

CONFERENCE AGENDA

*CONTENT SUBJECT TO CHANGE

SESSION COLOR KEY	■ Family Advisory Working Groups	■ Super Siblings	■ ASMD
	■ First Time Attendees	■ Legacy Families	■ NPC
	■ General Sessions		

THURSDAY July 10

TIME	SESSION	LOCATION
10:00am - 5:30pm	Conference Registration Open	Registration North
12:45pm - 4:00pm	Activity Zone Open <i>(For Family Advisory Working Group attendees only)</i>	Carolina
1:00pm - 4:00pm	Family Advisory Working Groups <i>(Pre-registration required)</i>	
4:30pm - 5:30pm	First Time Attendees Welcome Session	Concord I & J
5:45pm - 8:30pm	Activity Zone Open	Carolina
5:30pm	NNPDF Conference Welcome Dinner and Opening Session 5:30pm Welcome Dinner 6:00pm NNPdf Welcome and Conference Opening Liz Heinze, RN, NNPdf Board Chair 6:20pm 2024 - 2025 Memorial Video: We Remember Them <i>To honor and remember the Niemann-Pick Disease community members we lost in the past year.</i>	Concord F
6:45pm - 8:30pm	GETTING TO KNOW YOU BREAKOUT SESSIONS: ASMD: Individuals Diagnosed with ASMD (adults over age 18) ASMD: Parents and Caregivers of individuals diagnosed with ASMD Legacy Families: For families who have experienced the loss of a loved one to Niemann-Pick Disease NPC: Individuals Diagnosed with NPC and Spouses NPC: Parents and Caregivers of individuals diagnosed with NPC currently under age 18 NPC: Parents and Caregivers of individuals diagnosed with NPC currently over age 18 Super Siblings: Siblings (age 17+) of individuals diagnosed with Niemann-Pick Disease Facilitators: Serina Heinze and Sam Stites	Concord G Concord H Executive Boardroom Fairway A Fairway B Kannapolis A Kannapolis B

FRIDAY July 11

TIME	SESSION	LOCATION
6:00 - 9:00am	Hotel Breakfast	Atrium
8:00am	Conference Registration	Registration North
9:15am - 12:15pm	Activity Zone Open	Carolina
9:30 - 9:45am	Welcome, NNPdf Liz Heinze, RN, NNPdf Board Chair	Concord F
9:45 - 10:15am	Family Journey Jillian Arnold, Mom of Roman and Stella, ASMD	Concord F

10:15 - 12:15pm	Adult Hangout Space <i>For NPD diagnosed individuals ages 18+</i>	Fairway A
10:15 - 11:00am	Niemann-Pick Diseases: An overview of the diseases for families Josh Baker, DO, Attending Physician, Division of Genetics, Genomics, and Metabolism, Ann & Robert H. Lurie Children's Hospital of Chicago Caroline Hastings, MD, Director, Pediatric Hematology Oncology Fellowship Program, Children's Hospital & Research Center Oakland Moderator: Andrew Lieberman, MD, PhD, Professor, University of Michigan Medical School, NNPDP Scientific Advisory Board Chair	Concord F
11:00 - 11:15am	BREAK	
11:15 - 12:15 pm		
NPC PRE-CLINICAL UPDATES Phenotyping Neurological Abnormalities in Niemann-Pick Disease Type C Jaymin Upadhyay, PhD, Boston Children's Hospital NPC Survey: Impact of NPC on Quality of Life Toni Mathieson, INPDA Executive Secretary, NPUK Chief Executive Krista Casazza, PhD, Scientific Consultant, INDORE Update on Efavirenz Clinical Trial for NPC Patients Lola Ledesma, PhD, Centro Biologia Molecular Severo Ochoa, Madrid, Spain Moderators: Paul Merrigan and Mary Frances Harmon, NNPDP Board of Directors LOCATION: Concord F		ASMD PRE-CLINICAL UPDATE/SCREENING ASCEND Pediatric and Adult Trial Melissa Wasserstein, MD, Chief, Division of Pediatric Genetic Medicine, Children's Hospital at Montefiore ASMD at the Intersection of Carrier Screening and Newborn Screening Rachel Hickey, MS, LCGC, Genetic Counselor III, Ann & Robert H. Lurie Children's Hospital of Chicago, Division of Genetics, Genomics & Metabolism Moderator: Taylor Sabky, NNPDP Board of Directors LOCATION: Kannapolis A & B
12:15 - 1:30pm	LUNCH BREAK	Concord I & J
12:15 - 1:30pm	Super Siblings Lunch Meeting (AGE 17+) Invitation Only	Concord I & J
1:15 - 4:00pm	Activity Zone Open	Carolina
1:45 - 3:15pm		
NPC CLINICAL UPDATES Trappsol Cyclo (Cyclo Therapeutics) Lori Mckenna, Global Head of Patient Advocacy adrabetadex (Mandos Health) Elizabeth Berry-Kravis, MD, PhD, Rush University Medical Center nizubaglustat (Azafaros) Aidan Gill, MD, Medical Director Azafaros Caroline Hastings, MD, Director, Pediatric Hematology Oncology Fellowship Program, Children's Hospital & Research Center Oakland Moderator: Andrew Lieberman, MD, PhD, NNPDP Scientific Advisory Board Chair LOCATION: Concord F		ASMD UPDATES Preclinical Research to Understand and Treat Brain Pathology in ASMD Lola Ledesma, PhD, Centro Biologia Molecular Severo Ochoa, Madrid, Spain The Future of ASMD Research Ed Schuchman, PhD, Director, International Center for Types A & B Niemann-Pick Disease, Mount Sinai School of Medicine ASMD Patient Reported Outcome Justin Hopkin, MD, NNPDP Scientific Advisory Board Sandy Cowie, President, International Niemann-Pick Disease Alliance (INPDA) Moderator: Justin Hopkin, NNPDP Scientific Advisory Board LOCATION: Kannapolis A & B
3:15 - 3:30pm	BREAK	

3:30 - 4:45 pm

NPC APPROVED TREATMENTS

AQNEURSA (IntraBio)

Marc Patterson, MD, US Chief Medical Officer, IntraBio Inc.

MIPLYFFA (Zevra Therapeutics)

Barbara Burton, MD, Professor of Pediatrics Northwestern University Feinberg School of Medicine, Attending Physician of Genetics, Genomics and Metabolism, Ann & Robert H. Lurie Children's Hospital of Chicago

Orsini Specialty Pharmacy

Jacob Ruesch, CPhT, Director of Complex Therapy Operations, Orsini

Award Presentations

Liz Heinze, RN, NNPDP Board Chair

Moderators: Liz Heinze, NNPDP Board Chair & Paul Merrigan, NNPDP Board of Directors

LOCATION: Concord F

ASMD

Ask The Experts

Josh Baker, DO, Attending Physician, Division of Genetics, Genomics, and Metabolism, Ann & Robert H. Lurie Children's Hospital of Chicago

Justin Hopkin, MD, NNPDP Scientific Advisory Board

Melissa Wasserstein, MD, Chief, Division of Pediatric Genetic Medicine, The Children's Hospital at Montefiore

Ed Schuchman, PhD, Director, International Center for Types A & B Niemann-Pick Disease, Mount Sinai School of Medicine

Sanofi Patient Support Services (PSS)

Susan Dodge King, Head, Rare Disease PSS

Moderator: Taylor Sabky, NNPDP Board of Directors

LOCATION: Kannapolis A & B

5:00 - 7:30pm

Dinner on your own in Concord, NC

7:30 - 9:30pm

Family Fun Night: Join us for games and Karaoke!

Concord F

SATURDAY July 12

TIME	SESSION	LOCATION
7:30 - 10:30am	Hotel Breakfast	Atrium
8:45 - 12:00am	Activity Zone Open	Carolina
9:00 - 9:10am	Welcome Liz Heinze, RN, NNPDP Board Chair	Concord F
9:10 - 9:35am	Research: International Niemann-Pick Disease Registry (INPDR) Solomon Mbua, PhD, INPDR Consultant Moderator: Justin Hopkin, MD, INPDR Chief Scientific Officer	Concord F
9:35 - 10:00am	INPDA Updates Sandy Cowie, President, International Niemann-Pick Disease Alliance Toni Mathieson, INPDA Executive Secretary, NPUK Chief Executive	Concord F
10:00 - 10:15am	BREAK	
10:15 - 10:30am	Family Journey Dan Reynolds, Spouse of Dee, NPC	Concord F
10:30 - 11:45am	Super Siblings Panel Discussion Erol Ayik Serina Heinze Jenna Hill Avery Hopkin Grady Hopkin Sam Stites Moderator: Taylor Sabky, NNPDP Board of Directors	Concord F
12:00 - 1:00pm	Lunch	Concord I & J

1:15 - 3:30pm	Activity Zone Open	Carolina
1:30 - 2:30pm	Experts Panel Discussion: Continuing the Advancement of Diagnosis and Treatment of Niemann-Pick Disease Elizabeth Berry-Kravis, MD, PhD, Rush University Medical Center Sandy Cowie, President, International Niemann-Pick Disease Alliance Caroline Hastings, MD, Director, Pediatric Hematology Oncology Fellowship Program, Children's Hospital & Research Center Oakland Forbes (Denny) Porter, MD, PhD, Senior Investigator and Associate, Scientific Director for Translational Research, National Institutes of Health Justin Hopkin, MD, NNPDF Scientific Advisory Board Melissa Wasserstein, MD, Chief, Division of Pediatric Genetic Medicine, The Children's Hospital at Montefiore Josh Baker, DO, Attending Physician, Division of Genetics, Genomics, and Metabolism, Ann & Robert H. Lurie Children's Hospital of Chicago Moderators: Paul Merrigan, NNPDF Board of Directors	Concord F
1:30 - 2:15pm	Super Siblings Session for ages under 13 Facilitators: Serina Heinze and Sam stites	Kannapolis A
2:15 - 3:30pm	Super Siblings Session for ages 14+ Facilitators: Serina Heinze and Sam Stites	Kannapolis A
2:30 - 3:30pm	Newborn Screening Panel Justin Hopkin, MD, NNPDF Scientific Advisory Board Melissa Wasserstein, MD, Chief, Division of Pediatric Genetic Medicine, The Children's Hospital at Montefiore Josh Baker, DO, Attending Physician, Division of Genetics, Genomics, and Metabolism, Ann & Robert H. Lurie Children's Hospital of Chicago Moderator: Liz Heinze, NNPDF Board Chair	Concord F
3:30pm	Activity Zone Closes	
5:00 - 6:00pm	Capture the Moment: Family Photos	Concord Foyer
6:00 - 10:00pm	Gala Dinner Special dinner, DJ, dancing, and entertainment from the Activity Zone!	Concord F, I, & J

SUNDAY July 13

TIME	SESSION	LOCATION
7:30 - 10:00am	Hotel Breakfast	Atrium
8:15 - 12:00am	Activity Zone Open	Carolina
8:30 - 8:45am	Welcome Liz Heinze, NNPDF Board Chair	Concord F
8:45 - 10:15am		
IEP Working Session: Bring copies of your IEP and join an interactive session with Taylor Sabky and Dawn Stites LOCATION: Concord F	Legacy Family Time Together LOCATION: Concord G	
10:15 - 10:30am	BREAK	

10:15 - 12:00pm	ADULT HANGOUT SPACE for diagnosed individuals (Ages 18+)	Fairway A
10:30 - 12:00pm	Until Next Time Breakout Sessions:	
	ASMD: Moms of individuals diagnosed with ASMD	Concord H
	DADS: Dads of individuals diagnosed with Niemann-Pick Disease	Concord G
	Extended family/grandparents/friends/caregivers of individuals diagnosed with Niemann-Pick Disease	Fairway B
	Individuals diagnosed with Niemann-Pick and their spouses	Kitty Hawk
	NPC: Moms of children diagnosed with NPC currently under age 15	Concord F
	NPC: Moms of children diagnosed with NPC currently over age 16	Executive Boardroom
	Siblings: Age 18+	Blueridge
12:00pm	Farewell Lunch	Concord I & J