

ESMO STATEMENT
ON THE COUNCIL RECOMMENDATION
ON RARE DISEASES¹

The European Society of Medical Oncology (ESMO) welcomes the adoption on 5 June 2009 of the Council Recommendation on European action in the field of rare diseases.² The initiative is a major step towards overcoming barriers to the better treatment of rare cancers, a key category of rare diseases. The Recommendation will help promote coordinated action and the commitment of resources that will lead to higher quality care of rare cancer patients across Europe. We urge Member States to give a high priority to rare cancers and to implement the provisions of the Recommendation in full. While recognising the value of what has been achieved, we ask all stakeholders to continue to treat rare cancers as a priority topic and sign up to the recently launched “Call to Action Against Rare Cancers”.³

ESMO would also like to thank the European Parliament for its Resolution on Rare Diseases adopted on 23 April 2009. The Resolution was a useful contribution to the debate and a clear indication that MEPs treat the issue as a political priority. ESMO would like to especially thank Professor Antonios Trakatellis MEP, rapporteur for the report, for his commitment to driving the process forward.

Our priorities

As the leading European professional medical oncology organisation, ESMO welcomes the recognition by MEPs and by Member States that the EU brings a real added-value by promoting cooperation in the fight against rare diseases. We are pleased that attention was paid in the Recommendation to the following critical areas:

- **Cooperation and Coordination**
ESMO particularly welcomes the recognition of importance of the identification and promotion of centres of expertise and the call to link these to European reference networks. ESMO agrees that knowledge-sharing and improved education are two key elements for an improved strategy on rare diseases. The supportive idea in the Recommendation of developing supplementary tools, such as an inventory of rare diseases, registries and databases for an easier access to information, is also highly appreciated. As recognised in the text, a multidisciplinary approach, bringing together the diverse knowledge and expertise of various oncology communities, is particularly suited to the treatment of rare cancers. We would further call for EU consensus guidelines on multi-disciplinary treatment.
- **Adequate and sustained funding**
ESMO is pleased to see that the Recommendation recognises the need for adequate and sustained funding for centres/ networks of expertise. In addition, funding for clinical trials for rare cancers should be promoted by the EU and governments. It is extremely important that decisions with regard to funding are taken with a long-term perspective to ensure real benefits can be drawn.

¹ Adopted by ESMO on 20 July 2009

² The Council Recommendation can be found here:

http://www.consilium.europa.eu/uedocs/cms_data/docs/pressdata/en/lisa/108383.pdf

³ ESMO “Call to Action Against Rare Cancers” <http://www.rarecancers.eu/?-Call-to-Action->

- **Development of effective therapies**
ESMO endorses the idea that cancer sufferers are given full access to orphan drugs. It backs Parliament's reference to Regulation (EC) No. 141/2000 and supports its call for the use of medicinal products without marketing authorisation where no alternatives exist. ESMO calls for the optimal implementation of the Regulation so that proper incentives for development of orphan drugs can be provided to pharmaceutical companies.
- **Involvement of stakeholders**
ESMO appreciates the Parliament's call for the increased engagement of stakeholder advisory groups in the national decision-making to ensure that policymakers are given the necessary medical oncology expertise. It urges Member States to uphold such principles of decision-making.

Next steps

ESMO has been instrumental in placing rare cancer on the political agenda. In partnership with a range of stakeholder organisations, ESMO organised a conference on rare tumours in November 2008 and subsequently produced a series of political recommendations⁴. A second phase of this campaign recently began with the launch of a 'Call to Action' and a linked campaign website.⁵

As such, the Recommendation marks an important step in the global fight to improve the situation with rare cancers. We would call on all policymakers to take the following steps:

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 Directive⁶, 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 is a barrier to collecting definitive data through standard clinical trials. In the field of rare cancers, researchers and**

⁴ ESMO Political recommendations - <http://www.esmo.org/policy/position-statements-and-comments.html>

⁵ Call to Action on Rare Cancer - www.rarecancers.eu

⁶ Regulation (EC) No. 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products.

regulators must recognize these difficulties and encourage alternative study designs and methodological approaches.

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 show total transparency and effect their procedures over a reasonable time frame. They should include in the cost-effectiveness models 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.**

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.**

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 a 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 on an overall therapeutic strategy.**

While an important step, the Recommendation should not be the last. We would urge policymakers and stakeholders to sign up to the ESMO-led "Call to Action Against Rare Cancers" and thereby continue to high priority to quality treatment and care for patients with rare cancers.

About rare cancers

Rare diseases are so defined if they have a prevalence of less than 50 out of 100,000⁷. Rare cancers represent about 20 per cent of all cases of malignant neoplasms, including all cancers affecting children and teenagers and many affecting young adults. Due in part to unequal access to treatment, there are significant variations in survival rates for the same type of rare cancer in different Member States. Sub-optimal treatment outcomes are also common for rare cancers due to a lack of medical expertise.

⁷ ESMO recommendations

http://www.esmo.org/fileadmin/media/pdf/policies/ESMO_Rare_Cancers_RECOMMENDATIONS.pdf