P0278

Initiatives of the EAHAD Glanzmann Working Group (EGWG)

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Goals:

- The first goal of the working group is to share and disseminate knowledge among European health care providers on management of Glanzmann's Disease (GD).
- The second goal is to define and execute a European research pipeline for GD.

Introduction

- Glanzmann's Disease (GD) is an ultra-rare inherited platelet function disorder
- The estimated prevalence is 1:1.000.000
- It is characterised by dysfunctional platelets due to lack of Glycoprotein IIB/IIIA
- The inheritance is autosomal recessive
- Many clinical relevant questions are unanswered and current clinical practice is mainly based on anecdotal experience and still not optimal.
- These include basic management of surgery and bleeding, pregnancy management, prophylactic treatment in the context of anti-platelet antibodies and cardiovascular comorbidities.

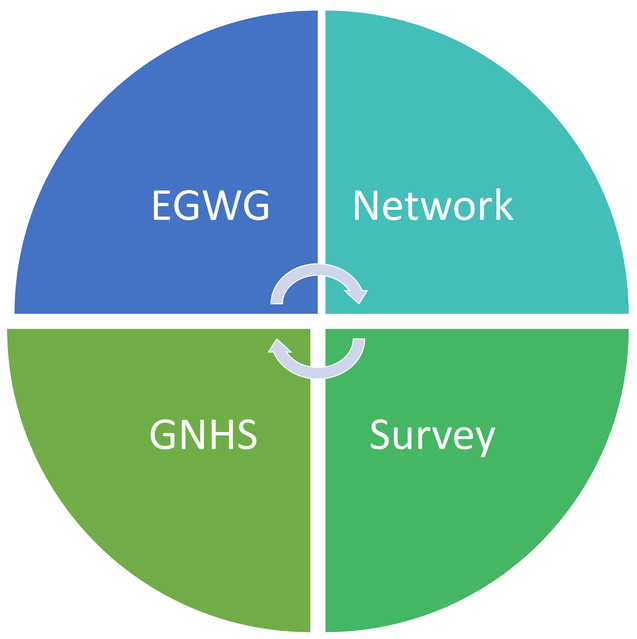
Methods

- The EAHAD Glanzmann Working Group was established in December 2022. It consists of at least four members from at least three different European countries (with a maximum of ten members) and 1 member from the European Haemophilia Consortium.

- We have initially designed 4 goals:
  - Creating the EGWG within EAHAD
  - Performing a cross-sectional survey
  - Creating a European digital network for caregivers
  - Starting a prospective Natural History Study (NHS)

- In future, additional projects will be covered:
  - Cardiovascular disease
  - Fertility and pregnancy
  - Molecular modifiers of disease serverity

Figure. Current initiatives of the EGWG



EGWG: EAHAD Glanzmann Working Group;
GNHS: Glanzmann Natural History Study

Results

1. The EGWG has been created and a digital instant messaging platform for medical purposes (Siilo®) has started for communication amomgst care givers
2. The pan-European cross-sectional survey has been conducted: see poster P0239 for details
3. The protocol for the GHNS has been finalised. We aim at including 200 patients with GD and follow them prospectively

Conclusion

With the initiation of the EAHAD Glanzmann Working Group, we are connecting health care providers and fill in the knowledge gaps on this ultrarare inherited platelet function disorder. This could be a model for other rare disorders next.

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