Issues in rare cancer research in EU
EORTC perspective

Anastassia Negrouk
Head of International Regulatory and Intergroup Office
Rare cancers need high quality clinical research

- Rare cancer cumulative incidence is as high as 22%
- Etiology and molecular pathology is poorly known
- Few therapeutic options based on low level of evidence
- Frequent off label use of drugs
  - Not as attractive for the pharmaceutical industry
  - Randomized clinical trials considered not possible
  - As of yet poor acceptance of adaptive designs and lower level evidence by regulators

*Thus randomized clinical trials are feasible (except for ultra-rare cancers)*

Require large international collaboration
Rare cancers and pathology: background issues

- Rare cancer diagnostics require expert opinion
  - Need for referral
  - No expert may be available in the country where patient lives
    - Cross-border referral

- Experts and reference centers are poorly known to patients and doctors:
  - Patient referral is sub-optimal
  - Long delays before patients get referred
  - Biological material may not be readily available
  - Quality of material not always adequate or quantity insufficient

- Need for a system in place that would swiftly direct rare cancer patient to an expert center together with appropriate data and good quality material
Prerequisites for clinical trials in rare cancers

• Strong referral systems
  • within each MS
  • cross-boarder

• Few reference centers in each country which are
  • quality controlled
  • transparent on
    • results of their research
    • data and material available
    • rules of access to data and material for other researchers
  • networking with each other (further reference of ultra-rare cases)

• involved in high quality international clinical trials
  (driven by both industry and academia)

• involved in high quality international translational research

• maximizing the use of data and material
SPECTA concept:
Screening Platform for Effective Clinical Trial Access

Molecular Screening Platform

1st line trial
2nd line trial
3rd line trial

Standard treatment (no open trial)
Standard treatment
Standard
Standard 1st line
Standard 1st line
Standard 2nd line

Academia investment
Industry cooperation

EORTC: European Organisation for Research and Treatment of Cancer

The future of cancer therapy
EORTC SPECTAprogram
Screen and Treat

**SPECTAplatforms**
- SPECTAcolor
- SPECTAbrain
- SPECTAmel
- others in preparation...

**SPECTApath**
- PathoBiology
- Biobanking
- Scientific/operational support

**SPECTAforum**
- Industry
- EMA (FDA)
- Patient representative

**SPECTAreg**
- Competent bodies
- Regulatory affairs research

The future of cancer therapy
Conclusion:

There is an urgent need systems and regulations enable:

*reference academic clinical research centers*

to *concert efforts* in order to

*have access to a critical mass* of rare cancer cases

to *perform robust & quality controlled*

*international research*
Thank you