Trial Description

Title

**EWOG - MDS 98 Myelodysplastic Syndromes in Childhood
Prospective Study of the Diagnosis and Treatment
of Myelodysplastic Syndromes (MDS) in Childhood - EWOG-MDS 98**

Trial Acronym

EWOG-MDS 98

URL of the trial

http://www.ewog-mds.org

Brief Summary in Lay Language

Myelodysplastic Syndrome of childhood is a rare disorder that is charcetrized by a reduced production of blood cells and the risk of leukemia development. The prognosis for children diagnosed with MDS is unfavorable. The aim of the study is to introduce standard diagnostic tests and subsequently collect data about the frequency of the differnt subtypes of MDS. Furthermore, the survival of children with MDS should be improved by standardized treatment with chemotherapy and/or bone marrow transplantation.

Brief Summary in Scientific Language

Myelodysplastic Syndromes (MDS) in childhood are a heterogenous group of clonal stem cell disorders accounting for less than 10% of all hematological malignancies in childhood. MDS is characterized by peripheral blood cytopenia, ineffective hematopoiesis, dysplasia of all 3 cell lineages and a high risk of transformation in acute leukemia. Classification of MDS in Childhood has been confusing and inconsistent, while the FAB classification has generally been accepted for adults with MDS. Prognosis of most children with MDS is poor and there is no consensus in the literature concerning the optimal treatment strategy. In general, allogeneic stem cell transplantation (SCT) is the therapy of choice.
The primary objective of this multi-center non-randomized study is to facilitate the diagnostic procedures for all children and adolescents with MDS (study patients)by a standardized review of morphology and standardized cytogenetic and molecular analyses. All cases of MDS will be classified according to a modified FAB classification. Using this approach the frequency of the differnt FAB subtypes and cytogenetic and molecular abnormalities are to be assessed.
The secondary objective of the study is to improve the survival for children and adolescents with primary MDS(protocol patients) over that reported in the literature. In the presence of an HLA-matched family donor, early SCT is the treatment of choice for MDS patients of all FAB subtypes. SCT will be performed according to the ongoing EWOG-MDS study. In the presence of a bone marrow blast count > 15%, AML induction therapy is recommended prior to SCT. AML therapy for these protocol patients will be administered according to the National AML studies. For patients lacking an HLA-identical family donor, SCT from an unrelated volunteer is recommended, though AML therapy without SCT can be offered as an alternative treatment option.

Do you plan to share individual participant data with other researchers?

[---]*

Description IPD sharing plan

[---]*

Organizational Data

- DRKS-ID: **DRKS00000001**
- Date of Registration in DRKS: **2008/08/07**
- Date of Registration in Partner Registry or other Primary Registry: **2002/10/03**
- Investigator Sponsored/Initiated Trial (IST/IIT): **yes**
- Ethics Approval/Approval of the Ethics Committee: **Approved**
- (leading) Ethics Committee Nr.: **099/98 , Ethik-Kommission der Albert-Ludwigs-Universität Freiburg**

Secondary IDs

- Primary Registry-ID: **NCT00047268 (ClinicalTrials.gov)**
- Partner Registry-ID: **UKF000876 (Register Klinischer Studien des Universitätsklinikums Freiburg)**

Health condition or Problem studied

- ICD10: **D46.9 - Myelodysplastic syndrome, unspecified**
- Free text: **Myelodysplastic syndrome, unspecified**

Interventions/Observational Groups

- Arm 1: **1.Diagnosis for all children and adolescents with MDS (study patients)by a standardized review of morphology and standardized cytogenetic and molecular analyses**
  2.Therapy: monitoring without treatment
- Arm 2: **1.Diagnosis for all children and adolescents with MDS (study patients)by a standardized review of morphology and standardized cytogenetic and molecular analyses**
  2.Therapy: treatment according to the national AML therapy
- Arm 3: **1.Diagnosis for all children and adolescents with MDS (study patients)by a standardized review of morphology and standardized cytogenetic and molecular analyses**
  2.Therapy: SCT will be performed according to the ongoing EWOG-MDS study

Characteristics

- Study Type: **Interventional**
- Study Type Non-Interventional: **[---]***
- Allocation: **Non-randomized controlled trial**
- Blinding: **[---]***
- Who is blinded: **[---]***
- Control: **Active control (effective treament of control group)**
- Purpose: **Treatment**
- Assignment: **Parallel**
- Phase: **[---]***
- Off-label use (Zulassungsüberschreitende Anwendung eines Arzneimittels): **[---]***

Primary Outcome

- To evaluate the frequency of the different FAB subtypes by a standardized approach
- To evaluate the frequency of cytogenetic and molecular abnormalities

Secondary Outcome

- To improve survival for children and adolescents with primary MDS over that reported in the literature
- To assess the rate of complete remission (CR) and the event-free survival (EFS) in children with primary MDS treated according to AML therapy
- To evaluate the relapse rate, morbidity and mortality in children with primary MDS treated by SCT
- To identify different subsets of patients with primary MDS benefiting from SCT or AML-type therapy

Countries of recruitment

- DE **Germany**
- DK **Denmark**
- SE **Sweden**
- NO **Norway**
- FI **Finland**
- IS **Iceland**
- NL **Netherlands**
-

CH **Switzerland**

- AT **Austria**
- IT **Italy**
- CZ **Czech Republic**

Locations of Recruitment

Recruitment

- Planned/Actual: **Actual**
- (Anticipated or Actual) Date of First Enrollment: **1998/07/03**
- Target Sample Size: **850**
- Monocenter/Multicenter trial: **Multicenter trial**
- National/International: **International**

Inclusion Criteria

- Gender: **Both, male and female**
- Minimum Age: **no minimum age**
- Maximum Age: **18 Years**

Additional Inclusion Criteria

All children and adolescents with MDS under the age of 19 diagnosed between July 1998 and June 2002 are registered as study patients.

Exclusion criteria

- Children with Down syndrome
- Children with the following cytogenetic or molecular abnormalities:
t(8;21)(q22;q22) [AML1/ETO fusion gene]
t(15;17)(q22;q12) [PML/RAR´´ rearrangement]
inv(16)(p13q22) [CBF$/MYH11rearrangement]
- Children with the typical clinical and cytogenetic features of AML FAB M7 who present with < 30% blasts in the BM and PB

- Concomitant illness precluding therapy according to protocol
- No informed consent by the patient or legal guardian
- Morphological diagnosis could not be confirmed

Addresses

■ **Primary Sponsor**

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■ **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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Status

- Recruitment Status: **Recruiting complete, follow-up complete**
- Study Closing (LPLV): **2006/12/31**

Trial Publications, Results and other documents

- Paper **Hematopoietic stem cell transplantation for advanced myelodysplastic syndrome in children: results of the EWOG-MDS 98 study**

** 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.*