EMA and EORTC STBSG WORKSHOP

HOW CAN WE DEVELOP NEW TREATMENTS IN ULTRA-RARE SARCOMAS, AS A MODEL FOR ULTRA-RARE TUMOURS?

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Disclosures*

Personal financial interests (honoraria, consultancy or advisory role):

Agenus, Bayer, Boheringer, Daiichi Sankyo, Ikena, Gentili, NEC Oncolimmunity, Novartis, Pharmamar, Pharma Essentia, Servier

Institutional financial interests:

Advenchen, Bayer, Blueprint, Boehringer, Daiichi Sankyo, Deciphera, Epizyme, Foghorn, Hutchinson, Inhbrix, Karyopharm, Novartis, Pharmamar, RainThera, Springworks

*last 2 years

BACKGROUND


EORTC
The future of cancer therapy
Anticancer Fund
EMA Innovation Task Force (ITF) / EORTC STBSG
Briefing Meeting
18 May 2020
Tyrosine kinase inhibitor plus
PDL1 inhibitor in the treatment of
Alveolar Soft Part Sarcomas
@EORTC


THE
EHE
FOUNDATION
Anticancer Fund
Scientific Advice
0000122255
SIROLIMUS IN
EPITHELIOID HEMANGIOENDOTHELIOMA
(EHE)
EUROPEAN
MEDICINES
AGENCY
Info on repurposing pilot from EMA
7th Meeting of the industry stakeholder platform on the operation of the centralised procedure for human medicines
1st December 2021, Session 1: 13:10 – 13:50 (CET)
Presented by Christelle Bouygues, Regulatory Affairs Office, EMA
An agency of the European Union

TO FIGHT DISCRIMINATION AGAINST RARE TUMOURS

EMA

EORTC STBSG

>90 MALIGNANCIES

MOST EXTREMELY RARE

De Pinieux G et al, Plos One 2021

RARITY & HETEROGENEITY

Limited
preclinical / clinical / epidemiological
data

R unfeasible

Lower level
of the available evidence

Larger degree of uncertainty
in clinical decision

Inconsistency
in pt care and
Pt discrimination

Few treatments available

Off-label

Less attractive
for the industry
(expecially for agents
already approved in other indications)

Less funding

Regulatory issues

Few (sponsored) prospective clinical studies

Few approved drugs

AGREE ON A DEFINITION

Original Article

Ultra-Rare Sarcomas: A Consensus Paper From the Connective Tissue Oncology Society Community of Experts on the Incidence Threshold and the List of Entities

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BACKGROUND: Among sarcomas, which are rare cancers, many types are exceedingly rare; however, a definition of *ultra-rare* cancers has not been established. The problem of ultra-rare sarcomas is particularly relevant because they represent unique diseases, and their rarity poses major challenges for diagnosis, understanding disease biology, generating clinical evidence to support new drug development, and achieving formal authorization for novel therapies. **METHODS:** The Connective Tissue Oncology Society promoted

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ULTRA-RARE SARCOMA

INCIDENCE </= 1/1,000,000/year

Stacchiotti S et al, Cancer 2021

TABLE 1. List of Ultra-Rare Soft Tissue Sarcomas Identified Based on Incidence and of Ultra-Rare Soft Tissue Sarcomas Identified on Expert Consensus Only

Incidence Based on Population-Based Registries (RARECARENet EU, Asia, NETSARC)	WHO (Soft Tissue and Bone Tumors, Gynecologic, Head and Neck, Hematologic)[a]
Adult fibrosarcoma	
Alveolar rhabdomyosarcoma	
Alveolar soft part sarcoma	
Angiomatoid fibrous histiocytoma	
Clear cell sarcoma	
Desmoplastic small round cell tumor	
Ectomesenchymoma	
Embryonal rhabdomyosarcoma	
Embryonal sarcoma of the liver	
Endometrial stromal sarcoma	High-grade *BCOR*-rearranged endometrial stromal sarcoma
	High-grade *YWHAE*-rearranged endometrial stromal sarcoma
Endometrial stromal sarcoma, low grade	
Epithelioid sarcoma	
Extrarenal malignant rhabdoid tumor	
Extraskeletal Ewing sarcoma	
Extraskeletal myxoid chondrosarcoma	
Extraskeletal osteosarcoma	
Fibroblastic reticular cell tumor	
Follicular dendritic cell sarcoma	
Giant cell tumor of soft tissues	
Hemangioendothelioma, composite	
Hemangioendothelioma, epithelioid	
Hemangioendothelioma, pseudomyogenic	
Hemangioendothelioma, retiform	
Histiocytic sarcoma	
Infantile fibrosarcoma	
Inflammatory myofibroblastic tumor	
Interdigitating dendritic cell sarcoma	Indeterminate dendritic cell tumor
	Interdigitating dendritic cell sarcoma
Intimal sarcoma	
Langerhans cell sarcoma	
Low-grade fibromyxoid sarcoma	
Low-grade myofibroblastic sarcoma	
Malignant glomus tumor	
Malignant granular cell tumor	
Malignant myoepithelioma/myoepithelial carcinoma	
Malignant tenosynovial giant cell tumor	
Myxoinflammatory fibroblastic sarcoma	
Ossifying fibromyxoid tumor, malignant	
Papillary intralymphatic angioendothelioma	
PEComa, excluding nonepithelioid angiomyolipoma	
Phyllodes tumor, malignant	
Phosphaturic mesenchymal tumor, malignant	
Pleomorphic liposarcoma	
Pleomorphic rhabdomyosarcoma	
Round cell sarcoma/Ewing-like sarcoma	*CIC*-rearranged sarcoma
	Round cell sarcoma with *EWSR1*–non-*ETS* fusions
	Sarcoma with *BCOR* genetic alterations
Sclerosing epithelioid fibrosarcoma	
Spindle cell/sclerosing rhabdomyosarcoma	
	Biphenotypic sinonasal sarcoma
	Inflammatory leiomyosarcoma
	Malignant melanotic nerve sheath tumor
	Metastasizing leiomyoma
	Myxoid pleomorphic liposarcoma
	NTRK-rearranged spindle cell sarcoma (emerging)

TABLE 2. List of Ultra-Rare Bone Sarcomas Identified Based on Incidence and Based on Expert Consensus Only

Incidence Based on Population-Based Registries (RARECARENet EU, Asia, NETSARC)	WHO[a]
Adamantinoma	
Angiosarcoma of bone	
Chondrosarcoma, clear cell	
Chondrosarcoma, dedifferentiated	
	Chondrosarcoma, periosteal
Chordoma, conventional	
Chordoma, dedifferentiated	
	Chordoma, poorly-differentiated
Epithelioid hemangioendothelioma of bone	
Fibrosarcoma of bone	
Leiomyosarcoma of bone	
Low-grade central osteosarcoma	
Malignancy in giant cell tumor of bone/giant cell tumor of bone, malignant	
Mesenchymal chondrosarcoma	
Osteosarcoma, parosteal	
Osteosarcoma, periosteal	
Osteosarcoma, high-grade surface	
Undifferentiated high-grade pleomorphic sarcoma of the bone	
	Rhabdomyosarcoma of the bone
	CIC-rearranged sarcoma
	Round cell sarcoma with *EWSR1*–non-*ETS* fusions
	Sarcoma with *BCOR* genetic alterations

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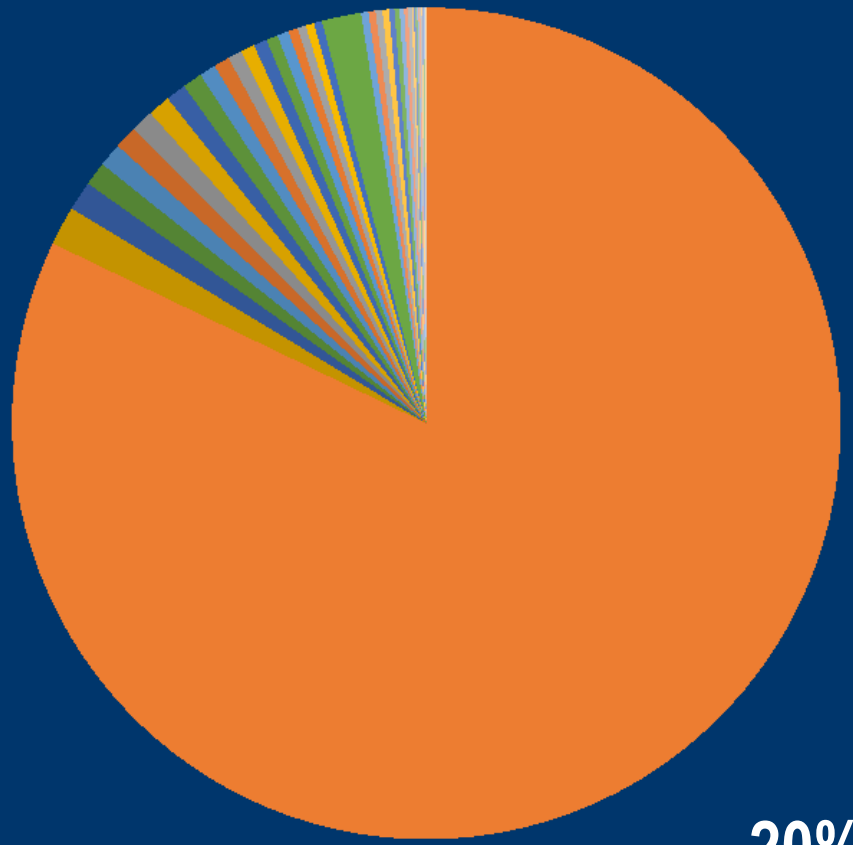
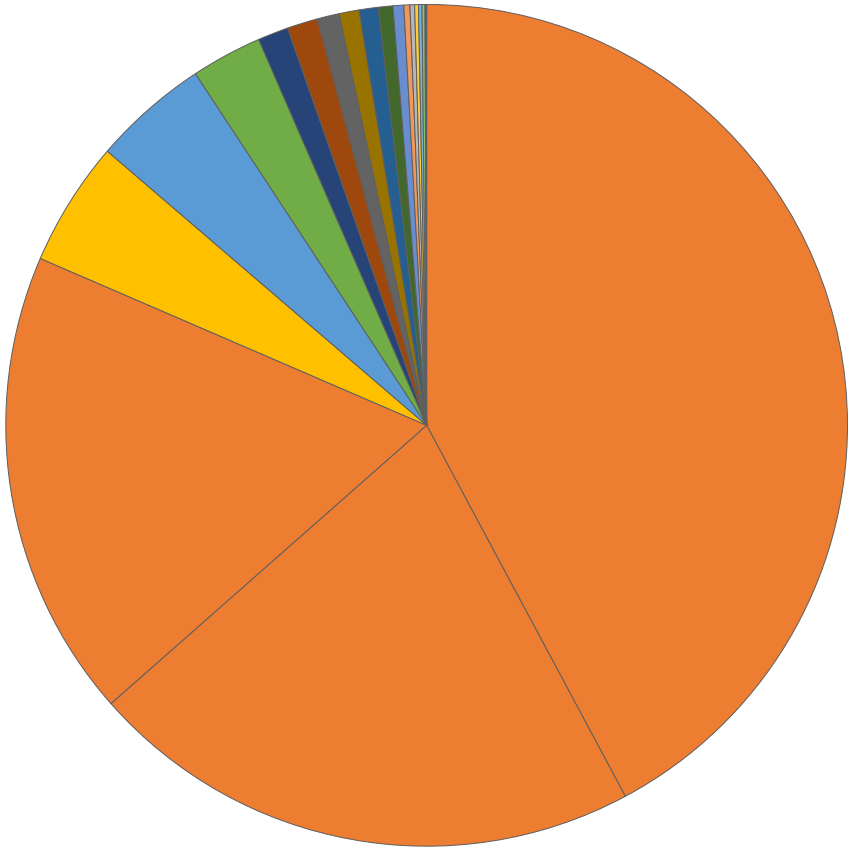
soft tissue, 56 types



bone, 22 types



20% of pts
with soft tissue bone sarcoma

Stacchiotti S et al, Cancer 2021

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Alveolar soft part sarcoma	
Angiomatoid fibrous histiocytoma	
Clear cell sarcoma	
Desmoplastic small round cell tumor	
Ectomesenchymoma	
Embryonal rhabdomyosarcoma	
Embryonal sarcoma of the liver	
Endometrial stromal sarcoma	High-grade *BCOR*-rearranged endometrial stromal sarcoma High-grade *YWHAE*-rearranged endometrial stromal sarcoma
Endometrial stromal sarcoma, low grade	
Epithelioid sarcoma	
Extrarenal malignant rhabdoid tumor	
Extraskeletal Ewing sarcoma	
Extraskeletal myxoid chondrosarcoma	
Extraskeletal osteosarcoma	
Fibroblastic reticular cell tumor	
Follicular dendritic cell sarcoma	
Giant cell tumor of soft tissues	
Hemangioendothelioma, composite	
Hemangioendothelioma, epithelioid	
Hemangioendothelioma, pseudomyogenic	
Hemangioendothelioma, retiform	
Histiocytic sarcoma	
Infantile fibrosarcoma	
Inflammatory myofibroblastic tumor	
Interdigitating dendritic cell sarcoma	Indeterminate dendritic cell tumor Interdigitating dendritic cell sarcoma
Intimal sarcoma	
Langerhans cell sarcoma	
Low-grade fibromyxoid sarcoma	
Low-grade myofibroblastic sarcoma	
Malignant glomus tumor	
Malignant granular cell tumor	
Malignant myoepithelioma/myoepithelial carcinoma	
Malignant tenosynovial giant cell tumor	
Myxoinflammatory fibroblastic sarcoma	
Ossifying fibromyxoid tumor, malignant	
Papillary intralymphatic angioendothelioma	
PEComa, excluding nonepithelioid angiomyolipoma	
Phyllodes tumor, malignant	
Phosphaturic mesenchymal tumor, malignant	
Pleomorphic liposarcoma	
Pleomorphic rhabdomyosarcoma	
Round cell sarcoma/Ewing-like sarcoma	*CIC*-rearranged sarcoma Round cell sarcoma with *EWSR1–non-ETS* fusions Sarcoma with *BCOR* genetic alterations
Sclerosing epithelioid fibrosarcoma	
Spindle cell/sclerosing rhabdomyosarcoma	
	Biphenotypic sinonasal sarcoma
	Inflammatory leiomyosarcoma
	Malignant melanotic nerve sheath tumor
	Metastasizing leiomyoma
	Myxoid pleomorphic liposarcoma
	NTRK-rearranged spindle cell sarcoma (emerging)

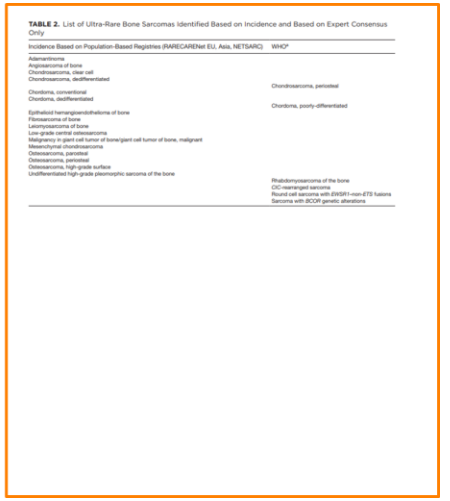

TABLE 2. List of Ultra-Rare Bone Sarcomas Identified Based on Incidence and Based on Expert Consensus Only

Incidence Based on Population-Based Registries (RARECARENet EU, Asia, NETSARC)	WHO[a]
Adamantinoma	
Angiosarcoma of bone	
Chondrosarcoma, clear cell	
Chondrosarcoma, dedifferentiated	
	Chondrosarcoma, periosteal
Chordoma, conventional	
Chordoma, dedifferentiated	
	Chordoma, poorly-differentiated
Epithelioid hemangioendothelioma of bone	
Fibrosarcoma of bone	
Leiomyosarcoma of bone	
Low-grade central osteosarcoma	
Malignancy in giant cell tumor of bone/giant cell tumor of bone, malignant	
Mesenchymal chondrosarcoma	
Osteosarcoma, parosteal	
Osteosarcoma, periosteal	
Osteosarcoma, high-grade surface	
Undifferentiated high-grade pleomorphic sarcoma of the bone	
	Rhabdomyosarcoma of the bone
	CIC-rearranged sarcoma
	Round cell sarcoma with *EWSR1–non-ETS* fusions
	Sarcoma with *BCOR* genetic alterations

ASPS

CEDIRANIB VS PLACEBO
CEDIRANIB VS SUNITINIB

Larotrectinib Entrectinib	NTRK-rearranged sarcomas
Avapritinib	PGDFRA D842V GIST
Crizotinib	Inflammatory myofibroblastic tumor (paed pts)

Larotrectinib Entrectinib	NTRK-rearranged sarcomas
Avapritinib	PGDFRA D842V GIST
Crizotinib	Inflammatory myofibroblastic tumor (all ages)
Pexidartinib	Tenosynovial giant cell tumor
nab-Sirolimus	PEComa
Tazemetostat	Epithelioid sarcoma
Atezolizumab	Alveolar Soft Part Sarcoma

ESMO GOOD SCIENCE BETTER MEDICINE BEST PRACTICE

ANNALS OF ONCOLOGY driving innovation in oncology

SPECIAL ARTICLE

Soft tissue and visceral sarcomas: ESMO–EURACAN–GENTURIS Clinical Practice Guidelines for diagnosis, treatment and follow-up☆

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☆Note: Approved by the ESMO Guidelines Committee, EURACAN and GENTURIS: August 2021. This publication supersedes the previously published version – *Ann Oncol.* 2018;29(suppl 4):iv51-iv67.

† Deceased.

Annals of Oncology 2021

STANDARD OF CARE
SURGERY
RADIATION THERAPY
SYSTEMIC THERAPIES
ANTHRACYCLINES
IFOSFAMIDE
TRABECTEDIN
PAZOPANIB
(ERIBULIN)

EORTC
The future of cancer therapy

EMA
EUROPEAN MEDICINES AGENCY

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

10:45 **Joining 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 (ad interim), EMA

Ultra-rare sarcoma: major challenges and opportunities the patient perspective 15'
Hugh Leonard, Epithelioid Haemangioendothelioma (EHE) Rare Cancer Charity, UK
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Ultra-rare tumours: major challenges and opportunities, the EORTC perspective 10'
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Generating the right data: randomized or not; observational or prospective 15'
Kit Roes, chair of the EMA Methodology Working Party

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Valerie Denux, Director of Europe and Innovation, ANSM

12:30 **Lunch Break**

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

Experience from ACCELERATE program for the paediatric community 15'
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Repurposing of old drugs in new ultra-rare indication: example of Sirolimus in EHE 10'
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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

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
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Saskia Litiere, EORTC

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

15:00 **Coffee Break**

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

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, FDA

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

16:50 **Closing Remarks**

Take home message and conclusions 10'
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PUSH
PLATFORM

Pushing Ultra Rare Sarcoma towards Hope

WORKING *ALONGSIDE* WITH THE REGULATORY AGENCIES

THANK YOU!

Silvia Stacchiotti

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HOW CAN WE DEVELOP NEW TREATMENTS IN ULTRA-RARE SARCOMAS, AS A MODEL FOR ULTRA-RARE TUMOURS?

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TO FIGH DISCRIMINATION AGAINST RARE TUMOURS

EMA  EORTC STBSG

OPTIMAL STUDY DESIGN
and
BEST USE OF AVAILABLE DATA

AGREE ON A DEFINITION

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2019 CTOS Annual Meeting

Ultra-rare sarcoma initiative

FONDAZIONE IRCCS ISTITUTO NAZIONALE DEI TUMORI

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