

## Rare Cancers in All Policies

Tuesday, 28 September 2021

On Tuesday 28th September, [Rare Cancers Europe](#) (RCE) hosted a [webinar on "Rare Cancers in All Policies"](#). The webinar brought together speakers from various EU institutions, representatives of European Reference Networks (ERNs), patient organisations and other crucial stakeholders to launch RCE's Call to Action, "[Rare Cancers in All Policies](#)".

The event included contributions from the European Commission, several Members of the European Parliament (MEPs) and the European Medicines Agency (EMA). In addition, interventions were made by representatives from the [European Hematology Association \(EHA\)](#) and the [European Society for Paediatric Oncology \(SIOPE\)](#), and by oncologists and e-PAG representatives from [ERN EURACAN](#), [ERN PaedCan](#), [ERN EuroBloodNet](#) and [ERN GENTURIS](#). Perspectives of the pharmaceutical industry were also provided, with the event including a broad scope of contributions from a wide range of stakeholders, thus allowing immediate visibility for Rare Cancers Europe's Call to Action.

### Executive summary

- All speakers were united in endorsing Rare Cancers Europe's Call to Action on *Rare Cancers in All Policies*, highlighting that 24% of all new cancer diagnoses, including all paediatric cancers, are rare forms of the disease. This results in 650,000 new rare cancer diagnoses annually, and a total of 5.1 million people in Europe living with a rare cancer.
- During the event, interventions highlighted that patients with rare cancers face a unique set of challenges linked to late or incorrect diagnosis, lack of access to appropriate therapies and expertise, lack of commercial feasibility in developing new therapies, difficulties in conducting well-powered clinical studies, and tissue banks.
- However, despite the scale of this challenge, in [Europe's Beating Cancer Plan](#) and other ongoing policy initiatives, rare adult cancers have not been considered. These include the [Pharmaceutical Strategy for Europe](#), the related [revision of the EU rules on Medicines for Children and Rare Diseases](#), the creation of the [European Health Data Space](#) and the [Cross-Border Healthcare Directive](#), among others. To tackle these challenges, and to ensure that they are addressed in the Cancer Plan and other initiatives, Rare Cancers Europe authored the Call to Action.
- The Call to Action includes 14 recommendations for action, including the introduction of a dedicated Flagship on rare cancers within the Cancer Plan, as well guaranteed funding for the continued functioning of European Reference Networks, amongst others. The Call to Action also calls for engagement with multistakeholder partnerships, such as Rare Cancers Europe, to ensure that the work done by the rare cancer community is reflected in policy actions.
- To date, the Call to Action has been endorsed by 39 organisations, the 4 European Reference Networks dedicated to rare cancers and 18 Members of the European Parliament (MEPs).

## Rare Cancers in All Policies – An RCE Call to Action

The context of the event is the challenge posed by rare cancers as a neglected area deserving attention from policymakers. 24% of all new cancer diagnoses are rare, including all paediatric cancers. Rare cancers belong to both cancers and rare diseases, and face challenges linked to their rarity, although their specificities make them closer to common cancers. Additionally, with 650,000 new diagnoses annually and around 5.1 million people in Europe living with a rare cancer, these cancers are not so rare.

The numbers alone do not tell the whole rare cancers story. Patients with a rare cancer face a unique set of challenges linked to late or incorrect diagnosis, lack of access to appropriate therapies and expertise, lack of commercial feasibility in developing new therapies, difficulties in conducting well-powered clinical studies, and tissue banks.

Rare Cancers Europe (RCE) was established in 2008 to highlight these challenges and place the topic on the European policy agenda. The organisation is a partnership comprised of 39 stakeholders, coordinated by the European Society for Medical Oncology (ESMO), and brings together scientific societies, patient and research organisations, healthcare and educational providers, and industry. The organisation is actively involved in driving achievements for rare cancer care by calling on regulators and policymakers to address treatment challenges and improve treatment quality.

The Call to Action also follows on from RCE's activities in previous years, with the webinar including an overview by RCE's Chair, Prof. Paolo G. Casali, of work on the Joint Action of Rare Cancers' (2016-2019). This culminated in the [Rare Cancer Agenda 2030: Ten Recommendations from the EU Joint Action on Rare Cancers](#), which now serves as a basis for RCE's advocacy activities. Prof. Casali stressed that the Call to Action, on *Rare Cancers in All Policies*, is the outcome of a rigorous exercise within the stakeholders of the rare cancer community and includes 14 recommendations for policymakers in the context of ongoing policy initiatives at EU-level.

### Rare Cancers in All Policies – An RCE 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.

## Perspectives from EU Institutions, policies to support rare cancer patients

During the webinar, all speakers were in agreement on the challenges faced by the European rare cancer community. This included a recognition of the important role played by Rare Cancers Europe in keeping the topic on the policy agenda, and the crucial timing of the Call to Action given the development of Europe's Beating Cancer Plan.

The MEPs attending the event all voiced their support for the Call to Action and stressed the importance of addressing rare cancers within the European Parliament and Europe's Beating Cancer Plan. **Cristian Silviu Buşoi MEP** cited the continued funding of European Reference Networks (ERNs) as an area for action, in line with Rare Cancers Europe's recommendations in the Call to Action. He highlighted that 5-year relative survival rates are worse for rare cancers than for common cancers, and that the differences in survival rates between countries suggest inequality in healthcare delivery across the EU. Buşoi stressed that these figures showed the need for coordinated European action, and that the ambitious targets set by the Commission in the Cancer Plan must be capitalised upon, to ensure that rare cancers are in focus and that the proposals in Rare Cancers Europe's Call to Action are addressed.

**Peter Liese MEP** and **Dolors Montserrat MEP** both welcomed the Commission's overall work on cancer but stated that cross-border medical cooperation needs to be strengthened to reach higher standards of care, highlighting that disparities in access to treatment need to be addressed. Mr Liese added that the importance of the topic was such that a special envoy on cancer, directly reporting to the President of the European Commission, is required to fully address the topic. Ms Montserrat also used her remarks to highlight the European Parliament's work on the [Pharmaceutical Strategy for Europe](#), where she is the rapporteur for the Parliament's [non-binding report](#). She particularly welcomed Rare Cancers Europe's call for research funding to be dedicated to rare cancers, adding that while European Union research programmes are among the best in the world, rare cancers, including rare adult solid cancers, haematological cancers and paediatric cancers, are an area of unmet medical need.

**Cyrus Engerer MEP** stressed his support for *Europe's Beating Cancer Plan*, but also said that adult rare cancers are not sufficiently addressed within the Cancer Plan as it stands. Engerer spoke in detail on his support of the creation of a dedicated Flagship on rare cancers within *Europe's Beating Cancer Plan*, to tackle the specific challenges faced by the European rare cancer community.

## Interventions from Members of the European Parliament:

### **Cristian Silviu Buşoi MEP:**

*“Let us be clear, that rare cancers require specific EU action and that specific initiatives should be included in ongoing policies.” “I support increased funding for European Reference Networks, as well as a strengthening of their role. The Cancer Plan and Cancer Mission offer us the opportunity to do that.”*

### **Dolors Montserrat MEP:**

*“In my role as ENVI rapporteur for the European Parliament’s report on the Pharmaceutical Strategy, I am committed to ensuring that we take this opportunity to bring forward solutions that are beneficial to the rare cancers community.”*

### **Peter Liese MEP:**

*“On rare cancer, it is clear that we need European action.” “We want to establish a special envoy on cancer, that is directly reporting to the President of the European Commission.”*

### **Cyrus Engerer MEP:**

*“As a member of the Special Committee on Beating Cancer, I have been working to raise the topic since becoming a Member of the European Parliament.” “We must ensure that there is a continued focus at EU level on rare cancers to provide patients with the best possible care.”*

During the event, speakers linked the rare cancer topic to a number of ongoing policy initiatives relevant to the rare cancer community. Speaking on behalf of the European Commission, DG SANTE, **Martin Dorazil** highlighted that the Cancer Plan includes 10 flagship initiatives, which aim to take action at every stage of the disease, alongside including the lessons learned from the COVID-19 pandemic. Dorazil added that the Commission’s overall objective on rare cancers is to ensure that all rare cancer patients receive access to the best knowledge and treatment available. The €4 billion of funding announced as part of the Cancer Plan also goes in tandem with the [EU4Health](#) programme, the EU’s response to COVID-19. Dorazil said that the EU4Health programme contains a total of €5.3 billion of funding, with €81.5 million being allocated to cancer actions in the first year and highlighted that this level of investment demonstrates the Commission’s commitment to action on cancer.

Dorazil also highlighted the importance of the European Reference Networks working on cancer, including those attending the webinar. He added that, since their establishment in 2017, ERNs have grown substantially and now bring together more than 900 healthcare units from more than 300 hospitals. As is highlighted in the Call to Action, Dorazil said that additional financial support for ERNs had been identified as necessary and had been secured from the EU4Health programme. He concluded his remarks by stating that the Commission will continue to support ERNs in their knowledge generation and research activities and that, looking further into the future, one of the next steps should be their integration into national healthcare systems.

The regulatory perspective was provided by Ms **Antonella Baron** from the **European Medicines Agency (EMA)**. Ms Baron stressed that the EMA is committed to helping developers of new treatments for rare cancers. She outlined the steps being taken by the EMA to prioritise breakthrough medicines that cover unmet medical need, via the priority medicines scheme ([PRIME](#)), which identifies candidate therapies for accelerated assessment. Although not specifically dedicated to rare cancers medicines, Ms Baron stated that this would help in areas of the greatest need, adding that while developments, such as PRIME, were helping rare cancer patients, there is a recognition that more needs to be done to address the topic. The EMA has a specific framework that is built for small populations, as there is a recognition that for diseases such as rare cancers, the rarity of the patient population presents a challenge for medicine developers. Based on the efforts made by the EMA, 16 new medicines were approved for rare solid cancer and rare blood cancer from 2018-2020.

However, Ms Baron stated that there is a recognition within the EMA that more needs to be done. This is in part due to the challenging nature of clinical research for rare and paediatric cancers, with there being a need to create and support the right infrastructure in Europe for systematically collecting, federating and sharing key data to create a learning healthcare system.

Non-institutional speakers, including interventions from coordinators and ePAG representatives from the 4 rare cancers European Reference Networks (ERNs), presented real-world examples of the challenges faced by the rare cancer community. Rare Cancers Europe’s “Rare Cancers in All Policies” Call to Action stresses the crucial role of ERNs, as one of the most important EU initiatives for rare cancers and rare diseases, with perspectives being presented at the webinar by representatives of **ERN EURACAN (Rare Adult Solid Cancers)**, **ERN PaedCan (Paediatric Cancers)**, **ERN EuroBloodNet (Rare Haematological Diseases)** and **ERN GENTURIS (Genetic Tumour Risk Syndromes)**. The non-institutional speakers also included an intervention from a representative of the pharmaceutical industry.

The specific challenges of managing haematological cancer were highlighted by Prof. **Elizabeth Macintyre**, from the **European Hematology Association (EHA)**. Prof. Macintyre highlighted that almost all haematological cancers are rare corresponding to the 6.5% of cancers in adults. The challenges on haematological cancers are not unique to patients, with Prof. Macintyre stating that the management of haematology requires networking amongst specialists, with many doctors in the field having received dual clinical and scientific training. She highlighted this combination of networking and training as a positive example for other disciplines.

In addition, Prof. Macintyre stressed the positive contribution of Rare Cancers Europe, as a multistakeholder coalition that brings together the entire rare cancer community, in optimising advocacy and communications at EU-level. Speaking on behalf of the **European Society for Paediatric Oncology (SIOPE)**, Prof. **Carmelo Rizzari** concurred with the importance placed on multistakeholder collaboration, adding that this is particularly important in the paediatric oncology field, as patients need to be monitored and supported for many years after their diagnoses, throughout adolescence and into adulthood. Prof. Rizzari stressed that SIOPE is committed to working alongside the rare cancer community and contributing to the advocacy efforts of Rare Cancers Europe. He also focused his remarks on the major health and socio-economic burden that paediatric cancers place on Europe. With 35,000 cases diagnosed annually, and more than 6,000 young patients dying each year, paediatric cancers are the highest cause of death by disease in children over the age of 1. Prof. Rizzari added that these figures are unfortunately teamed with unequal access to the best available multi-disciplinary care, with there being an up-to 20% difference in children’s survival rates across Europe.

The Call to Action stresses the crucial role of ERNs, as one of the most important EU initiatives for rare cancers and rare diseases, with professional and patient advocacy perspectives being presented at the webinar by representatives of **ERN EURACAN (Rare Adult Solid Cancers)**, **ERN PaedCan (Paediatric Cancers)**, **ERN EuroBloodNet (Rare Haematological Diseases)** and **ERN GENTURIS (Genetic Tumour Risk Syndromes)**.

The professional perspectives from the ERNs focused on the specific challenges faced when treating rare cancer patients, who often 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. The patient advocacy perspectives from the ERNs built on this and added a focus on the lived experiences of patients suffering from a rare cancer.

Providing the professional perspective from **ERN EURACAN**, Prof. **Jean-Yves Blay** highlighted EURACAN’s work in the development of guidelines and the importance of cooperation with scientific societies, such as the European Society for Medical Oncology. This point is echoed in the Call to Action, which calls for support for the recognition and adoption at national level of clinical guidelines developed or review by ERNs. Prof. Blay added that registries are crucial for collecting data on how patients are being treated and that they have shown that patient care is improved by the work of ERNs, particularly in terms of survival. However, given the number of rare cancer patients across Europe, digital tools such as the Clinical Patient Management System (CPMS) needs to be adapted for size and be fit for purpose. **ERN EURACAN’s ePAG representative**, Ms **Kathy Oliver**, provided her expertise as caregiver involved in the rare cancer community, which resulted from her son having been diagnosed with a brain tumour. She took participants back to the day of his diagnosis and said that patients and families dealing with a rare cancer diagnosis are often overwhelmed by feelings of isolation. She

added that now, more than ever, it is crucial for rare cancers to be on the policy agenda and, addressing policymakers directly, said that everyone involved with Rare Cancers Europe must united to ensure rare cancers are in all policies.

From **ERN PaedCan**, Prof. **Ruth Ladenstein** said that there remains a need for more efforts to overcome inequalities between countries in paediatric cancer care. This disparity is highlighted in the Call to Action, which states that rare cancer patients have the right to receive the highest quality of care and treatments, regardless of where they live. Prof. Ladenstein also underlined the special needs of children and young adults, and the need for dedicated care teams and specialists. **ERN PaedCan's ePAG representative**, Ms **Anita Kienesberger**, welcomed the fact that paediatric cancer was in the Cancer Plan. However, she added that survivorship is often overlooked, with the transition from active treatment to long term follow-up being an area in need of improvement in many European countries. Ms Kienesberger also added that, while the inclusion of paediatric cancer was welcome, the Cancer Plan still needed to address the needs of other rare cancers.

Speaking on behalf of **ERN EuroBloodNet**, Prof. **Pierre Fenaux** stressed the importance of guidelines and education for professionals working in the oncology field. Prof. Fenaux highlighted access to medicines as a crucial topic, with barriers to access still existing in some countries. In addition, he stressed the importance of clinical trials for rare cancers, as many clinical trials are not directed towards niche areas of unmet need. He concluded his remarks by noting the importance of cooperation with patient groups, stating that they are crucial stakeholders in the rare cancer community. **ERN EuroBloodNet's ePAG representative**, Ms **Ananda Plate** echoed the challenges for young cancer patients, adding that although survivorship is reflected in Europe's Beating Cancer Plan, there is no one-size-fits-all approach. This is particularly true for people diagnosed as teenagers or young adults, who often face challenges throughout their lives.

Prof. **Nicoline Hoogerbrugge**, representing **ERN GENTURIS**, underlined that while each individual rare cancer diagnosis may be rare by themselves, when taken together they represent 24% of all new cancer diagnoses in Europe. This means that, taken together, rare cancers are not so rare. Prof. Hoogerbrugge added that the introduction of ERNs has enhanced rare cancer care across the EU and reduced inequality, with the scope of work carried out by ERNs growing significantly since their establishment. However, in order to ensure that the work of ERNs is sustainable, further funding and support is needed. Prof. Hoogerbrugge stated that the best way to make ERNs sustainable is by introducing a dedicated flagship on rare cancers in Europe's Beating Cancer Plan. **ERN GENTURIS' ePAG representative**, Ms **Rita Magenheim** told participants her experiences as a cancer patient that suffers from Li-Fraumeni syndrome (LFS), an inherited familial disposition to a wide range of certain, often rare, cancers. Individuals with LFS have an approximately 50% chance of developing cancer by the age of 40, and up to a 90% chance by the age of 60. She said that patients with hereditary cancer, who will probably have cancer in the future, are often overlooked in comparison to current patients and cancer survivors. She also stressed the impact of hereditary cancers on patients and their families, as if one person carries this enhanced risk, so does the rest of their family. Given the enhanced risk for patients with one of the rare genetic tumour risk syndromes, further attention was needed for rare cancer 'previvors'.

Providing the industry perspective on the Call to Action, Ms. **Ivana Cattaneo** expressed the full support of the industry for the Call to Action. She added that, given the 5.1 million rare cancer patients across Europe, there is an urgent need for a dedicated flagship on rare cancer in the Cancer Plan to build on the work conducted by Rare Cancers Europe and the rare cancer community. This is teamed with the need for recognition of the specificities of rare cancers. She concluded her remarks by recognising the broad scope of the rare cancer community, with RCE providing its unified voice.

## Conclusions

Speakers were united in their call for additional EU action on rare cancers, particularly given there are around 5.1 million people in the Europe living with a rare cancer, with 650,000 new rare cancer diagnoses being made annually. They also linked the rare cancer topic to a number of ongoing policy initiatives relevant to the rare cancer community, particularly Europe's Beating Cancer Plan. The MEPs at the event added that the publication of the Call to Action was well-timed, with the European Parliament's [Special Committee on Beating Cancer \(BECA\)](#) being in the process of finalising its own-initiative report on the Cancer Plan. Speakers also stressed the need for a dedicated Flagship on rare adult cancer in Europe's Beating Cancer Plan, along with the need for further cross-border medical cooperation. The ERNs coordinators focused on the specific challenges faced when treating rare cancer patients, with the expert ePAG representatives from each of the 4 ERNs at the event building on these contributions to add a focus on the lived experiences of patients or caregivers.

**Rare Cancers Europe's Chair, Prof. Paolo Casali** closed the webinar by urging all participants to continue their engagement with Rare Cancers Europe, highlighting that the Call to Action will serve as a vehicle to bring the topic to the attention of policymakers. He also stressed that everyone involved in the webinar fully believed in the work of Rare Cancers Europe, and that it is vital for rare cancers to be fully recognised in ongoing initiatives at EU-level.

## Acknowledgements

Rare Cancers Europe wishes to thank the following speakers for participating in the webinar, and for their help in publicising the topic of rare cancers at a crucial time for the rare cancer community:

**ERN EURACAN, Rare Adult Solid Cancers:** Prof. Jean-Yves Blay (ERN coordinator), Ms Kathy Oliver (ePAG Representative, RCE Steering Committee).

**ERN EuroBloodNet, Rare Haematological Diseases:** Prof. Pierre Fenaux (ERN coordinator), Ms Ananda Plate (ePAG representative).

**ERN GENTURIS, Genetic Tumour Risk Syndromes:** Prof. Nicoline Hoogerbrugge (ERN coordinator), Ms Rita Magenheimer (ePAG Representative).

**ERN PaedCan, Paediatric Cancers:** Prof. Ruth Ladenstein (ERN coordinator), Ms Anita Kienesberger (ePAG Representative).

**European Commission, DG SANTE:** Mr Martin Dorazil.

**European Hematology Association:** Prof. Elizabeth Macintyre.

**European Medicines Agency:** Ms Antonella Barron.

**European Parliament:** MEP Cristian Silviu Buşoi, MEP Cyrus Engerer, MEP Peter Liese, MEP Dolors Montserrat.

**Rare Cancers Europe Steering Committee:** Ms Ivana Cattaneo (industry representative, RCE Steering Committee).

**European Society for Paediatric Oncology:** Prof. Carmelo Rizzari.

**Rare Cancers Europe & European Society for Medical Oncology:** Prof. Paolo G. Casali.