



nnpdf
Family Support & Medical Conference



2026 CONFERENCE AGENDA

SESSION COLOR KEY: ■ ASMD ■ NPC

*CONTENT SUBJECT TO CHANGE

THURSDAY July 9

TIME	SESSION	LOCATION
10:00am - 5:30pm	Conference Registration Open	Grand Registration
12:45pm - 4:00pm	Activity Zone Open <i>(For Family Advisory Working Group attendees only)</i>	Journey A
12:30pm - 1:00pm	Family Advisory Working Groups Registration	Ponce de Leon Foyer
1:00pm - 4:00pm	Family Advisory Working Groups <i>(Pre-registration required)</i>	
4:30pm - 5:30pm	First Time Attendees Welcome Session	Expedition
5:45pm - 8:30pm	Activity Zone Open	Journey A
5:30pm	NNPDF Conference Welcome Dinner and Opening Session 5:30pm Welcome Dinner 6:00pm NNPDF Welcome and Conference Opening 6:20pm Memorial Video: We Remember Them <i>A tribute in honor of Niemann-Pick disease community members we remember this year.</i>	Ponce de Leon I-III
6:45pm - 7:30pm	Family Breakout Sessions I:	
	ASMD: Moms of individuals diagnosed with ASMD	Bimini Ballroom
	DADS: Dads of individuals diagnosed with Niemann-Pick Disease	Voyage
	Extended family/grandparents/friends/caregivers of individuals diagnosed with Niemann-Pick Disease	Discovery A
	NPC: Moms of children diagnosed with NPC currently under age 15	Discovery B
	NPC: Moms of children diagnosed with NPC currently over age 15	Journey B
	Super Siblings: Age 18+ of individuals diagnosed with Niemann-Pick Disease Facilitators: Serina Heinze and Sam Stites	Ponce de Leon I-III

FRIDAY July 10

TIME	SESSION	LOCATION
7:00am - 9:00am	Breakfast	Deep Blu Seafood Grill
8:00am - 4:00pm	Conference Registration	Grand Registration
9:10am - 12:15pm	Activity Zone Open	Journey A
9:15am - 9:30am	Welcome & NNPDF Update Heather Teal, NNPDF Executive Director	Ponce de Leon I-III
9:30am - 10:10am	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, Professor of Pediatrics, UCSF Benioff Children's Hospital Oakland Moderator: Andrew Lieberman, MD, PhD, Professor, University of Michigan Medical School, NNPDF Scientific Advisory Board Chair	Ponce de Leon I-III
10:00am - 12:15pm	Adult Hangout Space <i>For Niemann-Pick diagnosed individuals ages 18+</i>	Discovery B
10:10am - 10:25am	BREAK	

10:25am - 11:15am		
Family Voices: Navigating Care and Life Transitions		
A Parent’s Perspective on Transition to Adulthood, Guardianship, and Planning Barb Lazarus, Mother of Daniel and David Trish Reiter, Mother of Allison Phoebe Ramsey, Certified Case Worker, LISW		How Palliative Care Helps Families Live Better Liz Heinze, Mother of Tyler, Katie, and Faith Krystal Samuelson, Mother of Willow
LOCATION: Ponce de Leon I-III		LOCATION: Expedition
11:15am - 12:15pm		
NPC Translational Updates NPC1 Gene Therapy: An Update on Preclinical Gene Therapy Cristin Davidson, PhD, Staff Scientist at NICHD, Porter Laboratory NPC Lived Experience Survey Krista Casazza, PhD, Scientific Consultant, INDORE NPC Consensus Clinical Guidelines Update Forbes D. Porter, MD, PhD, Senior Investigator, NICHD, NIH Moderator: Andrew Lieberman, MD, PhD, Professor, University of Michigan Medical School, NNPDP Scientific Advisory Board Chair LOCATION: Ponce de Leon I-III		ASMD Translational Updates Adults With ASMD Have Sustained Improvements in Clinical Outcomes With up to 5 Years of Olipudase Alfa ERT: ASCEND Trial Final Results Melissa Wasserstein, MD, Chief, Division of Pediatric Genetic Medicine, The Children's Hospital at Montefiore Development of Biobank/Observational Study for ASMD Josh Baker, DO, Attending Physician, Division of Genetics, Genomics, and Metabolism, Ann & Robert H. Lurie Children’s Hospital of Chicago Moderator: Justin Hopkin, MD, NNPDP Scientific Advisory Board LOCATION: Expedition
11:15am - 1:15pm	Cuddle Break Experience	Foyer
12:15pm - 1:30pm	LUNCH	Tesoro Cove
12:15pm - 1:30pm	Super Siblings Lunch Meeting (AGE 17+) Invitation Only	Tesoro Cove
1:30pm - 5:00pm	Activity Zone Open	Journey A
1:30pm - 3:15 PM		
NPC Clinical Updates Trappsol Cyclo (Cyclo Therapeutics) Josh Fine, COO, Cyclo Therapeutics TransportNPC™: A Phase 3 Study Update Caroline Hastings, MD, Professor of Pediatrics, UCSF Benioff Children's Hospital Oakland Beren Therapeutics: Progress Update and Looking Ahead Irene von Hennigs, PharmD adrabetadex (Beren Therapeutics) Adrabetadex Improves Survival, Slows Disease Progression and Normalizes Biomarkers in NPC Elizabeth Berry-Kravis, MD, PhD, Rush University Medical Center Update Clinical Program Azafaros including study information on Phase 3 NAVIGATE Chris Freitagh, CMO NIH Natural History Study Update: Clinical Aspects, Biomarkers and Genetic Modifiers Forbes D. Porter, MD, PhD, Senior Investigator NICHD, NIH Moderator: Andrew Lieberman, MD, PhD, Professor, University of Michigan Medical School, NNPDP Scientific Advisory Board Chair		ASMD Family Learning: Everyday Life and Shared Experiences Living Well with ASMD: Learning From Each Other Courtney Hopkin, Anne OConnor Smith, Evren Ayik ASMD Community Discussion: Shaping What's Next Heather Teal, NNPDP Executive Director Allie Stavrolakis, NNPDP Family Services Manager ASMD Clinical Guidelines Josh Baker, DO, Attending Physician, Division of Genetics, Genomics, and Metabolism, Ann & Robert H. Lurie Children’s Hospital of Chicago Moderator: Justin Hopkin, MD, NNPDP Scientific Advisory Board LOCATION: Expedition
3:15pm - 3:30pm	BREAK	

3:30pm - 4:30 pm		
NPC Approved Treatments AQNEURSA (IntraBio) Caroline Hastings, MD, Professor of Pediatrics, UCSF Benioff Children's Hospital Oakland MIPLYFFA (Zevra Therapeutics) Charting a Path in Niemann-Pick Disease Type C: Clinical Development Overview of Arimoclomol Caroline Hastings, MD, Professor of Pediatrics UCSF Benioff Children's Hospital Oakland What Happens After a Treatment Is Prescribed? A Practical Guide for NPC Families (Orsini Specialty Pharmacy) Dakota Hanson, Specialty Pharmacy Program Manager Jacob Ruesch, CPhT, Director of Complex Therapy Operations Walking the Journey Together: Supporting Families Living with NPC (Curant Rare) CJ Kovarik, PharmD, AAHIVP Geoffrey Fenich, PharmD, AAHIVP Moderator: Andrew Lieberman, MD, PhD, Professor, University of Michigan Medical School, NNPDP Scientific Advisory Board Chair LOCATION: Ponce de Leon I-III	ASMD Approved Treatment and Experts Panel CareConnectPSS and the Patient Journey (Sanofi) Sarah Nabors, MS, CGC Bevin Gray, Case Manager Ask The Experts Panel 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	
	LOCATION: Expedition	

4:30pm - 5:00pm	Know Your Rights: Accessing and Advocating for Niemann-Pick Treatment Coverage Erin Bradshaw, EVP, Development and Strategy, Patient Advocate Foundation	Ponce de Leon I-III
5:00pm	Dinner on your own in Orlando!	
7:30pm	Family Fun Night: Join us for Music Bingo & Karaoke	Expedition

SATURDAY July 11		
TIME	SESSION	LOCATION
7:00am - 9:00am	Breakfast	Deep Blu Seafood Grill
7:45am - 12:00pm	Activity Zone Open	Journey A
8:00am - 8:40am	INPDR Patient Reported Data Update/Focus Group	Expedition
9:00am - 9:10am	Welcome Heather Teal, NNPDP Executive Director	Ponce de Leon I-III
9:10am - 9:30am	International Niemann-Pick Disease Alliance (INPDA) Sandy Cowie, INPDA President Harry Koujaian, INPDA Vice President (US) Carina Satin, INPDA Vice President (EU)	Ponce de Leon I-III
9:30am - 10:00am	International Niemann-Pick Disease Registry (INPDR) Shaun Bolton, INPDR Chief Operating Officer Moderator: Justin Hopkin, MD, INPDR Chief Scientific Officer	Ponce de Leon I-III
10:00am - 10:15am	BREAK	
10:00am - 12:15pm	Adult Hangout Space <i>For Niemann-Pick diagnosed individuals ages 18+</i>	Discovery B
10:15am - 11:00am	Super Siblings Panel Discussion Serina Heinze, Spencer Hill, Avery Hopkin, Grady Hopkin, and Sam Stites Moderator: Taylor Sabky, NNPDP Board of Directors <i>*With Special Thanks to our Super Siblings Panel Discussion Sponsor Beren Therapeutics</i>	Ponce de Leon I-III

TIME	SESSION	LOCATION
11:00am - 11:55am	FAMILY BREAKOUT SESSIONS II:	
	ASMD: Individuals Diagnosed with ASMD (adults over age 18)	Bimini Boardroom
	ASMD: Parents and Caregivers of individuals diagnosed with ASMD	Expedition
	Legacy Families: For families who have experienced the loss of a loved one to Niemann-Pick Disease	Ponce de Leon IV-V
	NPC: Individuals Diagnosed with NPC and Spouses	Journey B
	NPC: Parents and Caregivers of individuals diagnosed with NPC currently under age 18	Ponce de Leon I-III
	NPC: Parents and Caregivers of individuals diagnosed with NPC currently over age 18	Voyage
11:15am - 1:15pm	Cuddle Break Experience	Foyer
12:00pm - 1:15pm	Lunch	Tesoro Cove
1:15pm - 3:30pm	Activity Zone Open	Journey A
1:15pm -2:15pm	Experts Panel Discussion: The Future of Niemann-Pick Disease Care, Research, and Advocacy Josh Baker, DO, Attending Physician, Division of Genetics, Genomics, and Metabolism, Ann & Robert H. Lurie Children’s Hospital of Chicago Elizabeth Berry-Kravis, MD, PhD, Rush University Medical Center Sandy Cowie, President, International Niemann-Pick Disease Alliance Caroline Hastings, MD, Professor of Pediatrics, UCSF Benioff Children's Hospital Oakland Justin Hopkin, MD, NNPDP Scientific Advisory Board Forbes D. Porter, MD, PhD, Senior Investigator NICHD, NIH Melissa Wasserstein, MD, Chief, Division of Pediatric Genetic Medicine, Children's Hospital at Montefiore Moderator: Andrew Lieberman, MD, PhD, Professor, University of Michigan Medical School, NNPDP Scientific Advisory Board Chair	Ponce de Leon I-III
1:15pm - 2:00pm	Super Siblings Breakout <i>for ages under 13</i> Facilitator: Serina Heinze & Emily McGlocklin	Expedition
2:00pm - 3:30pm	Adult Hangout Space <i>For Niemann-Pick diagnosed individuals ages 18+</i>	Discovery B
2:15pm -2:30pm	Navigating the NNPDP Family Toolkit: A Guided Walkthrough Taylor Sabky, Kelly Lee	Ponce de Leon I-III
2:15pm - 3:30pm	Super Siblings Breakout <i>for ages 14+</i> Facilitators: Serina Heinze & Sam Stites	Expedition
2:30pm - 3:30pm	Newborn Screening Update 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 Pam Andrews, Firefly Fund Moderator: Justin Hopkin, MD, NNPDP Scientific Advisory Board	Ponce de Leon I-III
3:30pm	Activity Zone Closes	
5:00pm - 6:00pm	Capture the Moment: Family Photos	Grand Registration Foyer
6:00pm - 10:00pm	Gala & Awards Dinner Special dinner, DJ, dancing, and entertainment from the Activity Zone!	Ponce de Leon I-III