Response to pharmaceutical strategy Roadmap submitted on 24 June 2020

ECL welcomes the initiative to build patient-centred Pharma Strategy with strong emphasis on addressing unmet need & achieving greater medicines access, availability, affordability and sustainability of health systems. For key policy recommendations, see page 12-18 of our BCP vision: https://bit.ly/3drdqPp

Strengths:

1. Ensure timely access to safe, quality and affordable medicines and financial sustainability of MS' health systems. ECL appreciates recognition of (i) high medicines prices and its negative impact on health systems and patients; (ii) need for enhanced cooperation in HS, HTA and P&R; (iii) need for increased transparency throughout the sector, including on costs and prices of medicines. ECL recommends to:

i. Prevent misuse and overuse of the orphan status and ensuring the right balance between investment in orphan medicines, particularly where there is no treatment alternative, and preventing unintended effects on affordability;
ii. Conduct a study on the role of price transparency, with attention to robust methods for the calculation of R&D and production costs, and suggesting ways toward EU-wide implementation of the WHO Transparency Resolution;
iii. Measure and disclosing the extent of public investment in R&D at both the EU and MS level and creating prerequisites for public investment to ensure publicly funded products are available at an affordable price;
iv. Establish High-Level multi-stakeholder WG to discuss a fair price definition and sustainable pricing models.

2. The need for new therapies to be clinically better than existing alternatives as well as cost-effective. It is crucial to align patient and public health needs with development of new medicines. It is important to maintain high regulatory and HTA standards. ECL recommends:

i. Full implementation of the Clinical Trials Regulation as soon as possible;
ii. Close collaboration between public authorities, patients and HCPs to identify and financially support areas of unmet medical need and low financial interest;
iii. High quality benefit-risk assessment of patient-relevant endpoints before granting market access, stressing the need for surrogate endpoints in CTs to be accompanied by hard endpoints reflecting improvements in overall survival and quality-of-life measures;
iv. iv. Systematic collection and submission of real-world evidence (including OS, adverse reactions and QoL improvements) once the medicine enters the market;
v. Pooling of resources and international cooperation between EU MS to prepare health systems for (i) the arrival of new medicines and technologies, (ii) conducting high quality HTA and (iii) sharing information about prices and pricing and reimbursement strategies, in order to enhance MS' ability to (a) prioritise medicines with higher clinical value, (b) review and adjust prices based on new evidence, and (c) effectively negotiate the prices of medicines.

3. Addressing and preventing medicine shortages. One country cannot address this growing problem alone. Please see our recommendations on how to prevent and manage medicine shortages in Europe here: https://bit.ly/2YqgxCE

Shortcomings:

1. More attention should be given to the need to maintain a robust regulatory environment for medicines coming to the EU market. This includes new ways of collecting evidence on medicines efficacy and safety (also post approval - RWE) and data requirements for developers which include regulators/payers demands early in the medicines R&D process. Connecting this Strategy with the recently published EMA Regulatory Science strategy is crucial.
2. The negative impact of IP protection and incentives on the affordability of products should be elaborated.
3. Acknowledging public investment in medical R&D and attaching it to conditionalities related to open science and affordability is key, e.g., for products funded through EU4Health or HEU.
4. Role and responsibilities of the EMA in the development of the Strategy should be clarified and enhanced

Attachment: ECL's Vision for Europe's Beating Cancer Plan pages 12-18 related to cancer medicines.