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Directorate-General for Internal Policies
Special Committee on Beating Cancer

SUMMARY

BECA Hearing
"Mind the gap: For equal access to cancer medicines and treatments"
Thursday 28 January 2021, 13:45 to 16:15 & 16:45 to 18:45

(Jósef Antall 4Q2 and with remote participation)

In the Chair: Bartosz ARŁUKOWICZ, Chair

The aim of the hearing was to look into the causes of unequal and delayed access to cancer drugs and treatment, and ways to create an enabling environment for optimising treatment and access in health care systems, including for rare and paediatric cancers.

In **Panel 1** - with the title "**Research and innovation, cancer drug development, multidisciplinarity of cancer care and authorisation**" - the following three expert speakers took the floor:

- **Emer Cooke**, Executive Director of the European Medicines Agency (EMA);
- **Prof. Maciej Krzakowski**, Chairman of the Polish National Council for Oncology at the Ministry of Health;
- **Dr Denis Lacombe**, Director-General of the European Organisation for Research and Treatment of Cancer.

In **Panel 2** - with the title "**Pricing and affordability of cancer treatment**" - spoke the following three expert speakers:

- **Prof. Thierry Philip**, President of Institut Curie, President of the Organisation of European Cancer Institutes;
- **Dr Ward Rommel**, Chair of the 'Access to Medicines' Task Force of the Association of European Cancer Leagues;
- **Dr Vlad Mixich**, Executive Director of the Romanian Health Observatory.

The expert speakers addressed the following **main issues:**

Availability of anti-cancer medicines in the EU and medicine shortages

There are large variations in the rate of anti-cancer drugs availability in Europe. Supply shortcomings are recurring. Moreover, there are delays in reimbursement. Disparities in access of cancer patients to cancer medicines and treatment are most significant in Eastern European Member States.

To remedy this situation, **the EU should undertake initiatives** in relation to a number of factors that influence the availability and accessibility of anti-cancer medicines, namely: **manufacturing, external pricing, Health Technology Assessments (HTA), internal pricing, and parallel trade** (this is when national wholesalers sell a medicinal product outside their country to obtain a better price).

The following measures were recommended:

- **Revising the EU pricing methodology**; although pricing and reimbursement decisions remain within the competence of Member States, there are many opportunities for the European Medicines Agency (EMA), Member States and developers to cooperate more at the EU level in order to increase access to cancer medicines;
- **Data transparency**: discontinuation or withdrawal of a medicinal product should be notified at least 1 year in advance;
- **Monitoring of available stocks**, at least at regional level;
- **Establishing alert thresholds** to identify risk of shortages for specific products;
- **Notification** of quantities and destination of **parallel traded products;**
- Setting out **special rules for essential medicines;**
- **Establishing a European Health Technology Assessment,** to speed-up the decisions;
- **Rules enforcement**.

Furthermore, the experts suggested the **following actions**:

- Ensuring **increased transparency** of the industry in relation to **supply chains** of anti-cancer medicines;
- Launching a **structured dialogue to identify vulnerabilities in the global supply chain** of anti-cancer medicines;
- **Addressing in legislation the market effects** impacting on affordability of cancer care;
- **Revising the system of incentives and obligations in legislation** to support innovation, access and affordability. National and EU public funding should be subject to conditionality. Public and private R&D should be stimulated and steered towards areas with limited treatment options, low survival and/or low commercial interest, such as rare and childhood cancers;
- Implementing **innovative payment models**;
- Enabling a proportionate and appropriate **Cross-Border Healthcare**;
- Establishing an **EU Health Emergency Response Authority**;
- Improving access to **generic and biosimilar medicines.**

Expert speakers also addressed the current **delays between the approval of new cancer medicines by the European Medicines Agency (EMA) and the national access to those medicines.** Such delays vary significantly between Member States: Eastern European Member

States are those where delays are the longest (to compare: average delay in Germany is 119 days; the EU average is 445 days; in Romania it is 775 days).

To decrease the time lag, as well as the differences between authorisation decisions by regulators and access decisions through HTA across Europe, efficient communication is needed, starting already during the phase of clinical research. Multi-stakeholder discussions on development plans is a key component to foster generation of evidence that is needed for decision making. The recently published "**European Medicines Authorities Network strategy to 2025**" has dedicated a specific chapter to access and availability. Since 2010, the EMA and **EUnetHTA, the European network for Health Technology Assessment**, have collaborated to improve the efficiency of the processes and mutual understanding of evidence needs, pursuing the goal of "one evidence generation plan for different decision makers". The proposed **Health technology assessment (HTA) Regulation**, which was presented by the Commission in 2018 and discussed in the European Parliament in 2019, will be instrumental to strengthen the legal basis and procedural rules for EMA to continue its long-standing cooperation with HTA bodies and, in that way, help reduce delays and disparities in access to medicines.

Pricing - the need for transparency

Fair pricing, affordability and sustainability of health systems should become a core element of EU pharmaceutical and cancer policy:

Existing structures (e.g. EURIPID database) should be expanded to share information on net prices of medicines and strive towards the full implementation of the **2019 World Health Assembly Resolution on price transparency** (World Health Assembly (WHA) is the decision-making body of the World Health Organisation).

A **High-Level Working Group on fair pricing** should be established; it would be facilitated by the European Commission and would connect relevant stakeholders to define a fair price and identify opportunities and challenges connected to different pricing models.

Urgent need for effective and affordable anti-cancer treatments

Expert speakers recommended the following actions:

- Establishing **private-public multi-stakeholder forums** (including industry, regulatory bodies, academia, patient advocacy groups) for research and innovation with an international scope to set research priorities;
- Developing new models of **partnership between commercial and non-commercial research**;
- Involving **academia and NGOs in drug development support**;
- **Training academia and civil society in regulatory science** for better translation of research into product development;
- Identifying most appropriate treatment pathways through **genomic profiling**;
- Adopting legislation providing for **cross-border participation in clinical trials**.

Transparency of decisions and sharing of information

Cooperation and dialogue is necessary between regulators, payers, governments, patients, health care providers, and industry.

Heath care should become **more patient centric** in the following ways:

- Methodologies should be modernised to **include patients' views in the benefit-risk assessment,** and to improve the communication of benefit-risk decisions to patients and doctors;
- **Datasets should be generated**, such as comparative evidence aiming at documenting the optimal treatment for cancer patients through the integration of clinical research -free of commercial interest- into the process of access to treatments, and inform healthcare systems.

Fostering the development of innovative cancer treatments for patients in need

Pharmaceutical legislation and regulatory practices provide a number of tools to incentivise innovation and breakthrough treatments in areas of high unmet need. For instance, shorter review times are foreseen for medicines of major interest for public health, particularly from the point of view of therapeutic innovation. The **European Medicines Agency (EMA**) has also developed the **"PRIME" scheme** to provide intensive support to so-**called priority medicines** during development in the form of in-depth scientific advice. **Orphan designation of drugs intended for rare diseases,** that are considered to be of significant benefit, also allows EMA to provide additional targeted support to developers. Conditional marketing authorisation can be granted when preliminary clinical evidence shows a positive benefit/risk for new medicines that address an unmet medical need or represent a major contribution to patient care.

The **EMA set of anticancer guidelines** are also a key reference for supporting efficient cancer drug development, and they undergo regular updating to reflect the latest experience and support more efficient research efforts. Similar tools and incentives are available to support further development of approved cancer medicines to target important new indications.

The need to optimise clinical trials

Currently, the **majority of clinical trials are conducted in less than a handful of EU Member States** and often in only a few centres.

It is necessary to design clinical trials that are fit for purpose from a patient perspective, ensuring that patient views are systematically considered throughout the development process. Patients expect to have the option to access clinical trials locally. There is an opportunity to bring trials closer to EU patients by promoting and enlarging the development of competences and trial readiness across Europe. EMA and other regulators are pursuing this goal through the renovation of the fundamental "**Good Clinical Practice guidelines" for clinical trials** in the context of the **International Council for Harmonisation.**

The EU has the opportunity to develop a "**learning health care system**" that can **maximise data generation** by federating data from different sources, like electronic health records and cancer registries. EMA is collaborating with the European Commission to create a **European Health Data Space** over the next years, contributing in particular through EMA's **DARWIN initiative.**

In the **Q&A sessions** that followed after Panels 1 and 2, BECA Members addressed a variety of topics, notably:

How to improve clinical trials, including clinical trials involving children; measures needed to cut down bureaucracy in the authorisation of cancer treatments in Member States; the impact of the COVID-19 pandemic on cancer research and the development of cancer medicines; precision medicine/oncology and treatment optimisation; insufficient innovation in the EU and

the need to reinvent partnerships; the need to encourage research without enabling the manipulation of health systems; the importance of quick vaccination of cancer patients against COVID-19; the need to establish an inequality registry; shortages of cancer medicines as an evidence of market failure; how to fix medicine prices more equitably; the need for joint procurement at EU level for orphan medicines; joint procurement in the case of COVID-19 vaccines can serve a model for cancer medicines.

In their **replies,** the expert speakers pointed out, among others, the following:

Fair pricing for cancer medicines requires more coordination at EU level; joint procurement of cancer medicines increases negotiation power and, thus, it is a means to address market failures; parallel trade is illegal; data transparency is paramount; all patients should have access to reference networks; the role of academic researchers should be reinforced and they should be trained in regulatory aspects.

Taking the floor, the **BECA Chair** pointed to the radical changes as a result of the pandemic; namely, the **joint procurement of vaccines shows the way for the procurement of cancer medicines.**

The DG SANTE Director **Andrzej Ryś** took the floor next, pointing out that **public procurement policies and affordability of cancer treatments form a part of the new Pharmaceutical strategy** of the European Commission. Talking of the importance of data collection, Director Ryś pointed to the project of the **European Health Data Space** that will be presented soon. He also referred to **cross-border clinical trials** as an issue addressed by the **Regulation on Clinical Trials.**

Taking the floor, the BECA Rapporteur **Véronique Trillet-Lenoir (RENEW, FR)** stressed the urgency of **adopting EU legislation to improve the affordability of anti-cancer drugs**. Access to generic medicinal products is very important, whereas a virtuous circle can be initiated through the **joint assessment of cancer medicines**, which is the object of the **proposed Health Technology Assessment (HTA) Regulation**. Everybody should have access to standard treatments in properly accredited health centres. Collective purchase programmes, such as in the case of COVID-19 vaccines, have shown what can be achieved by collective action; health nationalism should be avoided.

The second part of the hearing started with **Panel 3**, which focused on "**Rare cancers and paediatric cancers as regards access to treatments and clinical trials**". Two expert speakers took the floor:

- **Prof. Ruth Ladenstein**, Board Member of the European Society of Paediatric Oncology (SIOPE), and ERN PaedCan Coordinator;

- **Dr Kateřina Kopečková**, Oncologist at Charles University and University Hospital in Motol, Czech Republic.

Expert speakers made the following main points:

<u>Paediatric cancers as a major health and socio-economic burden</u>

- Paediatric cancers are the first cause of death by disease in children older than 1 year;
- 35,000 cases are diagnosed annually and more than 6,000 young patients die each year;

- There are more than 500,000 survivors; at least 60% of them experience adverse late-effects in adulthood;
- All individual paediatric cancer types are rare (incidence <6/100,000/year);
- There is a **significant lack of innovation in medicines for specific paediatric cancers, as compared with medicines for adult cancers:** only nine anti-cancer medicines have been authorised for a specific paediatric cancer indication in Europe since 2007, in contrast to over 150 for adult cancers.

Mission of the **European Reference Network in Paediatric Cancer** (ERN PaedCan):

- **Reduction of inequalities of childhood cancer outcomes across Member States:** enabling access to up to date diagnostics and treatments by facilitating the exchange of expertise and knowledge;
- **Implementation of Virtual Tumour Boards**: medical expertise and knowledge travels rather than travels of patients;
- **Proportionate and appropriate Cross-Border Healthcare:** to unite the best specialists across Europe to tackle complex or rare paediatric cancer conditions that require highly specialized interventions and a concentration of knowledge and resources.

<u>Paediatric cancers in Europe - needed actions to address the inequalities</u>

Improved treatments through policy and legislative actions:

- Facilitating and accelerating **therapeutic innovations in a new regulatory environment**;
- **Private–Public Multi-stakeholder Forums** (industry, regulatory bodies, academia, advocacy groups) for research and innovation with an international scope to set research priorities;
- Dedicated **international academic research platforms for innovative medicines for children and adolescents,** cross-linked and informed by adult cancer and basic research on paediatric malignancies;
- **Enhanced access to early trials** across the childhood cancer spectrum ensuring access to innovation in all Member States, including cross-border access;
- Ensuring equal access to diagnosis and therapeutic measures through **harmonised standards across Europe** for all paediatric cancer entities: Recognition of the forthcoming **European Paediatric Cancer Standard Practice Guidelines (ESCP)** by national health authorities to establish common understanding in relation to direct Cross-border Health Care;
- All newly diagnosed children and adolescent patients must be **presented to multidisciplinary tumour boards** in Member States **as a legal requirement** locally, nationally or cross-border;
- Ensuring access to **essential anticancer and supportive care medicines used in childhood cancer treatments** across Europe, alongside with adequate pricing and reimbursement, to secure timely access to essential medicines as defined by the SIOPE essential medicine list;
- Fostering **systematic registration** and **cross-linkage of databases** to capture outcomes, including moderate and severe long-term side effects of cancer treatments during young age.

Improved Quality of Life through Policy and Legislative Actions:

- **Survivorship Passport** (for childhood cancer) to enable health care providers supported by digital tools to deliver **appropriate, patient centered life-long follow-up care** and support in particular transition into adult care settings, as well as **surveillance of long-term side effects including the psychosocial dimension**, **cognitive or learning problems** as well as secondary cancers;
- **Shared decision making** at all levels with **affected young people and their families** in any care decisions;
- **Regulation on children's rights and carers' protection** (social protection and employment security) during severe illness and end-stage disease implemented in all Member States;
- A "**Right to be forgotten' regulation** should be adopted by all Member States.

Further actions needed to address inequalities:

- Sustainability of **European Cancer specific Reference Networks (ERNs)** to full potential and **integration in national health systems;**
- Foster the realisation of **patients' rights to cross-border care** and rules on reimbursement through:
    - the **Cross-Border Healthcare Directive** and
    - the **S2 mechanism** of the EU **Regulation on Coordination of Social Security Systems;**
- Enable faster and more efficient development of **innovative medicines** for children with cancer in relation to the **EU Paediatric Regulation** and ongoing inception **roadmap for its revision**;
- Adopt measures to counter **shortages of essential anticancer medicines** and foster availability of child-friendly formulations;
- Foresee appropriate **pricing and reimbursement strategies** for newly approved expensive medicines gradually becoming available for the paediatric population;
- Recognise **early clinical trial access for children with poor prognosis** as a standard of care.

Rare Cancers - Rare diseases

"Rare cancers - rare diseases" are those with a prevalence of less than 50 out of 100 000. Rare tumours represent 22% of all cases of cancer, with a cancer mortality of 52%.

The following actions were suggested as regards rare cancers:

- **Discussing the EU Regulation on Orphan Medicinal Products:** today there are more rare tumours from non-rare conditions, and there are more orphan medicinal products. It is the time of personalised medicine. Hence, the methodology of clinical trials has to be revised;
- **Amending the EU Regulation on Orphan Medicinal Products**: evaluation should not only be based on HTA&cost-effectiveness;
- **Genetic testing at the time of diagnosis** to find targetable alterations in the time of personalised medicine: find the proper patient for the proper treatment;

- Create an easily accessible **database of clinical trials in the EU**;
- Promote the **awareness of European Reference Networks,** and the implementation into national health care systems;
- **Horizon Europe Cancer Mission**: its published recommendations should be included into national cancer plans.

In the ensuing **Q&A session**, Members raised questions on various issues, namely:
Access to specialised Artificial Intelligence centres for paediatric cancers, and the main challenges of such centres; how to improve cross-border healthcare and the access to specialised reference centres despite persisting bureaucratic obstacles; the need to improve the treatment of children tumours through the involvement of European Reference Networks (ERNs); availability of impact assessments on ERNs; availability of specific studies into the causes of the increase in paediatric cancers (e.g., genetic factors; potential role of pesticides and endocrine disruptors); the need to ban carcinogenic substances such as certain food packages.

Expert speakers replied included the following:
There is some progress with Member States integrating more standard practices developed in ERNs but there is still room for improvement; awareness of new technologies and ERNs should increase, as everything goes down to the organisation of national health systems.
Willingness is here but budget resources are still scarce; EU involvement and financing is needed to improve cross-border health care and clinical trials; EU legislation does not leave enough room for early cross-border trials in children; however, clinical trials have improved, with many new drugs coming out of them.
As regards causes of cancer in children, predisposition is important but some cancers are also virus-associated; there are many factors involved.
Industry is still lacking interest in research and clinical trials with children patients, though the need for more innovative paediatric drugs is great; the creation of working groups carrying out better designed HTAs would be helpful; quality of life of patients should also be evaluated in HTAs.

Director Andrzej Ryś pointed out that the new pharmaceutical strategy is progressing well, with more evaluations of anti-cancer drugs to increase availability. Cross-border training programmes for specialists are being implemented. The Directive on cross-border health care is being reviewed and the first roadmap will be published soon; the Directive is interlinked with some other legislation, such as the Regulation on social security. Moreover, the revision of both the Regulation on Orphan Medicinal Products and the Paediatric Regulation will be ready by the end of 2021.

Panel 4 concerning "**Social determinants in accessing cancer care**" consisted of two expert speakers:

- **Prof. Mark Lawler**, Board Member European Cancer Organisation, Member of the European Cancer Patient Coalition Scientific Committee, professor at Queen's University Belfast;

- **Kathi Apostolidis**, Board Member of the European Cancer Patient Coalition.

The expert speakers focused on the following:

East-West Divide in cancer care:

- Hungary, Estonia, Croatia and Romania are the EU Member States with **the highest death rate per cancer;**
- Whereas, at global level, 81 countries have an **operational cancer plan**, more than half (56%) of **Central and Eastern European (CEE) Member States** do not have national cancer control plans;
- **Total healthcare spending** falls below the EU average in all CEE Member States.

The following can serve as **critical enablers** for CEE Member States:

- Robust **cancer intelligence**;
- Resilient **National Cancer Control Plans** (NCCPs);
- **Patient-centred multidisciplinary teams**;
- A research and innovation empowered culture.

The Critical Importance of Research for the Cancer Control Effort:

- Delivering a Cancer *Groundshot*: Identifying **Future Cancer Research Priorities** for Europe;
- **Identify the critical research gaps** which, if filled, would deliver the best solutions for improved cancer control;
- Deploy this evidence to inform a Cancer Groundshot that **prioritises a patient-centred Cancer Control effort** which delivers optimal solutions for enhanced, equitable, and affordable cancer outcomes across Europe;
- Call to **Action for all of Europe**, with an **emphasis on Eastern Europe**;
- Cancer patients **treated in research-active hospitals have better outcomes** than those who are not.

Cancer Country Dashboards

Cancer Country Dashboards can serve as "**Intelligence to Inform Policy Change**" in the following ways:

- Developing a **cancer "learning environment**" that benefits and informs cancer policy in CEE countries by highlighting key challenges and inequalities across the region;
- Ensuring that **evidence becomes an effective enabler** of action for stakeholders;
- Leveraging **key cancer policy indicators** to measure progress over time and provide a benchmark for best practice sharing across the region.

Actions to undertake without delay:

- Ensure widespread adoption of the **European Code of Cancer Practice**;
- Collect and deploy **intelligence on cancer control/cancer research activities**, illuminating the path to take that is best for our citizens and patients;
- Deliver **research-enabled cancer care** to ensure best outcomes for cancer patients;
- Develop a **cancer "learning environment**" to benefit the entire CEE region;
- Embed **Cancer country dashboards** to capture key intelligence, underpinning cancer policy decision-making and monitoring progress, locally, nationally and regionally;
- Facilitate **dissemination of citizen and patient-focussed information** to enhance opportunities for better health and wellbeing.

A picture of social disparities in Europe

- **Long waiting times for disability assessment** in many countries. Effects of cancer and treatments not considered as an additional element;
- **Lack of palliative care and hospice care** in many countries; patients and families pay out of pocket for these services;
- **Widely differing employment rights** across Europe: variable access to **paid sick leave,** workplace adjustments**, lack of protection from unfair dismissal**. No incentives or support to facilitate cancer patients and survivors to **return to work** or find new work;
- **Self-employed workers** have no or very limited **financial support**;
- Only half of the participating countries foresee for **pension contributions**, missed as a result of illness;
- Access to **health and life insurance, loans and mortgages** often restricted or more expensive as a result of a cancer diagnosis.

Recommendations:

- Faster **access to assessment** for disability benefits, and assessment criteria that recognise the **specific challenges of patients with cancer and of those who care for them;**
- Improve **harmonisation** across Europe with respect to **employment rights of patients and their carers,** and effective enforcement of legislation protecting cancer patients from unfair dismissal;
- **Compensate** for periods of ill-health resulting in **gaps in pension contributions** either due to illness or caring for a family member;
- Regulatory changes to ensure that **insurance, loan and mortgage providers** do not unfairly **discriminate against cancer survivors**;
- **Widespread cultural change** concerning the rights and social rehabilitation of cancer patients and family carers.

In the **Q&A session** that followed, Members raised questions on issues such as:

Reasons for the backlog in research: is it the lack of well-identified research priorities or lack of funding? How to tackle the health gap in screening programmes and prevention as regards people living in poverty, who generally avoid participating in such programmes; similarly, for other vulnerable groups such as the Roma community. Lack of access to medicines and diagnostics leave to more deaths in the CEE Member States but also in remote regions. Are there any data available about screening programmes that were suspended because of the pressure on health systems due to COVID-19?

The replies given to Members' questions included the following:

In some cases, insufficient levels of research have indeed more to do with lack of prioritisation than with lack of funding. As regards lack of treatment in disadvantaged regions, mobile teams of specialists have offered treatment on the spot in some cases; such a "partnership approach", including training opportunities for doctors and patients in disadvantaged regions, can offer some relief. As regards CEE countries, the brain drain of specialists is the most important problem; support is urgently needed to help them stay in their own countries. The Beating Cancer Plan is expected to offer support for solving a series of problems.

Throughout the hearing, several expert speakers and BECA Members noted that, as measures taken for the COVID-19 pandemic have shown, structured cooperation is key to achieving better health outcomes; this should serve as a model for future cancer care.

Rapporteur **Véronique Trillet-Lenoir (RENEW, FR)** took the floor last. She stressed that inequalities are found in all stages of cancer care, including studies, and they are even stronger when it comes to paediatric and rare cancers. As an example, she pointed out the case of new anti-cancer medicines developed since the old pharmaceutical strategy (2007): only a very small percentage of children patients had access to those new medicines.
The rapporteur stressed that with the Beating Cancer Plan, and along with the new pharmaceutical strategy, the effort will be to facilitate the access to more orphan medicines, reduce waiting times, achieve fair pricing and resolve the problem of shortages, as well as reinforce the budget for ERNs for rare diseases. Moreover, it is necessary to fully implement, when it comes to children patients, both the Directive on cross-border health care and the Clinical Trials Regulation.

The Chair concluded the meeting pointing out that it has been a dynamic hearing and underlining the urgency of eradicating all inequalities in cancer care.

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and https://multimedia.europarl.europa.eu/en/committee-on-beating-cancer_20210128-1645-COMMITTEE-BECA_vd