

MINUTES OF WEBEX MEETING_COST4KIDS

PDWP

Date: 2/07/2019. Webex meeting

Leaded by: Dr. Antonio Pérez Martínez (Hospital Universitario La Paz)

Participants:

1. Antonio Pérez Martínez (Hospital Universitario La Paz)
2. Peter Lang (Stem Cell Transplantation. University of Tubingen)
3. Selim Corbacioglu (University of Regensburg)
4. Cristina Ferreras (Hospital Universitario La Paz)

Main issues addressed:

A. Pérez presented 2 slides during the meeting. In the first slide he presented the queries received by the evaluation report of the COST Action (SHINE):

- ⇒ Q1: discuss alternative options to IS
 - ⇒ Q2: identify selected cases and conditions before starting the study.
 - ⇒ Q5: children with SOT and HSCT a small group.
 - ⇒ Q7: small sample size.
 - ⇒ Q8: develop young investigator exchange programmes
 - ⇒ Q9: dissemination (use of social network as communication strategic).
- A. *Pérez presented the second slide trying to address Q1-Q2 queries*
- ⇒ 3 groups of patients could be selected
 - Group 1: Patients where both, SOT and HSCT should be performed mandatory because there is no other option: the underlying disease, for example organ failure, in the context of primary immunodeficiencies or hematological malignancies because prolonged IS after SOT. In this group the approach is based on the NEED. However, there is not standardized protocol and results are still dismal with high TRM because previous morbidities.
 - Group 2: Patients who need a second or third SOT because fast rejection. In this group of patients new approaches are needed to induce tolerance and HSCT or cell therapies (MSCs, Tregs, hematopoietic progenitors...) should be explored.
 - Group 3: Patients who received a SOT with very good results (liver and kidney) but they do not want to take for life IS, they have adherence difficulties or sub-optimal quality of life or they want to enroll in a trial. In this group less risky approach would be cell therapies as MSCs or T regs as some groups are doing in liver (Tubingen) or kidney (Bambino Gesu).

Selim argued that the first group represents a high TRM group and it is not the main focus of the proposal. The focus is to immunomodulation properties of HSCT. Selim proposes a pilot trial with 10 patients (living donor liver transplant) and haplo transplant. If data from these trial are good

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enough we can present to our colleagues and move on into the new field and indications of HSCT. He proposed a one-day meeting with hepatologists and hematologists from Tübingen, Regensburg and Madrid to get together to brainstorm and develop a pilot trial proposal. He proposed to prepare a simple agenda with the objectives and expectations of the meeting.

Peter Lang argued two major points, first: answer the queries for the resubmission of the COST proposal and second: support the pilot trial as Selim proposes.

- ⇒ For the COST he argued that the group 1 of patients exist and they need to be considered in the proposal. He proposed to update retrospectively this cohort.
- ⇒ For the pilot trial he pushed for the meeting as Selim proposes

Next Steps:

Prepare an agenda for the face to face meeting (Tübingen-Regensburg-Madrid) for just 6 people (hematologists and liver transplant experts) or maybe try to invite only specialists of liver and hematologists the 27th of September Friday in Madrid

An agenda for the face to face meeting will be sent soon