RARE CANCERS EUROPE

Joining forces for action

RARE CANCERS IN ALL POLICIES

THE EUROPEAN PARLIAMENT, THE EUROPEAN COMMISSION AND THE COUNCIL OF THE EUROPEAN UNION,

- Having regard to Europe's Beating Cancer Plan
- Having regard to the establishment of the Conquering Cancer: Mission Possible
- Having regard to the EU Joint Action on Rare Cancers and the Rare Cancer Agenda 2030
- Having regard to Directive 2011/24/EU of the European Parliament and of the Council of 9 March 2011 on the application of patients' rights in cross-border healthcare, the Special Report N°7/2019 from the European Court of Auditors "EU actions for cross-border healthcare: significant ambitions but improved management required", and the European Parliament resolution of 12 February 2019 on the implementation of the Cross-Border Healthcare Directive (2018/2108(INI))
- Having regard to the Orphan Medicinal Products and the Paediatric Regulations
- Having regard to the EU Research and Innovation Programme (2021-27) and Horizon Europe
- Having regard to EU4Health and the European Semester Programmes
- Having regard to the Communication from the Commission to the European Parliament, the Council, the European Economic and Social Committee and the Committee of the Regions a European Strategy for Data
- Having regard to the Regulation (EU) 2016/679 of the European Parliament and of the Council of 27 April 2016 on the protection of natural persons with regard to the processing of personal data and on the free movement of such data, and repealing Directive 95/46/EC (General Data Protection Regulation)
- Having regard to the Regulation (EU) 536/2014 of the European Parliament and of the Council of 16 April 2014 on clinical trials on medicinal products for human use, and repealing Directive 2001/20/EC (Clinical Trials Regulation)
- Having regard to the establishment of the European Union's Erasmus+ programme in the fields of education and training
- Having regard to the Proposal for a Regulation of the European Parliament and of the Council on health technology assessment and amending Directive 2011/24/EU
- Having regard to the Communication from the Commission to the European Parliament, the Council, the European Economic and Social Committee and the Committee of the Regions Pharmaceutical Strategy for Europe
- Having regard to the Recommendations from the Rare 2030 foresight study, initiated by the European Parliament and co-funded by the European Commission Pilot Project and Preparatory Actions Programme

WHEREAS:

1. 24% of all new cancer diagnoses, including all paediatric cancers, across Europe each year are rare forms of the disease. With 650,000 new rare cancer diagnoses annually, and around 5.1 million people in the EU living with a rare cancer, rare cancers are not so rare. Whereas, patients with rare cancers face challenges linked to a late or incorrect diagnosis, lack of access to appropriate therapies and expertise, lack of understanding of underlying science, lack of commercial interest in developing new therapies, difficulties in conducting well-powered clinical studies, feelings of isolation, and few available registries and tissue banks. Whereas, patients with childhood tumours, face specific unmet needs distinct to the paediatric community.

2. Rare cancers, including all paediatric cancers, are defined as those with an annual incidence of less than six per 100 000 people in the European Union (EU).

3. Cross-border medical cooperation needs to be strengthened to reach higher standards of care. Whereas, the European Reference Networks (ERNs) are one of the most important EU initiatives for rare cancers and rare diseases, they still face significant challenges to ensure they are financially sustainable and are able to operate effectively within and across national healthcare systems. Whereas, there is a need for funding allocated to the ERNs and to the appointed national cancer institutes, including national healthcare providers, to create a robust networking system to ensure cross-country cooperation on health in Europe.

4. National networks of expert healthcare professionals, complementing the European Reference Networks (ERNs), are crucial for the success of ERNs, especially for adult rare cancers. Given the rarity of the cancers, it is important to increase awareness of rare cancers amongst primary and secondary healthcare professionals and establish well-functioning and interconnected networks at each Member State level, together with patient organisations, which would be connected to the umbrella rare cancer ERNs. This is essential and applies to the concept of the planned EU Network of National Comprehensive Centres, to be established under Europe's Beating Cancer Plan. Whereas, for the treatment of children and adolescents with cancer national clinical networks are already well established in the majority of European countries, interlinked with the ERN PaedCan.

5. Multistakeholder partnerships bringing together the entire rare cancer community including patientorganisations, scientificsocieties, academia, healthcare providers,education providers,and industry, such as Rare Cancers Europe are vital for representing a unified voice of the rare cancer community, and should be involved in the policymaking processes. Whereas, the voice of rare cancer patient organisations in policymaking processes is crucial.

6. The importance of harmonised, interoperable digital platforms to track, share, and discuss cases in a secure and sustainable manner, is vital for rare cancer and rare disease patients. Whereas, the current Clinical Patient Management System (CPMS) of the ERNs is not fully tailored to the needs of the specific ERNs and does not allow for cases to be shared/discussed in a user-friendly manner integrated into the daily cross-border patient care. Thus, it would be crucial to adapt and customise the IT infrastructure, including the CPMS, to create a system that is cross-functional across ERNs, taking into account the various uses of data being collected by ERNs (e.g. linking diagnostics and treatment data), but also linked to the national networks, especially for rare cancers.

7. The ERNs are responsible for creating guidance documents to treat rare cancer and rare disease patients. Whereas, it is vital to acknowledge that learned scientific societies have created guidelines, through robust, consensus-driven processes in the oncology area; and whereas EU Member States need to develop a mechanism to recognise and adopt at national level clinical guidelines developed or reviewed by ERNs.

8. Population-based cancer registries should be complemented by clinical registries linked to ERNs on rare cancers with the aim to improve patients' diagnosis with a special view to ultra-rare cancers. Whereas, there is a need to collect and harmonise clinically relevant data e.g., on detection, staging, treatment, to improve early detection and diagnosis of rare cancer patients. Thus, proper funding should be allocated to the development of such registries, while the potential threat to their existence posed by the fragmented implementation of the General Data Protection Regulation (GDPR) should be constructively addressed, in collaboration with the whole European cancer community.

9. Treatment of rare cancers and rare diseases is dependent upon the education and professional competence of the treating physician. Whereas for solid adult rare cancers this is especially difficult given the lack of cases and centres, and physicians with enough expertise. To ensure the optimal quality of cancer care is being delivered to patients, it would be crucial to ensure specific educational pathways corresponding to specific medical careers. The latter should be linked to the ERNs and done in cooperation with the EU university system and national postgraduate medical educational bodies. Whereas, educational programmes targeting patients, carers and patient representatives should also be supported, in conjunction with ERNs, to increase levels of health literacy in these populations, and ultimately help patients and their families make informed choices about treatment options and follow-up care.

10. Reducing the inequalities within EU healthcare systems, and discrepancies between the approval and access to medicines, is crucial. For rare cancers, the challenge is tremendous due to the rarity of cancers. Whereas, rare cancer patients have the right to receive the highest quality of care and treatments, regardless of where they live. Thus, the mandatory uptake of joint clinical assessments under the draft HTA regulation should be considered, to provide equal access to optimal cancer care to all patients.

11. The peculiarities of rare cancers compared with other rare diseases and the distinct specificities of childhood cancers have been clearly demonstrated in past EU-funded actions and projects (e.g. ERNs, Joint Actions). Initiatives such as the Joint Action on Rare Cancers (JARC) are testament to the need of having specific projects focusing on tackling rare adult cancers and dedicated initiatives for childhood cancers.

12. For rare cancers, it is difficult to generate new evidence and allow new agents to be available to patients as soon as possible. Whereas, flexible licensing approaches such as the European Medicines Agency's pilot on adaptive licensing leading to pan-European new evidence generation, as long as a new agent is already made available under strict conditions, might be an innovative solution for rare cancers. This could ensure the collection of robust data about new agents along with their availability within networks with medical expertise, e.g. ERNs, and the ability to generate new knowledge under a degree of control by national health systems.

13. There is a lack of biomarkers and limited access to biomolecular technologies for rare cancers, which are crucial to implement precision medicine: from early detection to an accurate diagnosis, and treatment. Whereas, in this context, genetic tumour syndromes are disorders in which genetic mutations may predispose individuals to develop tumours, sometimes with childhood onset.

14. Improving the quality of life for cancer patients, survivors and carers is one of the main pillars of Europe's Beating Cancer Plan. Whereas, patients affected by a rare cancer need to receive adequate psychological support, rehabilitation and monitoring of long-term side effects of treatments by professionals who understand their rare disease and the specificities linked to it. All patients with rare cancers should also be provided with a survivorship care plan. Whereas, carers for rare cancer patients (often parents or partners) also need access to specific psychosocial support to cope with the severity and complexity of the disease, and the significant burden of care which they take on.

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RARE CANCERS EUROPE: CALL TO ACTION

1. Dedicate research funding to rare cancers, which represent 24% of all new cancer cases, including rare adult solid cancers, haematological cancers and paediatric cancers (which are the leading cause of death by disease among children and adolescents in Europe) under the Mission on Cancer, including under UNCAN.eu – the European Initiative to Understand Cancer, among others; establish European strategic initiatives for rare cancers, such as a new Joint Action on Rare Cancers, including rare adult cancers and paediatric cancers.
2. Create a dedicated Flagship on rare cancers within Europe's Beating Cancer Plan, to tackle the specific challenges faced by the community representing almost one quarter of all new cancer cases; ensure dedicated funding under Horizon Europe for the creation of a European Partnership on Rare Cancers. It is vital that the EU continues to support and reinforce successful past EU-funded projects, such as the Joint Action on Rare Cancers.
3. Guarantee funding for the continued functioning of (European Reference Networks) ERNs, through the EU4Health Programme, the European Semester programme, Structural Funds, and through Article 195 of the Financial Regulation.
4. Encourage the creation of national networks for the rare cancer ERNs on rare adult solid cancers, haematological neoplasms, and rare genetic tumour risk syndromes.
5. Engage with multistakeholder partnerships such as Rare Cancers Europe, on a regular basis to ensure that the work done by the rare cancer community is reflected in policy actions.
6. Encourage the creation of user-friendly, interoperable and harmonised IT solutions to allow for easy exchange of cases, knowledge and discussion on complex cancer cases and treatment pathways.
7. Support the integration of existing robust, widely used clinical practice guidelines, developed by learned oncology societies, into EURACAN, EuroBloodNet, PaedCan, GENTURIS - the ERNs for rare adult solid cancers, rare haematological diseases, paediatric cancers and rare genetic tumour risk syndromes; Support the recognition and adoption at national level of clinical guidelines developed or reviewed by ERNs.
8. Develop clinical registries linked to ERNs and population-based disease registries, in collaboration with the European Network for Cancer Registries (ENCR), to encourage the collection of clinical data on rare and ultra-rare cancers.
9. Create a medical ERASMUS for ERNs, in collaboration with European and national educational institutions, and encourage mobility within ERNs, to strengthen the scarce professional competence to treat complex rare cancers; support increasing levels of health literacy of patients, carers and patient organisations' representatives.
10. Ensure the harmonisation and adoption of joint clinical assessments under the draft HTA regulation at EU level to overcome current disparities, inefficiencies and delays generated by unjustified duplicative actions and improve access to medicines, starting with cancer as a pilot.
11. Include a rare adult cancer component, as well as a component on paediatric cancers, in all national cancer plans, including links with the European Reference Networks, to ensure all rare adult cancer patients and paediatric cancer patients receive the same high-quality care as other patients.
12. Support novel regulatory approaches for evidence generation in rare cancers, such as the European Medicines Agency's adaptive licensing pilot, allowing to dynamically incorporate the generated new evidence into the decision-making process.
13. Recognise the difficulty in finding biomarkers for patients with a rare cancer and create a framework to incentivise research in biomarkers for rare genomic alterations to improve early detection and an accurate diagnosis.
14. Acknowledge the specificities of rare cancers in programmes dedicated to improving the quality of life for cancer patients, survivors and carers, and encourage specific training for professionals, other than healthcare providers, taking care of rare cancer patients.

*The abovementioned recommendations have been drafted by **Rare Cancers Europe (RCE)**, a 40 partner strong multi-stakeholder partnership, founded in 2008 and coordinated by the **European Society for Medical Oncology (ESMO)**, bringing together scientific societies, patient and research organisations, healthcare and education providers, and the industry. For more information please visit the **RCE website.***

For paediatric cancers, SIOP Europe, an RCE member, in partnership with CCI Europe and PanCare is leading the initiative focussing on paediatric cancer.

RARE CANCERS EUROPE'S CALL TO ACTION HAS BEEN ENDORSED BY:

EURACAN

EuroBloodNet

European Reference Network
for rare or low prevalence complex diseases
Network Genetic Tumour Risk Syndromes (ERN GENTURIS)

European Reference Network
for rare or low prevalence complex diseases
Network Paediatric Cancer (ERN PaedCan)

ENDORSEMENTS BY MEMBERS OF THE EUROPEAN PARLIAMENT:

Bartosz Arłukowicz	Poland	**Eero Heinäluoma**	Finland
Anna-Michelle Assimakopoulou	Greece	**Romana Jerković**	Croatia
Cristian-Silviu Buşoi	Romania	**Seán Kelly**	Ireland
Maria da Graça Carvalho	Portugal	**Peter Liese**	Germany
Sara Cerdas	Portugal	**Dolors Montserrat**	Spain
Olivier Chastel	Belgium	**Alessandra Moretti**	Italy
Cyrus Engerer	Malta	**Sirpa Pietikäinen**	Finland
Cindy Franssen	Belgium	**Maria Spyraki**	Greece
Jens Gieseke	Germany	**Hilde Vautmans**	Belgium