

Call to Action Against Rare Cancers

We call on all relevant stakeholders to work towards:

Substantially improved equity of care in rare cancers

We strongly encourage the consideration of equity, social justice and the interests of patients when setting public health priorities. While recognising the economic burden associated with the provision of healthcare, we emphasise that geographic borders, and other barriers, should not prevent patients from accessing the best standards of care.

Development of new effective therapies for rare cancers

While recognising the impact of the EU Orphan Drug Directive, we urge researchers, clinicians, the pharmaceutical industry and governments to further prioritise the development of new effective therapies for rare cancers.

Facilitating clinical studies in rare cancers

We call for more clinical studies designed to establish the efficacy of new medical treatments in rare cancers. The testing of new cancer therapies in rare cancer patients should be made an essential part of the clinical development process. Due to the rarity of the diseases, the low numbers of patients available for study accrual represents a barrier to collecting definitive data through standard clinical trials. In the field of rare cancers, researchers and regulators must recognize these difficulties and encourage alternative study designs and methodological approaches.

Building a knowledge-base

We encourage the establishment and further development of networks of clinical databases, rare cancer registries and tissue banks, which would improve the collection, provision and comparison of data on rare cancers.

Balanced, timely and transparent assessment of new therapies

We call upon all EU member states, the regulatory bodies, industry, patient representatives, and national health authorities to commit to meaningful and transparent dialogue with the rare disease-oriented research communities. It must be ensured that new effective therapies for rare cancers are appropriately developed and made available in a timely manner to all eligible patients with full reimbursement for as long as there is a need. Procedures for assessing the cost effectiveness of therapies must be totally transparent and be conducted in a timely manner. Cost-effectiveness models should include the wider social costs and benefits of effective treatment. Issues such as the off-label use of treatments in rare cancers should also be addressed more effectively.

Increased knowledge-sharing

We call for structured collaboration between rare cancer specialists and centres of expertise, through the creation of reference networks which will set a high standard for the provision of care to patients with rare cancers. Networking should not only take place within the individual Member States but also on a pan-European scale. Linking individual centres of expertise to European and global reference networks on rare cancers would provide the necessary structure for a more efficient exchange of experience, information, data and best practice. This would lead to an overall increase in knowledge as well as more efficient clinical research and improved care for patients with rare cancers.

Timely and accurate detection and provision of care

We encourage the development of innovative approaches to raising general practitioners' awareness about rare cancers in order to promote timely and appropriate referral of patients to centres of expertise and reference networks. Referral to centres of expertise is crucial, especially with regard to timely diagnosis and correct clinical decision-making in an overall therapeutic strategy.



Sign the **Call to Action**
Against Rare Cancers!

www.rarecancerseurope.org