

# The Inaugural International Conference on Deficiency of ADA2

## Agenda

**FRIDAY, NOVEMBER 11, 2016**

<b>7:00 am to 7:50 am</b>	Registration and Continental Breakfast
<b>8:00 am to 8:15 am</b>	Welcome/Introductions/Agenda Overview <b>Chip Chambers, M.D. - Founder &amp; President DADA2 Foundation</b> <b>Dan Kastner, M.D., Ph.D. - Scientific Director NHGRI, National Institutes of Health</b>

### SESSION 1: CLINICAL PRESENTATIONS/PHENOTYPES

<b>8:15 am to 8:40 am</b>	NIH Cohort Experience (Stroke/Liver Phenotype) <b>Amanda Ombrello, M.D. - NIH, NHGRI</b>
<b>8:40 am to 9:05 am</b>	Israel Cohort Experience (PAN Phenotype) <b>Yackov Berkun, M.D. - Hadassah Hebrew University Medical Center, Jerusalem, Israel</b>
<b>9:05 am to 9:25 am</b>	Italy Cohort Experience (B cell Phenotype) <b>Marco Gattorno, M.D. - Giannina Gaslini Institute, Genoa, Italy</b>
<b>9:25 am to 9:45 am</b>	Pure Red Cell Aplasia (Bone Marrow Failure Phenotype) <b>Alison Bertuch, M.D., Ph.D. - Texas Children's Hospital/Baylor College of Medicine</b>
<b>9:45 am to 10:00 am</b>	COFFEE BREAK and NETWORKING
<b>10:00 am to 10:20 am</b>	Finnish Cohort (Malignancy Phenotype) <b>Janna Saarela, M.D., Ph.D. - Institute for Molecular Medicine, Helsinki, Finland</b>
<b>10:20 am to 10:50 am</b>	Oral Abstract Presentations
<b>10:20 am to 10:30 am</b>	B Cells in DADA2 a Monocyte-Derived Antibody Deficiency <b>Michele Proeitti, Ph.D. - Center for Chronic Immunodeficiency, Freiburg, Germany</b>
<b>10:30 am to 10:40 am</b>	Spectrum of Peripheral Blood Findings and Bone Marrow Pathology in DADA2 <b>Jaya Balakrishna, M.D. - Department of Laboratory Medicine – Hematopathology, NIH</b>
<b>10:40 am to 10:50 am</b>	Screening of 180 CVID Patients for DADA2 Sheds New Light on the Disease in Adulthood <b>Johanna Schepp, M.D. - Center for Chronic Immunodeficiency, Freiburg, Germany</b>

## SESSION 2: DIAGNOSTIC / SCREENING APPROACH

10:50 am to 11:10 am	Screening for ADA2 Protein and Activity <b>Mike Hershfield, M.D. – Duke University School of Medicine</b>
11:10 am to 11:30 am	Range of ADA2 in Normal Individuals/English Cohort Experience <b>Paul Brogan, MB.ChB., Ph.D. - Great Ormond Street Hospital, London, England</b>
11:30 am to 11:50 am	Genetic Screening for DADA2 <b>Ivona Aksentijevich, M.D. - NIH/NHGRI</b>
11:50 am to 12:10 pm	PICK UP LUNCH/STRETCH BREAK
12:10 pm to 1:10 pm	Panel/Open Mic Discussion/Questions (1 hour)

## SESSION 3: MOLECULAR MECHANISMS

1:10 pm to 1:35 pm	What Does ADA2 do? Is Adenosine Deaminase Function Important? <b>Andrey Zavialov, Ph.D. - Turku Centre for Biotechnology, Turku, Finland</b>
1:35 pm to 1:55 pm	Do Type I Interferons Play a Role in DADA2? <b>Alexandre Belot, M.D., Ph.D. - Hospices Civils de Lyon, Lyon, France</b>
1:55 pm to 2:15 pm	Vascular Endothelium & the ADA2 Molecule <b>Sonia Sharma, Ph.D. - La Jolla Institute of Allergy and Immunology</b>
2:15 pm to 2:45 pm	Oral Abstract Presentations
2:15 pm to 2:25 pm	A New Validation Method Proves that ADA2 is a Lysosomal Protein <b>Ole Kristian Greiner-Tollersrud, Ph.D. - Institute of Medical Biology University of Tromsø, Norway</b>
2:25 pm to 2:35 pm	ADA2 Deficiency Attenuates M2 Macrophages Differentiation <b>Dan Yang, M.D., Ph.D. - NHLBI, NIH</b>
2:35 pm to 2:45 pm	Role of Adenosine and Neutrophils in Deficiency of Adenosine Deaminase 2 <b>Carmelo Carmona-Rivera, Ph.D. - NIAMS, NIH</b>
2:45 pm to 3:00 pm	COFFEE BREAK and NETWORKING

## SESSION 4: TREATMENT

3:00 pm to 3:20 pm	Supportive Care / Recommended Screening Labs / Studies <b>Debbie Stone, M.D. - NIH/NHGRI</b>
3:20 pm to 3:40 pm	Bone Marrow Transplant <b>Isabelle Meyts, M.D., Ph.D. - University Hospitals Gasthuisberg, Leuven, Belgium</b>
3:40 pm to 4:00 pm	The Experience of Gene Therapy for ADA1 and it's Potential for DADA2 <b>Maria Pia Cicalese, M.D., Ph.D. - San Raffaele Institute for Gene Therapy, Milan, Italy</b>
4:00 pm to 4:20 pm	Potential for PEG-ADA2 Therapy <b>Chris Thanos, Ph.D. - Halozyme Inc., San Diego, California</b>
4:20 pm to 4:40 pm	Oral Abstract Presentations
4:20 pm to 4:30 pm	ADA2 Deficiency Presenting as Bone Marrow Failure in Adulthood <b>Mark Hannibal, M.D., Ph.D. – University of Michigan Medical School</b>

4:30 pm to 4:40 pm	Successful Reduced Intensity HSCT in A Patient with Refractory Pure Red Cell Aplasia Due to DADA2 <b><i>Hasan Hashem, M.D. - Nationwide Children's Hospital</i></b>
4:40 pm to 5:40 pm	Panel/Open Mic Discussion/Questions/Next Steps (1 hour)

### SESSION 5: JOINT PHYSICIANS/SCIENTISTS & PATIENTS/FAMILIES

5:40 pm to 6:00 pm	SUMMARY OF THE DAY <b><i>Dan Kastner, M.D., Ph.D. - Scientific Director NHGRI, National Institutes of Health</i></b>
6:00 pm to 7:00 pm	JOINT RECEPTION FOR PHYSICIANS/SCIENTISTS & PATIENTS/FAMILIES