

CONSENSUS MEETING AND CONFERENCE

Improving the Methodology of Clinical Research on Rare Cancers

Hotel Bedford Brussels, 135-137, rue du Midi, 1000 Brussels, Belgium

9 February 2012 (Consensus Meeting)

10.30 – 11.00	Registration and Coffee
11.00 – 11.15	Welcome and Introduction <i>P. G. Casali, European Society for Medical Oncology (ESMO), IT</i>
11.15 – 13.00	Plenary Session <i>Chair: J-Y. Blay, European Organisation for Research and Treatment of Cancer (EORTC), FR</i> Reaching a Multi-Stakeholder Consensus on Improving the Methodology of Clinical Research on Rare Cancers <ol style="list-style-type: none"> 1. Rare cancers as an issue 2. Designs of clinical studies 3. End-points of clinical studies 4. Summarizing available evidence 5. Organizational and regulatory aspects of clinical studies
13.00 – 14.00	Lunch Break
14.00 – 15.30	Plenary Session Reaching a Multi-Stakeholder Consensus... (cont'd) <ol style="list-style-type: none"> 1. Rare cancers as an issue 2. Designs of clinical studies 3. End-points of clinical studies 4. Summarizing available evidence 5. Organizational and regulatory aspects of clinical studies
15.30 – 16.00	Coffee Break
16.00 – 18.00	Plenary Session Reaching a Multi-Stakeholder Consensus... (cont'd) <ol style="list-style-type: none"> 1. Rare cancers as an issue 2. Designs of clinical studies 3. End-points of clinical studies 4. Summarizing available evidence 5. Organizational and regulatory aspects of clinical studies
18.00 – 18.30	Plenary Session Wrap-Up and Concluding Remarks
18.30	Meeting Close
20.00	Dinner

10 February 2012 (Rare Cancers Conference)

8.00 – 8.30	Registration
8.30 – 8.40	<p>Welcome and Introduction</p> <p><i>P. G. Casali, European Society for Medical Oncology (ESMO), IT;</i> <i>Y. Le Cam, European Organisation for Rare Diseases (EURORDIS), FR,</i> <i>on behalf of EURORDIS and European Cancer Patient Coalition (ECPC)</i></p>
8.40 – 10.10	<p>Plenary Session</p> <p><i>Co-chairs: M. Pierotti, Organisation of European Cancer Institutes (OECI), IT;</i> <i>K. Oliver, International Brain Tumour Alliance (IBTA) and Cancer 52, UK</i></p> <p>Rare Cancers – An Overview</p> <ul style="list-style-type: none"> • The epidemiology of rare cancers in Europe <i>A. Trama, Fondazione IRCCS Istituto Nazionale dei Tumori, IT</i> • A list of rare cancers <i>A. P. Dei Tos, EuroBoNeT/EUROSARC, IT</i> • Significant benefit and clinical added value of orphan drugs <i>Y. Le Cam, EURORDIS, FR</i> • Rare cancers and drug development in Europe <i>B. Jonsson, Läkemedelsverket (Swedish Medical Products Agency, MPA), SE</i> • Remaining obstacles towards pharmaceutical development of treatments for rare cancers in Europe <i>A. Fehervary, Novartis Oncology Region Europe, IT</i> • Clinical practice guidelines to set standards at the European level <i>A. Cervantes, ESMO, ES</i> • Best practice examples <ul style="list-style-type: none"> ○ An attempt of a rare cancer community to set European multidisciplinary standards <i>L. Licitra, Fondazione IRCCS Istituto Nazionale dei Tumori, IT</i> ○ Le Cercle des Tumeurs Rares and INCa, France <i>J.-Y. Blay, EORTC, FR</i>
10.10 – 10.30	Discussion
10.30 – 10.50	Coffee Break
10.50 – 12.20	<p>Plenary Session</p> <p><i>Co-chairs: S. van Belle, University Hospital Ghent, BE;</i> <i>J. Geissler, CML Advocates Network, DE</i></p> <p>Clinical Research on Rare Cancers – An Introduction</p> <ul style="list-style-type: none"> • The changing environment of cancer science <i>TBC</i> • Regulatory and legal EU constraints to clinical studies on rare cancers <i>A. Negrouk, European Organisation for Research and Treatment of Cancer (EORTC), BE</i> • New collaboration models in rare cancers <i>D. Lacombe, EORTC, BE</i> • Methodological challenges <i>J. Bogaerts, EORTC, BE</i> • Best practice examples <ul style="list-style-type: none"> ○ The International Rare Cancers Initiative <i>M. Seymour, National Cancer Research Network, UK</i> ○ Paediatric oncology <i>G. Vassal, European Society for Paediatric Oncology (SIOPE), FR</i>

	<ul style="list-style-type: none"> ○ Patient involvement in clinical studies <i>Š. Narbutas, ECPC, LT</i>
12.20 – 12.40	Discussion
12.40 – 13.30	Lunch Break
13.30 – 14.30	<p>Plenary Session <i>Co-chairs: K. Redmond, Cancer World Magazine, European School of Oncology (ESO), CH; co-chair TBC</i></p> <p>Consensus Recommendations on Improving the Methodology of Clinical Research on Rare Cancers</p> <ul style="list-style-type: none"> ● Rare Cancers Europe <i>R. Schaefer, Rare Cancers Europe, DE</i> ● Designs of clinical studies in rare cancers <i>J. Bogaerts, EORTC, BE</i> ● Bayesian approaches to analysing studies and summarizing evidence <i>P. Bruzzi, National Cancer Research Institute, IT</i> ● Bringing evidence within clinical decision-making <i>P. G. Casali, ESMO, IT</i> ● Organizational and regulatory aspects of clinical studies <i>J.-Y. Blay, EORTC, FR</i>
14.30 – 14.50	Discussion
14.50 – 15.50	<p>Panel Discussion and Plenary Debate <i>Co-chairs: A. Cervantes, ESMO, ES; W. Yared, Association of European Cancer Leagues (ECL), BE</i></p> <p>What Stakeholders Can Do to Drive Clinical Research on Rare Cancers</p> <ul style="list-style-type: none"> ● Medical practice / Clinical research <i>P. G. Casali, ESMO, IT; J.-Y. Blay, EORTC, FR; A. Margulies, European Oncology Nursing Society (EONS), CH</i> ● Statistics / Epidemiology <i>J. Bogaerts, EORTC, BE; P. Bruzzi, National Cancer Research Institute, IT</i> ● Regulatory Affairs / Health administration <i>B. Jonsson, MPA, SE; EU Commission DG Research, BE, TBC</i> ● Pharmaceutical industry <i>S. Comis, Novartis Oncology, IT; Pfizer Oncology, TBC; Sanofi, TBC</i> ● Biology / Pathology <i>A. P. Dei Tos, European Society of Pathology (ESP), IT</i> ● Patient advocacy <i>S. Craine, The CML Support Group, UK; Jayne Bressington, GIST Support UK & PAWS-GIST, UK; J. Geissler, EURORDIS and CML Advocates Network, DE; J. Pelouchová, ECPC, CZ</i>
15.50 – 16.00	<p>Plenary Session</p> <p>Outlook and Concluding Remarks <i>P. G. Casali, ESMO, IT; F. De Lorenzo, ECPC, IT, on behalf of ECPC and EURORDIS</i></p>
16.00	Coffee and Departures

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 (EURORDIS), the European Cancer Patient Coalition (ECPC), the European Organisation for Research and Treatment of Cancer (EORTC), CONTICANET, EuroBoNeT, the Association of European Cancer Leagues (ECL), the Chronic

Myeloid Leukaemia Support Group, the International Brain Tumour Alliance (IBTA), Orphanet, the Chronic Myeloid Leukaemia Advocates Network, GIST Support UK & PAWS-GIST, Cancer 52, the Fondazione IRCCS Istituto Nazionale dei Tumori, the European Institute of Oncology (IEO), the European Society for Paediatric Oncology (SIOP Europe), the European School of Oncology (ESO), the European Oncology Nursing Society (EONS), the European Society of Pathology (ESP), 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 Takeda Pharmaceuticals Europe (Silver Industry Supporter).

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