4th International Symposium on MDS in Childhood

Freiburg, Germany
April 24-26, 2006

Registration and Accommodation:
Please see the website at www.ewog-mds.org. Registration is possible via the online form.

Location:
The meeting will take place at the Konzerthaus Freiburg, close to the central train station, within walking distance from the city center.
Freiburg can be reached conveniently from the Airports Frankfurt/Main (2 hours), Zürich (2.5 hours) or Mulhouse/Basel (45 minutes, buses not every hour).

Transfer to the Netherlands
For interested participants we offer on April 27 a transfer to the 17th meeting of the I-BFM Study Group (April 28-30, 2006), followed by the 5th Biannual Symposium on Childhood Leukemia (April 30 – May 2) in Noordwijkerhout. The transfer will include a scenic trip on the river Rhine. Please register on our website: www.ewog-mds.org

Organizing Committee:
Charlotte Niemeyer, Freiburg
Peter Bader, Frankfurt/Main
Irith Baumann, Bayreuth
Alexandra Fischer, Freiburg
Christian Kratz, Freiburg
Dirk Reinhardt, Hannover
Brigitte Schlegelberger, Hannover
For the European Working Group of MDS in Childhood (EWOG-MDS):
Jan Starý, Prague, Chairman

Secretary
Beate Batz
Dept. of Pediatrics and Adolescent Medicine, Division of Pediatric Hematology and Oncology
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D-79106 Freiburg
Germany
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symposium2006@ewog-mds.org

Sponsored by:
**Monday, April 24, 2006**

**8:30 - 12:00**

*Workshop: EWOG-MDS Pathology Board*
(Location: Institute of Pathology, University of Freiburg, Breisacher Str. 115a)

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**13:00**

*Opening of the 4th International Symposium on MDS in Childhood*

C. Niemeyer  
Chair of the Local Organizing Committee

J. Starý  
Chair of the European Working Group of MDS in Childhood (EWOG-MDS)

C. Peters  
Dean of the Medical Faculty, Albert-Ludwig University, Freiburg im Breisgau

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**13:30-15:30**

*JMML and other Myeloproliferative Disorders: Part 1*

Chair: R. Chan (Indianapolis, IN, USA), A. Biondi (Monza, Italy)

13:30

*Invited Lecture*

Role of JAK2 mutations in the pathophysiology of myeloproliferative disorders  
R. Skoda (Basel, Switzerland)

13:55

Erythrocytosis in childhood: What genes to look for?  
H. Cario (Ulm, Germany)

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**14:10**

Essential thrombocythaemia in children in the Czech Republic  
D. Pospisilova, V. Mihal, T. Votava, J. Hak, H. Ptoszkova, J. Blatny, M. Priwitzerova, V. Divoky, J. Cmejlova, R. Cmejla, K. Petrtylova, J. Starý (Olomouc, Czech Republic)

**14:25**

*Case Forum*

Idiopathic myelofibrosis in children – a rare disease curable by bone marrow transplantation.  
K. Zdrahalova, P. Sedlacek, R. Formankova, H. Ptoszkova, K. Michalova, G. Kerdrup, J. Starý (Prague, Czech Republic)

**Discussant:** I. Baumann (Bayreuth, Germany)

**14:40**

*Invited Lecture*

Murine Model Systems for studying JMML  
B. Braun (San Francisco, CA, USA)

**15:10**

Noonan syndrome and JMML: different diseases caused by deregulation of identical pathways  
C. Kratz, S. Schubbert, M. Zenker, S. Böll, G. Bollag, C. Niemeyer and K. M. Shannon (Freiburg, Germany)

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**15:30 – 16:00**

Coffee Break

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**16:00 - 18:00**

*JMML and other Myeloproliferative Disorders: Part 2*

Chair: B. Beverloo (Rotterdam, The Netherlands), J. Starý (Prague, Czech Republic)

16:00

*Case Forum*

An Interesting Case: Juvenile Myelomonocytic Leukemia  

**Discussant:** A. Manabe (Tokyo, Japan).
16:15  Clinical features and mutational status of the GM-CSF signaling pathway related genes in children with JMML
(Nagoya, Japan)

16:30  Bisphosphonate Zoledronic Acid (ZOL) inhibits the abnormal proliferation and differentiation of juvenile
myelomonocytic leukemia (JMML) cells
Y. Ohtsuka, A. Manabe, K. Hirihide, C. Daisuke, T. Takakuni, N. Tatsutoshi, T. Kohichiro (Nishinomiya, Japan)

16:45  Activating PTPN11 Mutants Aberrantly Promote Myelomonocytic Differentiation
R. Chan, C. Voorhorst, M. Hass, T. Kondo, E. Hashino (Indianapolis, IN, USA)

17:05  JAK2 gene mutation is not a common event in JMML and is not involved in the pathogenesis of the disease

17:20  No evidence for constitutively activated FLT3 in Juvenile Myelomonocytic Leukemia

17:35  Clinical characteristics of patients with JMML according to mutational status

8:00  Therapy of JMML
Chair: F. Locatelli (Pavia, Italy), M. Tuncer (Ankara, Turkey)

8:00  Case Forum:
Cutaneous manifestation of JMML without monocytosis in a newborn child
Discussant: H. Hasle (Aarhus, Denmark)

8:15  An Update of the Phase II Window Study of the Farnesyl Transferase Inhibitor (FTI), R115777, Followed By A Phase III Trial of 13-Cis Retinoic, Cytosine Arabinoside, Fludarabine And Stem Cell Transplantation (SCT) in Children with Juvenile Myelomonocytic Leukemia (JMML). A Children’s Oncology Group Study.
R. Castleberry, M. Loh, N. Jayaprakash, A. Peterson, V. Casey, M. Chang, B. Widemann, P. Emanuel (Birmingham, AL, USA)

8:40  Treatment of JMML other than Stem Cell Transplantation: A Retrospective Analysis of 129 Treatment Courses in 63 Patients
E. Bergsträßer, H. Hasle, T. Rogge, A. Fischer, M. Zimmermann, P. Nöllke, C. Niemeyer (Zürich, Switzerland)

8:55  Hematopoietic stem cell transplantation (HSCT) for juvenile myelomonocytic leukemia (JMML)

9:10  Hematopoietic stem cell transplantation (HSCT) in children with MDS and JMML – a single centre experience
M. Ussowicz, D. Wojcik, W. Pietras, K. Kalwak, E. Gorczynska, D. Turkiewicz, K. Pajdosz, A. Chybicka (Wrocław, Poland)
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<tr>
<th>Time</th>
<th>Session</th>
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| 9:25   | Single Nucleotide Polymorphisms (SNP) in the quantitative Realtime PCR as a highly sensitive marker for chimerism analyses in children with MDS and JMML after allogeneic stem cell transplantation  
A. Willasch, H. Kreyenberg, S. Reincke, N. Shayegi, T. Klingebiel, P. Bader  
(Frankfurt, Germany)  |
| 9:40   | **Round Table**  
How can we measure response to therapy in JMML?  
P. Bader, E. Bergsträßer, B. Castleberry, A. Manabe, C. Niemeyer |
| 10:00 – 10:30 | Coffee Break |
| 10:30 – 12:00 | **MDS: Diagnostic Criteria and Cell Cycle**  
Chair: I. Baumann (Bayreuth, Germany), H. Hasle (Aarhus, Denmark)  
**Case Forum**  
Natural course of the MPS/MDS overlap disease (JMML, CMML, RAEB-1) during 14 years follow-up in a child with NF-1  
J. Starý, D. Pospisilova, J. Houskova, K. Michalova, G. Kerndrup, C. M. Niemeyer (Prague, Czech Republic)  
**Discussant:** F. Locatelli (Pavia, Italy)  
**Minimal criteria for diagnosis of MDS**  
J. Bennett, G. Mufti (Rochester, NY, USA)  
Cell cycle studies in MDS  
**Myelodysplastic syndromes in childhood: epidemiological aspects**  
M. Hamaji, L.F. Lopes (Sao Paulo, Brazil) |
| 11:30   | Evaluation of children with myelodysplastic syndrome; a single center experience  
S. Aytaç-Elmas, M. Çetin, M. Tuncer, C. Hiçşönmez  
(Ankara, Turkey)  |
| 11:45   | **Case Forum**  
Successful Bone Marrow Transplantation for Myelodysplastic Syndrome Mimicking Refractory Idiopathic Thrombocytopenic Purpura: A Complicated Course  
**Discussant:** A. Biondi (Monza, Italy)  |
| 12:00 – 13:00 | Lunch |
| 13:00 – 15:00 | **Bone Marrow Failure Disorders**  
Chair: C. Niemeyer (Freiburg, Germany), S. Kojima (Nagoya, Japan)  
**Case Forum**  
Hereditary MDS or hereditary thrombocytopenia?  
E. Bergsträßer, M. Schmugge (Zürich, Switzerland)  
**Discussant:** B. Bader-Menieur (Paris, France)  
**Invited Lecture**  
The pathogenesis of dyskeratosis congenita: new insights into the causes of bone marrow failure  
T. J. Vuillami (London, United Kingdom)  
Detection of TERC Mutations in a Cohort of 80 Children with Hypoplastic Refractory Cytopenia  
C. Ortman, C. Niemeyer, A. Wawer, S. Honeff, W. Ebell, C. Klein, A. Yoshimi, I. Baumann, C. Kratz (Essen, Germany)  
**Cytogenetic studies in congenital bone marrow failure syndromes**  
G. Michaelsen, C. Kratz, C. Zeidler, C. Niemeyer, K. Welte, B. Schlegelberger (Hannover, Germany) |
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<tr>
<td>14:15</td>
<td>Hematological and genetic follow-up of 38 Italian Shwachman-Diamond Syndrome (SDS) patients</td>
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<td>14:30</td>
<td>Epidemiology of Childhood AA and MDS in Japan; 1990-2002</td>
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<tr>
<td></td>
<td>A. Ohara, S. Kojima, A. Manabe, A. Kikuchi, H. Mugishima, I. Tsukimoto, T. Nakahata (Tokyo, Japan)</td>
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<td>14:45</td>
<td>T-cell receptor (TCR) V beta repertoire analysis: a valuable diagnostic tool for pediatric MDS and SAA?</td>
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<td>15:00 – 15:30</td>
<td>Coffee Break</td>
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<td>15:30</td>
<td>Refractory Cytopenia</td>
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<td>Chair: M. M. van den Heuvel-Eibrink (Rotterdam, The Netherlands), P. Bader (Frankfurt, Germany)</td>
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<td>15:30</td>
<td>Invited Lecture</td>
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<td>Significance of immunosuppression for low grade MDS in adulthood</td>
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<td>A. Ganser (Hannover, Germany)</td>
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<td>15:55</td>
<td>Prospective multicenter trial of immunosuppressive therapy with antithymocyte globulin and cyclosporine in children with nonsevere aplastic anemia</td>
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<td>16:10</td>
<td>Response to immunosuppressive therapy for patients with very severe and severe aplastic anemia: results of study SAA-94</td>
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<td>M. Führer, C. Bender-Götze, U. Rampf, A. Faldum, I. Baumann, C. Niemeyer, A. Borkhardt (Munich, Germany)</td>
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<td>16:25</td>
<td>Immunosuppressive therapy with anti-thymocyte globin and cyclosporine A in children with refractory cytopenia</td>
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<td>A. Yoshimi, I. Baumann, M. Führer, D. Schneider, K.-W. Sykora, T. Klingebiel, S. Bielack, A. Fischer, C. Niemeyer (Freiburg, Germany)</td>
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<td>16:40</td>
<td>A reduced-intensity conditioning regimen for children with refractory cytopenia transplanted from an unrelated donor</td>
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<td>B. Strahm, C. Niemeyer, J. Vormoor, B. Kremens, F. Zinti, M. Führer, K.-D. Stachel, P. Bader, M. Sauer, P. Sedlacek, I. Baumann, F. Locatelli on behalf of the EWOG-MDS Study Group (Freiburg, Germany)</td>
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<td>16:55</td>
<td>Apoptotic myelopoetic cells and clonally expanded cytotoxic T cells in bone marrow trephines of patients with low grade myelodysplastic syndrome</td>
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<td>H. Pühlhorn, H. Harms, M. Herrmann, U. Reulbach, A. Jung, T. Kirchner, I. Baumann (Bayreuth, Germany)</td>
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<td>17:10</td>
<td>Invited Lecture</td>
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<td>Pancytopenia in MDS – friend or foe?</td>
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<td>C. Nissen (Basel, Switzerland)</td>
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<td>18:15</td>
<td>Tour to the “Markgräfler Land“</td>
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**Wednesday, April 26, 2006**

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<tr>
<th>Time</th>
<th>Event</th>
<th>Speaker(s)</th>
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| **8:30 – 10:30** | **Advanced MDS: Chromosomal Abnormalities and Immunophenotype**  
Chair: B. Schlegelberger (Hannover, Germany), D. Betts (Zürich, Switzerland) |                                                                                             |
| 8:30  | **Case Forum**  
A. Rao (London, United Kingdom)  
**Discussant:** C. Ortmann (Essen, Germany) |
| 8:45  | **Invited Lecture**  
Insights into leukemogenesis from cytogenetic and molecular studies of therapy-related myelodysplasia and acute myeloid leukemia | M. K. Andersen (Copenhagen, Denmark) |
| 9:10  | Abnormal clonal karyotypes with neoplastic associated aberrations pre and post myeloid disease: Are they always significant? | D. Betts, T. Güngör, T. Kühne, U. Carlsch, N. von der Weid, E. Bergsträßer (Zürich, Switzerland) |
| 9:40  | **Invited Lecture**  
Array-CGH and correlation with gene expression profile in adult AML with complex karyotype | K. Döhner, F. G. Rücker, L. Bullinger, H. A. Kestler, P. Lichter, H. Döhner (Ulm, Germany) |
| **10:00** | **Immunonophenotypic and morphological evaluation of MDS: The AIEOP experience with a combined approach** | L. Sainati, M. Veltroni, M. Spinelli, G. Tridello, S. Fenu, M. Zecca, G. Basso (Padova, Italy) |
| 10:30 – 11:00 | **Coffee Break** |                                                                                             |
| **11:00 – 12:45** | **Advanced MDS: Molecular Changes and Therapy-Related Disease**  
Chair: C. Kratz (Freiburg, Germany), A. Manabe (Tokyo, Japan) |                                                                                             |
| 11:00  | **Case Forum**  
Capillary leak syndrome - an unusual complication of MDS-RAEB | K. Tousovska, M. Klimovic, M. Seda, J. Starý, J. Blatný, J. Sterba (Brno, Czech Republic)  
**Discussant:** Peter Bader (Frankfurt, Germany) |
| 11:15  | **Invited Lecture**  
The Ras oncoprotein in 3 dimensions | A. Wittinghofer (Dortmund, Germany) |
| 11:40  | **Invited Lecture**  
Therapy-related myelodysplastic syndromes in childhood: report on a diagnostic surveillance protocol in a single institution | L. Farinasso, P. Saracco, S. Aschero, L. Garbarini, A. Linari, A. Iavarone, E. Gottardi, S. Vallero, F. Fagioli, N. Crescenzio, M. Basso (Turin, Italy) |
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<tr>
<td>12:05</td>
<td>Invited Lecture&lt;br&gt;Role of epigenetic modification in hematopoiesis&lt;br&gt;M. Lübbert (Freiburg, Germany)</td>
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<td>12:45 – 13:30</td>
<td>Lunch</td>
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<tr>
<td>13:30 – 15:30</td>
<td>Advanced MDS: Hematopoietic Stem Cell Transplantation&lt;br&gt;Chair: B. Strahm (Toronto, Canada), M. Zecca (Pavia, Italy)</td>
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<td>13:45</td>
<td>Allogeneic bone marrow transplantation for children with myelodysplastic syndrome and very high risk features&lt;br&gt;B. Kuşkonmaz, B. Tavil, M. Tuncer, G. Hiçsönmez, M. Çetin, D. Uçkan (Ankara, Turkey)</td>
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<td>14:00</td>
<td>Future strategies of HSCT in MDS&lt;br&gt;F. Locatelli (Pavia, Italy)</td>
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<td>14:20</td>
<td>High incidence of acute and chronic graft-versus-host disease after allogeneic stem cell transplantation for myelodysplastic syndrome in children&lt;br&gt;F. Scherer, T. Günsör, R. Seger, E. Bergsträßer, M. Schmugge (Zürich, Switzerland)</td>
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<td>14:35</td>
<td>Haemopoietic stem cell transplantation (HSCT) is the only curative treatment for advanced myelodysplasia in children, but there is little information about the use of donor lymphocyte infusions (DLI) in post HSCT relapse&lt;br&gt;R. Skinner, M. Velangi, N. Bown (Newcastle-upon-Tyne, United Kingdom)</td>
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<td>15:05</td>
<td>Round Table&lt;br&gt;What is the role of AML-specific therapy prior to SCT in MDS?&lt;br&gt;B. Castleberry, H. Hasle, F. Locatelli, LF Lopez, A. Manabe, D. Reinhardt</td>
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<td>15:30</td>
<td>Farewell&lt;br&gt;C. Niemeyer</td>
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<tr>
<td>16:00 – 18:00</td>
<td>EWOG-MDS Business Meeting</td>
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