Background and objectives

The European Medicines Agency (EMA) and the European Organisation of Research and Treatment of Cancer (EORTC) are organising a workshop on soft tissue and bone sarcoma specifically addressing the question on how we can develop new treatments in ultra-rare sarcomas, as a model for ultra-rare tumours. This workshop will bring together academia, learned societies, patients, non-profit organisation, medicines regulators to explore clinical and scientific aspects related to the development of medicines for ultra-rare cancers focusing on methodological aspects of clinical studies (such as the use of master protocols), repurposing medicines, and the use of retrospective and real-world data, and prospective registries for further data collection.

The aims of the workshop are to:

- Discuss points to consider for developing rare cancer medicines using ultra-rare soft tissue and bone sarcomas as examples;
- Facilitate interactions among relevant stakeholders aiming at international collaboration;
- Explore a framework for regular meetings between the adult sarcoma community and EMA.
How can we develop new treatments in ultra-rare sarcomas, as a model for ultra-rare tumours?

Chairled by Pierre Demolis and Silvia Stacchiotti

10:45 Jointing and technical checks

11:00 Welcome

Harald Enzmann (chair of the CHMP, EMA)

11:05 Introduction and meeting objectives

Silvia Stacchiotti (EORTC) and Pierre Demolis (EMA)

11:15 Session 1: Background

EMA regulatory framework for rare disease  15’
Ralf Herold, Head of work stream Regulatory Science and Academia, EMA

Ultra-rare sarcoma: major challenges and opportunities  15’
the patient perspective
Hugh Leonard, Epithelioid Haemangioendothelioma (EHE) Rare Cancer Charity, UK
Josh Sommer, Chordoma Foundation, US

Ultra-rare tumours: major challenges and opportunities,  10’
the EORTC perspective
Denis Lacombe, Chief Executive Officer, EORTC

Generating the right data: randomized or not;  15’
observational or prospective
Kit Roes, chair of the EMA Methodology Working Party

Lessons learned from compassionate use program  10’
Valerie Denux, Director of Europe and Innovation, ANSM

12:30 Lunch Break
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<tr>
<th>Time</th>
<th>Session 2: How to establish a framework for ultra rare sarcomas?</th>
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<tbody>
<tr>
<td>13:30</td>
<td>Experience from ACCELERATE program for the paediatric community 15’&lt;br&gt;Teresa De Rojas, Scientific Coordinator ACCELERATE</td>
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<td>How to collect retrospective data to be used for the development of new treatment for ultra-rare sarcoma 15’&lt;br&gt;Anna Maria Frezza, Department of Medical Oncology, IRCCS Fondazione Istituto Nazionale Tumori, Italy&lt;br&gt;Judith Bovee, Professor of Pathology of bone- and soft tissue tumors, NL</td>
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<td>Repurposing of old drugs in new ultra-rare indication: example of Sirolimus in EHE 10’&lt;br&gt;Pan Pantziarka, Anti-Cancer Fund, Belgium&lt;br&gt;Sandrine Marreaud, EORTC</td>
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<td>Prospective studies in ultra-rare sarcomas: nab-sirolimus in PEComa as an example 10’&lt;br&gt;Andrew Wagner, Associate Professor, Medicine, Harvard Medical School and Senior Physician, Adult Oncology, Dana-Farber Cancer Institute, US</td>
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<td>Prospective registries 15’&lt;br&gt;Annalisa Trama, Fondazione IRCCS Istituto Nazionale Tumori, Italy</td>
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<td>A prospective master protocol / platform to conduct international multicentric single-arm studies in ultra-rare sarcomas including real world data for external comparisons 15’&lt;br&gt;Lorenzo D’Ambrosio, Department of Oncology, San Luigi di Orbassano, Italy&lt;br&gt;Gautier Bouche, Anti-Cancer Fund, Belgium&lt;br&gt;Rosalba Miceli, Director of the Biostatistics for Clinical Research Unit at Fondazione IRCCS, Italy</td>
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<td>15:00</td>
<td>Coffee Break</td>
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<td>15:15</td>
<td>NCI childhood Cancer Data Initiatives 15’&lt;br&gt;Brigitte Widemann, Chief of the NCI Center for Cancer Research Pediatric Oncology Branch and special Advisor on Childhood Cancer to the NCI Director, US</td>
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<td>FDA perspectives on rare cancer development 15’&lt;br&gt;Caitlin Tydings, Clinical reviewer for the Sarcoma team, FDA</td>
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15:45  Panel Discussion

Moderator: Silvia Stacchiotti (EORTC), Pierre Demolis (EMA)

Panel discussion 60’
All speakers with additional panellists:
Martha Donoghue, Associate Director of Paediatric Oncology and Rare Cancers, FDA
Hugh Leonard – patient’s perspective
William Tap, Chief, Sarcoma Medical Oncology Service, Memorial Sloan Kettering Cancer Center, US
Wim Oyen, Nuclear Medicine Physician, Arnhem, The Netherlands

16:50  Closing Remarks

Take home message and conclusions 10’
Silvia Stacchiotti (EORTC) and Pierre Demolis (EMA)

17:00  End of meeting