

# Organizational aspects of clinical research in rare cancers

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I have no conflicts of interest

# From Dreams to Realities : Setting standards in cancer treatment

A private and non-profit  
cancer research organisation  
founded in 1962

Headquarters based in  
Brussels, Belgium

Core activities are related to  
the design and conduct of  
clinical trials & research across  
a Pan-European Network

## **Specialist skills and capabilities include:**

- Rare cancers
- Translational research
- Biomarkers
- Screening platforms
- Quality assurance
- Quality of life
- Pivotal clinical trials
- Survivorship issues

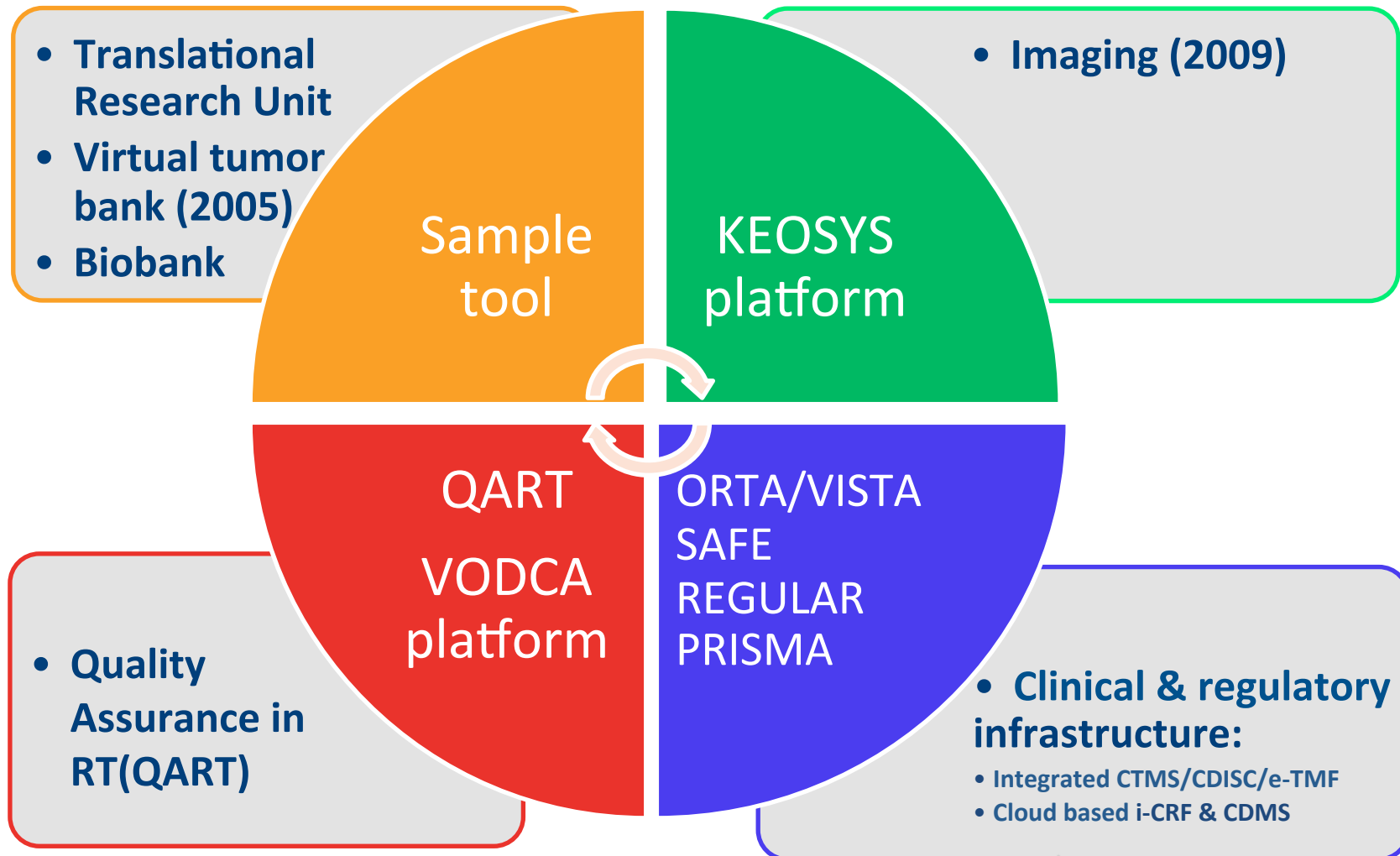
## **Extensive experience in working with:**

- Academic medical centres and other research organisations (150+)
- Pharmaceutical companies
- Regulators and other healthcare stakeholders

# EORTC 2015

- > 180,000 patients in the databases
- > 50,000 patients being followed-up
- > 2,000 collaborators (clinicians, pathologists, researchers,....)
- > 300 institutions in the network
- > 1600 publications with EORTC in the title
- 276 publications in 2012-2013
- > 32 different countries joining research
- 21 groups/task forces
- >40 trials open to patient entry

# EORTC Infrastructure to support new generation clinical trials



# Successes

- Changing the standards of care for many tumor types, including with pivotal /registration trials
- Working with large intergroup cooperation, global trials
- Setting up multidisciplinary infrastructures
- Implementing quality assurance programs
- Reaching out to cancer registries
- Changing the paradigms: translational agenda, bio-banking
- Setting up new tools for clinical research, new methodology
- Opening up to new partnerships
- Working with patient advocates

Always being profoundly convinced of the European capacity of excellence of all stakeholders involved in clinical research

# Fighting rare cancers: success stories

- **Soft Tissue Sarcoma :**
  - Gist Trial record breaking
- **Melanoma :**
  - Largest adjuvant trials in shortest time frame
- **Brain Tumors :** Adjuvant TMZ/XRT trial in GBM
- **Haemato-oncology**
  - Leukemia - trials / unique database
  - Lymphoma - trials / unique database
  - Children Leukemia - trials / unique database
- **Head and Neck Cancer:** Larynx preservation

# **EORTC HNCG STUDY - 1206**

**A randomised phase II study to evaluate the efficacy and safety of Chemotherapy (CT) vs androgen deprivation therapy (ADT) in patients with recurrent and/or metastatic, androgen receptor (AR) expressing, salivary gland cancer (SGCs)**

## **EORTC – NCI –UKCRN initiative**

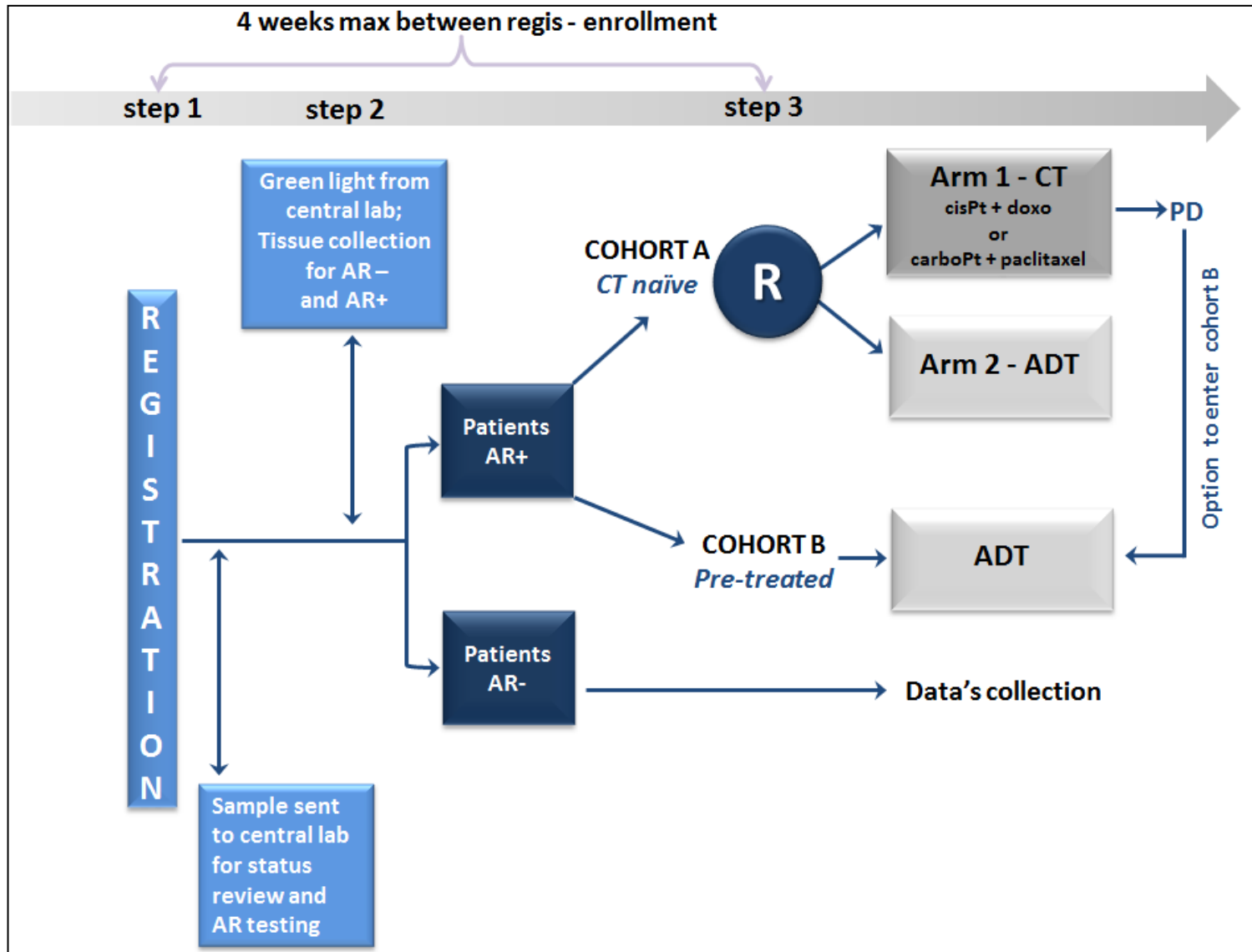
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Study Coordinator **Lisa Licitra**, Istituto dei Tumori, Milano



# Study design

152 patients; Primary endpoint:  
Progression Free Survival (PFS) for Cohort A  
Best Overall Response for Cohort B (according to RECIST 1.1)



# Realities of rare cancers

- Low numbers
- Varying and challenging diagnosis
- Etiology and molecular pathology is poorly known
- Few therapeutic options based on low level of evidence
- No consensus on standard of care & varying outcome
- Frequent off label use of drugs
  - Not enough attractive for the industry
  - Randomized clinical trials considered necessary, but not possible
  - As of yet suboptimal acceptance of adaptive designs by regulators
- Not enough information about ongoing research available to doctors and patients

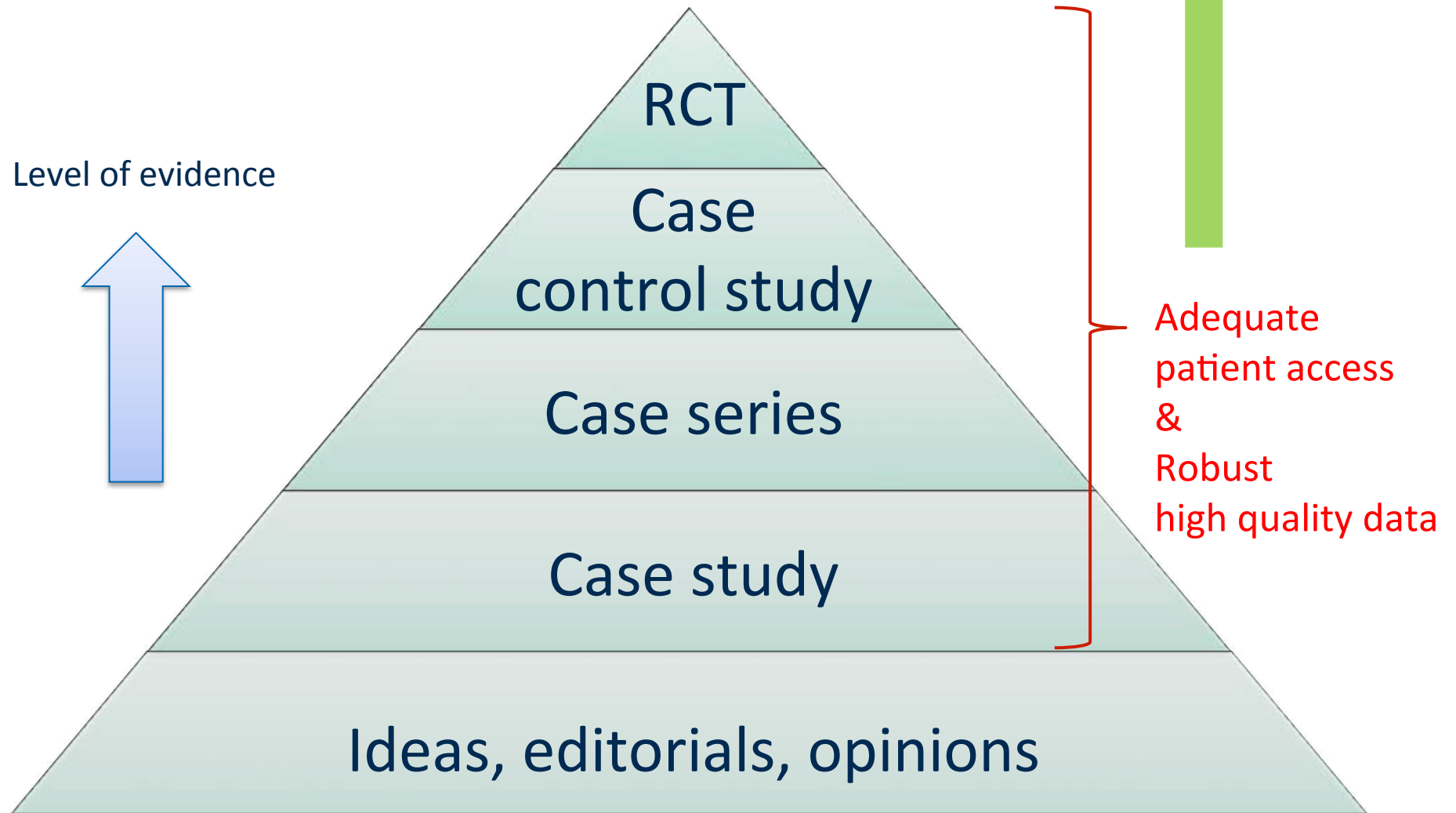
# Clinical research challenges

- Multi-stakeholder collaborations
  - New partnerships & models of risk sharing
- Centralised infrastructures to ensure timely review of diagnosis & pathology
- Generation of background knowledge to formulate hypothesis
- What is the standard treatment?
- Access to drug
  - Difficult access in academic setting
  - Limited to inexistent funding available, rarely international
  - No current solution to off label situation
- Access to patients and patient's access to research

# Evidence based medicine

includes MAPs & academic studies

Adequate infrastructures



# Clinical research requirements: RARE CANCERS

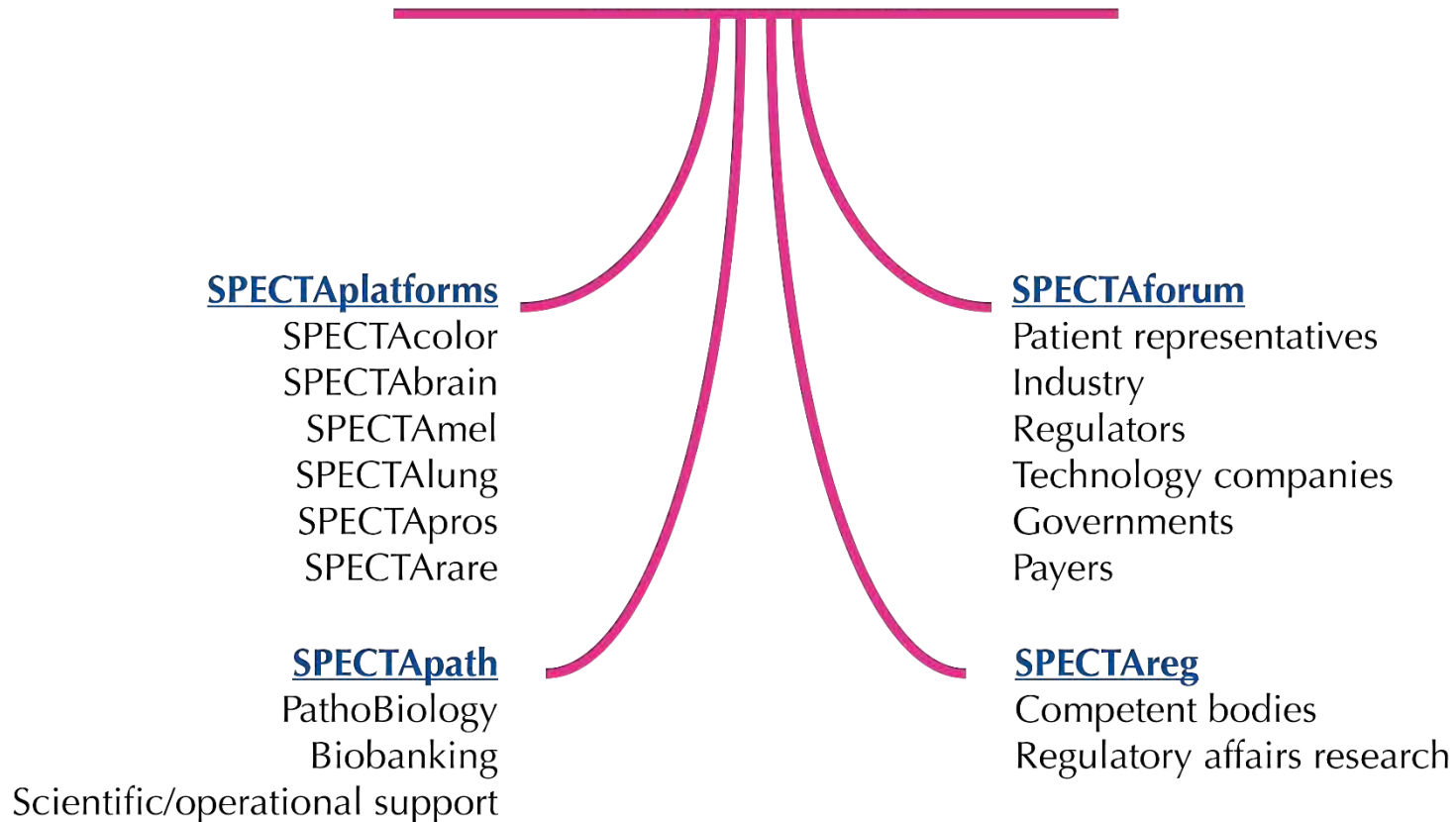
- Maximize patient access to existing research programs
- Provide high quality robust data (whatever level of evidence)
  - ...but also:
- Maximize meaningful efforts
- Ensure adequate screening (maximize timely inclusion)
- Enable to collect real life data & direct reporting by patients
- Ensure continuous & longitudinal research
- Obtain structuring overall effect by collecting all cases in prospective clinical and biologically documented databases
- Enable knowledge gathering, sharing and learning
- Enable development of new methodologies
- Enable new (*improbable / unexpected*) partnerships

ALLOW TRANSTUMORAL TALKATIVE /INTERACTIVE RESEARCH by

BUILDING PLATFORMS & INFRASTRUCTURE FOR KNOWLEDGE DEVELOPMENT

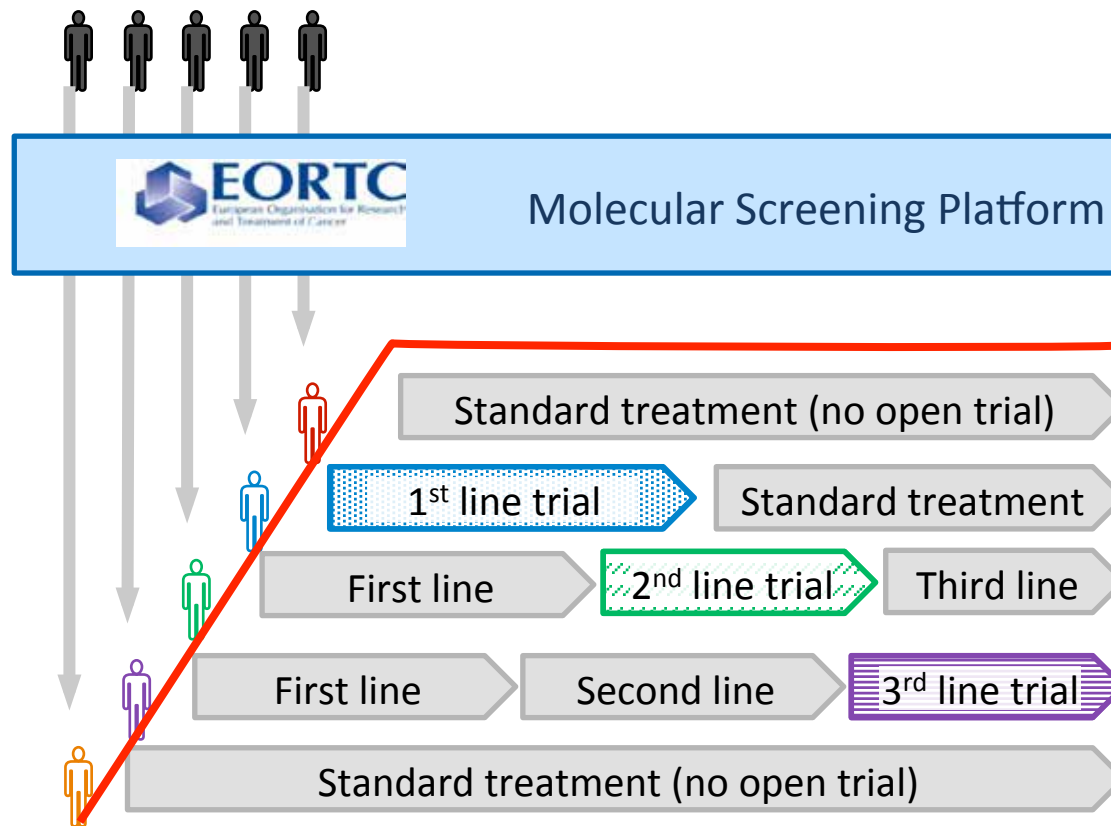
# SPECTA program: a forum for dialog and collaboration

## EORTC SPECTA Screening Patients for Efficient Clinical Trial Access *Screen and Treat*



# SCREENING PLATFORM: SPECTA

(Screening Platform for Efficient Clinical Trial Access)



SPECTAplatforms

SPECTA color

SPECTA rare

**SPECTArare**

Academia  
investment

Industry  
cooperation

# TAKE HOME MESSAGES

- CLINICAL RESEARCH & CLINICAL TRIALS CAN & MUST BE DONE IN RARE CANCERS: THERE IS NO ROOM FOR WAISTING OPPORTUNITIES FOR RESEARCH
- IT REQUIRES ADEQUATE ORGANISATION OF RESEARCH, but also ADEQUATE SYSTEMS and INFRASTRUCTURES THAT GUIDE PATIENTS TO THE RIGHT RESEARCH PROJECT IN THE RIGHT TIME
- THERE IS A NEED FOR TALKATIVE RESEARCH DATABASES & BIOBANKS ACROSS TUMORS
- THERE IS NO ROOM FOR DOING THINGS ALONE => RARITY PLEADS FOR COLLABORATIVE EFFORTS
- PARTNERSHIP IS ESSENTIAL:  
WE ALL NEED EACH OTHER IN THIS VENTURE



# Questions?

