Midterm Meeting on New Indications in Immune Dysregulatory, Autoinflammatory and Autoimmune Diseases

Brescia, Italy
22-24 June 2023

WELCOME

We are pleased to announce a unique initiative. Three EBMT working parties (Autoimmune, Inborn Errors and Paediatric) will all meet on 22 to 24 June 2023 for the Midterm Meeting in Immune Dysregulatory, Autoinflammatory and Autoimmune Diseases. The meeting will be held at the Auditorium di S.Giulia in Brescia (Italy) and will be part of the event 'The culture that cures', one of the scientific manifestations of the celebration "Bergamo and Brescia Capitali della Cultura", which are Italy Covid martyr towns. Why? It is about time to discuss the therapeutic approach to classical as well as emerging immunological diseases from an interdisciplinary viewpoint, bringing together both transplant physicians and disease specialists to evaluate the transplant and cellular therapy options.

Haematopoietic Stem Cell Transplantation (HSCT) is widely used to treat patients with malignant and non-malignant haematological disorders. During the last decade, major changes have occurred in the field of allogeneic HSCT, including the introduction of less aggressive conditioning regimens, improved patient selection, new cell manipulation techniques and modern supportive therapy, with substantial progress in reducing GvHD because of more accurate HLA-typing and better GvHD prevention, opening this procedure to a growing number of non-malignant disorders, including patients affected by diseases of the haematopoietic system and therefore of immunity who have a dramatically impaired quality of life. Progressively, the choice of donors and the sources of HSCs have enlarged, extending transplant indications to more patients.

Moreover, HSCT for autoimmune diseases (ADs) is today facing a unique developmental phase across EBMT. Autologous transplant has become an integral part of treatment algorithms in various ADs, providing treatment-free remissions by the reinduction of self-tolerance. More and more genetic backgrounds of autoimmune diseases are deciphered, and new monogenic diseases leading to loss of tolerance and AD are described, extending the indication for allogeneic HSCT. Recently, novel cellular therapies (ie CART cells, mesenchymal cells) have been successfully adopted as therapeutic options for severe ADs. Invited experts from all WPs will discuss the therapeutic options for the classical ADs, but also for emerging new diseases that overlap the specificities of the 3 WPs. The meeting welcomes contributions by colleagues who are invited to submit scientific abstracts and/or case presentations to be actively discussed in the sessions or as posters.

We look forward to meeting you all in Brescia,

Fulvio Porta, *local organiser*
Raffaella Greco, *Autoimmune Diseases Working Party chair*
Tobias Alexander *Autoimmune Diseases Working Party secretary*
Bénédicte Neven, *Inborn Errors Working Party chair*
Michael Albert, *Inborn Errors Working Party secretary*
Selim Corbacioglu, *Paediatric Diseases Working Party chair*
Katharina Kleinschmidt, *Paediatric Diseases Working Party secretary*

2023 BERGAMO BRESCIA Capitale Italiana della Cultura

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22-24 June 2023

Thursday, 22 June 2023

13:00 – 14:00	**Registration**	
14:00 – 15:00	**Session I: State of the art of HSCT in immunedysregulatory, autoinflammatory and autoimmune diseases** **Chairs: Fabio Candotti (IT) & Fulvio Porta (IT)**	
14:00 – 14:20	HSCT in inborn errors of immunity	Bénédicte Neven (FR)
14:20 – 14:40	Autologous vs. allogeneic HSCT in AD	Raffaella Greco (IT)
14:40 – 15:00	Future of cellular therapies	Selim Corbacioglu (DE)
15:00 – 16:30	**Session II: Pathophysiology** **Chairs: Aleixo Muise (CA) & Raffaele Badolato (IT)**	
15:00 – 15:20	Genetics in lupus	Christian Hedrich (UK)
15:20 – 15:40	Mechanism of autoimmunity in IEI	Eleonora Gambineri (IT)
15:40 – 16:00	Plasma cells as drivers of chronic inflammation in autoimmune diseases	Tobias Alexander (DE)
16:00 – 16:20	T-cell compartment	Maria Teresa Cencioni (UK)
16:20 – 16:30	Discussion	
16:30 – 17:00	**Coffee Break**	
17:00 – 18:30	**Session III: Key steps in the procedure** **Chairs: Eleonora Gambineri (IT) & John Snowden (UK)**	
17:00 – 17:20	Transplant Indications according to EBMT guidelines	John Snowden (UK)
17:20 – 17:40	Conditioning regimen and ATG in AD	Raffaella Greco (IT)
17:40 – 18:00	Conditioning and serotherapy for IEI	Arjan Lankester (NL)
18:00 – 18:20	The role of low-dose radiotherapy	Fulvio Porta (IT)
18:20 – 18:30	Discussion	

All talks include 5 minutes for Q&A at the end

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Friday, 23 June 2023

08:30 – 10:00	**Session IV: Neurologic diseases** **Chairs: Alice Mariottini (IT) & Lucia Moiola (IT)**	
08:30 – 08:50	HSCT in MS	Joachim Burman (SE)
08:50 – 09:10	HSCT in ADA2 deficiency	Isabelle Meyts (BE)
09:10 – 09:30	HSCT in rare neurologic indications	Basil Sharrack (UK)
09:30 – 09:40	Case presentation: Impact of AHSCT on acute and chronic neuroinflammation in aggressive multiple sclerosis	Giacomo Boffa (IT)
09:40 – 09:50	Case presentation: Fludarabine and cyclophosphamide as safety and affective lymphoablative conditioning regime for multiple sclerosis	Anatolij Rukavitsyn (RU)
09:50 – 10:00	Discussion	
10:00 – 11:30	**Session V: Lupus and vascular diseases** **Chairs: Tobias Alexander(DE)**	
10:00 – 10:25	Transplant in lupus	Tobias Alexander (DE)
10:25 – 10:50	HSCT in interferonopathies	Marie Louise Fremond (FR)
10:50 – 11:15	AHSCT in BEHCET's disease	Mathieu Puyade (FR)
11:15 – 11:25	Case presentation: Long-term Disease Remission of Neuromyelitis Optica with Allogeneic Hematopoietic Stem Cell	Giorgio Orofino (IT)
11:25 – 11:30	Discussion	
11:30 – 12:00	**Coffee Break**	
12:00 – 13:40	**Session VI: Hematopoiesis** **Chairs: Bénédicte Neven (FR) & Alessandro Aiuti (IT)**	
12:00 – 12:20	Autoimmune dysregulation and purine metabolism in adenosine deaminase deficiency	Alessandro Aiuti (IT)
12:20 – 12:40	Autologous HSCT in pediatric cytopenia	Federica R. Achini (CH)
12:40 – 13:00	Monogenetic causes of cytopenia	Jérome Hadjadj (FR)
13:00 – 13:20	VEXAS	Carmelo Gurnari (IT)
13:20 – 13:30	Case presentation: How to investigate VEXAS: early identification and characterization of HSCT candidates	Bruno Alessandro (IT)
13:30 – 13:40	Discussion	
13:40 - 14:30	**Lunch Break**	
14:30 - 16:00	**Session VII: Scleroderma and other Skin diseases** **Chairs: Dominique Farge (FR) & Arjan Lankester (NL)**	
14:30 – 14:55	CTLA-4 Insufficiency	Thomas Fox (UK)
14:55 – 15:20	HSCT in ARPC1B and related diseases (Actinopathies)	Stefano Volpi (IT)
15:20 – 15:45	HSCT vs. MSC in reversing skin fibrosis	Nicoletta del Papa (IT)
15:45 – 15:55	Case presentation: Esophageal motility in systemic sclerosis before and after autologous hematopoetic cell transplantation	Jan Storek (CA)
15:55 – 16:00	Discussion	
16:00 – 17:30	**Session VIII: Systemic Inflammation** **Chairs: Matteo Doglio (IT) & Despina Moshous (FR)**	
16:00 – 16:25	Allogeneic hematopoietic stem cell transplantation for severe, refractory juvenile idiopathic arthritis	Juliana M. F. Silva (UK)
16:25 – 16:50	IEI with inflammatory osteoarticular involvement	Tadej Avcin (SI)
16:50 – 17:10	Case presentation: Sideroblastic anemia with B-cell immunodeficiency, periodic fevers, developmental delay (SIFD) due to novel bi-allelic TRNT1 mutations successfully treated with Hematopoietic Stem Cells Transplantation (HSCT)	Giulia Baresi (IT)
17:10 – 17:30	Discussion	

All talks include 5 minutes for Q&A at the end

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Saturday, 24 June 2023

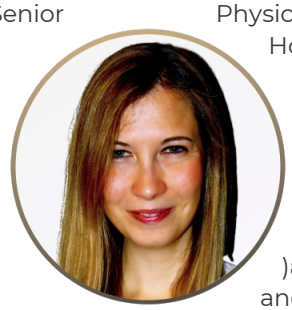
08:00 – 09:00	**Session IX: Heart and Lung** **Chairs: Tobias Alexander (DE) & Selim Corbacioglu (DE)**	
08:00 – 08:20	Pulmonary alveolar proteinosis and HSCT	Michael Albert (DE)
08:20 – 08:40	Improvement of lung function in SSc after HSCT	Dominique Farge (FR)
08:40 – 08:50	Case presentation: Triple hematopoietic stem cell transplantation in a boy with refractory systemic juvenile idiopathic arthritis – a case report	Oktawiusz Wiecha (PL)
08:50 – 09:00	Discussion	
09:00 – 10:45	**Session X: Gastroenterologic inflammatory diseases** **Chairs: Michael Albert (DE) & Francesco Onida (IT)**	
09:00 – 09:20	Monogenetic causes of IBD	Daniel Kotlarz (DE)
09:20 – 09:40	Genetic Predisposition to IBD	Aleixo Muise (CA)
09:40 – 10:00	Autologous HSCT in refractory CD: improving safety while maintaining efficacy and immune re-setting	Azucena Salas (SP)
10:00 – 10:20	Nonmyeloablative allogeneic HSCT in Crohn´s Diseases	Richard Burt (USA)
10:20 – 10:30	Case presentation: IL10R deficiency and Hemopoietic cell transplantation (HCT): outcome of 2 patients treated in a single Center with CBU HCT	Stefano Rossi (IT)
10: 30 – 10:45	Discussion	
10:45 – 11:15	**Coffee Break**	
11:15 – 12:45	**Session XI: Non-transplant approaches** **Chairs: Raffaella Greco (IT) & Bénédicte Neven (FR)**	
11:15 – 11:40	Targeted therapy in monogenetic IEI	Markus Seidel (AT)
11:40 – 12:05	CD19 CAR T-cells in SLE	Fabian Müller (DE)
12:05 – 12:30	CAR-Tregs in autoimmunity	Matteo Doglio (IT)
12:30 – 12:40	Case presentation: Severe immune effector cell associated HLH-like syndrome: a case report of successful treatment in a pediatric patient after CAR-T cell infusion	Benedetta E. Di Majo (IT)
12:40 – 12:45	Discussion	
12:45 – 13:00	**Closing**	

All talks include 5 minutes for Q&A at the end

Fabio Candotti (Italy)

Fabio Candotti is Full Professor of Medicine at the University of Lausanne and Head Physician in the Division of Immunology and Allergy of the Lausanne University Hospital, in Lausanne, Switzerland. Dr. Candotti received his MD from the University of Brescia (Italy) and completed his residencies in Pediatrics and Pediatric Allergy and Immunology at the Universities of Pavia (Italy) and Brescia (Italy), before joining the National Institutes of Health in the USA where he performed postdoctoral research in the field of gene therapy. Dr. Candotti then joined the faculty of the National Human Genome Research Institute of NIH as a Tenured Senior Investigator/Attending Physician from 1998 to 2014. During this time, Dr. Candotti focused his clinical activities on rare inherited disorders of immunity, with particular interest on the Wiskott-Aldrich syndrome and adenosine deaminase deficiency. In 2014, he was recruited by the University Hospital of Lausanne, Switzerland where he currently heads the clinical Unit on Primary Immunodeficiencies of the Division of Immunology and Allergy. Dr. Candotti's clinical and research interests are the discovery of the genetic and molecular bases of primary immunodeficiencies and the development of gene therapy approaches for these diseases. He has published more than 170 indexed articles and 60 reviews and book chapters on primary immunodeficiency diseases and gene therapy. He currently serves as President of the European Society for Immunodeficiency, Associate Editor of Pediatric Allergy and Immunology, and Specialty Chief Editor of the Primary Immunodeficiencies section of Frontiers.

Raffaella Greco (Italy)



Senior Physician in the Haematology and Blood/Marrow Transplant (BMT) Unit of the IRCCS San Raffaele Hospital in Milano, Italy. Haematologist involved in hematopoietic stem cell transplantation (HSCT) and cellular therapies in all spectrum of haematological cancers and non-malignant indications, including autoimmune diseases. Her expertise in this field encompasses allogeneic and autologous stem cell transplantation (malignant and non-malignant diseases), cellular therapies (i.e. CART cells), immune reconstitution, biomarkers, transplant complications (i.e graft versus host disease, infections). Her career has been focused on several clinical research projects on HSCT and cellular therapies. She has (co-)authored many research articles in peer reviewed journal as well as reviews, book chapters and best-practice guidelines in the field. She has been significantly involved with the European Group for Blood and Marrow Transplantation (EBMT), as Autoimmune Diseases Working Party (ADWP) Chair, Scientific Council Representative with the Education Portfolio, active member of the ADWP and Cellular Therapy & Immunobiology Working Party (CTIWP). Area of expertise: haematology, allogeneic and autologous hematopoietic stem cell transplantation (malignant and non-malignant diseases, including autoimmune diseases), cellular therapy and immunotherapy, immune reconstitution, transplant complications, graft versus host disease, infections in immunocompromised patient.

Tobias Alexander (Germany)

Charité University Medicine Berlin. Autoimmune Diseases Working Party secretary.

Isabelle Meyts (Brussels)

Prof. dr. Isabelle Meyts is a pediatric hemato-oncologist and immunologist at the Department of Pediatrics in University Hospitals Leuven, Belgium where she heads the Division of Primary Immunodeficiencies, which she founded. She is a professor at the KU Leuven and leader of the Laboratory for Inborn Errors of Immunity. Her research focuses on the immunological and genetic analysis of patients with inborn errors of immunity next to ERC funded research on ADA2 deficiency.

Carmelo Gurnari (Italy)

After graduating from Pavia (Italy) medical school in 2015 with a thesis on Paediatric Haematology/Oncology, Dr. Gurnari completed his training in Haematology at the University of Rome, Tor Vergata. He then decided to continue his education at Tor Vergata, and he is currently enrolled in a Ph.D program in "Immunology, Molecular Medicine and Applied Biotechnology". Simultaneously, Dr. Gurnari has spent the last 3 years working at the Department of Translational Haematology and Oncology Research of Cleveland Clinic (USA) where he has been focusing on the genomics of bone marrow failure disorders and myeloid neoplasia. His clinical and translational research expertise encompasses myeloid malignancies with a special focus on acute promyelocytic leukemia, myelodysplastic syndromes, VEXAS, and acute myeloid leukemia. Dr. Gurnari is a member of the European Haematology Association (EHA) for which serves as a Mentor of the Classical Master Class Program, and of the European Society for Blood and Marrow Transplantation (EBMT), and he is actively involved in the activities of the Trainee Committee. He is also a member of other national and international societies such as the American Association for the Advancement of Science, the Italian Haematology Association and the International Paroxysmal Nocturnal Haemoglobinuria Interest Group. In these years, Dr. Gurnari has been awarded several international prizes such as the Tito Bastianello Young Investigator Award in MDS research and the ASH-IPIG Award for PNH research (2020 and 2022).

Arjan Lankester (Netherlands)

Arjan C Lankester, MD, PhD, Professor of Paediatrics and Stem Cell Transplantation, Willem-Alexander Children's Hospital, Leiden University Medical Center, The Netherlands After obtaining his M.D. from the University of Leiden and a PhD on B cell receptor signaling from the University of Amsterdam, he was trained as paediatrician-immunologist at Leiden University Medical Center (LUMC). Since 2009 he is clinical director of the JACIE-accredited Paediatric Stem Cell Transplantation program which serves as the national centre for stem cell therapy in patients with inherited immune disorders. In 2016 he was appointed as professor of Paediatrics and Stem Cell Transplantation at the University of Leiden. He is heading the LUMC expert centre on inherited immune disorders and stem cell transplantation (SCT) which is full member of ERN-RITA. His primary research interest is to improve efficacy and safety of stem cell therapy with particular focus on optimizing conditioning regimens and immune reconstitution after SCT. He has conducted and coordinated many single and multicenter studies including investigator-initiated adoptive cellular therapy trials, and is PI of a first-in human gene therapy trial for RAG1 SCID. He is the past chair of Inborn Errors Working Party of the European Society for Blood and Marrow Transplantation and European Society for Immune Deficiencies.

Jérome Hadjadj (France)

Saint-Antoine Hospital / Sorbonne University

Michael Albert (Germany)

Dr. von Hauner University Children's Hospital, LMU. Inborn Errors Working Party secretary.

Juliana Montibeller Furtado e Silva (United Kingdom)

Great Ormond Street Hospital.

Fulvio Porta (Italy)

Dr. Fulvio has worked for over 24 years as a general consultant in Pediatrics covering on-calls for all pediatric diseases. He is currently working at the second largest teaching hospital in Italy. His unit consists of 26 beds, with 7 doctors, 30 nurses and technicians. They follow children affected by immnological diseases (primary or secondary),and oncohematological diseases (leukemia, solud tumors, lymphoma). Dr. Fulvio graduated in Medicine at Pavia University in 1983 and started the Specialization School of Pediatrics in fall of 1983 and continued his activity within the immunology laboratory of the Pediatric University Clinic in Pavia with major interests on cellular immunology. In 1987, He entered specialization school in Hematology and completed in 1991. He became the head of Oncohematology and BMT unit of the Pediatric Department of hte Children Hospita in Spedali Civili di Brescia in 1990 and in 2003, he became the Director of hte Pediatric Oncology Department of the Brescia Province. He was the secretary of hte Inborn Errors working Party of the European Group of BMT from 2009-2013 and the President of the Italian Association of Oncology and Hematology from 2010 to 2012. This organization coordinates the activity of 53 national cancer centers offering common national diagnostic and therapeutic protocols. His main lines of research in the Stem Cell laboratory that he coordinated are regenerative therapies by stem cell of genetic diseases, prenatal and post-natal stem-cell therapies, BMT in solid tumor patients by alloreactive HLA non identical KIR family donors. Moreover, he is also an author for more than 130 publications quoted in PubMed.

Matteo Doglio (Italy)

San Raffaele Scientific Institute.

Thomas Fox (United Kingdom)

I am a clinical academic haematologist at University College London with an interest in allogeneic stem cell transplantation and gene therapy. My subspecialty interest is in the use of this curative technique in adolescents, young adults and adults with inborn errors of immunity. I earned my PhD at University College London under the supervision of Professors Emma Morris, Siobhan Burns, Claire Booth and David Sansom and defended my thesis to Pavel Tolar and Alessandro Aiuti. My current academic research is focused on T cell gene editing to treat T cell mediated inborn errors of immunity. I am working on translating a T cell gene therapy approach for CTLA-4 Insufficiency into the clinic.

Stefano Volpi (Italy)

I'm a pediatrician with a PhD in genetics, caring for patients with primary immunodeficiencies and supervising basic research projects in the field of immune dysregulation and autoinflammatory syndromes. Beside my clinical training at Gaslini Children Hospital, in Genova, I followed several projects of basic immunology at the Institute for Research in Biomedicine in Bellinzona, in Gigi Notarangelo's lab at Boston Children's Hospital, Harvard University and in Fabio Candotti's lab at Lausanne University Hospital. My research focus is the study of immune dysregulation and inflammation using in vitro and in vivo models of primary immunodeficiencies.

Joachim Burman (Sweden)

Dr Joachim Burman graduated from the Medical School of Uppsala University in 2001 and finished his residency training in Neurology in 2009. In 2017 he was appointed Associate Professor of Neurology at Uppsala University. He is currently the head of the Multiple Sclerosis Outpatient Clinic at Uppsala University Hospital. In his research, he has investigated biological and clinical effects of autologous hematopoietic stem cell transplantation for multiple sclerosis in order to understand why this treatment is effective, which patients benefit most from the procedure and what the adverse events and long-term outcomes of autologous hematopoietic stem cell transplantation are.

Mathieu Puyade (France)

Head of the transplant program for AID in Poitiers, France. Member of the ADWP.

Eleonora Gambineri (Italy)

Professor Gambineri has a long-standing expertise in the field of inborn errors of immunity (IEI), and a well-established network of collaborations worldwide. Her background and strong research interest have always been revolved around immune dysregulation disorders. Since 2006 she is a board member of the European Society for Immunodeficiencies, serving with different roles. Her expertise was recently transferred towards the haemato-oncology field. She is leading the Immune-haemato-oncology Unit at Anna Meyer Children's Hospital. The focus of her work is to define the role of immune system in common paediatric haematological diseases and malignancies, ultimately translating new findings from IEI into oncology/haematology, which fits the goal of the present proposal. She will fully supervise the study and her professional background and networks will contribute to refer patients nationally and internationally.

Dominique Farge (France)

As Professor of Internal Medicine since 1994 at St-Louis APHP and Paris Cité Univeristy, I have a long-standing commitment to the field of transplantation and stem cell therapy. In the last 30 years I have focused on stem cell therapy in rare autoimmune diseases. I conducted clinical and translational research, and participated in clinical activity, in hematopoietic stem cells transplantation (HSCT) and mesenchymal stem cell (MSC) therapy for autoimmune diseases. I have been involved in the development of several European guidelines for stem cell therapies and worked at both French and European levels to coordinate several clinical and translational research programs on rare autoimmune diseases, including Systemic Sclerosis (SSc), Systemic Lupus Erythematosus (SLE), severe forms of Multiple Sclerosis (MS) and Crohn's disease. I am a co-founding elected board member of the European Scleroderma Trials and Research Group (EUSTAR). I have been a member of the Autoimmune Diseases Working Party (ADWP) of the European Bone Marrow Transplant Association (EBMT) since 1998, acting as secretary of the ADWP in 2004 and then elected chair for 2 mandates from 2010 to 2016. While coordinating the first clinical trials SSc (PI for PHRC for HSCT 1997, MSC 2011), SLE, MS, Diabetes and Crohn's disease, I founded the French MATHEC (Maladies Auto Immunes et Thérapie Cellulaire) network dedicated to stem cell therapy in auto-immune disease, which has been labelled and funded as Center of Reference for Rare Autoimmune Diseases in Ile de France since 2017 (www.mathec.com). Since 2017, I have been a member of the International Society of Cell Therapy www.ISCT.org and was elected as Board Member of Directors (2021-2023). Amongst 500 peer-reviewed articles (HI: 72), I contributed as Editor to 4 books, including the "Hematopoietic stem cell transplantation and cellular therapies for auto-immune diseases 2021. Editors R Burt, D Farge, RJ Snowden, R Saccardi, Milton CRC Press, Chicago, 690 Pages".

Federica Achini-Gutzwiller (Switzerland)

Federica Achini-Gutzwiller is a pediatric hematologist. Her research focus is on pediatric stem cell transplantation and in particular on serotherapy pharmacokinetics and pharmacodynamics in children with severe congenital immunological and hematological diseases.

Aleixo Muise (Canada)

Hospital for Sick Children.

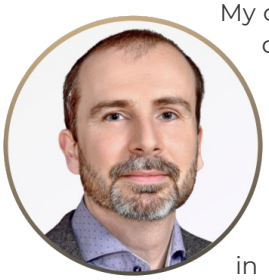
Benedicte Neven (France)

Necker children hospital.

Tadej Avcin (Slovenia)

Tadej Avcin is Head of Department of Allergology, Rheumatology and Clinical Immunology at the Children's Hospital, University Medical Center Ljubljana (Slovenia) and Professor of Pediatrics at the University of Ljubljana, Faculty of Medicine (Slovenia). He studied medicine and pediatrics at the Faculty of Medicine, University of Ljubljana (Slovenia) and completed fellowship in pediatric rheumatology at the Hospital for Sick Children, University of Toronto (Canada). His clinical and research focus are systemic autoimmune and auto-inflammatory diseases with an emphasis on juvenile idiopathic arthritis, systemic connective tissue diseases and diseases of immune dysregulation. He has published more than 160 indexed publications and 19 book chapters on pediatric rheumatology and immunology. He is currently Secretary of the Paediatric Rheumatology European Association (PReS), past Chairman of the European League Against Rheumatism Standing Committee on Pediatric Rheumatology, and Member of the Advisory Council of Paediatric Rheumatology International Trials Organisation.

Christian Hedrich (United Kingdom)



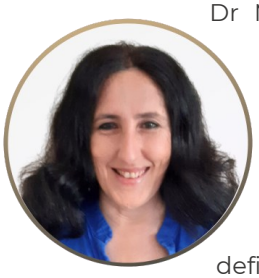
My core research interests are molecular mechanisms of cytokine regulation and their effects on disease expression in the spectrum from autoinflammation to autoimmunity. Special foci include the autoinflammatory bone disorder chronic nonbacterial osteomyelitis (CNO), the mixed-pattern disease psoriasis, and the prototypical autoimmune disorder systemic lupus erythematosus (SLE). SLE is a largely T cell-mediated autoimmune disorder that can affect most organs of the human body. In my laboratory, we are investigating genetic and epigenetic variants in jSLE and their effects on age at disease-onset and associations with disease outcomes. This may allow us to predict disease courses and define therapeutic targets in the future. Another focus are transcription factor networks contributing to epigenetic alterations. Disturbed transcription factor expression and activation in T cells from SLE patients result in effector T cell phenotypes in the CD4+ and the TCR+CD3+CD4-CD8- (so-called double negative, DN) T cell compartments. Applying CRISPR/Cas9 technology, we are eliminating single transcription factors that are considered central for SLE to determine their exact contribution to altered T cell responses. We hope that our work will contribute to novel therapeutic options targeting pathological effector T cell responses in SLE and other autoimmune disorders. Another focus of my work in the mixed-pattern disorder psoriasis that combines features from autoinflammatory and autoimmune disorders. This is of special interest, since psoriasis shares key molecular signatures with SLE. My research group is interested in the contribution and phenotypes of CD4+, CD8+ and DN T cells in health and disease.

Applying state-of-the-art technology, we identified effector DN T cells as contributors to pro-inflammatory cytokine expression in the skin of patients with psoriasis. After the definition of T cell phenotypes within the CD4+, CD8+ and DN T cell compartments, we are aiming to i) define signature cytokine expression patterns in effector T cells in health and disease, and ii) determine molecular mechanisms orchestrating T cell lineage determination. As mentioned above, effector T cells also reflect a key contributor to inflammation and tissue damage in SLE. Thus, research in (more easily accessible) samples from psoriasis patients may guide future directions and developments in SLE.

Marie-Louise Frémond (France)

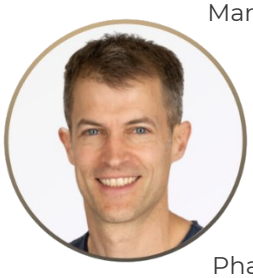
Dr Frémond is a clinician scientist (MD-PhD, Associate Professor) at Necker Hospital and Imagine Institute (Université Paris Cité) with developing expertise in monogenic autoinflammation driven by interferon. She works as a paediatric rheumatologist at the Necker Hospital, an internationally recognised centre of excellence in paediatric rare diseases. She joined the Crow laboratory (Imagine Institute, Paris) in 2015 to pursue her thesis and carries on her research work there, covering a translational theme in type I interferonopathies.

Maria Teresa Cencioni (Italy)



Dr Maria Teresa Cencioni is a senior research associate at Imperial College London in the department of Brain Sciences where is working with Professor Paolo Muraro in the field of Neuroimmunology and immunotherapy. Dr Cencioni was trained at Tor Vergata University of Rome and was awarded a PhD at Fondazione Santa Lucia in Rome. After completing her PhD, she pursued her interest in translational medicine and joining the laboratory of Professor Paolo Muraro at Imperial College London in 2014 with the support of FISM and Fondazione Umberto Veronesi. The research activity is related to understand mechanisms by which the immune system contributes to the autoimmune disease, Multiple Sclerosis and to define the mechanisms that regulate the tolerance to the antigens in immuno-modulatory treatment and haematopoietic stem cell transplantation (HSCT).

Markus Seidel (Austria)



Markus Seidel is a Professor of Translational Pediatric Hematology and Immunology at the Medical University of Graz, Austria. In October 2022 he was elected as chairperson of the ESID registry with 25.000 patients with inborn errors of immunity for his aims to facilitate clinical research in this important field of rare diseases. Furthermore, for the next five years, he heads the Styrian Children's Cancer Research Unit, an infrastructural project at the Medical University of Graz. He studied medicine and specialized in pediatric hematology-oncology with a main interest in stem cell transplantation at the Medical University / St. Anna Children's Hospital in Vienna, Austria. He worked as post-doc in Michael Freissmuth's lab at the Institute of Pharmacology in Vienna in the field of MAPK signaling and in Tom Look's lab in apoptosis signaling research at the Dana-Farber Cancer Institute (Peds Hem/Onc), Harvard Medical School, in Boston. His current own clinical focus and research interests are inborn errors of immunity with immune dysregulation and cancer predisposition, and he is leading a multicenter, prospective registry and biomarker study for severe immune cytopenias and heading the outpatient clinic for pediatric hematology-oncology with stem cell transplantation in Graz.

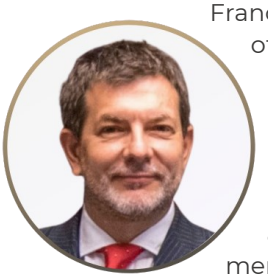
Raffaele Badolato (Italy)

Raffaele Badolato is a Pediatric immunologist with a special expertise in the field of Inborn Errors of Immunity. He is Chair of Pediatrics at ASST Spedali civili, Full Professor of Pediatrics at University of Brescia. He has received Fellowships from AIRC and Telethon and research grants from Telethon , Italian Health Ministry, EU 7th program and Italian, MIUR, and eRare. He is member of numerous Scientific Societies, including the European Society of Immunodeficiency, the Society of Leukocyte Biology, the Italian Society of Pediatrics, the Italian Society for Pediatric Research. He serves as Reviewer for Journal of Clinical Investigation, Blood, Journal of Immunology, Pediatric Infectious Diseases Journal, is Chief editor of Frontiers in Pediatric immunology and is member of the Journal of Leukocyte Biology and Pediatric Allergy and Immunology Editorial Boards.

Basil Sharrack (United Kingdom)

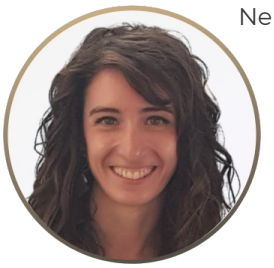
Professor Sharrack is a Consultant Neurologist at the Sheffield Teaching Hospitals NHS Foundation Trust and a Professor of Clinical Neurology at the University of Sheffield. Professor Sharrack was trained at the National Hospital for Neurology and Neurosurgery, UCL, London and was awarded a PhD from King's College, London. His principal academic areas of interest are neuro inflammation and degeneration with reference to multiple sclerosis and autologous haematopoietic stem cell transplantation (AHSCT) in immune mediated neurological disease. He has been awarded a number of prestigious awards including the 2019 National Future NHS Parliamentary and the 2020 Queen Anniversary Awards.

Francesco Onida (Italy)



Francesco Onida is Associate Professor of Hematology at the University of Milan in the Department of Oncology and Hemato-Oncology and Director of the Hematology Unit at the ASST Fatebenefratelli-Sacco, Milan, Italy. He received his medical degree in 1995 from the University of Milan and obtained the Italian Hematology Board Certification in 1999. From 1999 to 2002 he worked as a Postdoctoral Research Fellow, at the Leukemia Dept, The University of Texas MD Anderson Cancer Center, Houston, TX, USA. In 2006 he obtained the Italian Board Certification in Oncology and was hired as Assistant Professor of Hematology at the University of Milan, Department of Clinical Sciences and Community Health. Active member in the American Society of Hematology (ASH), American Society of Clinical Oncology (ASCO), European Hematology Association (EHA), Italian Society of Hematology (SIE), Italian Society of Experimental Hematology (SIES), professor Onida is also active member in the European Group for Blood and Marrow Transplantation (EBMT) Chronic Malignancies Working Party and Practice Harmonisation & Guidelines Committee, in the MDS/MPN International Working Group, in the Italian Foundation for the study of Myelodysplastic Syndromes (FISM), The Myelodysplastic Syndromes Foundation, inc. (MDS Foundation) and in the Italian Group for Bone Marrow and Hemopoietic Stem Cell Transplantation and for Cellular Therapy (GITMO). Since 2021, Professor Onida is also member of the EMA Scientific Advisory Group (SAG) Oncology and of the Oncology Special Interest Community (ESEC). Professor Onida has been an invited speaker at numerous national and international meetings, including the EBMT and ASCO annual meetings. He is author of numerous peer-reviewed papers published in peer reviewed international journals.

Alice Mariottini (Italy)



Neurologist, currently employed as clinical researcher in the Department of Neurosciences, University of Florence, Italy. Research activities mainly focused on AHSCT in aggressive multiple sclerosis, collaborating since 2013 with the Cellular Therapy and Transfusion Medicine Unit (Director Dr. Saccardi) of the Careggi University Hospital in Florence, Italy. Honorary association with Imperial College London as Visiting Researcher in the Department of Brain Sciences, Clinical Neurology.

Alessandro Aiuti (Italy)

IRCCS San Raffaele Hospital/Vita-Salute San Raffaele University.

Fabian Müller (Germany)

Hematologist and oncologiste, Head of the CAR T cell program (experimental & commercial); Clinical Focus on B cell malignancies. Principal Investigator of a Max Eder Junior Research Group on targeted immunotherapeutics; Scientific Focus on antibody-based therapeutics, CAR T cells, and effects of disease and treatment and the immune system.

Nicoletta Del Papa (Italy)

Dip. Reumatologia, Università degli Studi di Milano, ASST G. Pini-CTO

Selim Corbacioglu (Germany)

Universitätsklinikum Regensburg. Paediatric Diseases Working Party chair.

Richard Burt (United States)

Dr. Richard K. Burt (https://astemcelljourney.com/about/drrichardburt/) is a Fulbright Scholar, Professor of Medicine at Scripps Health Care, tenured retired Professor of Medicine at Northwestern University, and CEO of Genani biotechnology. He endeavored for thirty-five years, first with animal models then with some of the world's first clinical trials, to bring the field of stem cell and cellular therapy to the patient's bedside. Dr. Burt has published more than 145 mostly first author articles and is the Editor of four medical textbooks. He was the first Autoimmune Committee Chairperson for the International Bone Marrow Transplant Registry (IBMTR) and was the principal investigator of a National Institute of Health (NIH) $10,000,000 multi-center contract to develop stem cell clinical trials for autoimmune diseases. Professor Burt performed America's first hematopoietic stem cell transplant (HSCT) for multiple sclerosis (MS), systemic lupus erythematosus (SLE), Crohn's disease (CD), stiff person syndrome (SPS), and chronic inflammatory demyelinating polyneuropathy (CIDP) and published the world's first randomized clinical stem cell transplantation trials for systemic sclerosis and multiple sclerosis. He has been awarded Leukemia Scholar of America, the Lupus Foundation of America Fidelitas Award, the Van Bekkum Award by the European Society for Blood and Marrow Transplantation, the Distinguished Clinical Achievement Award by the Clinical Research Forum, and the European Group for Blood and Marrow Transplantation Clinical Achievement Award. Dr. Burt was presented in Vatican City, Rome with the "Keys to the Vatican", was speaker at the Festival of Thinkers in Leadership in Healthcare in the United Arab Emirates, and chaired the biotechnology session at the Baku Azerbaijan International Humanitarian Forum. Dr. Burt was recognized by Science Illustrated for accomplishing one of the Top 10 medical breakthroughs for the next ten years, and by Scientific American as one of the Top 50 individuals, teams, or organizations for improving humanity and outstanding leadership.

John Snowden (United Kingdom)

Professor Snowden is Consultant Haematologist and Director of the Sheffield BMT & Cellular Therapy Programme in the UK. He trained in the UK, Australia and New Zealand. Nationally, he has delivered on key leadership roles in BMT and haemato-oncology at a UK public health level, including Clinical Lead for various NICE Guidelines, Clinical Lead of the NHS England Clinical Reference Group for BMT, Chair of the Intercollegiate Committee on Haematology to the Royal College of Physicians and Royal College of Pathologists and President of the British Society of Blood and Marrow Transplantation and Cellular Therapy (2021-22). Internationally, he has a long association with the EBMT and 'JACIE', including JACIE Medical Director (2012-16) and Chair of the JACIE Committee (2015-20). He has been a member of the EBMT Board and Scientific Council as Autoimmune Diseases Working Party Chair (2016-20) and now as EBMT Secretary (2020-24). Academically, he has contributed to the fields of BMT, haematology-oncology and autoimmunity with authorship of over 300 publications (H-index >61), along with clinical trials, grant awards, educational meetings, teaching, supervision, examination, journal editorship and scientific peer review. He has been awarded honorary professorships by The University of Sheffield and University College London.

Despina Moshous (France)

I am a paediatrician working in the Unit for Paediatric Immunologie, Haematology and Rheumatology in Necker-Enfants Malades Hospital in Paris, France, affiliated to the National Reference for inherited immunodeficiencies CEREDIH, which I coordinate. My research activity is in the Laboratory “Genome Dynamics in the Immune System” at Institut Imagine. As a professor of paediatrics I am affiliated to Université Paris Cité. My main interests in clinical research are primary HLH and combined immunodeficiencies linked to DNA repair defects.

Azucena Salas (Spain)

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