RD-ACTION, EMA and DG Health workshop
ERNs and Clinical Research

Case study: EURACAN clinical research

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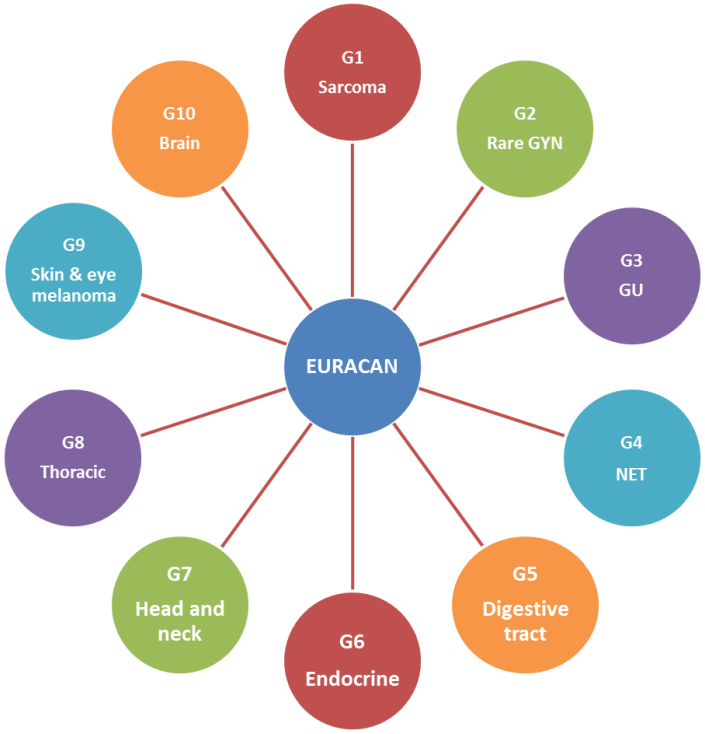
On behalf of EURACAN

European Reference Network

for rare or low prevalence complex diseases

Network Adult Cancers (ERN EURACAN)

EURACAN



Melanoma Patient Network Europe

RARE SOLID ADULT CANCERS

Research projects

- **Added value of EURACAN?** Cross tumors. Reinforce access to patients and expertise.
- **RP-1759 – AYA 12-29 year-old with high grade glioma and non-grade 1 bone and soft tissue sarcoma**
  - To understand better the biology of the tumor, and compare it to children and adults with similar disease.
  - PILOT to improve the inclusion of young adults into clinical trials.
  - 50 HGG and 50 sarcoma. FFPE tissue and blood.
  - Analyses: WES, RNAseq and methylation assay.
- **RP-1544: Collaboration with Ignyta for enrollment in clinical trial.**
  - Locally advanced or metastatic solid tumor: Soft Tissue Sarcoma, Cholangiocarcinoma, Neuroendocrine Tumors, Ovarian Cancer, Exocrine Pancreas.
  - To screen for NTRK1/2/3, ROS1 or ALK gene rearrangement to assess eligibility for the STARTRK-2 clinical trial.
- More to come incl. phase 2 trials

Lessons learned in rare cancers clinical research

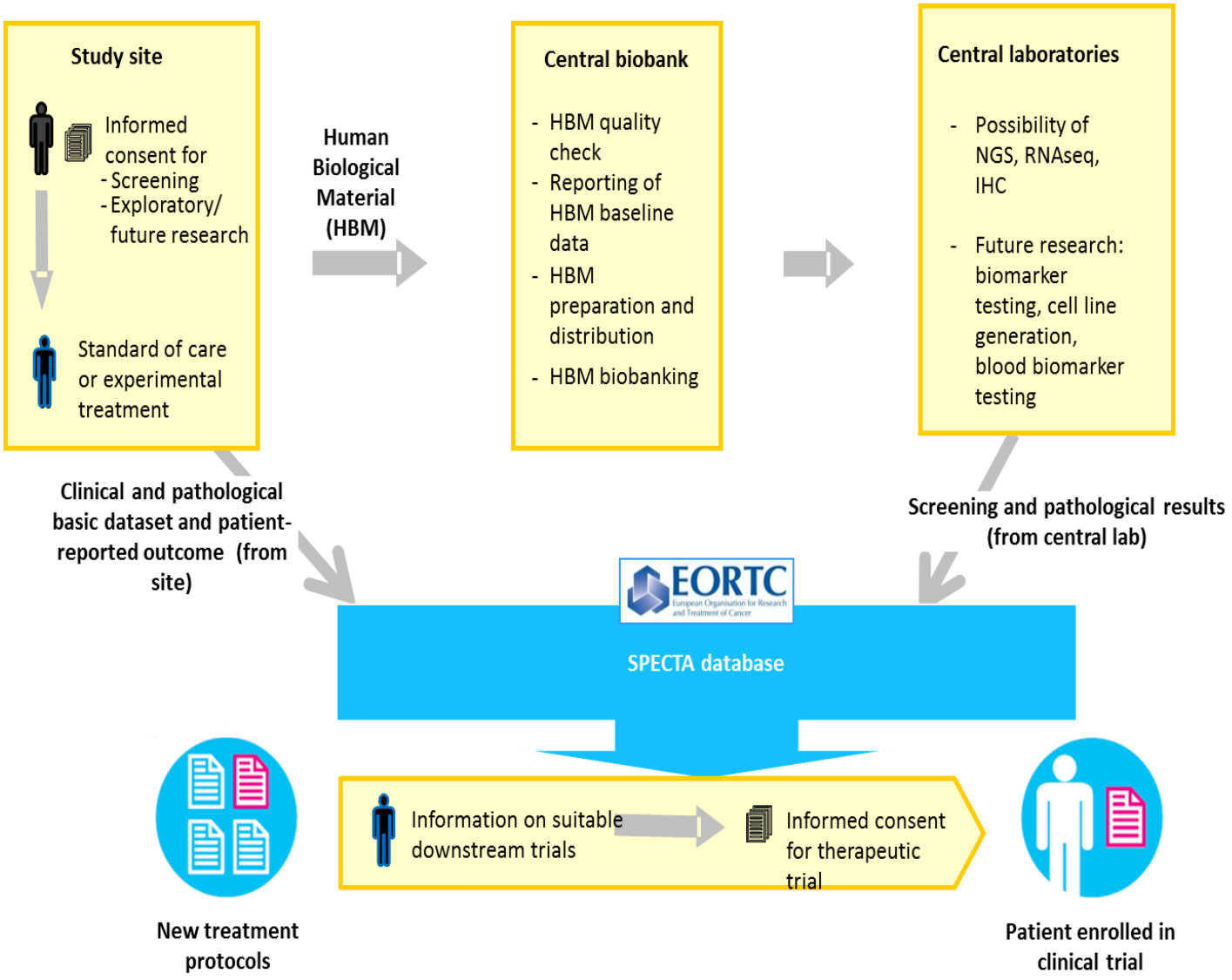
- Patients, scientific and clinical expertise are rare.
  - Go for international research reaching the critical mass in a reasonable timeframe.
  - Avoid small & irrelevant study. Use robust methodology generating results. Rare patients disserve high level of evidence.
  - Participating clinical sites should be selected on recruitment capacity, facilities and expertise (clinical, pathology,..).
  - Only centralised database and biobank will ensure homogeneous collection and full availability for research incl. future use.
  - Use central laboratory using consistent methodology and offering optimal quality.
  - Histopathological confirmation of the rare cancer diagnostic by central review is critical
  - QA/QC program should be implemented incl. audit.

Overview of SPECTA

SPECTA is a EORTC platform enabling robust molecular testing, for clinical trials or translational research projects.

- A protocol for longitudinal collection of cancer patient data and HBM without immediate interventional intent.
- An informed consent form allowing future unspecified use provided ethical committee approval without repeat consent
- A logistics, biobanking, and testing infrastructure, to be activated according to the needs of the attached clinical trials or research projects.
- Any tumor type incl. rare cancers. Target 100 centers from 20 countries. 40 clinical sites from EURACAN.
- EURACAN research infrastructure.

SPECTA – workflow



Any question?