RARECARE
Surveillance of Rare Cancers in Europe

LIST OF RARE CANCERS AND ITS RATIONALE
Wednesday 10 June 2009
Carlton Hotel, Treviso

Participants

Riccardo Audisio	European Society of Surgical Oncology
Gianni Bisogno	SIOP EUROPE (The European Society for Paediatric Oncology)
Fredrik Bosman	European Society of Pathology
Marco Bregni	European Group for Blood and Marrow Transplantation
Riccardo Capocaccia	Istituto Superiore di Sanità, Rome
Paolo Casali	Fondazione IRCCS "Istituto Nazionale dei Tumori", Milan
Maria Luisa Clementi	Epidemiologia e Prevenzione
Angelo Paolo Dei Tos	Treviso Regional Hospital
John Dowling	European Prostate Cancer Coalition, Europa Uomo
Joanne Fleming	Treviso Regional Hospital
Gemma Gatta	Fondazione IRCCS "Istituto Nazionale dei Tumori", Milan
Jan Geissler	European Cancer Patients Coalition
Margherita Greco	Fondazione IRCCS "Istituto Nazionale dei Tumori", Milan
Graeme Heron	University of Edinburgh
Ian Kunkler	University of Edinburgh
Tezer Kutluk	International Union Against Cancer
Denis Lacombe	European Organisation for Research and Treatment of Cancer
Lisa Licitra	Fondazione IRCCS "Istituto Nazionale dei Tumori", Milan
Sandra Mallone	Istituto Superiore di Sanità, Rome
Carmen Martinez	Escuela Andaluza de Salud Publica, Granada
Marc Maynadie	HAEMOCARE
Torgil Moller	Lund University Hospital
Kathy Oliver	International Brain Tumour Alliance
David Perry	University of Edinburgh
Riccardo Soffietti	European Association of Neuro Oncology
Samba Sowe	Fondazione IRCCS "Istituto Nazionale dei Tumori", Milan
Hana Stankusova	European Society for Therapeutic Radiology and Oncology
Annalisa Trama	Fondazione IRCCS "Istituto Nazionale dei Tumori", Milan
Markus Wartenburg	Global GIST Network

Welcome and overview of the RARECARE project

Dr Gemma Gatta (leader of the RARECARE project) welcomed the participants and provided a brief overview of the project aims and actions. She recalled that information on rare cancer[ ] burden indicators is limited and differences in surveillance have been observed across EU and over time. In this context, the RARECARE project aims are:

- to develop an operational definition of rare cancer and a list of rare cancers meeting that definition
- to estimate the burden of rare cancers in Europe
- to improve the quality of data on rare cancers
- to develop strategies and mechanisms for the diffusion of information among all the key players

She stressed the importance of the project since it represents a unique opportunity to provide epidemiological indicators on the basis of population-based cancer registries (CRs). The first 2 years of activities were dedicated to the development of the list of rare cancers and to the estimation of the burden

indicators. The list of rare cancers and its rationale are available on the project web-site (www.rarecare.eu). The report on the indicators for rare cancers (incidence, prevalence survival and mortality) will be available within a few weeks.

She concluded by listing the objectives of the meeting:

- to endorse the rare cancers list
- to share and agree upon the rationale behind the list
- to discuss and identify dissemination strategies and opportunities

The meeting proceeded with presentations on the list, its rationale, what to do with the list and some results on the indicators. A brief summary of the presentation and of the following discussion is reported below.

The process for the definition of "rare cancers" and the list

Paolo Dei Tos recalled the steps undertaken to develop of the list and the rationale behind it. The list is based on the ICD-O3 classification since the RARECARE objective was not to create a different classification. The aim of the effort was to develop a list of rare cancers by clinically meaningfully grouping WHO-coded entities on the basis of a common clinical management. The list was hierarchically structured in three layers based on various combinations of ICD-O morphology and topography to respond to different needs: layer 1) families of tumours (relevant for the health care organisation), layer 2) clinically meaningful tumours (relevant for clinical decision making and research), layer 3) WHO tumour entities. The list was distributed to oncologic societies and patient associations. The comments received were all very supportive.

Paolo Dei Tos concluded that the list it is not fixed; it will change according to the evolution of the classification system.

The group discussion appreciated and agreed with the rationale of the list. Accordingly it was suggested to widely disseminate the rationale together with the list.

Presentation of the results

Riccardo Capocaccia presented the results on the indicators.

The discussion focussed mainly on the survival data. According to the analysis, rare cancers have a lower survival than the common ones, survival differs across EU geographical areas and it seems to be associated with a country's GDP. Different reasons were listed for explaining such differences: rare cancers usually have a worse prognosis, are more problematic and more aggressive (different pathology process).

The group concluded that a difference in survival was expected, however, further analysis will be necessary to explain the extent of the difference and to understand what should be done. It was agreed on that the results will be uploaded on the project web-site and methodological notes will be developed to guide interpretation and understanding of the results.

The list of rare cancers: what to do with it?

Paolo Casali proposed different uses of the list recalling also the ESMO recommendations on challenges and solutions for rare tumours in Europe. According to the presentation, the list could be used for:

- Call for networks on rare cancers (European reference networks)
- Call for a focus on treatment and timely diagnosis
- Call for investigating the off-label use in rare cancers
- Call for a greater involvement of disease-oriented research communities for providing advice to the pharmaceutical industry on the development of new drugs
- Call for alternative methodologies for rare cancer research
- Involving patients in the clinical decision-making process

Casali concluded that it will be important to build political pressure to ensure the implementation of the ESMO recommendations and participants were invited to visit and download the recommendations from ESMO website for the "European Action Against Rare Cancers" www.rarecancers.eu

The meeting continued by discussing the point of view of oncologic societies and other stakeholders on the use of the list. In general, they were all supportive and in agreement with the list. Specific contributions followed.

- The representative of the European Association of Neuro Oncology proposed some minor changes to the list with regard to the Central Nervous System.

- The representative of the European Society of Pathology commented that some rare cancers are not problematical from the clinician's point of view whereas clinicians are more concerned with problematic cancers, be they rare or not. He concluded suggesting that the list will be useful for increasing awareness among pathologists on limitations/challenges in rare cancers. The Society could contribute to increasing awareness through training initiatives and its web-site.

- The representative of the European Group for Blood and Marrow Transplantation commented that many rare cancers in the RARECARE list are targets for BMT, making it important to promote the development of guidelines on procedures (indications for BMT). Finally, he mentioned that the EGMBT has a registry and they are willing to collaborate with the RARECARE project in the future.

- The representative of the European Organisation for Research and Treatment of Cancer confirmed that the EORTC considers rare cancers one of their priorities and supports the RARECARE list. However, he mentioned that it would be important to have some EU endorsement of specific procedures for clinical trials addressing rare tumors. He stressed the importance of clinical trials as the only way to structure therapeutic advances for rare tumors, suggesting that clinical trials should be a high priority for impact on the list (immediate community wide impact with possible deliveries in terms of therapeutic progress within 2-3 years)

- The representative of the SIOP Europe called for a special chapter on paediatric tumours considering the peculiarities of such tumours (all paediatric tumours are rare cancers, common adult cancers have specific and different characteristics in paediatric age patients, among the paediatric tumours there are some ultra rare cancers).

Representatives of patients associations representatives commented that the list of rare cancers will be very useful for:

- Uncovering Europe-wide inequalities between Member States in handling rare cancers
- Lobbying for a greater focus of attention on rare cancers, and for orphan drug development.
- Health Technology Assessment (HTA)
- Informing HTAs of the special challenges that rare cancers present.

They are very supportive of the list and will promote the dissemination of the list through patient associations as follows:

- Emailing Update to ECPC Membership
- Asking to cascade information down to PAG's websites.
- Adding a link to RARECARE's website.
- Dissemination to other rare cancer advocacy groups
- ECPC Website: Homepage & "Rare Cancer" Section
- Incorporation in ECPC rare cancer position paper
- Promotion of the RARECARE list at cancer conferences & meetings

The representative of the International Union Against Cancer (UICC) was also very supportive of the list and proposed different means for its dissemination (presentation and/or dedicated section on rare cancers at the next UICC congress, UICC web-site, UICC common interest group).

Marc Maynadie discussed the importance of the list for supporting and orientating research. He mentioned that the list should be used to create a research network and biobanks for collecting biological material on rare tumours, to promote collaboration with industry, to inform researchers on expected numbers and prognosis, to define research priorities and research programmes.

Carmen Martinez provided the epidemiological and public health point of view. First of all she stressed the importance of having achieved a consensus on what constitutes a rare cancer. The list will be useful to shift

the focus of cancer statistics to rare cancers, to promote collaboration among epidemiologists and researchers, to improve data quality for rare cancers, to create reference centres, to establish preventive measures (where possible) and to uncover and address inequalities.

Annalisa Trama presented the European Commission Communication on Rare Diseases: Europe's challenges and the recently adopted EU Recommendations. Detailed information is available on the EU health portal http://ec.europa.eu/health/ph_threats/non_com/rare_10_en.htm.

Margherita Greco described the psychosocial aspects connected to rare cancers and the special and unique needs of rare cancer patients (need to belong to a wider population (rarity), to reduced feelings of loneliness and isolation, the need to learn new ways of adjustment through a reciprocal model (between patients), to have links that easily connect the patients to referral centres.
It was also felt that the key word to addressing psychosocial needs is networking since networking allows patients to feel part of a wider population.

Gemma Gatta summarised the discussion concluding that all comments had been very positive and supportive of the list and its rationale. Accordingly the list (with the EANO minor changes) should be considered final.

The final list will be uploaded on the RARECARE web-site and will be disseminated widely accordingly to the suggestions and availability expressed by participants. RARECARE will follow-up with patient associations and oncologic societies to finalise procedures and to ensure the dissemination of the list to all different stakeholders.

There was no other business, the meeting ended at 4.30pm