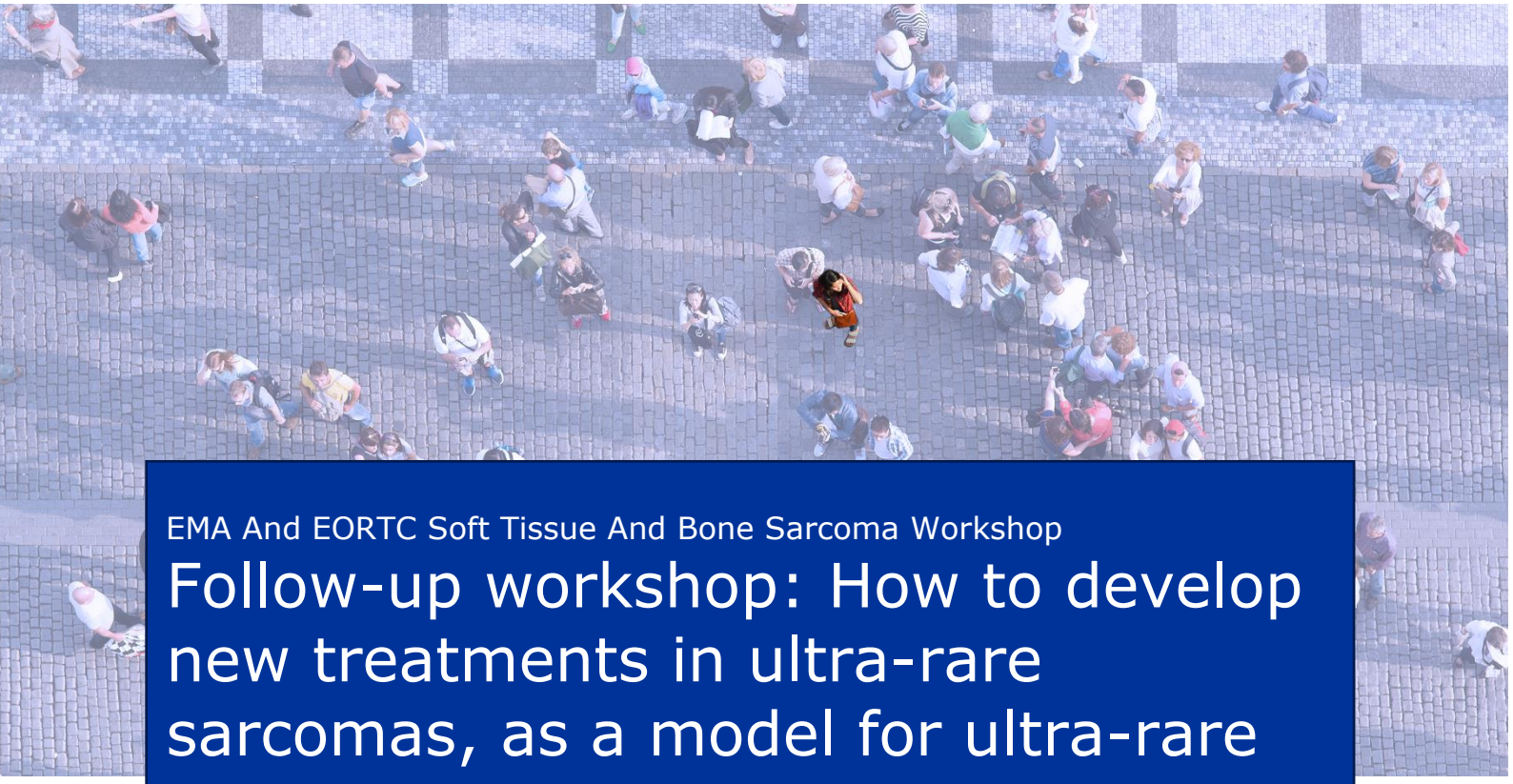

EMA And EORTC Soft Tissue And Bone Sarcoma Workshop

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 12th 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

10:30 Joining and technical checks

11:00 Welcome

Harald Enzmann (chair of the CHMP, EMA)

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

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

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

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

12:20 Discussion

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

All speakers with additional panellists:

Caitlin Tydings, Clinical reviewer for the Sarcoma team FDA (TBC)

Nicole Scobie, Accelerate

13:00 **Lunch Break**

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

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

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

Gerard van Oortmerssen, SPAGN

External control: The challenge in ultra-rare cancers and how to do better in the future **15'**

Kit Roes, chair of the Methodology working Party, EMA (TBC)

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

15:30 **Coffee Break**

15:50 **Discussion**

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

All speakers with additional panellist:

Martha Donoghue, Associate Director of Paediatric Oncology and Rare Cancers, FDA (TBC)

16:45 **Closing Remarks**

Take home message and conclusions **10'**

Silvia Stacchiotti (EORTC) and Pierre Demolis (EMA)

16:50 **End of meeting**