



MEDICAL PROFESSIONALS, PATIENTS & FAMILIES:

Once a year, the ECD medical community, patients and their families come together to share information and stories with one another. We are optimistic that this event will enlighten and encourage all that attend. Thank you to all presenters and participants for your dedication, time, and hope!

ASK THE EXPERTS

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ERDHEIM-CHESTER DISEASE GLOBAL ALLIANCE

ECD Global Alliance
P.O. Box 775
DeRidder, LA 70634 USA
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ECD Patient & Family Gathering Attendees

September, 15, 2016
Paris, France

Dear Attendees,

Welcome to Paris, France and the 4th Annual International ECD Family and Patient Gathering!
We are happy to be in Europe and are looking forward to meeting each of you!

Thank you for your attendance and involvement in this year's event. We truly believe with one another's help we will move forward in increasing ECD studies and research, approving treatment options and aiding in the quality of life for each patient. Our ultimate goal is to find a cure for ECD. This year's presentations and events are based heavily on feedback provided by our members from past events. For this reason, we are optimistic that you will enjoy the ECD community fellowship and find the diverse presentations helpful.

We are hopeful that the gathering is informative and promising to you and your family. There will be opportunities to hear from experts about treatment and research information, to interact with other ECD patients and family members, and to learn about ways to help the ECD organization continue on its mission.

The ECD community is extremely grateful for your involvement and sharing of experiences with others. Please let us know how the organization can best serve you and if you are interested in getting more involved in the work of the organization. The contributions of you and others have proven to be instrumental in the success of this organization!

Please enjoy your visit!

Sincerely,

THE ECDGA STAFF & BOARD OF DIRECTORS

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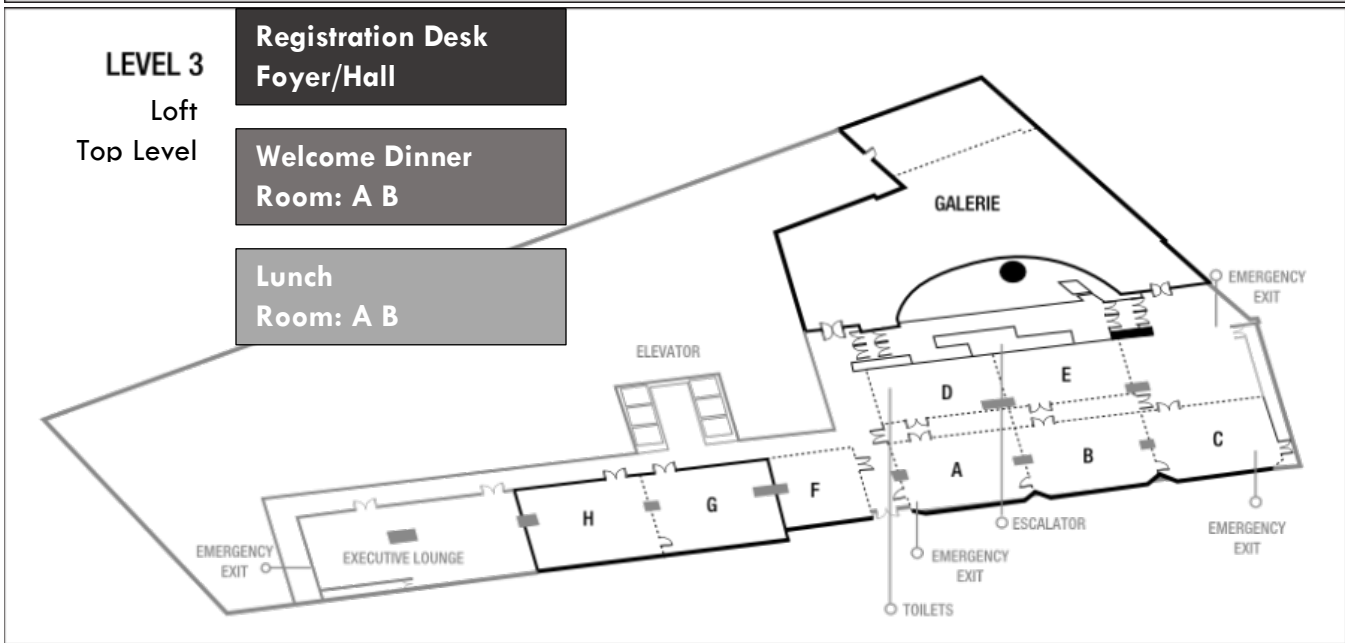
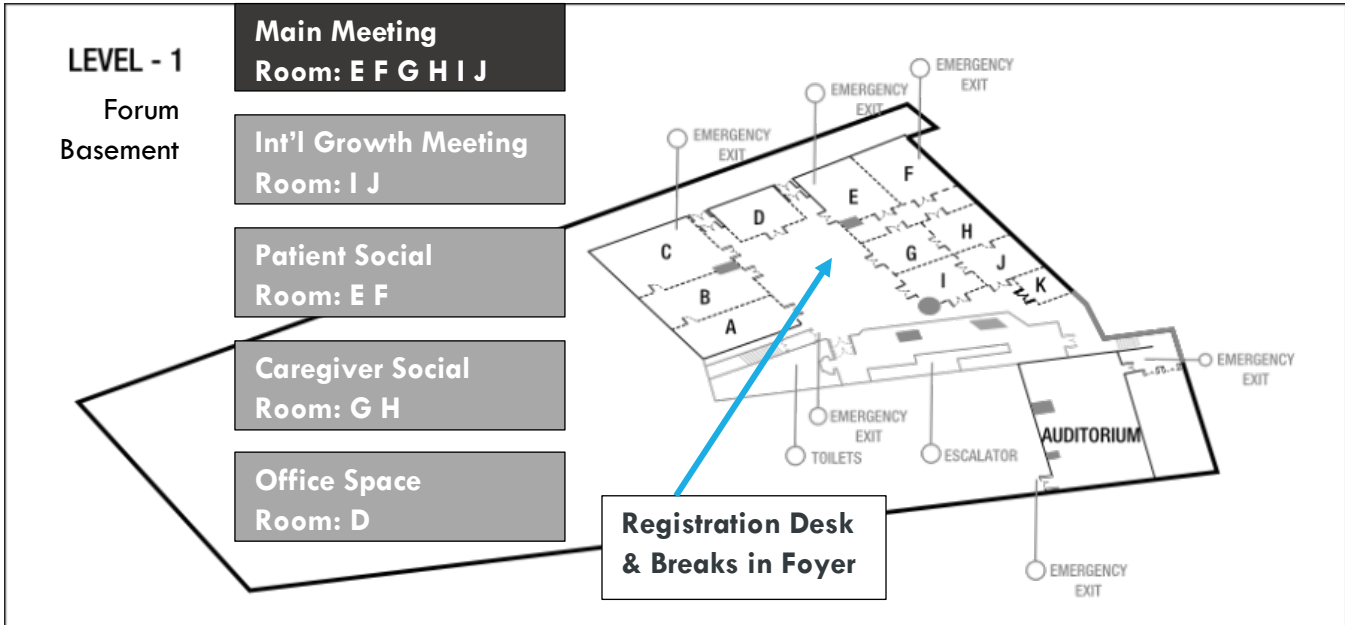
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ECD Patient & Family Gathering

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Hotel Floor Maps

*Wi-Fi is available to the hotel guest by choosing the network "Marriott Guest" and entering password "ECDGA2016".



Schedule of Events | September 15 - 16, 2016

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DAY	TIME	EVENT	LOCATION
September 15	5:30 – 7:00 pm	Registration / Help	Level 3 Foyer
	7:00 – 8:30 pm	Welcome Dinner	Level 3 Room AB
September 16	8:00 am– 7:00 pm	Registration / Help	Level 1 Foyer
	9:00 am – 5:00 pm	Patient & Family Gathering Presentations	Level 1 Room EFGHIJ
	12:40 – 12:55 pm	Group Photo	Location to be announced
	12:55 – 2:10 pm	Lunch	Level 3 Room AB
	6:00 – 7:00 pm	Caregiver Social	Level 1 Room GH
	6:00 – 7:00 pm	Patient Social	Level 1 Room EF

Presentation Agenda | September 16, 2016, 9:00 am to 5:00 pm

TIME	PRESENTATION TOPIC	PRESENTER
9:00 - 9:10	Welcome	Prof. Julien Haroche, MD, PhD
9:10 - 9:30	ECD101 + Mutations & Findings Overview	Juvianee Estrada-Veras, MD
9:30 - 10:10	ECD Studies & Trials Panel + Findings, Updates & Needs	Achille Aouba, MD, PhD Lorenzo Dagna, MD Eli L. Diamond, MD Juvianee Estrada-Veras, MD Julien Haroche, MD, PhD Filip Janku, MD, PhD Kenneth McClain, MD, PhD Augusto Vaglio, MD, PhD
10:10 - 11:00	Doctor Q & A	Same as above
11:00 - 11:30	BREAK	
11:30 - 11:40	Clinical & Radiological Signs of ECD in ENT	Mylène Drouillard, MD
11:40 - 12:00	ECD - Care & Pain Management	Prof. Lorenzo Dagna, MD
12:00 - 12:20	ECD - Neurological Issues	Eli L. Diamond, MD
12:20 - 12:40	Renal & Ureteral Involvement in ECD	Augusto Vaglio, MD, PhD
12:40 - 12:55	GROUP PHOTO	
12:55 - 2:10	LUNCH	
2:10 - 2:40	ECD Scan & Radiology Panel + Q & A	Marcus Chen, MD Philippe Cluzel, MD Philippe Grenier, MD Philippe Maksud, MD
2:40 - 3:00	ECD - Lung & Heart Issues	Giulio Cavalli, MD
3:00 - 3:15	Endocrinological consequences of ECD	Carine Courtillot, MD
3:15 - 3:30	ECD - Skin Issues	Stephane Barete, MD
3:30 - 4:00	BREAK	
4:00 - 4:30	Doctor Q & A	All remaining speakers
4:30 - 5:00	ECDGA Close & Evaluations	ECDGA

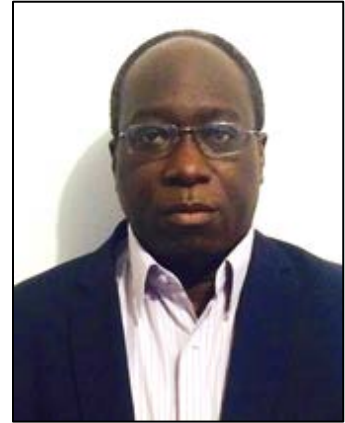
Presenter Biographies

Achille Aouba, MD, PhD

Department Of Hematology, Hôpital Necker-Enfants Malades, Assistance Publique-Hôpitaux De Paris, Université De Paris 5 René-Descarte
Presentation: ECD Trials & Studies

Born in 1964 in Paris, Dr. Achille Eric Aouba is a French Physician who studied Medicine and Medical Ethics in West Africa & Paris.

After entering the French Army, he worked for the Public Assistance of Paris Hospitals (A.P.H.P) during 20 years as: (1) Head of a Hematology Unit in Necker Hospital- Paris, (2) Voluntary Physician for the “Prison de la Santé”- Paris, (3) Head of Internal Medicine and Clinical Immunology Unit in Cochin Hospital- Paris, and (4) Head of Internal Medicine and Clinical Immunology Unit in Antoine-Béclère Hospital- Paris.



In 2014, he decided to move to Normandy to join the University Hospital of Caen-Normandy as M.D.-Ph.D., Associate Professor and Specialist of auto-inflammatory & auto-immunity diseases, Langerhans & Non-Langerhans Histiocytosis (Erdheim-Chester Disease), and blood coagulation disorders.

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Stéphane Barete, MD, PhD

Dermatology, Sorbonne Universités, UPMC Univ Paris VI, Assistance Publique-Hôpitaux De Paris, Département De Dermatologie Et Allergologie, Hôpital Tenon, Centre De Référence Des Mastocytoses, Paris, France
Presentation: ECD & Skin Matters

Contact Information

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Giulio Cavalli, MD

Internal Medicine, Clinical Immunology, Vita-Salute San Raffaele University, San Raffaele Scientific Institute
Presentation: ECD & Lung and Heart Involvement

Contact Information

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Marcus Y. Chen, MD

Director, Cardiovascular CT, Advanced Cardiovascular Imaging, National Heart, Lung and Blood Institute, National Institutes of Health
Presentation: Cardiovascular Involvement of ECD (Scans Panel)

Dr. Marcus Chen is a Clinical Investigator at the National Institutes of Health located in Bethesda, Maryland, USA. Dr. Chen is a graduate of the California Institute of Technology. He earned his MD degree at the University of Wisconsin at Madison and completed training in internal medicine, nuclear medicine, cardiology, cardiovascular CT and MRI. He is the director of Cardiovascular CT at the National Heart, Lung and Blood Institute. Dr. Chen's clinical and research interests include radiation reduction, technical development of new imaging methods and multimodality non-invasive cardiac imaging. He has co-authored over 60 peer-reviewed papers and serves as an associate editor for the Journal of Cardiovascular CT.



Dr. Chen supports the imaging for all the ECD patients that Dr. Juvianee Estrada-Veras evaluates at NIH.

Contact Information

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Philippe Cluzel, MD, PhD

Cardiovascular Radiology, Hôpital Pitié-Salpêtrière, Paris, France

Presentation: ECD Scans Panel

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Carine Courtillot, MD

Endocrinology, Metabolism and Reproductive Medicine, Hôpital Pitié-Salpêtrière, Paris, France

Presentation: Endocrinological consequences of ECD

Carine Courtillot, MD personally sees, for the endocrine evaluation and the endocrine follow-up, every patient with ECD admitted in the department of Internal Medicine at IE3M, Pitié-Salpêtrière. To date she has seen around 110 patients with ECD, having conducted a study to evaluate the endocrine consequences in ECD patients. In January 2016, Dr. Courtillot published her experience on 64 patients in the Journal of Clinical Endocrinology and Metabolism.

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Prof. Lorenzo Dagna, MD, FACP

Associate Professor of Medicine, Vita-Salute San Raffaele University, IRCCS San Raffaele Scientific Institute, Milano, Italy

Presentations: ECD Trials & Studies, ECD Care & Pain Management

Lorenzo Dagna graduated in Medicine in 1999 and is Board Certified in Internal Medicine. He is currently Associate Professor of Medicine and Vice-Coordinator of the San Raffaele International MD Program at Vita-Salute San Raffaele University, in Milano, Italy. Prof. Dagna is also Coordinator for Clinical Immunology and Immunopathology at the Department of Internal Medicine at San Raffaele Scientific Institute, Milano.



Besides being a general internist, the clinical activity of Prof. Dagna is focused on the care of patients with immunological and rheumatic diseases, in particular those with rare immune-mediated disorders. He is also involved in both basic and clinical research on the immunopathogenesis and immunotherapy of those diseases. Lorenzo Dagna is a Fellow of the American College of Physician (ACP), Honorary Fellow of the European Federation of Internal Medicine (EFIM), and member of the Italian Society for Internal Medicine (SIMI).

Dr. Dagna serves as the Editor for Immunology and Rheumatology for the European Journal of Internal Medicine. Prof. Dagna is Chair of the Rare Disease Working Group of the European Federation of Internal Medicine, member of the Commission Expert Group on Rare Diseases, European Commission (Luxembourg, L), member of the Healthcare Professionals Working Party of the European Medicine Agency (EMA, London, UK), member of the Medical Advisory Board of the Erdheim-Chester Global Alliance (DeRidder, LA), member of the International Foundation of Medicine Oversight Committee of the National Board of Medical Examiners (NBME, Philadelphia, PA).

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Eli L. Diamond, MD

Neuro-Oncology, Neurology, Memorial Sloan Kettering Cancer Center, New York, NY, USA

Presentations: ECD Trials & Studies and ECD & Neurological Issues

Eli Diamond, MD, is a neuro-oncologist who specializes in the care of patients with brain tumors and ECD, along with the neurologic complications of cancer. He works together with neurosurgeons and radiation oncologists to provide the highest quality medical treatment and supportive care for patients with gliomas and glioblastomas, primary central nervous system lymphomas (brain and spinal cord), ependymomas, and other types of brain tumors. Together with his colleagues in the Memorial Sloan Kettering Counseling Center, he performs clinical



research about quality of life, symptom management, and patient-physician communication for patients with brain tumors.

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Mylène Drouillard, MD

ENT, Hôpital Pitié-Salpêtrière, Paris, France

Presentation: Clinical and radiological signs of Erdheim Chester Disease in ENT

Dr. Drouillard is currently investigating ENT symptoms and signs in Erdheim-Chester patients. A questionnaire will be given at the end of the meeting to every patient. Thanks to the collected data, she and her team will be able to classify ENT disorders present in ECD. The final goal is to improve early diagnosis of ECD.



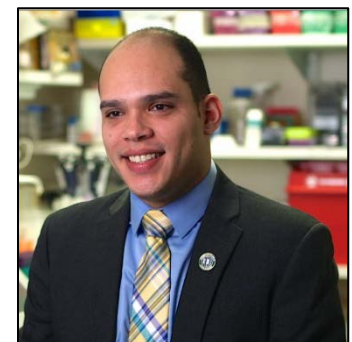
For the questionnaire, e-mail ECDquestionnaire@gmail.com.

Juvianee Estrada-Veras, MD

Genetics, National Human Genome Research Institute, National Institutes of Health, Bethesda, Maryland, USA

Presentations: ECD101 and ECD Trials & Studies

Juvianee Estrada-Veras, MD, is a Clinical Investigator and Staff Clinician at National Human Genome Research Institute (NHGRI) at the National Institutes of Health (NIH) in Bethesda, Maryland, USA.



Dr. Estrada-Veras received his MD magna cum laude from the Instituto Tecnológico de Santo Domingo (INTEC), Dominican Republic in 2003. He completed his residency at the State University of New York Health Science Center at Brooklyn and Downstate Medical Center with the Department of Pediatrics in 2009, and a fellowship at the National Institutes of Health/National Human Genome Research Institute, Bethesda, Maryland in Clinical Genetics and Medical Biochemical Genetics in 2011.

He is the Principal Investigator of the “Erdheim Chester Disease Natural History Protocol” and the “Dabrafenib and Trametinib in People with BRAF V600E Mutation Positive Lesions in Erdheim Chester Erdheim-Chester Disease Global Alliance

Disease” studies at the NIH, and an Associate Investigator with Dr. William Gahl and colleagues in the study for diagnosis and treatment of inborn errors of metabolism at the NHGRI.

Dr. Estrada-Veras is a member of the ECD Global Alliance Board of Directors as the US Federal Liaison for the ECD Global Alliance.

Contact information

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Philippe Grenier, MD

Service de Radiologie Polyvalente et Oncologique, Hôpital Pitié-Salpêtrière, Paris, France

Presentation: Thoraco-Abdominal Imaging in ECD (Scans Panel)

Philippe A. Grenier is Professor of Radiology at Pierre & Marie Curie University, and Chief of Section of Diagnostic Radiology at Pitié-Salpêtrière Hospital, Paris, France. He is also the Chairman of the Medical Board of Pitié-Salpêtrière Hospital.

A respected expert in chest imaging and respiratory disease, Dr. Grenier is author or coauthor of 240 peer-reviewed articles, and more than 65 books or book chapters. He has given more than 160 invited lectures at international scientific meetings or universities. He has obtained 12 grants for research and received several awards for scientific exhibitions from international societies.

Dr. Grenier was awarded honorary membership from radiological societies of North America, Japan, Austria, Belgium, Italy, Argentina, and Sweden. He is also honorary fellow of the Royal College of Radiologists and honorary Professor of Universidad de Buenos Aires. He was President of the European Congress of Radiology (ECR), President of the European Society of Thoracic Imaging and President of the Fleischner Society (a multidisciplinary international group dedicated to chest disease). Dr. Grenier is awarded the gold medal of the European Association of Radiology.

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Prof. Julien Haroche, MD, PhD

*Medical Advisory Board Member, Service de Médecine Interne,
Hôpital Pitié-Salpêtrière, Paris, France*

Presentations: Welcome and ECD Trials & Studies

Julien Haroche is a professor in internal medicine, at Pitié-Salpêtrière, Paris, France. Since 2003, his main research field is Erdheim-Chester disease upon which he has acquired a world-renowned expertise. To date, he has seen more than 150 patients at his institution. His other research fields are the other histiocytoses, such as Langerhans cell histiocytosis, mixed histiocytosis and Rosai-Dorfman disease. He is also interested in vasculitis, lupus and antiphospholipid syndrome. Dr. Haroche serves on the ECD Global Alliance Medical Advisory Board.



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Prof. Filip Janku, MD, PhD

*Assistant Professor, Oncology, the University of Texas MD Anderson
Cancer Center, Houston, TX, USA*

Presentation: ECD Trials & Studies

Filip Janku, MD, PhD is Assistant Professor, Department of Investigational Cancer Therapeutics, Division of Cancer Medicine at The University of Texas MD Anderson Cancer Center, Houston, TX. Dr. Janku received his MD and PhD degrees from the Charles University, Prague, Czech Republic. In addition to his expertise in Erdheim-Chester disease, Dr. Janku is principal investigator for numerous phase I trials, most of which involve novel, targeted agents, some of those being first-in-human studies. He also runs a laboratory program focused on genomic analysis of liquid biopsies. Dr. Janku received multiple awards for his research efforts, including four Merit Awards from ASCO as well as an American Association for Cancer Research Scholar-in-Training Award and highly prestigious Kimmel Award.



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Philippe Maksud, MD

Radiology, Hôpital Pitié-Salpêtrière, Paris, France

Presentation: ECD Scans Panel

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Kenneth L. McClain, MD, PHD

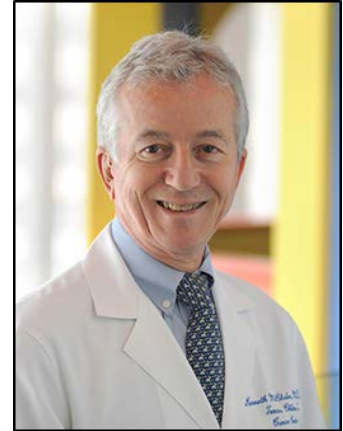
Director, Histiocytosis Program, Texas Children's Cancer Center;

Professor, Department of Pediatrics, Section Of Hematology-Oncology,

Baylor College Of Medicine, Houston, TX

Presentations: ECD Studies & Trials

Dr. Kenneth McClain is Director of the Histiocytosis Program at Texas Children's Cancer and Hematology Centers. He is a member of the leukemia and lymphoma teams of the Texas Children's Cancer Center. His research is focused on the clinical and biologic aspects of Langerhans cell histiocytosis (LCH). He is a key member of the clinical trials group of the Histiocyte Society.



Dr. McClain and Dr. Carl Allen see 100 new patients with a variety of histiocytic diseases each year. A number of clinical studies are in progress or development including new therapeutic interventions for CNS LCH. Current biologic studies in his lab are focused on determining the gene expression profiles Langerhans cells and lymphocytes from control and patient tissues and making clinical correlations.

Dr. Kenneth McClain is a member of the Lymphoma Program and Director of the Histiocytosis Program.

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Augusto Vaglio, MD, PhD

*Nephrology Specialist, Consultant Nephrologist, Nephrology Unit,
Parma University Hospital, Parma, Italy*

*Presentations: ECD Trials & Studies and Renal & ureteral
involvement in ECD*

Augusto Vaglio, MD, PhD, is a Nephrology Consultant with the Dept. of Clinical Medicine and Nephrology at the University Hospital of Parma, Italy. Dr. Vaglio received his MD magna cum laude from the School of Medicine at Parma University, Italy in 2000. His fellowship in nephrology was completed in 2005 at the University of Parma, magna cum laude.



Dr. Vaglio's clinical activity has focused mainly on the clinical management of patients with immune-mediated diseases, particularly retroperitoneal fibrosis and systemic vasculitis, and renal transplant patients. His main fields of interest are: retroperitoneal fibrosis and related diseases, systemic small vessel vasculitis, immunotherapy for renal cell cancer and pathogenesis of autoimmune diseases. He is the coordinator of a pilot trial of prednisone and sirolimus in patients with Erdheim-Chester Disease and is actively involved in clinical and pathogenetic studies on this topic. He is also the proponent of a large multicentre survey aimed to explore the presence of different clinical subsets of Erdheim-Chester.

Dr. Vaglio has authored 76 articles published in international peer-reviewed journals, with a cumulative Impact Factor of 550 and an h-index of 20.

Dr. Vaglio is a member of the ECD Global Alliance Medical Advisory Board.

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Dr. Augusto Vaglio,

Unità Operativa di Nefrologia,

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Thank you to all of our donors and generous benefactors who have contributed to making this event, and the work of the ECD Global Alliance, possible.



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