11th International Symposium of EWOG-MDS/SAA

On the Verge to Malignancy: Cytopenia and Beyond

Program

www.ewogberlin2025.org
September 18 - 20, 2025 Berlin

BERLIN ewog

Thursday, 18.09.2025

7:30	*Breakfast*
08:30 – 08:45 - Opening of the symposium - For the Organizing Committee: • Charlotte Niemeyer • Brigitte Strahm • Edoardo Muratore	
08:45 – 12:00 - Education session (organized by Young EWOG) Moderators Part I: • Marc Bierings (Utrecht, The Netherlands) • Felicia Andresen (Freiburg, Germany) Moderators Part II: • Barbara de Moerloose (Ghent, Belgium) • Edoardo Muratore (Bologna, Italy)	
08:45	**SAA beyond the state of the art** Invited experts: • Brigitte Strahm (Freiburg, Germany), • Carlo Dufour (Genova, Italy), • Mirjam Belderbos (Utrecht, The Netherlands)
09:30	**Well begun is half done: How to find the right diagnostic path in MDS** Invited experts: • Marcin Wlodarski (Memphis, USA), • Michael Dworzak (Vienna, Austria), • Martina Rudelius (Munich, Germany)
10:15	*Coffee break*
10:30	**The journey begins with a single step: clonal evolution in germline predisposition and clinical management** Invited experts: • Marlene Pasquet (Toulouse, France) • Marc Bierings (Utrecht, The Netherlands) • Henrik Hasle (Aarhus, Denmark)
11:15	**JMML preclinical models: One size does not fit all - choosing the right model for the right question** Invited experts: • Miriam Erlacher (Ulm, Gemany) • Silvia Bresolin (Padova, Italy)
12:00	*Lunch*

13:00 – 14:30 - Aplastic anemia I Moderators**:** • Tania Masmas (Copenhagen, Denmark) • Michael Amrein (Zurich, Switzerland)	
13:00	**Keynote lecture: The role of somatic T-cell mutations and immune dysregulation in SAA** Satu Mustjoki (Helsinki, Finland)
13:30	**Alternative donor peripheral stem cell transplantation with TCRαβ/CD19 depletion for pediatric patients with bone marrow failure** Timothy S. Olson*, C. W. Elgarten, J. H. Oved, L. Wray, K. Venella, P. Nicholas, S. Kadauke, Y. Wang, S. Grupp, and T. S. Olson *(Philadelphia, USA)
13:45	**Outcome of second hematopoietic stem cell transplantation in pediatric patients with severe aplastic anemia** Nienke Wieringa*, M. Amrein, M. Dworzak, V. Labarque, M. Schmugge, J. Stary, F. Locatelli, O. Smith, M. Bierings, J. Buechner, K. Pawelec, B. Strahm *(Utrecht, The Netherlands)
14:00	**Late effects of immunosuppressive therapy in acquired bone marrow failure: Long-term follow-up of Czech patients with aplastic anemia and refractory cytopenia** Martina Sukova*, B. Cabalkova, E. Mejstrikova, M. Reiterova, P. Riha, P. Keslova, Z. Novak, O. Zapletal, L. Mastikova, I. Janotova, J. Stary *(Prague, Czechia)
14:15	**Improved engraftment following a treosulfan-fludarabine conditioning regimen compared to thiotepa-fludarabine in patients with refractory cytopenia of childhood** Brigitte Strahm*, A. Yoshimi, I. Bodova, J. Buechner, A. Catala, V. De Haas, B. De Moerloose, M. Dworzak, H. Hasle, K. Kallay, F. Locatelli, R. Masetti, P. Noellke, M. Schmugge, O. Smith, J. Stary, D. Turkiewicz, M.Ussovicz, L. Vinci, M. Erlacher, C.M. Niemeyer *(Freiburg, Germany)

14:30 – 15:30 - Aplastic anemia II

Moderators:

- Brigitte Strahm (Freiburg, Germany)
- Julian Sevilla (Madrid, Spain)

Mini Panel: Long-term consequences of therapy in aplastic anemia
Setting the stage: Therapeutic concepts in pediatric SAA Carlo Dufour (Genova, Italy)
Relevance of clonal hematopoiesis following IST and HSCT Mirjam Belderbos (Utrecht, The Netherlands)
Risk of malignancy following IST and HSCT Akiko Shimamura (Boston, USA)
Risk of infertility Ayami Yoshimi (Freiburg, Germany)
How to design long term FUP study Jean Hugues Dalle (Paris, France)

15.30	*Coffee break*

16:00 – 17:30 The path to clonal myeloid malignancy I

Moderators:

- Kirsi Jahnukainen (Helsinki, Finland)
- Barbora Cabalkova (Prague, Czech Republic)

16:00	**Assessment of genetic testing following current comprehensive clinical diagnostic evaluation for pediatric severe aplastic anemia** Helen Reed*. J. De Jong, M. Gaviria, A. Koppayi, Y. Ahmed, I. Atkinson, M. Joos, M. Malsch, M. McClung, P. Nicholas, M. Shah, E. Sullivan, H. Xie, S. Zavarella, Y. Zhou, B. Zorman, L. Godley, T. Nakano, T. Olson, A. Bertuch, A. Shimamura) *(Boston, USA)
16:15	**Molecular surveillance in bone marrow failure: Predictive value of clonal hematopoiesis** Nathan Gray*, E. Attardi, M. Boals, S. Lewis, P. Shaker, K. Ray, J. Uhrich, M. Hale, A. Kennedy, M. Wlodarski *(Memphis, USA)
16:30	**Refractory cytopenia of childhood with normal karyotype: Long-term results of an observational approach** Beatrice Drexler*, S. Schwarz-Furlan, I. Baumann, M. Rudelius, P.Noellke, D. Lebrecht, S. Ramamoorthy, N. Rotari, A. Karow, S. Hirabayashi, F. Beier, Y.L. Behrens, G. Goehring, R. Kalb, M. Wlodarski, B. Strahm, M. Erlacher, C.M. Niemeyer, A. Yoshimi *(Basel, Switzerland)

16:45	**Single-cell and spatial transcriptomics of pediatric myelodysplastic syndrome with refractory cytopenia of childhood reveal a disrupted bone marrow niche driving impaired hematopoiesis** Patrycja Fryzik*. L-T. Chen, E.S. Hanemaaijer, I.J. Kal, T. Candelli, B.M. TePas, N. Epskamp, V. De Haas, M. Scheijde-Vermeulen, W.J. De Jonge, T. Margaritis, M. Belderbos *(Utrecht, The Netherlands)
17:00	**Concordance of somatic genetic testing in blood vs. bone marrow in Shwachman-Diamond syndrome** Felicia Andresen*, K. C. Myers, A. Guttierez, D. Schwarz, B. Goldberg, E. Weller, C. Reilly, K. Brundige, S. Loveless, L. Cheng, C. Lindsley, A. Shimamura *(Boston, USA)
17:15	**In-vivo mouse model of acute myeloid leukemia development in congenital neutropenia** Jeremy Haaf*. M. Ritter, P.A. Tutusaus, S. Kandabarau, M. Klimiankou, J. Skokowa *(Tübingen, Germany)

17:30 – 19:00 - The path to clonal myeloid malignancy II

Moderators:

- Miriam Erlacher (Ulm, Germany
- Kirsten Thus (Utrecht, The Netherlands)

Keynote Lecture: How risky is clonal hematopoiesis?

Luca Malcovati (Pavia, Italy)

Mini panel: Clonal hematopoiesis in aging and germline Predisposition: Is it the same?
Impulse statement Luca Malcovati (Pavia, Italy)
Battle of clones: Somatic genetic rescue in congenital bone marrow failure disorders Akiko Shimamura (Boston, USA)
Genetic outfit and modes of stem cell aging Julia Skokowa (Tübingen, Germany)
Clonal evolution in aplastic anemia – what is different? Marcin Wlodarski (Memphis, USA)

19:00	End of scientific program
19:00	*Welcome reception*

Friday, 19.09.2025

7:30	*Breakfast*
08:30 – 10:00 - Inherited predispositions to myeloid neoplasms and their clinical implications Moderators: • Shlomit Barzalai (Tikva, Israel) • Marena Niewisch (Ulm, Germany)	
08:30	**Keynote lecture: GATA2 deficiency: from pathophysiology to clinical care** Marlene Pasquet (Toulouse, France)
09:00	**St. Jude.org/GATA2: online catalogue of 900 individuals with germline *GATA2* mutations** Lili Kotmayer*, K. Gangwani, B. Csaba, Z. Xin, M. Wlodarski *(Memphis, USA)
09:15	**Mitotic defects impaired CD34+ proliferation in GATA2 deficiency** Maria Magallon-Mosella*, D. Romero-Moya, C. Calvo, J. Pera, E. Torralba-Sales, M. Erlacher, O. Molina, A. Giorgetti *(Hospitalet de Llobregat, Spain)
09:30	**Superior survival following hematopoietic stem cell transplantation for high-risk marrow features in patients with Shwachman Diamond syndrome** Kasiani Myers*, F. Andresen, B. Goldberg, E. Weller, C. Reilly, T. Nakano, A. Bertuch, A. Geddis, M. Joos, K. Coyne, K. Brundige, S. Loveless, L. Cheng, A. Shimamura *(Cincinnati, USA)
09:45	**Impulse Statement: Tailoring pre- and post HSCT therapy in advanced MDS** Ricardo Massetti (Bologna, Italy)
10:00	***Poster viewing*** *- Coffee served*
10:00	**Poster Walk 1: SAA (incl. therapy)** Moderator: • Joanne Yacobovich (Tikva, Israel) • Katarzyna Pawelec (Warsaw, Poland) **Poster Walk 2: Predisposition** Moderator: • Marena Niewisch (Ulm, Germany) • Mikael Sundin (Stockholm, Sweden) **Poster Walk 3: Therapy (other than SAA)** Moderator: • Kristian Juul-Dam (Aarhus, Denmark) • Kavicic Marko (Ljubljana, Slovenia)

10:45	**Poster Walk 4: Classical bone marrow failure** Moderator: • Krisztián Kállay (Budapest, Hungary) • Shinsuke Hirabayashi (Hokkaido, Japan) **Poster Walk 5: MDS** Moderator: • Sarah Basali (Ulm, Germany) • Pierre Goncalves (Lisbon, Portugal) **Poster Walk 6: JMML** Moderator: • Marek Ussowicz (Wroclaw, Poland) • Ingrid Furlan (Ulm, Germany)
11:30 – 13:00 - Inherited bone marrow failure and telomeropathies Moderators: • Sophia Polychronopoulou (Athens, Greece) • Juncal Fernandez-Orth (Ulm, Germany)	
11:30	**Keynote lecture: germline disorders linked to telomere maintenance and dysfunction** Sharon Savage (Bethesda, USA)
12:00	**Functional analysis of non-canonical *FANCJ* variants in Fanconi anemia** Reinhard Kalb*, A. Droste, A. Repczynska, P. Bydgoszcz, B. Fiebig, J. Vodopiutz, A-R. Janecke, J. Knaup, R. Hark, R. Kulka, A. Sobeck *(Würzburg, Germany)
12:15	**Clonal hematopoiesis in congenital neutropenia: Insights from somatic mutation analysis** Maksim Klimiankou*, S. Kandabarau, C. Zeidler, S. Kadah, D. Pogozhykh, Y.L. Behrens, D. Dale, V. Makaryan, M. Kelley, A. Bolyard, M. Mezger, C. Gruenes, P. Lang, C. Lengerke, K. Welte, J. Skokowa *(Tübingen, Germany)
12:30	**Transcriptional contingency in the early hematopoiesis of inherited bone marrow failure syndromes** Alfredo de Jesús Rodríguez Gómez*, A. De La Cruz, P. Siliceo, P. Leal Anaya *(Mexico City, Mexico)
12:45	**In vitro model of stage-specific evolution of clonal hematopoiesis in congenital neutropenia to CN/AML** Natalia Alejandra Borbaran Bravo*, A-S Hellmuth, S. Kandabarau, M. Klimiankou, J. Skokowa *(Tübingen, Germany)
13:00	*Lunch*

13:30 – 14:30 - (Epi-)genomic landscape in JMML

Moderators:

- Anupuma Rao (London, UK)
- Maximilian Schönung (Heidelberg, Germany)

13.30	**Comprehensive characterization of somatic *PTPN11*-mutated JMML** Edoardo Muratore*, V. De Haas, D. Bresters, C. Flotho, P. Goncalves, M. Hofmans, M. Kavcic, T. Lammens, D.B. Lipka, L. Petrikkos, Charlotte M. Niemeyer for the EWOG-MDS JMML Working Group, Peter Nöllke, Senthilkumar Ramamoorthy, Dirk Lebrecht, Riccardo Masetti, Maximilian Schönung, EWOG-MDS National Coordinators for EWOG-MDS *(Bologna, Italy)
13:45	**The watch-and-wait approach in patients with juvenile myelomonocytic leukemia (JMML): Results of the French national cohort** Neven Quentin*, C. Arfeuille, A. Caye Eude, P. Durand, E. Lainey, B. Nelken, M. Nolla, A. Sterin, C. Thomas, C. Khouri, M-E. Dourthe, M. Fahd, F. Millot, B. Neven, A. Petit, J-H. Dalle, A. Baruchel, H. Cavé, M. Strullu *(Paris, France)
14:00	**Long-term outcome of an observational approach in patients with *NRAS*-mutated juvenile myelomonocytic leukemia and absence of high risk features** Jolien De Waele*, M. Hofmans, A. Fischer, A. Catala, M. Dworzak, M. Erlacher, H. Hasle, R. Masetti, M. Schmugge, M. Ussowicz, S. Keogh, O. Tufekci, M. A. Yesilipek, A. Yoshimi, D. Lebrecht, M. Schönung, P. Nöllke, B. De Moerloose, C. M. Niemeyer *(Ghent, Belgium)
14:15	**Single-cell trajectories of monosomy 7 driven *KRAS*-mutant JMML** Lili Kotmayer*, S. Sahoo, Y. Masanori, A. Frisanco, F. Andresen, C. Flotho, M. Erlacher, C. M. Niemeyer, M. Wlodarski *(Memphis, USA)

14:30 – 15:15 - JMML risk factors for outcome

Moderators:

- Christian Flotho (Freiburg, Germany)
- Daniel Lipka (Heidelberg, Germany)

Mini Panel: Towards an international consensus on risk criteria
One size fits all? Risk criteria and genetic groups Hélène Cavé (Paris, France)
Is a DNA methylation score mandatory? Hideki Muramatsu (Nagoya, Japan)
What is the role of subclonal mutations in risk definition? Elliot Stieglitz (San Francisco, USA)
How many parameters do we need? Edoardo Muratore (Bologna, Italy)

14:00 - 15:00	**Meeting of reference pathologists EWOG pathology: MDS/MPN and MPN– clear cut or overlapping?** • Martina, Rudelius (Munich, Germany) Room: Strasbourg, Estrel Hotel
15:15	*Coffee break*
15:30 – 17:00 - Classification and clinical approach to overlap syndromes and rare MPDs Moderators: • Valérie De Haas (Utrecht, The Netherlands) • Chyzyński Bartosz (Warsaw, Poland)	
15:30	**Biological and clinical implications of subclonal genetic events in juvenile myelomonocytic leukemia revealed via error-corrected next-generation sequencing** Daichi Sajiki*, H. Muramatsu, M. Wakamatsu, K. Ogawa, D. Yamashita, A. Yamamori, K. Narita, S. Kataoka, Y. Takahashi *(Nagoya, Japan)
15:45	**Predicting the unpredictable: chimerism as a relapse indicator in JMML post-haematopoietic stem cell transplant** Susanne Kricke Orszulik*, A. Rao, E. Louka, S. Adams *(London, UK)
16:00	**Myeloproliferative neoplasms (MPN) diagnosed in childhood and adolescence – prospective data on clinical and genetic characteristics from the German national MPN childhood registry** Axel Karow*, Y.L. Behrens, T. Reinkens, W. Hofmann, A. Gumann, R. Strasser, P. Wanjek, N. Di Donato, L. Seitz, M. Pontones, M-P. Hitz, Z. Wotschofsky, B. Schlegelberger, T. Ripperger *(Erlangen, Germany)
16:15	**Impulse statement: classification of overlap syndromes and rare MPD** Martina Rudelius (Munich, Germany)
16:30	End of scientific program
17:00	*Departure for evening program*

Saturday, 20.09.2025

7:30	*Breakfast*
7:30	Closed EWOG Meeting
08:30 – 10:15 - Advanced MDS - from clones to therapy Moderators: • Jochen Buechner (Oslo, Norway) • Hila Dias-Polak (Haifa, Israel)	
08:30	**Keynote lecture: Therapy-induced clonal hematopoiesis** Sam Behjati (Cambridge, UK)
09:00	**Genetic origins and clonal trajectories of monosomy 7 in pediatric MDS** Sushree Sahoo*, V.P. Pastor, M. van Roosmalen, D. Lebrecht, S. Ramamoorthy, C.G. Harris, A. van Leeuwen, L. Kotmayer, K. Schratz, A. Yoshimi, B. Strahm, P. Noellke, A. Gebert, R. van Boxtel, C.M. Niemeyer, M. Erlacher, M. Wlodarski *(Memphis, USA)
09:15	**Genetic landscape of myelodysplastic syndrome in children** Masataka Hasegawa*, K. Mimura, R. Ono, D. Keino, S. Tsujimoto, K. Isobe, T. Deguchi, H. Iwafuchi, H. Moritake, A. Manabe, S. Ogawa, K. Yoshida, D. Hasegawa (Tokyo, Japan)
09:30	**Genetic landscape of primary myelodysplastic syndromes with excess of blasts (MDS-EB) in children and adolescents** Davide Leardini*, L. Vinci, B. De Moerloose, H. Hasle, K. Heitink-Polle, M. Kavčič, K. Thus, D. Turkiewicz, C.M. Niemeyer, M. Erlacher, R. Masetti, M. Dworzak, and EWOG-MDS National Coordinators *(Bologna, Italy)
09:45	**Genetic landscape and outcomes after hematopoietic stem cell transplantation in children and adolescents with therapy-related myeloid neoplasms** Luca Vinci*, S. Ramamoorthy, E-V. Kornemann, V. De Haas, B. De Moerloose, M. Dworzak, M. Erlacher, G. Goehring, H. Hasle, K. Jahnukainen, K. Kallay, R. Masetti, P. Noellke, M. Schmugge, J. Stary, D. Turkiewicz, M. Ussowicz, F. Locatelli, T. N. Masmas, P. Sedlacek, M. Wlodarski, A. Yoshimi, M. Zecca, C. M. Niemeyer, B. Strahm *(Freiburg, Germany)
10:00	**Advanced myelodysplastic syndrome. The Dutch experience** Katja Heitink-Polle*, K. Thus, D. Bresters, V. De Haas, A. Vissers, A. Kors, M. Belderbos, L. Kester, E. Waanders, M. Bierings *(Utrecht, The Netherlands)
10:15	*Coffee break*

10:35 – 11:45 - Innovations in therapy and diagnostics for MDS Moderators: • Dominik Turkiewicz (Lund, Sweden) • Wolfgang Novak (Vienna, Austria)	
10:35	**Integrated immunophenotyping and drug response profiling in pediatric MDS-EB: The Zurich-Vienna experience** Zurich (Switzerland): Nastassja Scheidegger-Egloff, S. Gutnik, C. Moeller, F. Steffen, A. Arpagaus, L. Schori, S. Schuehle, S. Bohler, B. Haladik, A. Hurt, C.M. Koebl, M. Maurer-Granofszky, B. De Moerloose, H. Hasle, C.M. Niemeyer, M. Ussowicz, L. Vinci, A. Yoshimi-Noellke, K. Boztug, M.N. Dworzak, J-P. Bourquin, M. Erlacher, M. Schmugge, B. Bornhauser Vienna (Austria): Ben Haladik, M. Maurer-Granofszky, A. Hurt, C.M. Koelbl, N. Scheidegger, S. Gutnik, F. Steffen, B. De Moerloose, H. Hasle, M. Ussowicz, L. Vinci, A. Yoshimi-Noellke, J-P. Bourquin, C.M. Niemeyer, M. Schmugge, B. Bornhauser, M. Erlacher, M.N. Dworzak, K. Boztug
11:00	**BH3-mimetic treatment reveals subtype-specific BCL-2 protein dependency in pediatric MDS with excess blasts** Sheila Bohler*, A. Yoshimi-Noellke, N. Scheidegger, B. Haladik, B. Bornhauser, M.N. Dworzak, K. Boztug, M. Schmugge, C. M. Niemeyer, M. Erlacher *(Freiburg, Germany)
11:15	**Stratus Prime™ GMP compliant process to create high numbers of HLA compatible definitive HSPCS from IPSCS using Piezo1 agonists** Michael P Cooke*, A. Das, G. Singh, Z-J. Liu, Q. Lin, S.K. Patel, J. Li, V. G. Tirunagaru *(Cambridge, USA)
11:30	**Impulse statement: Myeloid targets in CAR T-cell therapy** Tobias Feuchtinger (Freiburg, Germany)
11:45 – 13:00 - Advances in molecular diagnostics for bone marrow failure Moderator: • Marcin Wlodarski (Memphis, USA) • Markus Schmugge (Zurich, Switzerland)	
11:45	**Keynote lecture: Establishing a diagnostic pipeline for bone marrow failure and inherited predisposition syndromes in Australia** Piers Blombery (Melbourne, Australia)

12:15	**Mini Panel: The future in germline diagnostics**
	Setting the stage: Germline testing concepts in hematologic diseases Piers Blombery (Melbourne, Australia)
	Germline testing using nails and buccal swabs: Lessons from JMML Hideki Muramatsu (Nagoya, Japan)
	Dual platform (WGS and WES) testing from direct skin biopsy Victor Pastor (Memphis, USA)
	Hair follicles NGS: Fast and reliable method for confirmation of germline status Dirk Lebrecht (Freiburg, Germany)
13:00	*Adjourn*

Poster

P1	C. Dufour	OUTCOMES OF IMMUNOSUPPRESSIVE AND TRANSPLANT THERAPIES IN PEDIATRIC PATIENTS WITH MODERATE APLASTIC ANEMIA: A SYSTEMATIC LITERATURE REVIEW
P2	C. Kelaidi	FAVORABLE LONG-TERM OUTCOMES WITH IMMUNOSUPPRESSIVE THERAPY IN CHILDREN WITH SEVERE APLASTIC ANEMIA
P3	E. Picotti	CLINICAL AND LABORATORY CHARACTERISTICS OF IMMUNE SUPPRESSION THERAPY RESPONDERS AND NON-RESPONDERS IN SWISS PEDIATRIC PATIENTS WITH APLASTIC ANEMIA OR HYPOCELLULAR REFRACTORY CYTOPENIA OF CHILDHOOD
P4	D. Montes	BONE MARROW FAILURE SYNDROMES AND APLASTIC ANAEMIA: GENOMIC LANDSCAPE AND OUTCOMES – ANALYSIS FROM A TERTIARY CARE CENTER OF WESTERN INDIA
P5	D. Dulla	HEPATITIS-ASSOCIATED VS IDIOPATHIC PAEDIATRIC APLASTIC ANAEMIA: CLONAL ARCHITECTURE AND DISEASE TRAJECTORY
P6	K. Ray	BUILDING RESOURCES FOR DISCOVERY AND TREATMENT: THE INSIGHT-HD BIOBANKING INITIATIVE FOR PEDIATRIC BONE MARROW FAILURE AND MYELODYSPLASTIC SYNDROMES
P7	M. Komonova	FULMINANT HEPATITIS ASSOCIATED APLASTIC ANEMIA PRESENTED CONCOMITANTLY WITH LIVER INJURY AND AFFECTED BY LIVER TRANSPLANTATION: A CASE SERIES
P8	A. Sharathkumar	ROMILOSTIM FOR TRTEATMENT OF CHILDREN AND YOUNG ADULTS WITH SEVERE APLASTIC ANEMIA AND MYELODYPLASTIC SYNDROME: A SINGLE CENTRE PILOT STUDY

P9	A. Delgado Beltran	CLINICAL CHARACTERISTICS AND OUTCOMES IN CHILDREN WITH SEVERE APLASTIC ANEMIA WHO RECEIVED IMMUNOMODULATORY THERAPY AND/OR TRANSPLANTATION AT THE MISERICORDIA PEDIATRIC HOSPITAL FOUNDATION 2015 TO 2024
P10	D. Karapinar	A FAMILY WITH MYSM1 MUTATION
P11	J. Fernandez-Orth	EPIGENETIC MECHANISMS AND THERAPEUTIC VULNERABILITIES IN A GATA2 HAPLOINSUFFICIENT MOUSE MODEL
P12	M. Alcaide Miranda	EXPLORING THE IMPACT OF GATA2 HAPLOINSUFFICIENCY ON DNA DAMAGE REPAIR IN MURINE HEMATOPOIETIC CELLS
P13	N. Lelli	WHEN STANDARD TESTS FALL SHORT: SOLVING A COMPLEX CASE OF INHERITED BONE MARROW FAILURE SYNDROME THROUGH WHOLE-EXOME SEQUENCING
P14	A. Frisanco Oliveira	GATA2 GENE HAPLOINSUFFICIENCY: A CLINICAL AND LABORATORY CHARACTERIZATION OF BRAZILIAN PATIENTS REGISTERED IN GCB-SMD-PED.
P15	P. Leal-Anaya	GENETIC LANDSCAPE OF INHERITED BONE MARROW FAILURE SYNDROMES IN PEDIATRIC MEXICAN PATIENTS: A NATIONAL REGISTRY OVERVIEW
P16	M. Vasileva	INHERITED BONE MARROW FAILURE WITH A PREDISPOSITION TO MYELODYSPLASTIC SYNDROMES/ACUTE MYELOID LEUKEMIA IN CHILDREN
P17	P. Shaker	THE ROLE OF GENETIC COUNSELORS IN BONE MARROW FAILURE: A CASE-BASED APPROACH TO SRP72 AND DHX34 VARIANTS OF UNCERTAIN SIGNIFICANCE
P18	V. Pastor Loyola	INTEGRATION OF A BONE MARROW FAILURE GERMLINE PANEL INTO THE COMPREHENSIVE CLINICAL GENOMICS PIPELINE AT ST. JUDE CHILDREN'S RESEARCH HOSPITAL: INSIGHTS FROM 22 MONTHS' EXPERIENCE
P19	B. Urbański	CANCER AND CANCER PREDISPOSITION SYNDROMES IN A POLISH INHERITED THROMBOCYTOPENIA COHORT
P20	J.B. Lang	BONE MARROW FAILURE IN LIGASE IV DEFICIENCY CURED BY ALLOGENEIC HSCT
P21	E. Sebastián	WHEN A "ZERO" CAN BE A DIAGNOSIS IN PEDIATRICS
P22	M. Muñoz	CLONAL EVOLUTION AND SOMATIC GENETIC RESCUE IN SAMD9 SYNDROME: PRESENTATION OF FOUR CASES FROM THE SPANISH REGISTRY OF PEDIATRIC MYELODYSPLASTIC SYNDROMES
P23	N.S. Malyasova	CO-EXITANCE OF BIALLELIC GERMLINE AND ACQUIRED SOMATIC RAD50 VARIANTS IN PEDIATRIC PATIENT WITH COMBINED PRIMARY IMMUNODEFICIENCY WITH SIGNS OF BONE MARROW FAILURE
P24	J. Nyiro	HIGH PREVALENCE OF GERMLINE PREDISPOSITION IN PAEDIATRIC MYELODYSPLASTIC SYNDROME: A SINGLE-CENTRE RETROSPECTIVE ANALYSIS
P25	S. Hirabayashi	NATIONWIDE RETROSPECTIVE STUDY OF GATA2 DEFICIENCY IN JAPAN: CLINICAL SPECTRUM AND TRANSPLANT OUTCOMES

P26	D. Bresters	IMPROVED DISEASE FREE SURVIVAL IN JMML. THE DUTCH EXPERIENCE.
P27	A. Frisanco Oliveira	HAPLOIDENTICAL HEMATOPOIETIC STEM CELL TRANSPLANTATION WITH POST-TRANSPLANT CYCLOPHOSPHAMIDE FOR CHILDREN WITH JUVENILE MYELOMONOCYTIC LEUKEMIA
P28	R. Balceiro	AZACITIDINE FOR PEDIATRIC PATIENTS WITH ADVANCED MYELODYSPLASTIC SYNDROME: EXPERIENCE FROM BRAZILIAN COOPERATIVE GROUP OF PEDIATRIC MYELODYSPLASTIC SYNDROME (GCB-SMD-PED).
P29	M. Maurer-Granofszky	A PILOT STUDY FOR MUTATION- BASED MRD ASSESSMENT TO GUIDE POST-TRANSPLANT THERAPEUTIC INTERVENTION IN JUVENILE MYELOMONOCYTIC LEUKEMIA.
P30	P. Riha	FOUR CASES OF DYSKERATOSIS CONGENITA TINF2 PATIENTS DIAGNOSED WITH BMF AND TRANSPLANTED IN CZECHIA BETWEEN 2002 AND 2024.
P31	L. Xiaolan	A SINGLE-CENTER RETROSPECTIVE ANALYSIS OF TRANSPLANTATION OUTCOMES IN PATIENTS WITH JUVENILE MYELOMONOCYTIC LEUKEMIA
P32	L. Xiaolan	IMPROVED OUTCOMES FOR PEDIATRIC PATIENTS WITH DE NOVO CHRONIC MYELOID LEUKEMIA IN BLAST PHASE BY EARLY STAGE ALLOGENEIC HEMATOPOIETIC STEM CELL TRANSPLANTATION
P33	X. Qin	RISK-ADAPTED THERAPY FOR JUVENILE MYELOMONOCYTIC LEUKEMIA: A MULTICENTER, PROSPECTIVE STUDY IN CHINA
P34	M. Kastamoulas	HEMATOPOETIC STEM CELL TRANSPLANTATION (HSCT) IN PEDIATRIC PATIENTS WITH SEVERE APLASTIC ANEMIA (SAA): THE GREEK EXPERIENCE
P35	T. Aksu	TELOMERE BIOLOGY DISORDERS: PHENOTYPE AND GENETIC FINDINGS
P36	A. Bataev	THE EFFECT OF THROMBOPOIETIN MIMETIC ELTROMBOPAG (EPAG) ON EX VIVO HAEMATOPOIETIC PROGENITORS CELLS PROLIFERATION AND DIFFERENTIATION IN DIAMOND BLACKFAN ANEMIA (DBA) PEDIATRIC PATIENTS
P37	A. Catala	FANCONI ANEMIA AND T-LYMPHOBLASTIC LYMPHOMA. REPORT OF TWO CASES
P38	M. Nasri	CRISPR/CAS9-BASED GENE EDITING FOR ELANE-ASSOCIATED CONGENITAL NEUTROPENIA: A PATH TO CURE AND LEUKEMIA PREVENTION
P39	N. Karadas	MALIGNANCY IN FANCONI APLASTIC ANEMIA; SINGLE CENTER EXPERIENCE
P40	M. Yousef	OUTCOMES OF RABBIT VS. HORSE ANTI-THYMOCYTE GLOBULIN IN IMMUNOSUPPRESSIVE THERAPY FOR REFRACTORY CYTOPENIA OF CHILDHOOD
P41	M. Boals	A CASE OF SEVERE CONGENITAL THROMBOCYTOPENIA WITH HETEROZYGOUS PATHOGENIC NBEAL2 MUTATION.
P42	F. Pegoraro	CSF3R MUTATIONS IN CONGENITAL NEUTROPENIA, A LONG STORY: DATA FROM THE ITALIAN REGISTRY

P43	H. Reed	GENETIC RE-EVALUATION OF SHWACHMAN-DIAMOND SYNDROME-LIKE AND NEUTROPENIA CONDITIONS
P44	A.Kennedy	PARADIGM: ST. JUDE INITIATIVE TO ADVANCE DEVELOPMENT OF INDIVIDUALIZED BASE AND PRIME EDITING THERAPIES FOR BONE MARROW FAILURE SYNDROMES
P45	A.Droste	FANCE MISSENSE VARIANT WITH MILD PHENOTYPE BUT RETAINS HIGH MMC SENSITIVITY
P46	N. Karadas	A RARE BONE MARROW FINDING IN CHILDHOOD: MYELOFIBROSIS
P47	L. Kotmayer	REPORT OF A FIRST CASE OF PEDIATRIC MDS WITH VEXAS SYNDROME
P48	S.M. Makkeyah	CLINICAL SPECTRUM OF AN EGYPTIAN COHORT OF CHILDREN WITH MYELODYSPLASTIC SYNDROME
P49	M. Pavlovic	HUMAN HERPES VIRUS 6 INFECTION WITH BACTERIAL SEPSIS MIMICKING MYELODISPLASTIC SYNDROME IN AN INFANT
P50	T. Aksu	MYELODYSPLASTIC SYNDROME IN CHILDHOOD: THE HACETTEPE COHORT
P51	A.K. Gupta	THE GENOMIC LANDSCAPE OF JUVENILE MYELOMONOCYTIC LEUKEMIA ON WHOLE EXOME SEQUENCING
P52	J. Wang	MACROPHAGE POLARIZATION AND CD47 BLOCKADE IN PTPN11-MUTATED JUVENILE MYELOMONOCYTIC LEUKEMIA
P53	A.Frisanco Oliveira	FLOW CYTOMETRY IMMUNOPHENOTYPING AND JUVENILE MYELOMONOCYTIC LEUKEMIA: JUST FOR BLAST COUNT?
P54	D. Yamashita	GENETIC MUTATIONAL PROFILE INFLUENCES THE MORPHOLOGICAL CHARACTERISTICS OF ABNORMAL MONOCYTES IN PATIENTS WITH JUVENILE MYELOMONOCYTIC LEUKEMIA
P55	Z. Wehbe	MODELING JUVENILE MYELOMONOCYTIC LEUKEMIA USING PATIENT-DERIVED INDUCED PLURIPOTENT STEM CELLS TO UNRAVEL EPIGENETIC AND METABOLIC INTERPLAY
P56	S. Jin	CHARACTERIZATION OF MURINE STEM AND PROGENITOR CELLS HARBORING ONCOGENIC PTPN11 OR KRAS MUTATIONS
P57	R. Majhi	SH2B3 GENE MUTATION IN JUVENILE MYELOMONOCYTIC LEUKEMIA (JMML)- A FREQUENT CO-EXISTING MUTATION WITH RAS MUTATIONS
P58	J. Rajak	SILENT CONVERSATIONS: JMML'S INTERPLAY WITH T CELL
P59	S. Frahia Bento da Silva	MOLECULAR PROFILING OF JUVENILE MYELOMONOCYTIC LEUKEMIA: CHARACTERIZATION OF PATIENTS WITH MULTIPLE ONCOGENIC VARIANTS USING A TARGETED NGS PANEL
P60	E. Muratore	COAGULATIVE ABNORMALITIES IN PATIENTS WITH CBL SYNDROME
P61	J. Werner	CELLULAR IMMUNOTHERAPY TARGETING CLL-1 FOR JUVENILE MYELOMONOCYTIC LEUKEMIA

The Symposium would not have been possible without the financial support from:

Förderverein für krebskranke Kinder e.V. Freiburg i.Br.

Deutsche Kinderkrebsstiftung

Stiftung Lichterzellen

We kindly acknowledge support from the following sponsors:

Bristol Myers Squibb

Medac GmbH

Pfizer Pharma GmbH

Neovii Biotech GmbH

Novartis Pharma GmbH

www.ewogberlin2025.org