpdesk for SMEs which we believe could be a valid tool also for academia and public research centers that run scientific projects.

Supporting a competitive and innovative European pharmaceutical industry

Undoubtedly, the European Union is home of a strong pharmaceutical industry, research centers of excellence, and the pharmaceutical sector is a key player in the market.

Yet, ECL calls on the European Commission, the other EU institutions, and the Member States to:

- Dedicate special attention to the pharmaceutical sector more broadly, beyond the industry, if the objective is to have a competitive and innovative ecosystem;

- Better define the terms 'availability', 'affordability', and 'accessibility' in order to identify the root causes behind the inequalities across Europe and provide adequate legislative and non-legislative solutions;

- Investigate and address the (marketing) tactic of withdrawing off-patent drugs and reintroducing similar medicines with new indications and higher prices[2]. The revision of the Directive 2001/83 will be critical in this regard.

We call on the European Commission to discourage commercial practices that hamper competition by:

- Discouraging practices that extend market exclusivity, prolong intellectual property protection, lead to competition distortion and profit maximization, through the strategic use of intellectual property rights, such as incremental patenting of existing products ("ever-greening" strategies);

- Promoting generic competition for off-patent rare disease drugs. To this end, advanced market commitments and subsidies for non-for-profit manufacturers should be considered;

- Considering repurposing of existing drugs for treatments without viable markets. This can sometimes be done at low cost and has the potential to decrease both the time-frame and the costs;

- Stepping up cooperation between Member States on the affordability and cost-effectiveness of medicines as well as cooperation between national pricing and reimbursement authorities and healthcare payers, including fostering transparency of price information.

In addition, ECL wishes to stress that while accelerated product access and a degree of regulatory flexibility might be needed, it should not contribute to the lowering of regulatory standards in product development and evaluation. This poses serious threats to patient safety and would stimulate an unhealthy mechanism where a producer aims at receiving the marketing authorisation by providing little evidence on the safety, efficacy, and real added value on the health technology.

[2] See the Pharmaceutical Sector Inquiry by the European Commission.

ECL calls on the European Commission and Member States to:

- Urgently establish clear criteria and a common European definition of 'medicine shortages'. This is an essential action in order to establish a coordinated approach for reporting and managing shortages. An EU definition of 'shortage' would allow appropriate interpretation and identification of the phenomenon and the development of appropriate legislative solutions;

- Set clear obligations and sanctions alongside the definition of a 'shortage'. This would provide scope to act in case a marketing authorisation holder does not fulfil their responsibilities or cause availability issues due to withdrawals;

- Request to marketing authorisation holders mandatory prevention plans to avoid shortages along with the mitigation plans should a shortages occur. This could find a legal basis in the revision of the Regulation on the role of EMA;

- Consider medicine shortages as a cross-border health threat as it puts national health systems under pressure and poses risks to patient's health and may lead to non-treatment, under-treatment, and possible medication errors from attempts to substitute missing medicines[3];

- Design two scenarios of medicine shortages: in 'normal' times and shortages during public health emergencies;

- Make efficient use of existing instruments to improve the communication channels between stakeholder such as the Single Point of Contact system (SPOC) and Industry Single Point of Contact (i-SPOC) system;

- **Strive for greater transparency and availability of (i) medicines shortage data**, (ii) early detection, and assessment of potential shortages, (iii) consistency of reporting, (iv) increased access to the information available across the supply chain, (v) improved data infrastructure, and (vi) collaborative governance processes;

[3] https://www.who.int/medicines/publications/druginformation/WHO_DI_30-2_Medicines.pdf?ua=1

On the last day of the 72nd session of the World Health Assembly (WHA) which took place in Geneva between 20 and 28 May 2019, Member States adopted the resolution on 'Improving the transparency of markets for medicines, vaccines and other health products'. The final resolution is clear about public transparency on real medicine prices. Member States are urged to 'take appropriate measures to publicly share information on net prices of health products'.

ECL calls on the European Commission and Member States to:

- Restore the balance in pricing and reimbursement negotiations between governments/payers and multinational pharmaceutical companies. Currently, there exists an informational asymmetry, where the industry knows the product prices in the countries where they decided to launch the product, but national payers do not have access to such data beyond their borders;

- Increase transparency with regard to public contributions and incentives received on research and development (R&D) programs;

- Establish a mechanism that allows the national healthcare systems to receive from pharmaceutical companies information on marketing, sales and reimbursement in other countries, including negotiated prices.

Any treatment, no matter if innovative or not, does not have any value unless reached by the patient. Affordability is therefore key.