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 EU 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.

Rare Cancers Europe is a joint initiative based on a partnership between the European Society for Medical Oncology (ESMO), the European Organisation for Rare Diseases (EURODIS), the European Cancer Patient Coalition (ECPC), the European Organisation for Research and Treatment of Cancer (EORTC), Confinet, EuroBoNet, the World Sarcoma Network (WSN), the Association of European Cancer Leagues (ECL), the Chronic Myeloid Leukemia Support Group, the International Brain Tumour Alliance (IBTA), Orphanet, the Chronic Myeloid Leukemia Advocates Network, the Sarcoma Patients EuroNet Association (SPAEN), GIST Support UK & PAWS-GIST, Cancer S2, the International Kidney Cancer Coalition (IKCC), the Choroida Foundation, the Fondazione IRCCS Istituto Nazionale dei Tumori, the European Institute of Oncology (IEO), the European Society for Paediatric Oncology (SIOP Europe), the European Society of Surgical Oncology (ESSO), the Grupo Español de Tumores Huérfanos e Infrecuentes (GEITH), the European School of Oncology (ESO), the European Oncology Nursing Society (EONS), canercare, the European Society of Pathology (ESP), the European, Middle Eastern and African Society for Biopreservation and Biobanking (ESBB), Novartis Oncology (initiating sponsor and industry partner), Pfizer Oncology (industry partner), and Sanofi (industry partner). The campaign is moreover supported by additional corporate supporters, including Amgen (silver industry supporter) and Takara Pharmaceuticals Europe (silver industry supporter).

For more information about this European initiative, please visit our Web site: www.rarecancerseurope.org or contact us:

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More common than you think!

Rare cancers represent about 20% of all cancer cases diagnosed in the European Union each year, including all cancers in children.*

4 mill.

Rare cancers affect around 4 million people in the EU alone.*

500,000

Each year, more than 500,000 people in the EU are newly diagnosed with a rare cancer.*

* Source: www.rarecare.eu

More challenges than you think!

» Late or incorrect diagnosis
» Difficulties finding clinical expertise and accessing appropriate treatments
» Difficulties carrying out clinical studies due to the small number of patients

» Possible lack of interest in developing new therapies
» High uncertainty in clinical decision-making
» Scarcity of available registries and tissue banks

Call to Action

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 recognise these difficulties and encourage alternative study designs and methodological approaches.

More joint action than you think!

Rare Cancers Europe is a multi-stakeholder initiative addressing methodological and regulatory barriers in rare cancer care, the need for centres of expertise and European reference networks, barriers to patients’ access to care, education of healthcare professionals, and access to information on rare cancers.

The strategic focus of the initiative is to develop scientific-based platforms, gather scientific findings, educate and raise awareness with target audiences, build consensus among professionals and the patient advocacy community, and advocate recommendations for political and stakeholder implementation.

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.