AGMT | ARBEITSGEMEINSCHAFT MEDIKAMENTÖSE TUMORTHERAPIE

PATIENT REGISTRY
Autoimmune Hemolytic Anemia (AIHA) with corresponding Biobank

Protocol Number: AGMT_AIHA

Coordinating Investigator: U. Jäger[1]

[1]Medical University of Vienna, Department of Internal Medicine I, Div. of Hematology and Haemostaeology

Protocol Synopsis:

Indication: Autoimmune hemolytic anemia (AIHA)

Planned sample size: 100 patients

Design: retrospective and prospective, multicenter research initiative

Recruitment: First patient in (FPI): Q4 2016

Objectives:

- o Epidemiological evaluations
  - Assessment of AIHA subtypes in Austria
  - Assessment of specific characteristics and frequency of AIHA
- o Patient care and treatment in Austria
  - Treatments used, sequence of treatments
  - Efficacy and toxicity
- o Establishment of a central biobank to provide a basis for future AIHA related research (optional)

Rationale:

Autoimmune hemolytic anemia (AIHA) is caused by autoimmune-mediated destruction of red blood cells (RBCs) by autoantibodies with various properties and target specificities. AIHA is a heterogeneous disease with respect to the type of the antibody involved and the absence or presence of an underlying condition.

There is considerable a lack of evidence regarding effectivity of second- or third-line treatments. In addition, the algorithm of treatment for patients resistant to steroids, rituximab or splenectomy is unclear. Therefore, there is a strong medical need to generate data on long-term follow-up which may help to develop novel therapies.

This Patient Registry is set up to collect real-world experience in the management of patients with AIHA in Austria. The aim is to gain valuable insights on epidemiology and patient care and treatment of this rare disease.

Given the low incidence of AIHA the incorporation of a biobank into a well characterized clinical registry will enable research for the benefit of AIHA patients in Austria and around the world.

Design:

This registry is a prospective and retrospective, multicentre collection of data on patients with AIHA in Austria. All disease characteristics, medical histories and also treatment sequences are documented in anonymised form. Additionally patients will be asked to complete the FACIT-Fatigue questionnaire. For documentation in the registry no further diagnostic or therapeutic measures are required than those already necessary in general. Participation in the project must not interfere with treatment routines.

Data will be collected from all sites in Austria willing to participate. An estimated 100 patients are expected to be included; this number may be revised over time as interest and demand dictates.

To help maintain patient confidentiality, each patient will be assigned a unique patient identifying number upon enrolment; this number will accompany the patient's medical and other information throughout the lifetime of the project.

Biobank:

For scientific purpose the biomaterial will also be transferred to different specialised laboratories in Austria and abroad (e.g. True North Therapeutics Inc. in San Francisco). True North Therapeutics is working on new drugs against AIHA by testing antibody drug response. These results can be correlated with routine examinations performed in the University Department of Internal Medicine of the Vienna General Hospital to assess whether a patient will benefit from a certain therapy or not.

Inclusion Criteria:

- Signed written informed consent
- Age 18 years or over
- Clinical and laboratory signs of AIHA

Due to the non-interventional design of the registry there are no exclusion criteria.

Status (as of March 2017):

No	*Sites*	*Patients*
01	AKH MUW / Med. I Hämatologie	54
02	PMU Salzburg / III. Med	3
	TOTAL	**57**

An academic registry

Sponsor: Arbeitsgemeinschaft medikamentöse Tumortherapie gemeinnützige GmbH, Clinical-Scientific Director: R. Greil