Promotion Unicancer

Portfolio études onco sarcomes - Juin 2021

A Randomized Phase II, placebo-controlled , multicenter study evaluating efficacy and safety of regorafenib in patients with metastatic bone sarcomas
REGOBONE

Cohort: CIC-rearranged sarcoma

Nom du Centre porteur : UNICANCER
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Centres participants

- CHU La Timone Adultes
- CENTRE LEON BERARD
- INSTITUT CURIE SITE PARIS
- INSTITUT DE CANCEROLOGIE DE L OUEST SITE RENE GAUDUCHEAU
- INSTITUT CLAUDIUS REGAUD
- CENTRE ANTOINE LACASSAGNE
- CENTRE FRANCOIS BACLESSE
- CENTRE HOSPITALIER UNIVERSITAIRE DE BESANCON - HOPITAL JEAN MINJOZ
- INSTITUT DE CANCEROLOGIE DE LORRAINE ALEXIS VAUTRIN
- CENTRE GEORGES FRANCOIS LECLERC
- CENTRE OSCAR LAMBRET
- INSTITUT BERGONIE
- Institut Paoli Calmettes
- Gustave Roussy
- ICM Val d'Aurelle
- Hopital Cochin
- Centre Eugene Marquis
- Institut de Cancérologie Lucien Neuwirth (ICLN)
- CHU Bretonneau

Molécules, schéma de traitement

- **Randomization 2:1 to receive either REGORAFENIB or PLACEBO, until disease progression**
- REGORAFENIB : 120 mg /d, 3 weeks on/1 week off OR
- PLACEBO 120 mg/d, 3 weeks on/1 week off
- REG or PLA dosed until unacceptable toxicity or progression
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  Pts initially randomized to PL could **cross-over to open-label REG after PD confirmation closed**

Indication : metastatic/locally advanced disease, in 2d, 3d or 4th line of treatment

Main inclusion criteria

- Osteosarcoma,Ewing sarcoma of bone, Chondrosarcoma, Chordoma, **CIC-DUX4 sarcoma (either of bone or soft tissue)**
- Progressive disease at study entry
- (within prior 3 mo. period for OsteoS and Ewing , CIC-DUX4 and within 6 mo. for ChondroS and Chordoma)
- Metastatic disease/local. advanced disease not amenable to surgery or radiation with curative intent
- Measurable disease (RECIST 1.1)
- Prior treatment: at least 1, but no more than 2 prior (combination) chemo regimen for metastatic disease (neo-adjuvant /maintenance therapy are not counted towards this requirement.) for OS, ES, CS **For CIC-DUX4 sarcoma at least 1 but no more than 3 prior (combination) chemo regimen**
- Age ≥ 10 years, ECOG PS < 2 (Karnofsky ≥ 60%)
- Dated+ signed the informed consent form

Endpoints and statistics

Primary endpoint: Non-progression rate at 8 weeks

Secondary end points: PFS (per modified RECIST 1.1), OS, Toxicity per NCI-CTC AE v4

Statistical Design per A'Hern's single-stage design for phase II trials (A'Hern et al 2001)

- **H0 25%, H1 55%, one-sided α: 5%; β = 80%**
- **18 patients planned in REGO arm**
- 2:1 Randomization ➔ 27 patients total randomized population
- **≥ 8 progression-free pts at 8 weeks for 'success'**
- **No formal statistical comparison between REGO and PLACEBO**

Etat d'avancement

Etat du recrutement

- Nombre de patients inclus : **3**
- Nombre de patients restant à inclure : **24**
- En théorie:
- Fin des inclusions prévue en septembre 2022 et fin du suivi en septembre 2024

Problème/ Amendement à venir

- **Augmenter le nombre de centres**
- **En rajoutant 3 centres italiens et 2 centres espagnols**
- **Revoir les objectifs statistiques ?**