



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

22 March 2018  
EMA/187787/2018  
Human Medicines Research & Development Support Division

European Society for Medical Oncology (ESMO),  
Joint Action on Rare Cancers (JARC),  
Rare Cancers Europe (RCE)  
&  
Oncology Working Party (ONCWP)

Licensing in rare cancers: a role for  
ERNs?

Programme

16 April 2018

European Medicines Agency, London, United Kingdom

Meeting **room 2F**



# Programme details

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## **09:00-11:00**    **Session 1 - Introduction**

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**Chairs:** Paolo G. Casali, *ESMO & JARC*  
Francesco De Lorenzo, *European Cancer Patient Coalition (ECPC)*  
Ruth Ladenstein, *European Reference Network on Paediatric Cancer (ERN PaedCan)*  
Francesco Pignatti, *EMA*

### **09:00-09:15**    **Aims of the meeting**

Chairs

### **09:15-09:30**    **The problem of rare cancer patients**

Francesco De Lorenzo, *ECPC*

### **09:30-09:45**    **The new scenario of ERNs**

Paolo G Casali, *ESMO & JARC*

### **09:45-10:00**    **Small populations – regulatory perspectives**

Daniel O' Connor, *MHRA*

### **10:00-10:15**    **Expanding the label**

Elias Pean, *EMA*

### **10:15-10:30**    **The concept of a disease-“broad advice”**

J. Borras, *JARC*

### **10:30-11:00**    **Discussion**

## **11:00-11:30**    **Coffee break**

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## **11:30-12:30**    **Session 2 – Case study 1: Chordoma**

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**Chairs:** Paolo G. Casali, *Fondazione IRCCS Istituto Nazionale dei Tumori, Italy*  
Kathy Oliver, *RCE*  
Kristina Larsson, *EMA*

### **11:30-11:45**    **The disease: challenges and potential**

Silvia Stacchiotti, *Fondazione IRCCS Istituto Nazionale dei Tumori, Italy*

### **11:45-12:00**    **A perspective on patient's challenge**

J. Sommers, *Chordoma Foundation*

**12:00-12:30**      **Discussion**

**12:30-13:30**      **Lunch break**

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**13:30-14:30**      **Session 3 – Case study 2: Salivary gland tumours**

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**Chairs:**            Lisa Licitra, *Fondazione IRCCS Istituto Nazionale dei Tumori, Italy*  
Olivier Collignon,  
Jeffrey Kaufman, *Adenoid Cystic Carcinoma Research Foundation*

**13:30-13:45**      **The disease: challenges and potential**

Lisa Licitra, *Fondazione IRCCS Istituto Nazionale dei Tumori, Italy*

**13:45-14:00**      **A perspective on patient's challenge**

Jeffrey Kaufman, *Adenoid Cystic Carcinoma Research Foundation*

**14:00-14:30**      **Discussion**

**14:30-15:00**      **Coffee break**

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**15:00–16:30**      **Session 4 – Plenary discussion: Broad advice, accelerating patient access and label expansion within a ERN**

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**Chairs:**            Paolo G. Casali, *ESMO & JARC*  
Francesco De Lorenzo, *ECPC*  
Francesco Pignatti, *EMA*

**15:00-15:15**      **An industry perspective**

Catherine Weil, *Rare Cancers Europe*

**15:15-15:30**

**Accelerating access to patient: Challenges in Biostatistics**

Olivier Collignon

**15:30-15:45**      **ERNs and continuous evidence generation**

S. Boselli, *EURORDIS Rare Diseases Europe*

**15:45-16:15**      **Discussion**

**16:15-16:30**      **Take-home messages**

**Chairs 16:30**      **End of the meeting**

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