Summary of last workshop and objectives

Pan Pantziarka

Anticancer Fund

Areas of agreement

- Ultra rare sarcomas are an exemplar of ultra rare cancers in general
- There is an urgent need to address the issues that are a consequence of low patient numbers
  - Difficulty in accruing patients in clinical trials – making randomisation problematic
  - Lack of interest from commercial drug developers
  - Gaps in knowledge of natural history of the disease
  - Gaps in knowledge of the biology of the disease – especially in those histologies with high phenotypic and genotypic heterogeneity

Areas of agreement…

- Regulatory pathways are better suited to diseases where there is less uncertainty in knowledge (i.e. more patients, randomised controlled trials etc)
- Clinicians and academics in the ultra rare sarcoma space may be less familiar with regulatory requirements
- Programs such as ACCELERATE can help bring together regulators, patients, clinicians and pharma to speed up and deepen collaboration
- Need to prepare for the positive changes that may arise from the new pharma regulation (e.g. article 48, article 164, article 84 etc)

Areas of challenge - objectives

- How to integrate non-traditional data streams (e.g. prospective registries, new trial designs etc) into regulatory decision making without compromising regulatory standards
- How to strengthen non-randomised data collection (e.g. pre-specification)
- Building a collaborative environment – agile, regular meetings, co-creation (e.g. PUSH)

Agenda for today

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:
Martha Donoghue, associate Director of Paediatric Oncology and Rare Cancers, FDA
Nicole Scobie, Accelerate
Kit Roes, chair of the Methodology working Party, EMA

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

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, 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**

15:45 **Discussion**

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

The bottom line…

There are very high unmet patient needs that demand that we act together to affect change – and that we do this quickly and rigorously