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

- Translational Research Unit
- Virtual tumor bank (2005)
- Biobank

Sample tool

KEOSYS platform

QART

VODCA platform

ORTA/VISTA
SAFE
REGULAR
PRISMA

Quality Assurance in RT(QART)

Clinical & regulatory infrastructure:
- Integrated CTMS/CDISC/e-TMF
- Cloud based i-CRF & CDMS

Imaging (2009)
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

Study Coordinator Lisa Licitra, Istituto dei Tumori, Milano
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.

Level of evidence:

- RCT
- Case control study
- Case series
- Case study
- Ideas, editorials, opinions

Adequate infrastructures:
- Adequate patient access &
  Robust high quality data
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 \textit{(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*

**SPECTAplatforms**
- SPECTAcolor
- SPECTAbrain
- SPECTAmel
- SPECTAlung
- SPECTApros
- SPECTArare

**SPECTAforum**
- Patient representatives
- Industry
- Regulators
- Technology companies
- Governments
- Payers

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

**SPECTAreg**
- Competent bodies
- Regulatory affairs research
SCREENING PLATFORM: SPECTA

(Screening Platform for Efficient Clinical Trial Access)

Molecular Screening Platform

1st line trial

Standard treatment (no open trial)

Standard treatment

2nd line trial

First line

Third line

2nd line trial

Second line

3rd line trial

Standard treatment (no open trial)

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?