AGMT_HMA REGISTRY

Registry on Hypomethylating Agents in Myeloid Neoplasms, including Myelodysplastic Syndromes (MDS), Chronic Myelominocytic Leukemia (CMML) and Acute Myeloid Leukemia (AML)

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Protocol Synopsis

Indication: Myeloid neoplasms, including MDS, CMML, AML

Design: Retrospective and prospective, observational, multi-center, multi-national research initiative

Recruitment: First patient in (FPI): Q1 2009

Number of patients: 1.500 (planned)

Objectives:
- Number of cycles and dosage of VIDAZA® and/or DACOGEN®
- Response evaluation
- Toxicities
- Severe adverse reactions
- Overall survival
- Prognostic factors for overall survival and response

Design

This registry is set up to collect real-world experience in the management of patients with myeloid neoplasms, in particularly in patients with MDS, CMML or AML, treated with hypomethylating agents in Austria and potentially other participating countries. This registry will collect data in a retrospective as well as in a prospective manner at various sites. The aim is to gain valuable insights on both efficacy and toxicity of these drugs in a routine clinical setting in patients with various comorbidities.

An estimated 1.500 patients are expected to be included; these numbers may be revised over time as interest and demand dictates.
No pre-defined visits, medical tests, laboratory tests, procedures, or interventions are required. Physicians who have already treated patients with hypomethylating agents or are planning to initiate treatment with hypomethylating agents can include patient data in this registry. Electronic Case Report Forms (eCRF) will be used for data collection.

Additionally from some patients with myeloid neoplasms, blood or tissue samples will be stored for further analyses. These samples will also be obtained from patients, who are not or not yet treated with hypomethylating agents, to comprise a control.

2019 this initially treatment-specific registry shall be converted into a disease-specific registry (planned number of patients 2000 in total), assessing the therapeutic landscape of patients with MDS, CMML and AML in Austria. The aim is to document patients from initial diagnosis to the present status and thus gain valuable insights on both efficacy and toxicity, as well as the sequence of use of different treatments in a routine clinical setting in patients with various comorbidities.

Recruitment

Sites	Patients
PMU Salzburg / Innere Medizin III	312
Hanusch KH Wien / 3. Medizin	131
Ordensklinikum Linz / Interne I	130
Klinikum Wels Grieskirchen / Innere Med. IV	125
UK Innsbruck / Innere Medizin V	116
UK Graz / Innere Medizin - Hämatologie	115
SMZ Ost / 3. Medizin	68
LKH Feldkirch / Innere Med. II - Interne E	59
LKH Hochsteiermark / Dep. Hämato- Onkologie	58
Kepler UK, Med Campus III. / UK Hämatologie	52
Wilhelminenspital Wien / 1. Medizin	45
UK Krems / Innere Medizin II	37
Clinical Hospital Mekur, Zagreb	29
KH Hietzing / 5. Medizin	26
AKH Wien / Innere Medizin I - Hämatologie	20
KH Klagenfurt / 1. Medizin	15
LKH Fürstenfeld / Innere Medizin	12
LKH Steyr / Innere Medizin II	11
Rudolfsstiftung / 1. Medizin	3
TOTAL	**1.364**

Publications

Falantes J, Pleyer L, Thepot S, Almeida AM, Maurillo L, Martinez-Robles V, et al. Real life experience with front-line azacitidine in a large series of older adults with acute myeloid leukemia stratified by MRC/LRF score: results from the expanded international E-ALMA series (E-ALMA+). *Leuk Lymphoma.* 2018;59(5):1113-20.

Huemer F, Weiss L, Faber V, Neureiter D, Egle A, Geissler K, et al. Establishment and validation of a novel risk model for estimating time to first treatment in 120 patients with chronic myelomonocytic leukaemia. *Wiener klinische Wochenschrift.* 2018;130(3-4):115-25.

Leisch M, Weiss L, Lindlbauer N, Jungbauer C, Egle A, Rohde E, et al. Red blood cell alloimmunization in 184 patients with myeloid neoplasms treated with azacitidine - A retrospective single center experience. *Leukemia research.* 2017;59:12-9.

Almeida AM, Prebet T, Itzykson R, Ramos F, Al-Ali H, Shammo J, et al. Clinical Outcomes of 217 Patients with Acute Erythroleukemia According to Treatment Type and Line: A Retrospective Multinational Study. *International journal of molecular sciences.* 2017;18(4).

Pleyer L, Dohner H, Dombret H, Seymour JF, Schuh AC, Beach CL, et al. Azacitidine for Front-Line Therapy of Patients with AML: Reproducible Efficacy Established by Direct Comparison of International Phase 3 Trial Data with Registry Data from the Austrian Azacitidine Registry of the AGMT Study Group. *International journal of molecular sciences.* 2017;18(2).

Pleyer L, Burgstaller S, Stauder R, Girschikofsky M, Sill H, Schlick K, et al. Azacitidine front-line in 339 patients with myelodysplastic syndromes and acute myeloid leukaemia: comparison of French-American-British and World Health Organization classifications. *J Hematol Oncol.* 2016;9:39.

Ramos F, Thepot S, Pleyer L, Maurillo L, Itzykson R, Bargay J, et al. Azacitidine frontline therapy for unfit acute myeloid leukemia patients: clinical use and outcome prediction. *Leukemia research.* 2015;39(3):296-306.

Pleyer L, Burgstaller S, Girschikofsky M, Linkesch W, Stauder R, Pfeilstocker M, et al. Azacitidine in 302 patients with WHO-defined acute myeloid leukemia: results from the Austrian Azacitidine Registry of the AGMT-Study Group. *Annals of hematology.* 2014;93(11):1825-38.

Pleyer L, Germing U, Sperr WR, Linkesch W, Burgstaller S, Stauder R, et al. Azacitidine in CMML: matched-pair analyses of daily-life patients reveal modest effects on clinical course and survival. *Leukemia research.* 2014;38(4):475-83.

Pleyer L, Stauder R, Burgstaller S, Schreder M, Tinchon C, Pfeilstocker M, et al. Azacitidine in patients with WHO-defined AML – Results of 155 patients from the Austrian Azacitidine Registry of the AGMT-Study Group. *Journal of Hematology & Oncology.* 2013;6(1):1-13.

Melchardt T, Weiss L, Pleyer L, Steinkirchner S, Auberger J, Hopfinger G, et al. Complications of 5-azacytidine: Three cases of severe ischemic colitis in elderly patients with myelodysplastic syndrome. *Oncology letters.* 2013;6(6):1756-8.

Weiss L, Melchardt T, Neureiter D, Kemmerling R, Moshir S, Pleyer L, et al. Complete remission of Waldenstrom macroglobulinemia with azacitidine and rituximab. Journal of clinical oncology : official journal of the American Society of Clinical Oncology. 2011;29(24):e696-8.

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An academic registry
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