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

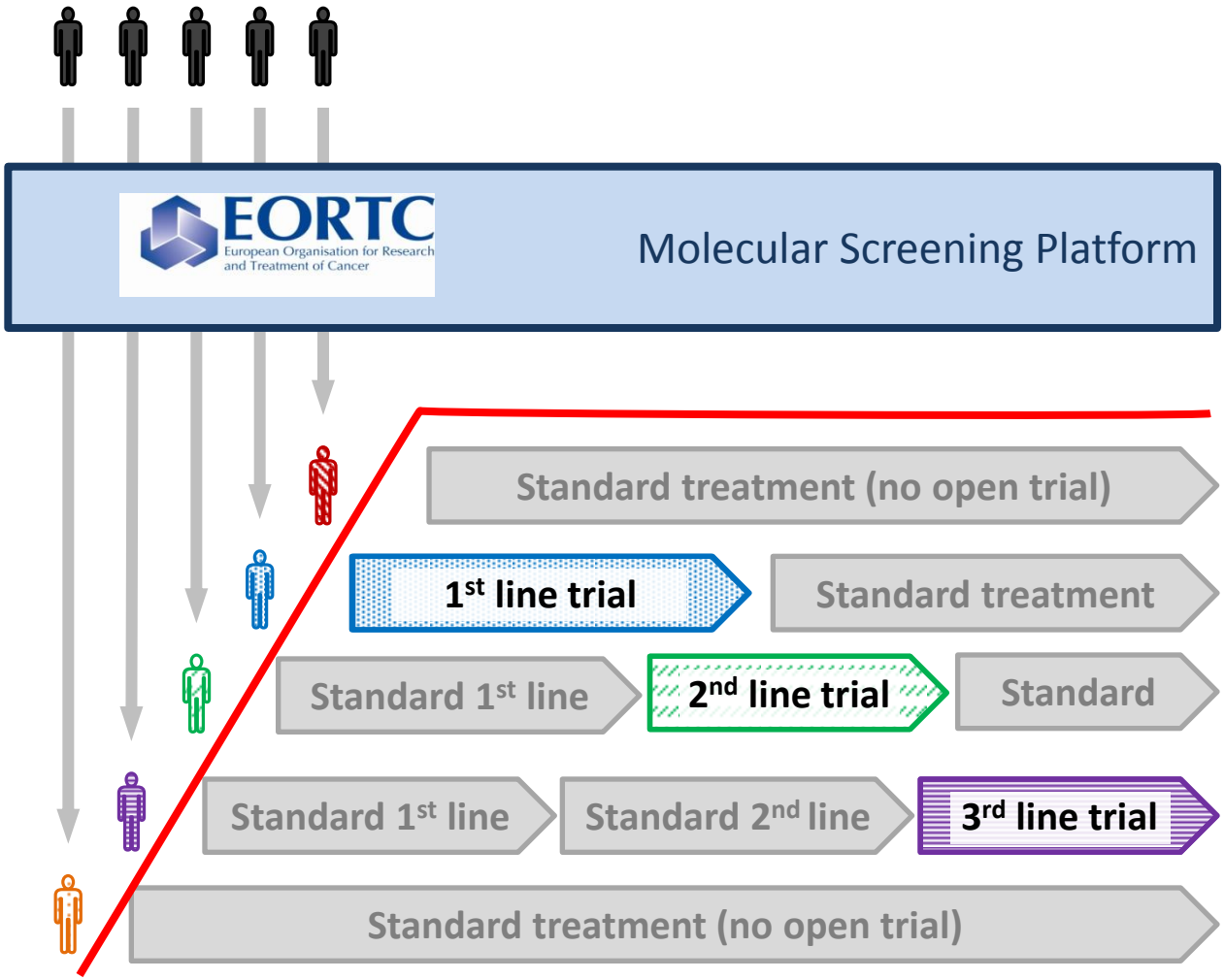
- *Need for a system in place that would **swiftly***
  *direct **rare cancer patient to** an **expert center***
  *together with appropriate **data and good quality material***

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

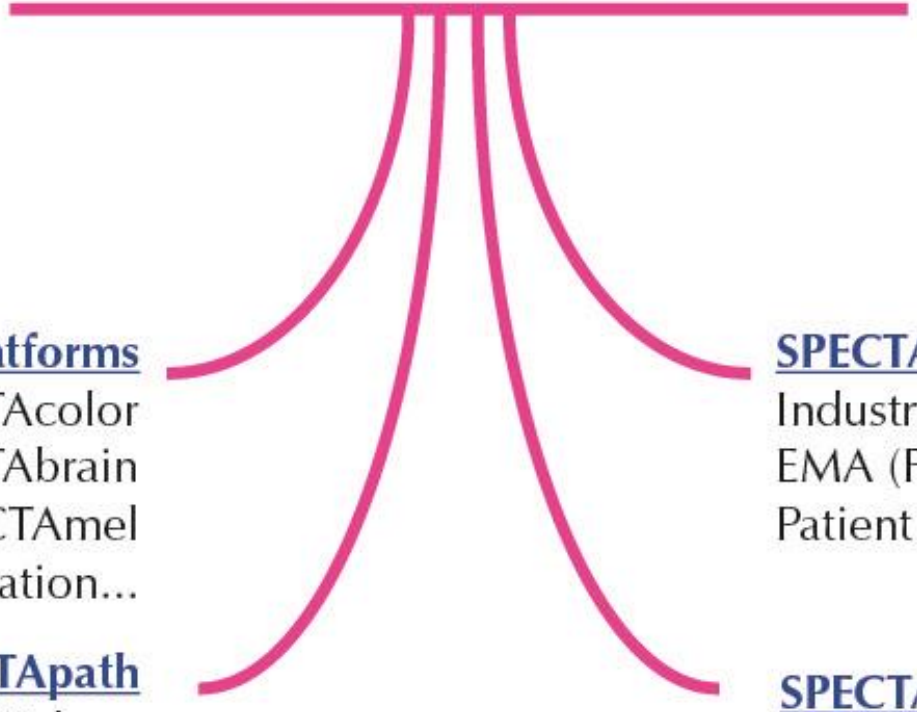


Academia investment

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