

Issues in rare cancer research in EU EORTC perspective

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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

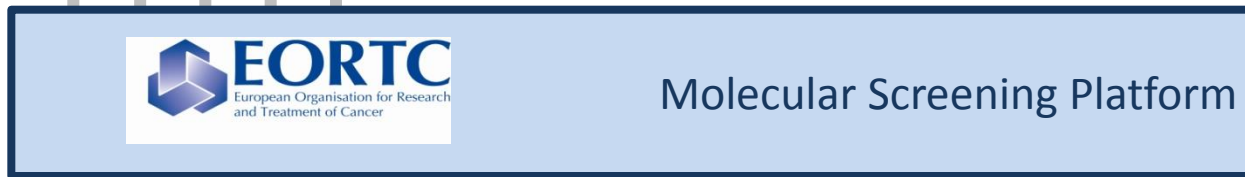
- Rare cancer diagnostics require expert opinion
 - Need for referral
 - No expert may be available in the country where patient lives
 - Cross-border referral
- Experts and reference centers are poorly known to patients and doctors:
 - Patient referral is sub-optimal
 - Long delays before patients get 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

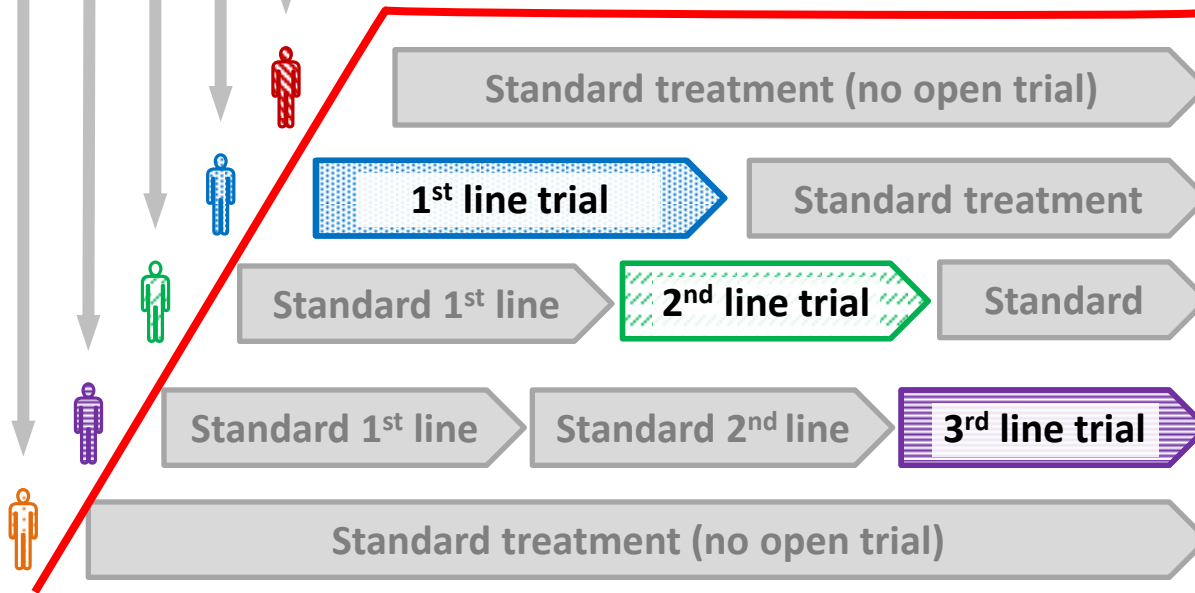
- Strong referral systems
 - within each MS
 - cross-boarder
- Few reference centers in each country which are
 - *quality controlled*
 - *transparent* on
 - results of their research
 - data and material available
 - rules of access to data and material for other researchers
 - *networking* with each 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



Academia investment



Industry cooperation

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

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