



## Red Blood Cell Disorders Committee

<http://www.bhs.be>

### MEETING January 25, 2018

7:00 PM

#### **Participants:**

Ann Van Damme, Anna Vanderfaeillie, Laurence Dedeken, Cécile Boulanger, Ann Devos, Marie-Françoise Dresse, Bram De Wilde, Olivier Ketelslegers, Phu Quoc Lé, Veerle Labarque, Marie-Agnès Azerad, Alice Ferster, Béatrice Gulbis.

#### **Minutes:**

##### 1. Clinical trials

- a. Pyruvate kinase deficiency: registry and clinical trials ([Christian ROSE](#) - Service d'onco hématologie - Hôpital Saint Vincent de Paul - Université Catholique de Lille - Univ Nord de France, Lille, France)

New drug AG-348 activator of PK activity.

The response factors are not the dose but the genotype (non-responding nonsense mutations) (some residual protein is needed for the drug to work).

The drug is well tolerated (are known to be related to the drug: hypertriglyceridemia and worsening of sudden anemia if the drug is stopped abruptly)

The drug also has a mild anti aromatase activity that prevents its use in children.

The trials will start in mid-2018 against placebo (only missens mutation, exclusion of nonsens) but there is a crossover at 6 months which allows all patients to benefit from this innovative drug (first in its category).

For inclusion of your patients, please contact Pr ROSE.

For the registries, there are two of them but not necessarily competitors.

1. The European descriptive register of PK cases: RADEEP is a joint venture conceived in the core of the ERN-EuroBloodNet ([www.eurobloodnet.eu](http://www.eurobloodnet.eu)), as an umbrella for both new and already existing European patients' registries in rare anaemias (RAs).

2. A long-term international registry: PEAK registry: Pyruvate Kinase Global Deficiency Longitudinal International Registry sponsored by AGlos.

- b. myTomorrows and L-Glutamine in the treatment of SCD ([Frederik BUIJS](#) – My tomorrows – Amsterdam, The Netherlands):

<https://mytomorrows.com/en/about/what-we-do/>

##### 2. Recommendations to be updated and new ones; publication in the Belgian Journal of Hematology

- a. Recommendation for management of a sickle cell crisis in pediatrics ([Bram De Wilde](#)): document in preparation.

##### 3. Difficult cases



- a. Anemia in an 18-year-old girl with neurological disorders ([Anna VANDERFAEILLIE](#)): presented and discussed.
  
4. SCD meeting – February 22
  - a. Final programme ([Lucie PECHEUX](#)): presented and in attachment.
  
5. BHS meeting: GAM presentation ([Béatrice GULBIS](#)): final version in attachment.