



EMA AND EORTC SOFT TISSUE AND BONE SARCOMA WORKSHOP

# How can we develop new treatments in ultra-rare sarcomas, as a model for ultra-rare tumours?

**12 January 2024**

Hybrid meeting - EMA (meeting room 2A), Amsterdam and virtual

## **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?

Chaired by Pierre Demolis and Silvia Stacchiotti

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## 10:45 Joining and technical checks

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## 11:00 Welcome

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*Harald Enzmann (chair of the CHMP, EMA)*

## 11:05 Introduction and meeting objectives

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*Silvia Stacchiotti (EORTC) and Pierre Demolis (EMA)*

## 11:15 Session 1: Background

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### **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 the patient perspective** **15'**

*Hugh Leonard, Epithelioid Haemangioendothelioma (EHE) Rare Cancer Charity, UK*

*Josh Sommer, Chordoma Foundation, US*

### **Ultra-rare tumours: major challenges and opportunities, the EORTC perspective** **10'**

*Denis Lacombe, Chief Executive Officer, EORTC*

### **Generating the right data: randomized or not; observational or prospective** **15'**

*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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**13:30**      **Session 2: How to establish a framework for ultra rare sarcomas?**

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**Experience from ACCELERATE program for the paediatric community**      **15'**  
*Teresa De Rojas, Scientific Coordinator ACCELERATE*

**How to collect retrospective data to be used for the development of new treatment for ultra-rare sarcoma**      **15'**  
*Anna Maria Frezza, Department of Medical Oncology, IRCCS Fondazione Istituto Nazionale Tumori, Italy*  
*Judith Bovee, Professor of Pathology of bone- and soft tissue tumors, NL*

**Repurposing of old drugs in new ultra-rare indication: example of Sirolimus in EHE**      **10'**  
*Pan Pantziarka, Anti-Cancer Fund, Belgium*  
*Sandrine Marreaud, EORTC*

**Prospective studies in ultra-rare sarcomas: nab-sirolimus in PEComa as an example**      **10'**  
*Andrew Wagner, Associate Professor, Medicine, Harvard Medical School and Senior Physician, Adult Oncology, Dana-Farber Cancer Institute, US*

**Prospective registries**      **15'**  
*Annalisa Trama, Fondazione IRCCS Istituto Nazionale Tumori, Italy*

**A prospective master protocol / platform to conduct international multicentric single-arm studies in ultra-rare sarcomas including real world data for external comparisons**      **15'**  
*Lorenzo D'Ambrosio, Department of Oncology, San Luigi di Orbassano, Italy*  
*Gautier Bouche, Anti-Cancer Fund, Belgium*  
*Rosalba Miceli, Director of the Biostatistics for Clinical Research Unit at Fondazione IRCCS, Italy*

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**15:00**      **Coffee Break**

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**15:15**      **Session 2: How to establish a framework for ultra rare sarcomas? (continued)**

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**NCI childhood Cancer Data Initiatives**      **15'**  
*Brigitte Widemann, Chief of the NCI Center for Cancer Research Pediatric Oncology Branch and special Advisor on Childhood Cancer to the NCI Director, US*

**FDA perspectives on rare cancer development**      **15'**  
*Caitlin Tydings, Clinical reviewer for the Sarcoma team, FDA*

**15:45 Panel Discussion**

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*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**

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**Take home message and conclusions**

**10'**

*Silvia Stacchiotti (EORTC) and Pierre Demolis (EMA)*

**17:00 End of meeting**

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