Response of the European Cancer Organisation to the Roadmap consultation on a new EU Pharmaceutical Strategy.

07 July 2020

The European Cancer Organisation welcomes the commitment to producing a new EU Pharmaceutical Strategy, and the accompanying commitment to public consultation on its content. This is an encouraging recognition of not only the range of concerns that exist in relation to such issues as access, affordability, availability, transparency, and the research, incentive and regulation frameworks in operation. It also acknowledges the opportunities being highlighted by new tools, approaches and proposed paradigms, such as better use of data tools and patient reported outcomes.

This response includes comment relating to the current research landscape for pharmaceutical (and other) treatment in Europe, noting opportunities to reduce burden and improve access to clinical trials.

In responding to this consultation, we wish to strongly express that in respect of cancer treatment is multi-modal and best outcomes for the patient are achieved by a range of interventions, including multidisciplinary care. So while welcoming attention to pressing policy needs for the pharmaceutical domain we encourage policy makers at both European and national level to be ever mindful of the need for attention to all components of care and treatment. Distortion of policy attention in this respect, should be avoided. Policy makers should always strive for an holistic and comprehensive approach to improving outcomes for patients.

Related to this, we emphasise the importance of the new EU Pharmaceutical Strategy not sitting in isolation. It should be considered and understood to be part of a broader need to re-orientate and improve health systems generally. This includes an overall need to improve the balance of incentives to ensure that access to improvements in ALL treatment modalities is achieved, and across the care continuum i.e. in respect to prevention, detection, diagnosis and follow up care. For this reason it is important that the Pharmaceutical Strategy seek coherence with other EU initiatives such as digital, research and industrial strategies, and of course, Europe's Beating Cancer Plan. A common overall social purpose and vision should be pursued by these strategies, encompassing improved access of patients to treatment and care.

This response should be read in conjunction with the responses of others from the cancer community, such as that of:

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- **the European Organisation for Research and Treatment of Cancer (EORTC),** (feedback reference F533681) which emphasises among other matters the value and needs of independent clinical research for achieving the optimal use of pharmaceuticals.
- **the European Association of Urology (EAU),** (feedback reference F532897), which highlights opportunities for reducing trial burden, making innovation more patient-centred and making better use of PROMs and digital technology within the pervading regulatory landscape.
- **the European Society of Oncology Pharmacy (ESOP),** (feedback reference F532879) which raises recommendations for improving access, availability and affordability of pharmaceutical treatment.
- **the Association of European Cancer Leagues (ECL),** (feedback reference F525494) which emphasises a series of recommendations related to pricing, affordability, transparency, cost-effectiveness and medicines shortages.
- **[the European Association for Palliative Care (EAPC),** (feedback reference F536504) which highlights particular needs to be met in respect of access disparities to opioids and other analgesics across Europe.
- **the European Society for Paediatric Oncology (SIOP Europe),** (feedback reference F536497) which emphasizes the urgent need to address the specific needs of children and adolescents with cancer, including the dire lack of innovative medicines for young patients in the current regulatory framework.

RECOMMENDATION 1: MAKE SURE THE NEW PHARMACEUTICAL STRATEGY IS AMBITIOUS AND COMPREHENSIVE

The new EU Pharmaceutical Strategy should be bold and ambitious in achieving a timely update of regulatory and incentive models, that takes account of new developments in science and practice and tackles burning concerns about access, affordability, transparency and the weighting and balancing of incentives. The Strategy should also serve to achieve a lasting upgrade of the modes of cooperation between EU member states in ensuring equitable and timely access for patients to medicines.

An auspicious start has been made in this direct by ensuring the conduct of open forms of consultation with all interested stakeholders. We urge careful attention to all perspectives and recommendations brought forward. The Strategy authors should have the fullest sense of the critical moment in which the Strategy proposals are being developed. Opportunity abounds to institute significant corrections and reforms to the pervading regulatory and incentive structures with the promise of enormous long-term benefit to cancer patients, and all patients – *if* the moment is well taken.

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RECOMMENDATION 2: REMODEL INCENTIVE STRUCTURES FURTHER TOWARDS VALUE & OUTCOMES; SUPPORT INNOVATION IN ALL AREAS OF TREATMENT AND CARE

The time is overdue to reconsider the incentive frameworks operating in Europe in respect to research, development and access to new treatments as well as the delivery of improvements in the way we detect, diagnose and provide follow up care for cancer and other diseases. In stating that, it should be emphasised that health systems are not currently suffering from a lack of new treatments and innovations coming forward per se. Rather, there are observable difficulties in respect to such matters as ensuring critical unmet needs are met (example, the ongoing need for new antimicrobials) and that reward for innovation is well targeted towards meaningful improvement of outcomes and quality of life for patients.

This targeting of incentive and reward should, as far as possible, apply regardless of the sector from which, and for, an innovation is developed e.g. whether in diagnostics, imaging, surgery, radiation therapy, medical treatment, IT application or other. In other words, we should seek to achieve a more holistic approach to the reward of innovation in health and cancer care, more orientated towards concepts of outcome and value.

Achieving such an ambition should guide the further development of the Pharmaceutical Strategy, targeted consultation activity to follow, as well as other EU initiative.

See also:

- Aapro M, Astier A, Audisio R, et al (2017). Identifying critical steps towards improved access to innovation in cancer care: a European CanCer Organisation position paper. Eur J Cancer. doi:10.1016/j.ejca.2017.04.014
- Lievens Y, Audisio R, Banks I, et al. (2019). Towards an evidence-informed value scale for surgical and radiation oncology: a multi-stakeholder perspective. The Lancet Oncology. doi: 10.1016/S1470-2045(18)30917-3

DRUG REPURPOSING

Within this, we additionally urge careful attention to the opportunities provided by the emergence of a new EU Pharmaceutical Strategy to achieve improvements in the regulatory and incentive framework in respect of drug repurposing.

Drug repurposing, also known as drug repositioning, corresponds to a development strategy predicated on the reuse of existing licensed medicines for new indications. Despite being

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affordable and safe, it is a largely untapped approach for improving clinical treatment options . For example, the Repurposing Drugs in Oncology (ReDO) project, launched by the Anticancer Fund, cites over 300 non-cancer drugs as having shown some evidence of anticancer effects. Of these, 50% are supported by relevant human data and 16% are supported by data from at least one positive clinical trial. Example of initiatives in this regard include ongoing investigations into aspirin for recurrence and survival in colon cancer, and repurposing of an angina pectoris medication as a lung cancer treatment.

However current pharmaceutical regulations principally focus on the development of new medicines, not new indications for existing medicines, and there is a clear lack of EU and national pathways to facilitate drug repurposing. We urge the authors of the new EU Pharmaceutical Strategy not to neglect the particular area of potential improvement in respect of drug repurposing.

RECOMMENDATION 3: RESOLVE THE CURRENT POLITICAL IMPASSE ON THE EU HTA COOPERATION PROPOSAL

More than two years have passed since the European Commission published its legislative proposal for improving the coordination of health technology assessment across EU member states. Provided with wide support from the European Parliament and stakeholders including patient, consumer and payer organisations, health NGOs, academia and industry, its blocked status in Council is a political logjam that must be broken. The longer its passage is delayed, the longer an obvious opportunity for efficiency and greater coordination of access is missed, with patients in need the main losers.

RECOMMENDATION 4: LEVERAGE DATA AND NEW TOOLS IN A MORE COHERENT AND POWERFUL WAY

A DIGITAL AND DATA AMBITION AND VISION WITHIN THE PHARMACEUTICAL STRATEGY

The EU is well placed to help Europe to fully embrace the digital revolution, and give leadership to help ensure such tools result in a new world of highly evidence-based decision-making, including in respect to pharmaceutical regulation and access. The creation of a harmonised European Health Data Space, a post-COVID-19 'Digital Marshall plan', helping to achieve better standardisation of health data quality, collection and interoperability (including with respect to cancer registries in Europe), are all important roles for the EU in this respect. It is encouraging therefore to read the acknowledgement of this in the Roadmap consultation, for example in recording the need for the new EU Pharmaceutical Strategy to be aligned with the EU Digital Strategy.

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We encourage an accompanying digital and data ambition and vision to be well expressed within the Pharmaceutical Strategy.

Promote the use of Patient Reported Outcomes within European regulatory structures.

From a patient perspective, the integration of quality of life measurements as endpoints for clinical trials, involving both psychological and medical aspects, is an increasingly relevant need. In the realm of cancer treatment for example, success is not only related to increasing survival, but also achieving meaningful improvements to a patient's quality of life.

To meet this need, in the past decade there has been sustained development of the regulatory concept of Patient Reported Outcome Measures as a normalising part of the trial landscape. A patient-reported outcome (PRO) is defined as any report of the status of a patient's health condition that comes directly from the patient, without interpretation of the patient's response by a clinician or anyone else.

Patient-reported outcomes usually include information about health-related quality of life, symptoms, function, satisfaction with care or symptoms, adherence to prescribed medications or other therapy, and perceived value of treatment.

Patient Reported Outcome Measures are helping to collect new forms of data that can be used to guide changes in clinical and health policy decisions, to improve treatments, reduce secondary effects, increase workflow efficiency, and enhance patient-physician communication.

In the context of a new emerging EU Pharmaceutical Strategy, any adaptation of the regulatory landscape for the assessment and approval of medicines should seek to support the greater use of patient reported outcome measures in clinical research.

Embrace the Treatment Optimisation agenda

Due to the particular development challenges associated with precision oncology, including the more limited number of potential users, a number of new personalised medicine therapies have been authorised. However, some concern is raised about the more limited knowledge about dosage, sequencing, combination and duration of such treatments. This in turn is raising concern about potential sub-optimal administration, prospective generation of unnecessary toxicity for patients, and negative impact on healthcare budgets. Taken together, this has served to highlight a growing need for clinical, post-market authorisation research that more

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thoroughly investigate the optimal way to use medicines, or other treatments, after they are authorised for use.

To help lead reform in this respect, the European Organisation for Research and Treatment of Cancer (EORTC) has developed a 'Treatment Optimisation Manifesto' addressing these challenges, and which commands broad support from the European cancer community. The manifesto calls for such changes as:

- the generation of treatment optimisation evidence at an earlier stage of a prospective treatment's development, i.e. as soon as the safety and efficacy profiles are known;
- establishing treatment optimisation research as an official and mandatory step in the treatment access path to market; and,
- public funding for treatment optimisation research, to ensure it is free of commercial consideration, including via the EU Cancer Mission and Horizon Research programme.

We recommend the strongest consideration of the recommendations of the Treatment Optimisation Manifesto during the development and finalisation stages of the new EU Pharmaceutical Strategy.

RECOMMENDATION 5: REDUCE BUREAUCRACY IN THE CLINICAL TRIAL LANDSCAPE

Numerous barriers to the conduct of cancer and other health research across Europe are observable and should aim to be tackled within the context of the new EU Pharmaceutical Strategy, for example, in relation to the regulatory environment for pharmaceutical research.

Tackling the GDPR problem

A prominent example in this respect include the concerns expressed by some members of the European cancer research community about the burdens and restrictions on research imposed by the EU's General Data Protection Regulation (GDPR), which came into legal force in 2018. Criticisms include:

- Additional hurdles presented in respect to European participation in global cancer research projects; and
- Barriers imposed on the conduct of secondary analysis due to the interpretation of the regulation's patient consent requirements.

We therefore welcome attention being given by the European Commission to this issue presently, including a very recent Survey assessing the Member States' rules on health data in the light of GDPR.

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All opportunities for ameliorating GDPR's burden on research should be acted upon.

Go further on improving patient access to clinical trials across borders

Further efforts should be conducted at European and national levels to reduce the barriers that prevent cancer patients from accessing innovative treatment across borders, including via clinical trials. In the absence of EU legislation or guidelines to facilitate patients' participation in trials in locations outside their particular country, patients who travel to another country for clinical trials face issues such as the lack of clarity on protocols for follow-up after their return home, and how national insurance covers costs associated with their participation in the trial. The operation and content of the Cross Border Healthcare Directive should be reviewed with the intent of identifying and securing opportunities to increase citizen rights and access to participate in clinical trials across borders.

RECOMMENDATION 6: URGENTLY ADDRESS THE MEDICINES SHORTAGES CRISIS

There is a growing experience of medicines shortage across Europe and the world, which is adversely impacting care across all therapeutic areas including in cancer. Recent investigations of the topic by the European Society of Medical Oncology (ESMO), in collaboration with the Economist Intelligence Unit, evidence that countries large and small, highly resourced and low resourced, are experiencing the real-life daily impacts of the medicines shortage crisis.

In respect to cancer care, delays and interruptions to chemotherapy caused by medicine shortage can be highly distressing for patients, families, carers and healthcare professionals in view of the vital nature of treatment, which in the curative setting is often highly dependent on keeping its dose-intensity stable. Furthermore, cancer medicines affected by shortages often have few or no proven effective alternatives.

Common and well-established cancer medicines being reported as in periodic shortage in Europe include essential drugs, like carboplatin and tamoxifen, and other drugs, such as methotrexate and 5-fluorouracil. These are widely used in the treatment of cervical cancer, head and neck cancer, lung cancer, colorectal cancer and breast cancer.

Healthcare professionals on the frontline of care often cite lack of information about the reason for the shortage, and the expected length of its duration as one of the many frustrations they experience, making it harder to provide accurate information to patients and make robust and timely contingency plans. This comes alongside the lost valuable clinician time that must be rediverted to making alternative treatment plans to manage the shortage situation.

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Against this backdrop, policy recommendations to better prevent and manage medicines shortages in the EU, also expressed by stakeholders at European level, include:

- strengthening of EU pharmaceutical legislation in respect to early notification of forthcoming supply issues;
- improved requirements for marketing authorisation holders to have strong plans in place for the prevention of shortages;
- clearer legal guidance for EU member states in respect to the situations when parallel trade of medicines may be restricted to prevent or manage shortage;
- better arrangements for the sharing of information between countries on medicines in shortage, including publicly; and,
- stronger onus in ensuring prevention of generic medicine shortage by encouraging all EU health systems to tender for generic medicine supply in forms that enable more than one supplier to enter the market.

We urge that the authors of the EU Pharmaceutical Strategy provide the fullest attention to these recommendations on how to tackle medicines shortages.

See also the European Public Health Alliance (EPHA) position paper on medicines shortages (April 2020): https://epha.org/position-medicine-shortages/

RECOMMENDATION 7: WORK WITH MEDICAL AND HEALTHCARE PROFESSIONAL SOCIETIES AND PATIENT ASSOCIATIONS IN CREATING THE BEST POSSIBLE SET OF CHANGES

We welcome the approach of the European Commission in conducting two public consultations in the development phase of forming a new EU Pharmaceutical Strategy. It is important to draw on the perspectives, experiences and ideas of a wide range of interested parties. We wish to emphasise that there should not be a strong sense of hierarchy of stakeholder in this process. Producer and purchaser interest should be heard on an equal level to user interest.

The European Cancer Organisation hosts a network of 31 member organisations and 20 patient organisations touching points of interest in many areas across the diverse spectrum of cancer care. A great many will be providing individual response to the Commission on the points raised in the consultation. We urge ongoing and proactive forms of engagement and outreach to all in the conduct of further consultation activity to ensure the creation of the strongest possible Pharmaceutical Strategy. The opportunity to achieve lasting and significant improvement is too important to do otherwise.

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