SSC Subcommittee Project

DEFINITIONS IN ACQUIRED HEMOPHILIA

Subcommittee on Factor VIII, Factor IX and Rare Coagulation Disorders

- Person responsible (Chair / Principal Investigator):

  Andreas Tiede (Hannover Medical School, Hannover, Germany. tiede.andreas@mh-hannover.de)

- Design:

  Consensus building

- Aim/Objective/Rationale (Needs assessment / Reason):

  Although AHA is an extremely rare disorder, European and North American Registries have made progress in better describing outcomes and prognostic factors (*e.g.* United Kingdom Surveillance Study[1], EACH2 registry,[2,3] GTH-AH 01/2010 study,[4] publication of HTRS registry pending). In view of these studies, however, it becomes apparent that outcomes of hemostatic treatment and immunosuppression were defined differently. In particular, we lack definitions of cessation of bleeds as well as remission and relapse of disease. Ideally, uniform definitions for these endpoints should be used globally to ensure valid reporting of study results. This is particularly important in the light of planned studies to optimize immunosuppression and of new hemostatic treatments, like porcine recombinant factor VIII, entering the market.

- Methodology (Data expected to collect, sample size and statistical analysis):

  Systematic literature review and consensus discussion

- Study population (Inclusion, exclusion, eligibility) (patient population; recruitment of participating institutions/physicians and subjects; minimum number needed; expected number):

  Relevant patient group: patients with acquired hemophilia A (AHA)

  Participating investigators (core group, further members to be determined):

Peter Collins (Arthur Bloom Haemophilia Centre, University Hospital of Wales, School of Medicine, Cardiff University, Cardiff, UK. peter.collins@wales.nhs.uk)

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Cindy A. Leissinger (Louisiana Center for Bleeding and Clotting Disorders, Tulane University Medical Center, New Orleans, LA, USA, cleissi@tulane.edu)

Laszlo Nemes (National Haemophilia Centre and Haemostasis Department, Medical Centre of Hungarian Defence Forces , Budapest, Hungary. lnemes@t-online.hu)

- Expected timeline:
    - Project stage/set up: 01/2016
    - Launch: 01/2016
    - Duration: 6-8 months
    - Finalization/analysis: 10/2016
    - Reporting: 2017

- Expected outcomes (ie. publications):
    - Publication type: SSC Communication

- Description of project set/up and management, needed infrastructure and resources (summary):

  The PI will coordinate the project. No additional resources needed.

- Possible references:

1. Collins PW, Hirsch S, Baglin TP, et al. Acquired hemophilia A in the United Kingdom: a 2-year national surveillance study by the United Kingdom Haemophilia Centre Doctors' Organisation. *Blood*. 2007;109(5):1870-1877.
2. Collins P, Baudo F, Knoebl P, et al. Immunosuppression for acquired hemophilia A: results from the European Acquired Haemophilia Registry (EACH2). *Blood*. 2012;120(1):47-55.

3. Knoebl P, Marco P, Baudo F, et al. Demographic and clinical data in acquired hemophilia A: results from the European Acquired Haemophilia Registry (EACH2). *J Thromb Haemost*. 2012;10(4):622-631.
4. Tiede A, Klamroth R, Scharf RE, et al. Prognostic factors for remission of and survival in acquired hemophilia A (AHA): results from the GTH-AH 01/2010 study. *Blood*. 2015;125(7):1091-1097.