

# The Third International Conference on Deficiency of ADA2

## AGENDA

TUESDAY | NOVEMBER 17, 2020

**SESSION 1: WORLD-WIDE DADA2 COHORTS – TELL US SOMETHING NEW!**

7:00 to 7:40 EST	Sign into Virtual Conference   Check Internet   Check Audio & Video Connection
7:40 to 8:10 EST	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, USA</b>
8:10 to 8:25 EST	India: DADA2 Cohort - <u>Inaugural DADA2 Conference Lecture</u> <b>Aman Sharma, M.D. – Postgraduate Institute of Medical Education &amp; Research, Chandigarh, India</b>
8:25 to 8:40 EST	Belgium: DADA2 Cohort <b>Isabelle Meyts, M.D., Ph.D. – University Hospitals Gasthuisberg, Leuven, Belgium</b>
8:40 to 8:55 EST	Canada: DADA2 Cohort <b>Kelly Brown, Ph.D. – BC Children’s Hospital Research Institute, Vancouver, BC, Canada</b>
8:55 to 9:10 EST	China DADA2 Cohort <b>Qing Zhou, Ph.D. – Zhejiang University, Hangzhou, China</b>
9:10 to 9:25 EST	England: DADA2 Cohort <b>Samantha Cooray, M.D., Ph.D. – UCL Institute of Child Health, London, England</b>
9:25 to 9:40 EST	Finland: DADA2 Cohort <b>Janna Saarela, M.D., Ph.D. - Institute for Molecular Medicine, Helsinki, Finland</b>
9:40 to 9:55 EST	France: DADA2 Cohort <b>Alexander Belot, M.D., Ph.D. - Hospices Civils de Lyon, Lyon, France</b> <b>Sophie Georgin-Lavialle, M.D., Ph.D. - Pierre and Marie Curie University, Paris, France</b>
9:55 to 10:05 EST	<b>BREAK</b>
10:05 to 10:20 EST	Germany :DADA2 Cohort <b>Bodo Grimbacher, M.D. – Center for Chronic Immunodeficiency, Freiburg, Germany</b>

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10:20 to 10:35 EST	Israel: DADA2 Cohort <b>Yackov Berkun, M.D. - Hadassah Hebrew University Medical Center, Jerusalem, Israel</b>
10:35 to 10:50 EST	Italy: DADA2 Cohort <b>Marco Gattorno, M.D. - Giannina Gaslini Institute, Genoa, Italy</b>
10:50 to 11:05 EST	Japan: DADA2 Cohort <b>Kazushi Izawa, M.D., Ph.D. – Kyoto University Hospital, Kyoto, Japan</b>
11:05 to 11:20 EST	Netherlands: DADA2 Cohort <b>Joris van Montfrans, M.D., Ph.D. – University Medical Center Utrecht, Utrecht, The Netherlands</b>
11:20 to 11:35 EST	NIH   USA: DADA2 Cohort <b>Karyl Barron, M.D. – NIAID/NIH, Bethesda Maryland, USA</b>
11:35 to 11:50 EST	South America: DADA2 Cohort <b>Gustavo Santo, M.D. – Hospitais da Universidade, Coimbra, Portugal</b>
11:50 to 12:05 EST	Spain DADA2 Cohort <b>Juan Ignacio Aróstegui, M.D., Ph.D. – Hospital Clinic-Biomedical Diagnostic Center, Barcelona, Spain</b>
12:05 to 12:20 EST	Turkey: DADA2 Cohort <b>Seza Ozen, M.D. - Hacettepe University, Dept of Pediatrics, Ankara, Turkey</b>
12:20 to 12:30 EST	<b>Q &amp; A</b>

**WEDNESDAY | NOVEMBER 18, 2020**

**SESSION 2: NEW INSIGHTS IN DADA2 – THINGS YOU NEED TO KNOW**

7:00 to 7:40 EST	Sign into Virtual Conference   Check Internet   Check Audio & Video Connection
7:40 to 8:10 EST	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, USA</b>
8:10 to 8:30 EST	Neurological Phenotype of DADA2 – The Neurologists' Perspective <b>Timothy Bernard, M.D. - University of Colorado School of Medicine, Denver, Colorado USA</b>
8:30 to 8:50 EST	Dermatological Phenotype of DADA2 – The Dermatologists' Perspective <b>Edward Cowen, M.D. – Dermatology Branch   NCI/NIH, Bethesda, Maryland USA</b>
8:50 to 9:10 EST	Non-Invasive Vascular Testing & Cardiac Phenotype of DADA2 – The Cardiologists' Perspective <b>Alessandra Brofferio, M.D. – NHLBI/NIH, Bethesda, Maryland USA</b>
9:10 to 9:30 EST	Immunodeficiency Phenotype of DADA2 – The Immunologists' Perspective <b>Troy Torgerson, M.D., Ph.D. – Allen Institute for Immunology, Seattle, Washington USA</b>
9:30 to 9:50 EST	Efficacy of TNF-Inhibitors in Treating DADA2 <b>Amanda Ombrello, M.D. – NHGRI/NIH, Bethesda, Maryland USA</b>
9:50 to 10:00 EST	<b>BREAK</b>
10:00 to 10:20 EST	Genotype & Functional Correlates of Disease Phenotype in DADA2 <b>Pui Lee, M.D., Ph.D. – Harvard Medical School, Boston, Massachusetts USA</b>

10:20 to 10:40 EST	Analysis of DADA2 Pathogenesis Based on Single Cell RNA Sequencing of Monocytes <i>Alice Wu, M.D., Ph.D. – NHLBI/NIH, Bethesda, Maryland USA</i>
10:40 to 11:00 EST	How Not to Miss the Diagnosis of DADA2 <i>Ivona Aksentijevich, M.D. – NHGRI/NIH, Bethesda, Maryland USA</i>
11:00 to 11:20 EST	The Diagnosis of DADA2 in 30 mins at the Point of Care – Is it Possible? <i>Carson Chen, Ph.D. - iXensor, Taipei City, Taiwan</i>
11:20 to 11:40 EST	Can the Prevalence of DADA2 be Estimated? <i>Anne O'Donnell Luria, M.D., Ph.D. – Broad Institute at MIT, Cambridge, Massachusetts USA</i> <i>Samantha Baxter, MS, LGC – Broad Institute at MIT, Cambridge, Massachusetts USA</i>
11:40 to 12:30 EST	<b>Q &amp; A</b>

**THURSDAY | NOVEMBER 19, 2020**

**SESSION 3: FIRE SIDE CHATS – TOPICS THAT NEED A CONSENSUS**

7:00 to 7:40 EST	Sign into Virtual Conference   Check Internet   Check Audio & Video Connection
7:40 to 8:00 EST	Welcome   Introductions   Agenda Overview <i>Chip Chambers, M.D. - Founder &amp; President DADA2 Foundation</i> <i>Dan Kastner, M.D., Ph.D. - Scientific Director NHGRI, National Institutes of Health, USA</i>
8:00 to 8:20 EST	What to Do About the Asymptomatic DADA2 Patient? <i>Taryn Youngstein, M.D. - Imperial College Healthcare NHS Trust, London, England</i>
8:20 to 8:40 EST	Interpreting Variants of Unknown Significance in Patients with Sign/Symptoms of DADA2 <i>Pui Lee, M.D., Ph.D. – Harvard Medical School, Boston, Massachusetts USA</i>
8:40 to 10:10 EST	What is the Function of ADA2? – Can We Design a Research Agenda to Answer that Question? <i>Sonia Sharma, Ph.D. – La Jolla Institute for Immunology, La Jolla, California USA</i> <i>Ole Kristian Greiner-Tollersrud, Ph.D. – Institute of Medical Biology University of Tromsø, Norway</i> <i>Michele Proeitti, M.D. Ph.D. – Center for Chronic Immunodeficiency, Freiburg, Germany</i> <i>Peter Grayson, M.D. – NIAMS/NIH, Bethesda, Maryland USA</i> <i>Kazushi Izawa, M.D., Ph.D. – Kyoto University Hospital, Kyoto, Japan</i> <i>Dan Kastner, M.D., Ph.D. – Moderator   Scientific Director, NHGRI/NIH, Bethesda, Maryland USA</i>
10:10 to 11:00 EST	What is the Best Approach to Hematopoietic Stem Cell Transplant for DADA2? <i>Jennifer Kanakry, M.D. – NCI/NIH, Bethesda, Maryland USA</i> <i>Isabelle Meyts, M.D., Ph.D. – University Hospitals Gasthuisberg, Leuven, Belgium</i> <i>Mrinal Patnaik, MBBS – Moderator   Mayo Clinic, Rochester, Minnesota, USA</i>
11:00 to 12:10 EST	Can DADA2 be Cured by Gene Therapy & Gene Editing? <i>Despina Eleftheriou, MBBS, MRCPCH, Ph.D. - UCL Institute of Child Health, London, England</i> <i>Alessandra Mortellaro, Ph.D. - San Raffaele Telethon Institute for Gene Therapy, Milan, Italy</i> <i>Rachel Diamant, MSc Eng – EMENDO Biotherapeutics, New York, New York, USA</i> <i>Harry Malech, M.D. – Moderator   NIAID/NIH, Bethesda, Maryland USA</i>
12:10 to 12:30 EST	<b>Conclusion 3<sup>rd</sup> International Conference on the Deficiency of ADA2</b>