



Ágnes Cser

Member of the Civil Society Organisations' Group
European Economic and Social Committee
Rue Belliard 99/101
1040 Brussels

Subject: Feedback on the 'Leaving no one behind: European Commitment to Tackling Rare Diseases' opinion.

Lugano, 10 October 2024

Dear Ms Cser,

We are writing to you on behalf of the European Society for Medical Oncology's (ESMO) [Rare Cancers Working Group \(RCWG\)](#) - a multi-stakeholder partnership which brings together scientific societies, patient and research organisations, healthcare and education providers, and industry – to provide comments on the European Economic and Social Committee's (EESC) - 'Leaving no one behind: European Commitment to Tackling Rare Diseases' - opinion.

ESMO RCWG's key objectives are to support the rare cancers community towards the effective implementation of the **Rare Cancer Agenda 2030**¹ and advocate for fostering rare cancers research and improving care. Rare and ultra-rare cancers have an annual incidence of less than 6 per 100.000 people, and they correspond to 650.000 new diagnosis annually in Europe. Thus, rare cancers are not so rare, as 5.1 million people live with a rare cancer in Europe, and they represent the 24% of all new cancer diagnoses, including all cancers in children which have additional specificities, as outlined in the dedicated chapters of the Rare Cancers Agenda 2030.

The ESMO RCWG welcomes the opinion which provides a vital opportunity to bring much-needed attention at EU level to rare diseases of which rare cancers are an often-overlooked subset and can be overshadowed by more prevalent cancers. By raising awareness, advocating for research and funding, providing support, encouraging early detection and by reducing stigma, such an initiative plays a pivotal role in improving the lives of those affected by rare cancers.

Rare cancers are the rare diseases of oncology - they share the specificities of both cancer and rare diseases. These specificities require a tailored policy approach to overcome their challenges. Rare cancers face difficulties linked to access to early and correct diagnosis, lack of access to cancer care, including treatment and development of new therapies, lack of feasibility in developing new therapies and difficulties in conducting well-powered clinical studies which lead to a need for the development of methodological research applicable to rare cancers given the low numbers.

The aforementioned challenges should be tackled through greater cooperation at EU level and by addressing rare cancers in dedicated EU initiatives. It is recognised that progress has been made in the current mandate, however the ESMO RCWG regrets that rare cancers neither have a dedicated action area under the Europe's Beating Cancer Plan (EBCP), contrary to paediatric cancers, nor are included in the European Rare Diseases Research Alliance's (ERDERA)² work programme. This provides missed opportunity for directing sufficient attention rare cancers deserve.

The ESMO RCWG would like to highlight that the opinion's scope does not include rare cancers, while clearly rare diseases go beyond 'congenital rare conditions in children'. Whilst the opinion focuses on diagnostics and support, it lacks a sufficient consideration regarding treatment. While it aligns with traditional congenital diseases, it not only entirely fails rare cancer patients but even conditions where new treatments (e.g. gene therapies) become available.

¹ <https://www.esmo.org/policy/rare-cancers-working-group/rare-cancers-in-europe/rare-cancer-agenda-2030>

² <https://www.ejprarediseases.org/erdera/>



The opinion also suggests that the future APRD should ensure access to diagnosis in due time as well as comprehensive life-long care regardless of where a patient lives in the EU and it recommends strengthening and integrating the 24 European Reference Networks (ERNs) and national networks of specialised service providers. These include ERNs on rare cancers and the ESMO RCWG believes that it provides a rationale to include rare adult and paediatric cancers respectively in the scope of the APRD, including cross-referencing initiatives, such as the EBCP and Cancer Mission, and recognising that rare adult and paediatric cancers belong to the realm of rare diseases and of oncology, and so stand to benefit from initiatives in both domains.


Therefore, the ESMO RCWG would suggest including a separate paragraph on rare cancers including:

- To recognise that rare adult and childhood cancers, respectively, are both rare and oncological diseases and stand to benefit from initiatives in both domains, therefore a coordinated approach and interference between the European Action Plan on Rare Diseases (APRD) and EU cancer initiatives is needed.
- A Steering Group for the APRD shall also include experts from other relevant stakeholders, in addition to Member States and EURORDIS, to ensure horizontal coordination and cooperation.
- The shared and different aspects with congenital rare conditions shall be addressed.
- The need for pathways for diagnosis and treatment within centres and/or networks of excellence, both, nationally as well as cross-border.
- The importance of diagnostics and reliance on precision medicine with timely access to affordable medicines supported by appropriate measures adopted as a result of the ongoing revision of the EU's Pharmaceutical legislation in the field of orphan and paediatric drugs.
- There is an urgent need to collect data at both EU and international level by using or establishing clinical registries for rare cancers to increase research on these cancers to better understand the natural history, predictive and prognostic factors and improve the quality of care.
- Accelerated development of clinical, translational and basic research specifically dedicated to rare cancers is urgently needed.
- Due to the rarity of each disease, there is a need to increase number of clinical trials and facilitate access to cross-boarder clinical trials as well as to accept a higher degree of uncertainty also from the regulatory standpoint due to the difficulties to carry out high-power clinical trials in rare and ultrarare cancers.
- The EU and national governments should recognise that rare adult and paediatric cancers respectively belong to both rare diseases and oncology fields and ensure synergies between rare diseases and cancer initiatives.
- Future APRD shall set common and measurable goals that will help define and implement national plans and strategies for rare cancers and encourage Member States to include rare cancers into their National Cancer Plans.

We hope that the aforementioned recommendations will be taken into consideration for inclusion and the ESMO RCWG is committed to working with EU institutions during the new mandate to ensure that rare cancers' specificities continue being part of the EU health agenda to deliver improved health outcomes to rare cancers patients.

We look forward to hearing from you and would be delighted to elucidate further, either in person or by continued correspondence.

Yours sincerely,



Prof Paolo Casali
Chair of ESMO RCWG

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