



EMA And EORTC Soft Tissue And Bone Sarcoma Workshop

# Follow-up workshop: How to develop new treatments in ultra-rare sarcomas, as a model for ultra-rare tumours?

**24 May 2024**

## **Background and objectives**

The European Medicines Agency (EMA) and the European Organisation of Research and Treatment of Cancer (EORTC) organised a workshop on soft tissue and bone sarcoma on 12<sup>th</sup> January 2024 specifically addressing the question on how to develop new treatments in ultra-rare sarcomas, as a model for ultra-rare tumours. This workshop brought together academia, learned societies, patients, non-profit organisations, and 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, repurposing medicines, and the use of real-world data.

This second workshop is organised to discuss more in depth certain aspects as a follow-up discussion. Specifically, the workshop will cover topics such as how to support an ecosystem for ultra-rare cancers and discuss lessons learned on specific examples.

# How to 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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## 10:30 Joining and technical checks

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## 11:00 Welcome

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

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## 11:05 Meeting objectives and discussion on how to develop new treatment in ultra-rare sarcoma, as a model for ultra-rare tumours

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*Moderators: Silvia Stacchiotti (EORTC) and Pierre Demolis (ANSM, EMA)*

**Setting the scene** 10'  
*Pierre Demolis, ANSM, EMA*

**Summary of last workshop and objectives** 10'  
*Pan Pantziarka, Anticancer Fund*

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## 11:30 Support an ecosystem for ultra rare cancers from diagnosis to treatment

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*Moderators: Ralf Herold (EMA): Winan Van Houdt (EORTC)*

**Patient involvement – hospital cohorts and mobilising the patient community** 15'  
*Hugh Leonard, EHE Rare Cancer Charity*

**Identifying new drugs in ultra-rare indications and off label use** 15'  
*Robin Jones Royal Marsden, London, UK*

**Case example from PUSH: LGFMS/SEF and immunotherapy** 15'  
*Andrew Wagner, DFCC, Boston, US*

**What could the development of medicines in ultra rare indication look like?** 10'  
*Pierre Demolis, chair of the Oncology Working Party and SAWP vice chair, EMA*

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## 12:20 Discussion

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*Moderator: Ralf Herold (EMA), Winan Van Houdt and Silvia Stacchiotti (EORTC)* 40'

*All speakers with additional panellists:*

*Martha Donoghue, associate Director of Paediatric Oncology and Rare Cancers, FDA*

*Nicole Scobie, Accelerate*

*Kit Roes, chair of the Methodology working Party, EMA*

**13:00 Lunch Break**

**14:00 Practical cases: what have we learned?**

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*Moderators: Caroline Voltz (EMA), Denis Lacombe (EORTC)*

**What is important for patients in addition to RECIST and overall survival? 15'**

*Gerard van Oortmerssen, SPAGN*

**Use of real word data to complement prospective studies: case example in alveolar soft parts sarcoma and epithelioid sarcoma 15'**

*William Tap, MSKCC, New York, US*

**Developing new criteria for response assessment: Case example of epithelioid haemangioendothelioma 15'**

*Lorenzo D'Ambrosio, University of Turin, Turin, Italy*

**Repurposing: case example of sirolimus in epithelioid haemangioendothelioma**

*Denise Robinson, EHE Group, US 15'*

**Engaging companies in academic trials of Ultra Rare Tumours – Hopes and hurdles 15'**

*Gauthier Bouche, Anticancer Fund*

**15:15 Coffee Break**

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**15:45 Discussion**

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*Moderators: Caroline Voltz (EMA): Denis Lacombe (EORTC)*

*All speakers with additional panellists:*

*Caitlin Tydings, clinical reviewer for the Sarcoma team FDA*

*Kit Roes, chair of the Methodology working Party, EMA*

**16:45 Closing Remarks**

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**Take home message and conclusions 10'**

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

**16:50 End of meeting**

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