

The Second International Conference on Deficiency of ADA2

Agenda

FRIDAY, NOVEMBER 9, 2018

7:00 to 7:50 Registration and Continental Breakfast

8:00 to 8:20 Welcome/Introductions/Agenda Overview

Chip Chambers, M.D. - Founder & President DADA2 Foundation Dan Kastner, M.D., Ph.D. - Scientific Director NHGRI, National Institutes of Health

SESSION 1: NEW CLINICAL MANIFESTATIONS OF DADA2

8:20 to 8:35	How Much Agreement is There on the Diagnosis & Treatment of DADA2 – A World Wide Survey <i>Taryn Youngstein, M.D. – Imperial College Healthcare NHS Trust, London, England</i>	
8:35 to 8:55	Deficiency of ADA2 a Perspective from ADA1 Deficiency <i>Mike Hershfield, M.D. – Duke University School of Medicine</i>	
8:55 to 9:10	Deficiency of ADA2 a Year-in-Review Ivona Aksentijevich, M.D. – NHGRI, NIH, Co-Chair 2 nd International DADA2 Conference	
9:10 to 9:25	What is Unique about DADA2 Patients of European Descent? Marco Gattorno, M.D Giannina Gaslini Institute, Genoa, Italy	
9:25 to 9:35	What is Unique about DADA2 Patients of Middle Eastern and Eastern Mediterranean Descent? Seza Ozen, M.D. – Hacettepe University of Medicine, Ankara, Turkey	
9:35 to 9:50	Diamond-Blackfan Anemia Phenotype Caused by Deficiency of Adenosine Deaminase 2 Marcin Wlodarski, M.D., Ph.D. – St. Jude Children's Research Hospital	
9:50 to 10:00	ADA2 Deficiency: Clonal Lymphoproliferation in a Subset of Patients Janna Saarela, M.D., Ph.D Institute for Molecular Medicine, Helsinki, Finland	
10:00 to 10:20	COFFEE BREAK and NETWORKING	
10:20 to 10:30	Is Deficiency of Adenosine Deaminase 2 Associated with Lymphoma? Fahad Alabbas, M.D., Prince Sultan Military Medical City, Riyadh, Saudi Arabia	

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10:30 to 10:40	ADA2 Deficiency Mimicking Idiopathic Multicentric Castleman's Disease Erika Van Nieuwenhove, M.D University Hospitals Gasthuisberg, Leuven, Belgium
10:40 to 10:50	DADA2 Presenting as Bone Marrow Failure in Adulthood & Response to TNF-Inhibition Thomas Michniacki, M.D. – University of Michigan Medical School
10:50 to 11:00	Early Diagnosis of an Infant with DADA2 – Japanese Cohort Kazushi Izawa, M.D., Ph.D. – Kyoto University Hospital, Kyoto, Japan
11:00 to 11:15	Can Carriers Express the DADA2 Phenotype & Require Treatment? <i>Pui Lee, M.D., Ph.D., - Harvard Medical School</i>
11:15 to 12:15	Q & A WITH ALL PRESENTERS ON STAGE
12:15 to 12:45	PICK UP LUNCH

SESSION 2: NEW INSIGHTS INTO THE PATHOPHYSIOLOGY OF DADA2		
12:45 to 12:55	12:55 Variable Clinical Phenotypes and Relation of Interferon Signature with Disease Activity in DADA2 Antonella Insalaco, M.D I.R.C.C.S. Ospedale Pediatrico Bambino Gesu, Rome, Italy	
12:55 to 1:10	Disrupted N-Linked Glycosylation as a Disease Mechanism in Deficiency of ADA2 <i>Pui Lee, M.D., Ph.D Harvard Medical School</i>	
1:10 to 1:25	Vascular Endothelium & the ADA2 Molecule Rekha Dhanwani, Ph.D La Jolla Institute of Allergy and Immunology	
1:25 to 1:45	ADA2 is a Lysosomal Nuclease Involved in Nucleic Acid Sensing of Myeloid Cells Ole Kristian Greiner-Tollersrud, Ph.D Institute of Medical Biology University of Tromsø, Norway Michele Proeitti, Ph.D. – Center for Chronic Immunodeficiency, Freiburg, Germany	
1:45 to 2:00	Deficiency in Human Adenosine Deaminase 2 Alters Cellular Responses to Extracellular Adenosine Andrey Zavialov, Ph.D. – Guangzhou Institute of Pediatrics, Guangzhou (Canton), China	
2:00 to 2:15	ADA2 Deficiency Triggers Adenosine - Mediated NET Formation and TNF Production in Patients with DADA2 <i>Peter Grayson, M.D. – NIAMS, NIH</i>	
2:15 to 2:30	Vascular Inflammation in DADA2 Patients: Pre and Post TNF-Inhibition Dan Yang, M.D., Ph.D. – NHLBI, NIH	
2:30 to 2:45	Deficiency of Adenosine Deaminase 2 in Patients Diagnosed with PAN Oskar Schnappauf, Ph.D. – NHGRI, NIH	
2:45 to 3:00	Expanded Spectrum of Peripheral Blood Findings and Bone Marrow Pathology in DADA2 Kathy Calvo, M.D., Ph.D., - Department of Laboratory Medicine - Hematopathology, NIH	
3:00 to 3:50	Q & A WITH ALL PRESENTERS ON STAGE	
3:50 to 4:10	COFFEE BREAK and NETWORKING	

SESSION 3: TREATMENT OF DADA2		
4:10 to 4:25	A Decision Tree for the Genetic Diagnosis of DADA2: A French Reference Centers Experience Sophie Georgin-Lavialle, M.D., Ph.D Pierre and Marie Curie University, Paris, France	
4:25 to 4:40	Pre-Clinical Study on Gene Therapy for DADA2 – Great Ormond Street Hospital's Experience Despina Eleftheriou, MBBS, MRCPCH, Ph.D UCL Great Ormond St Institute of Child Health, London, England	
4:40 to 4:55	Pre-Clinical Study on Gene Therapy for DADA2 – San Raffaele Hospital's Experience Alessandra Mortellaro, Ph.D San Raffaele Telethon Institute for Gene Therapy, Milan, Italy	
4:55 to 5:15	Analysis on the Efficacy of Anti-TNF in the Treatment of Patients with DADA2 - The NIH Experience Amanda Ombrello, M.D. – NHGRI, NIH	
5:15 to 5:30	Bone Marrow Transplant for DADA2 – Where are we now? Isabelle Meyts, M.D., Ph.D University Hospitals Gasthuisberg, Leuven, Belgium	
5:30 to 6:00	Q & A WITH ALL PRESENTERS ON STAGE	

SESSION 4: JOINT PHYSICIANS/SCIENTISTS & PATIENTS/FAMILIES		
6:00 to 6:30	SUMMARY OF THE DAY Dan Kastner, M.D., Ph.D Scientific Director NHGRI, National Institutes of Health	
6:30 to 7:30	JOINT RECEPTION FOR PHYSICIANS/SCIENTISTS & PATIENTS/FAMILIES	

SATRUDAY	, NOVEMBER	10, 2018
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7:30 to 8:00	Continental Breakfast	White Oak Foyer
8:00 to 8:30	Introduction to day # 2	White Oak A
8:30 to 12:00	Scientific Advisory Board	Forest Glen
8:30 to 12:00	Diagnosis & Screening DADA2 Collaborative Group	Oakley Room
8:30 to 12:00	Gene Therapy DADA2 Collaborative Group	Middlebrook Room
8:30 to 12:00	Hematopoietic Stem Cell Transplant	Timberlawn Room
8:30 to 12:00	Neurological Phenotype	Strathmore Hall Room
8:30 to 12:00	Pathophysiology	Linden Oak Room
8:30 to 12:00	Patient Reported Outcomes	Glen Echo Room
12:00	CONCLUSION OF THE DADA2 CONFERENCE	