Trial Description

Title

Registry for BCR-ABL negative myeloproliferative neoplasms

Trial Acronym

Freiburg MPN Register

URL of the trial

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Brief Summary in Lay Language

The BCR-ABL negative myeloproliferative neoplasms (MPN) include several disease entities of the bone marrow, e.g., polycythemia vera (PV), essential thrombocythemia (ET), primary myelofibrosis (PMF) and myelofibrosis evolved from PV or ET (post-PV or -ET MF), systemic mastocytosis (SM) und unclassifiable MPN (MPN-U). Our understanding of the disease course and our insight into the underlying pathophysiology increased substantially during the past decade. Nevertheless, many questions remain open or rose in consequence of this progress. We want to address those unsolved problem in research projects. Based on clinical and experimental research, our major goal is to develop new methods for diagnostics as well as treatment options. In order to achieve this, we want to include MPN patients in this MPN registry study along with selected characteristics of those patients and their disease and to update this information regularly. Furthermore, we want to collect, store and analyze biomaterial (e.g. blood) to further improve the basic knowledge and shed more light on the pathophysiology of MPN.

Brief Summary in Scientific Language

This is a registry for BCR-ABL negative myeloproliferative neoplasms (MPN). Patient and disease features as well as biomaterials will be collected and analyzed. BCR-ABL negative MPNs are polycythemia vera (PV), essential thrombozythemia (ET), primary myelofibrosis (PMF), post PV or ET myelofibrosis (post-PV or -ET MF), systemic mastocytosis (SM) and unclassifiable MPN. Mutations in genes involved in proliferation signalling pathways are characteristic for these MPNs. The JAK2 gene is mutated in almost 100% of the patients with PV and approximately 50% of the PMF and ET patients. Patients with PMF or ET without JAK2 mutation often have mutations in the CALR or MPL genes. Among patients with SM, more than 95% have a mutation in the KIT gene. In addition, gene mutations are detectable in the MPNs that are also present in other hematologic neoplasias (e.g. in TET2, DNMT3A, ASXL1, EZH2, IDH1 or IDH2). The mutations refer to the biology of the disease and its clonal character, and the mutations clinically help in making a therapy decision and estimation of prognosis. Despite the advances in the genetic characterization and in the understanding of the biology of MPNs, the allogeneic hematopoietic stem cell transplantation remains to be the only curative treatment option. Targeted therapies are being tested in clinical trials, and a JAK2 inhibitor has been recently approved for the treatment of PMF and PV. However, so far, the targeted therapies do not cure

from the disease. Under consideration of the heterogeneity and low incidence of the MPNs, the Freiburg MPN Register aims to serve the clinical and basic research of MPNs, with the overarching goal to develop novel diagnostic and therapeutic treatment strategies.

Organizational Data

- DRKS-ID: **DRKS00010288**
- Date of Registration in DRKS: **2016/04/18**
- Date of Registration in Partner Registry or other Primary Registry: **[---]***
- Investigator Sponsored/Initiated Trial (IST/IIT): **yes**
- Ethics Approval/Approval of the Ethics Committee: **Approved**
- (leading) Ethics Committee Nr.: **558/15 , Ethik-Kommission der Albert-Ludwigs-Universität Freiburg**

Secondary IDs

Health condition or Problem studied

- ICD10: **D47.1 - Chronic myeloproliferative disease**
- ICD10: **D47.3 - Essential (haemorrhagic) thrombocythaemia**
- ICD10: **D47.4 - Osteomyelofibrosis**
- ICD10: **D45 - Polycythaemia vera**
- ICD10: **D47.0 - Histiocytic and mast cell tumours of uncertain and unknown behaviour**

Interventions/Observational Groups

- Arm 1: **Collection of selected characteristics of patients with a myeloproliferative neoplasm and of their disease and repeated update of this information. Collection, storage and analysis of the biomaterial (e.g. blood).**

Characteristics

- Study Type: **Non-interventional**
- Study Type Non-Interventional: **Observational study**
- Allocation: **Single arm study**
- Blinding: **[---]***
- Who is blinded: **[---]***

Study Type: **Non-interventional**

Study Type Non-Interventional: **Observational study**

Allocation: **Single arm study**

Blinding: **[---]***

Who is blinded: **[---]***

- Control: **Uncontrolled/Single arm**
- Purpose: **Other**
- Assignment: **Single (group)**
- Phase: **N/A**
- Off-label use (Zulassungsüberschreitende Anwendung eines Arzneimittels): **N/A**

Primary Outcome

Completeness of the register, event free survival (EFS), overall survival (OS), quality of life, symptom burden

Secondary Outcome

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Countries of recruitment

- DE **Germany**

Locations of Recruitment

- University Medical Center **Universität Freiburg - Innere Medizin I, Freiburg im Breisgau**

Recruitment

- Planned/Actual: **Actual**
- (Anticipated or Actual) Date of First Enrollment: **2016/04/18**
- Target Sample Size: **1000**
- Monocenter/Multicenter trial: **Monocenter trial**
- National/International: **National**

Inclusion Criteria

- Gender: **Both, male and female**
- Minimum Age: **18 Years**
- Maximum Age: **120 Years**

Additional Inclusion Criteria

- Age ≥18 years
- Patients with BCR-ABL negative MPN of the following entitities: primary myelofibrosis (PMF), polycythemia vera (PV), essential thrombocythemia (ET), post-PV or ET myelofibrosis (PPVMF/PETMF), systemic mastozytosis (SM), unclassifiable myeloproliferative neoplasm (MPN, U) or acute myeloid leukemia (AML) after MPN
- Signed informed consent

Exclusion criteria

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Addresses

- **Primary Sponsor**

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  79106 Freiburg
  Germany

  Telephone: **[---]***
  Fax: **[---]***
  E-mail: **[---]***
  URL: **[---]***

- **Contact for Scientific Queries**

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Sources of Monetary or Material Support

■ **Institutional budget, no external funding (budget of sponsor/PI)**

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Fax: **[---]***
E-mail: **[---]***
URL: **[---]***

Status

- Recruitment Status: **Recruiting ongoing**
- Study Closing (LPLV): **[---]***

Trial Publications, Results and other documents

** This entry means the parameter is not applicable or has not been set.*

**** This entry means that data is not displayed due to insufficient data privacy clearing.*