



# OECD CONSULTATION: SUSTAINABLE ACCESS TO INNOVATIVE THERAPIES

## Reply from European Cancer Patient Coalition

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### 1. Reflecting on the last 5-10 years, what do you think have been the major changes affecting access to medicines?

The European Cancer Patient Coalition knows that European cancer patients live a paradox today: advances in the understanding of cancer biology have allowed the development of new treatments and medicines, but these treatments and medicines are not equally available to all cancer patients who would benefit from them. Many life-preserving/life-enhancing medicines are relatively inexpensive, yet cancer patients in many European countries are denied access.

There are huge disparities between wealthier and poorer European countries in terms of survival rates. According to EURO CARE 5, in North and Central Europe the survival rate for colorectal cancer is around 60%, whilst the same rate in Eastern Europe shrinks to only 45%. In breast cancer, for instance, there is an 86% chance of survival in Sweden, but only a 66% in Lithuania. The difference in budget allocated to cancer care contributes to the burden of inequalities, as it affects access to medicines. However, survivorship is not correlated directly to spending, but it is true that less spending equals less long-term investments, and in turn also affects access to care. Studies show that countries do not spend in cancer care according to the burden of the disease. Rather, the spending correlates with their economic status.

The profound disparities are also caused by inequalities in access to treatments. Cancer Control plans are inadequately resourced in many European countries. Social and economic deprivation and the influence of recent austerity measures have further exacerbated many of these inequalities. National healthcare budgets rarely match the burden of cancer – even if in some cases the expenditure has increased, it does not match the needed levels of spending and investment in the medium and long term. There are also variations in access to medicines between countries of similar economic status, which suggests room for improvement not only in economic terms, but in terms of efficient policies.

All in all, the uptake rate of new medicines is not as fast as it should be, compared to the rate in which such new medicines are being developed recently. New types of medicines, such as immunotherapies, are only becoming available piecemeal. It is also important to note that other factors affect to the development and approval of new medicines, such as the availability of patients to participate in clinical trials. In this case, the Clinical Trials Directive, in force from 2001 to 2016, was responsible for the dramatic drop of international trials by 25%.



## 2. What are the top three (3) issues that must be addressed to ensure access to innovative medicines while maintaining financial sustainability of health systems?

**First and foremost**, Health Technology Assessments need to be harmonized at the EU level to the greatest extent possible, to speed up the time needed for the assessment and approval of new medicines and to reduce inequalities between countries. Currently, health technology assessments are undergone by EU Member States separately, which creates duplicities in evaluations, suboptimal allocation of resources, disparities among countries, and most importantly great inequalities in the timings: some countries with bigger healthcare systems and regulators cope with the assessments within months, while in other countries such decisions can take up to 10 years or more. Taking into account that HTA should inform pricing and reimbursement decisions, such inequalities entail unnecessary delays and impede timely access for patients to new meaningful medicines. Therefore, the harmonization of joint Health Technology Assessments at the European level can save time and resources, ultimately helping ensure access to innovative medicines to the right patients. Finally, the European Commission should ensure the enforcement of the Transparency Directive, which poses a limit of 180 days for competent bodies to adopt pricing and reimbursement decisions after the approval of new medicines by the EMA.

**Second**, ECPC supports fast track approvals, insofar as transparency on the criteria is ensured. The European Medicines Agency has developed various approaches to help support early access to innovative medicines that address unmet needs, such as the adaptive pathways. This concept of adaptive pathways entails marketing approval for medicines with data gathered from smaller groups of trials, under the condition that more real-world data will be gathered after approval.

We support the system of adaptive pathways if it ensures certain guarantees. The evidence generation needs to make use of real-world data, and patients need to effectively be involved to, *inter alia*, help identify unmet medical needs. Transparency is also a precondition, by ensuring that the main results from clinical trials are timely reported publicly. All in all, these guarantees are aimed at ensuring high standards of safety: safety should not be compromised due to the establishment of adaptive pathways.

Patients should also be involved at the early dialogue stages in the research of new products, whether it is done at EMA level or during health technology assessments.

**Third**, to solve the dire situation of drug pricing, we need new pricing models. Most countries in the EU negotiate a national price using “international reference pricing”, which leads to inefficiencies in the way prices are negotiated, leaving smaller and poorer states with less negotiation leverage and in turn hampering their capacity to access new medicines. As a result of the current system, decisions on pricing and reimbursement are often driven only by financial issues, rather by considerations regarding the overall value of the services brought to the patients. Therefore, we support the introduction of pay-for-outcome schemes, following the principle of outcomes-based pricing: the goal is to reward improved outcomes for patients and healthcare systems rather than volume of usage. Such schemes must collect patient-reported outcome measures, as well as other clinical, economic and legal/ethical information, in order to provide a comprehensive picture of the real impact of drugs within national healthcare systems. Pricing should also be flexible over time, reflecting changes in



assessed outcomes and cost-effectiveness during the lifetime of the medicine in question. At the same time, pricing and reimbursement decisions ought to be transparent.

However, these pay-for-outcome schemes need certain preconditions to be met, in order to ensure their functioning. It requires the establishment of suitable means to collect real-world patient data, through well-functioning and interoperable eHealth infrastructure in all collaborating countries, to create working and useful registries of such data. Collaboration of agencies and governments is essential to achieve this. Luckily, some projects on this issue have already been conducted, such as epSOS project which delivered concrete guidelines on how to establish eHealth systems for the benefit of cancer patients and the redesign of the regulatory framework.



### 3. Why do you think there are issues in ensuring access to innovative medicines while maintaining financial sustainability of health systems?

In cancer care, the main obstacle that prevents access to innovative therapies is the small proportion of healthcare budget dedicated to cancer, which does not correlate with the real burden of the disease in terms of incidence. In 17 out of 28 EU countries, cancer has overtaken cardiovascular disease as the leading cause of premature death. Moreover, cancer confers the second largest disease burden in Europe, accounting for 19% of all disease. However, EU Member States invest only 4-6% of their healthcare budgets on cancer. This proportion does not reflect the major and rising contribution of cancer to the total disease burden.

The unacceptable imbalance between the total disease burden and the amount of budget allocated for its care is further exacerbated between the differences among EU Member States, which is reflected in inequalities in survival outcomes. Moreover, the disease burden of cancer is expected to increase in the upcoming years, thus it is imperative to find approaches to match the required spending without compromising financial sustainability.

Several EU stakeholders (e.g. ESMO) have reported that the high prices of innovative cancer medicines are a principal barrier to access, which was reflected in the Council of the EU Conclusions of 17 June 2016 on 'strengthening the balance in the pharmaceutical systems in the EU and its Member States'. As we have said in the previous questions, the regulatory framework for approval, pricing and reimbursement of medicines is suboptimal. Current procedures in Europe pose administrative barriers to innovation, with undue delays and inefficiencies.

It is very important to understand, however, that the economic burden of cancer care is not only comprised of medicines. The total cost of cancer care is 126 billion euros per year only in Europe, and 60% of that spending is on indirect costs (early death, disability, lost working days and informal care). Thus, 40% of cancer care are direct costs. The point is that among those direct costs, only 23% is spending on medicines, and the rest is comprised of other treatments and inpatient hospital care. If inpatient hospital care can be made more efficient (with, for instance, better mHealth technologies), then more resources will be allocated for reimbursement of medicines and other treatments.

It is of outmost importance, therefore, to evaluate the performance of health systems (including that of cancer patient pathways) in a reliable, comprehensive manner, to identify inefficiencies and waste that consume resources that can otherwise be redirected to accessing innovative medicines. This also links with the need to inform disinvestment efforts. Disinvestment specifically refers to resource allocation decisions based on withdrawing funding from no or low added-value health interventions, freeing up these resources for reinvestment in other health technologies that meet the criteria of safe and cost-effective care.

Moreover, patients are not involved in R&D and approval of medicines, which hampers the possibilities for gathering valuable information on the effectiveness and need of different medicines. Therefore, patients should be routinely involved in further collaborations with regulators, academia and industry in the design and operation of regulatory models for innovative cancer medicines.



#### 4. What changes would you like to see happen to improve access to innovative therapies?

A basic measure to improve access and guarantee sustainability is to better evaluate the performance of the healthcare systems. The question of access to innovation is deeply intertwined with the sustainability of healthcare systems. Healthcare systems will be able to provide access to critical, meaningful therapies if there are efficient procedures that ensure sustainability of the systems.

In the EU, this can be done through timely and comprehensive recommendations from European Semester. It is therefore crucial to enhance the work of the European Semester and make sure that the EU governments have proper guidance from these recommendations, to make healthcare sustainable and allow for innovations.

To improve the available information of the performance of healthcare systems, cancer registries can be crucial to collect of real-world data on disease burden, the effectiveness, safety and cost-effectiveness of innovative treatments, service provision and care quality. Data from cancer registries can inform patient care and healthcare planning, as well as providing data for public health policy and research. Cancer registries ought to be linked and interoperable, through harmonization of data quality and collection methods.

Interoperability should also refer to eHealth infrastructures as a whole, which allows for the establishment of innovative pricing and reimbursement methods. The final objective is to allow pay-for-outcome schemes, to reward improved outcomes for patients and healthcare systems rather than volume of usage.

Health Technology Assessments shall routinely inform reimbursement decisions; therefore, it is also crucial to fully harmonize joint HTA at the EU level, or subsidiarily enable a complete exchange of information between agencies. Otherwise, current inequalities will keep entailing unnecessary delays and impede timely access for patients to new life-preserving or life-enhancing medicines.

Access to innovation is not only related to medicines, but also to many other health technologies. In cancer, care is inherently multidisciplinary, and comprises other questions such as innovative diagnostics (i.e. biomarkers), innovative radiation oncology and innovative surgery. Better literacy among healthcare professionals is also needed to guarantee proper radiation oncology and surgery. At the same time, innovative diagnostics, which are increasingly personalized, need to be reimbursed to the greatest extent.

At the same time, any effort at national and European level to better assess the economic, clinical, societal and ethical/legal value of existing cancer care services should take into account the potential effect of disinvestment policies.

Cancer is a complex disease, and the best way to treat it is in a multidisciplinary way, making optimal use of all therapeutic modalities. There are already existing sets of clinical recommendations and best care models proposed, which need to be urgently implemented. The best example is European Commission-led Joint Action on Cancer Control (CanCon), formed by authorities of the EU Member States, which produced a European Guide on Quality Improvement in Comprehensive Cancer Control and several policy papers. We urge for the full uptake and implementation of these recommendations.

Last, we demand that patients are involved in all steps of decision making, to better communicate their needs and better inform policymakers of the existence of such recommendations.