Promotion Institut Curie

Portfolio études onco sarcomes - Juin 2021

MYKID STUDY :

MOLECULAR IDENTITY CARD FOR KIDS, ADOLESCENT AND YOUNG ADULT WITH NON RHABDOMYOSARCOMAS SOFT TISSUE SARCOMA

Centre porteur : Institut Curie, paris

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MYCKIDS: Molecular Identification and Characterization of non-Rhabdomyosarcoma Soft Tissue Sarcoma in Kids, Adolescents and Young Adults: an EpSSG NRSTS study

International sponsorship: Prinses Máxima Center, Utrecht

PI's: M van Noesel, D Orbach, A Ferrari

		Material	Number of cases	Investigations
Organization, international sponsorship, data management				
Utrecht	WP1	fresh	200	1. WES 2. mRNAseq 3. DNAmeth (plus clinical data)
	WP2	FFPR	250	GI vs CINSARC vs grading
	WP3	fresh	30	organoids
	WP4	blood serum		liquid biopsies
Therapeutic Recommandations for all NRSTS histiotypes				

WP1 and **WP2** will start from the beginning, with the available money

WP3 finances are included in the organizational money

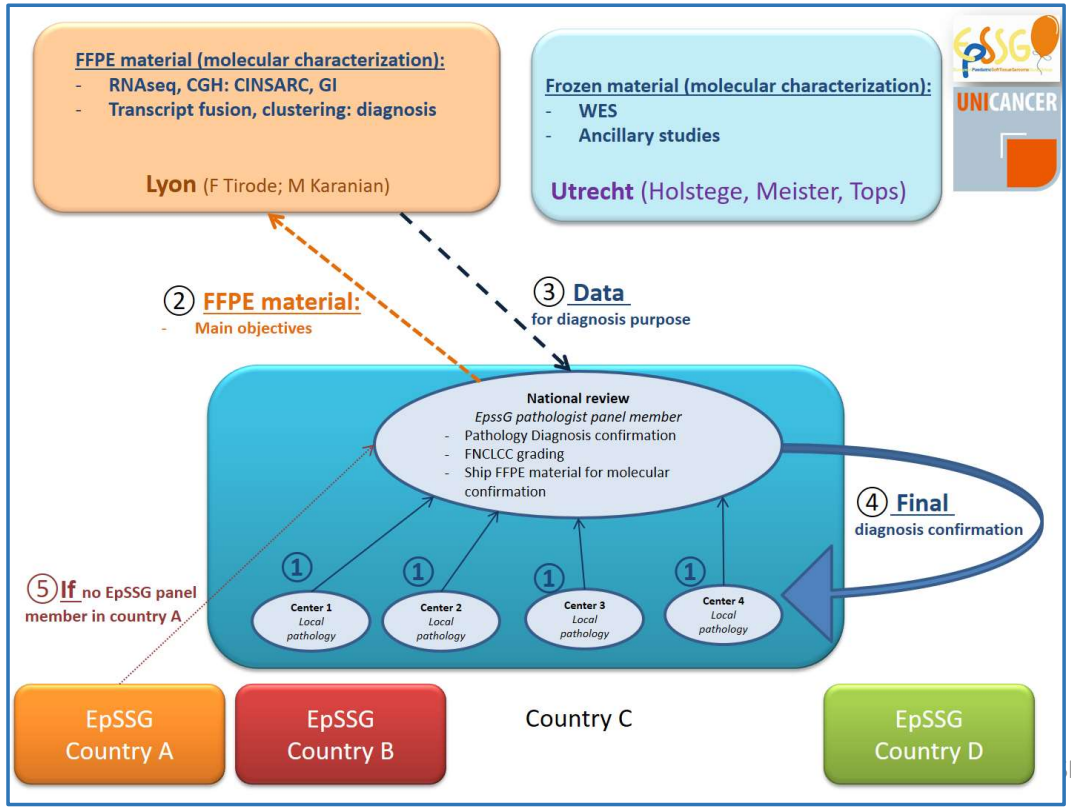
WP4 will start when needed money will become available (from an international application, from a single country, or from a single center)

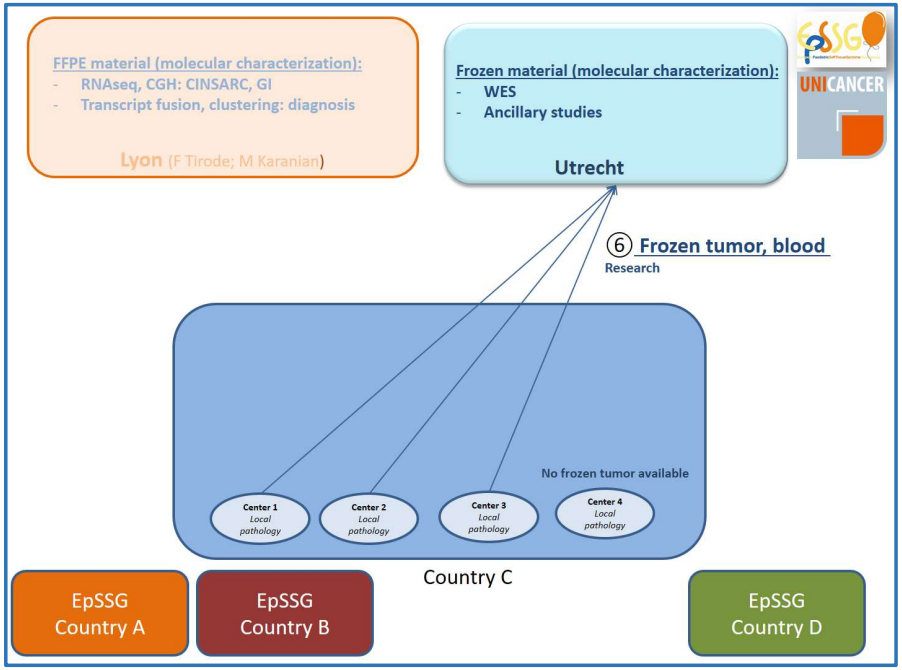
The study finances will cover organization, international sponsorship, data management, shipping materials *AND* **WP1** *AND* **WP2** *AND* **3**

Centres participants

- Centres SFCE/GSF-GETO
- Centres EpSSG

Molécules, schéma de traitement





Indication : 1ière ligne, tous stades

Principaux critères d'inclusion

- **Patients age: 0 to 25 years**
- **Newly diagnosed localized or metastatic tumor**
- **With Non RMS tumor or with intermediate tumor (including desmoid)**
- **Diagnosis confirmed by national pathological review (RRePS pathologist network in France and other networks in Europe)**
- **Treatment in one European EpSSG centre,**
- **Whatever therapy is decided (observation, surgery, radiotherapy or chemotherapy)**
- **Patients able to provide a FFPE or frozen sample of the tumor at diagnosis for research purposes**
- **Written patient/ parents' consent**

Endpoint et stat succinctes

- Hypothèses stat :
  - **For the main question**: With 80% power, alpha 5%, 50% prevalence of a high Cinsarc signature, HR associated with 4:16 metastases must then be observed in the whole sample. For this purpose, if the average 5-year metastasis rate is 15%, then 109 subjects must be included. US, ATF and SS represent 45% of the overall EpSSG NRSTS population. In case of inclusion of **30 subjects per year**, it will take **4 years** to include **120 subjects** (10% of potential data not retrospectively available) and then you will have a median regression of 5 years (group A).
  - **Group B** which represents 55% of all tumors will include **147 subjects**
- Endpoint 1aire :
  - **To compare the prognostic value of CINSARC, Genomic index and FNCLCC grading, for group A.**
  - **To integrate central pathology review and molecular profiling, for the diagnosis of all NRSTS in Europe (groups A and B).**
  - **To improve treatment and outcomes for patients with NRSTS by establishing a clinically annotated cohort of newly diagnosed patients with NRSTS with longitudinal collection of bio specimens.**
  - **To determine the feasibility of recruiting patients to an international study with high-quality bio specimen collection.**
- Endpoints 2aires :
  - **Molecular profiling of NRSTS**
  - **Correlate clinical entities to molecular profiles/patterns/ clusters.**
  - **Identify new groups of NRSTS entities in this cohort according to biological characterizations,**
  - **To assess targetable alterations for future possible treatments.**
  - **Developing ancillary studies based on this bio-clinical cohort.**
- Nombre de patients nécessaires : 267 pts

Etat d'avancement

Etat du recrutement

- Nombre de patients inclus : 0
- Nombre de patients restant à inclure : tous
- Fermeture prévue le : Non encore ouvert

(Courbe de recrutement éventuelle)

Problème/ Amendement à venir