



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 11

TIME	SESSION	LOCATION
10:00am - 5:30pm	Conference Registration Open	Amethyst Pre-Function
12:45pm - 4:00pm	Activity Zone Open (For Family Advisory Working Group attendees only)	Amethyst Ballroom
12:30pm - 1:00pm	Family Advisory Working Groups Registration	Amethyst Pre-Function
1:00pm - 4:00pm	Family Advisory Working Groups (Pre-registration required)	
4:30pm	First Time Attendees Welcome Session	Olympus
5:45pm - 8:30pm	Activity Zone Open	Amethyst Ballroom
5:30pm	<p>NNPDF Conference Welcome Dinner and Opening Session</p> <p>5:30pm Welcome Dinner</p> <p>6:00pm NNPDF Welcome and Annual Update Joslyn Crowe, NNPDF Executive Director Becky McGuire, NNPDF Board Chair</p> <p>6:20pm 2023 - 2024 Memorial Video: We Remember Them To honor and remember the Niemann-Pick Disease community members we lost in the past year.</p>	Capitol Ballroom
6:45pm - 8:30pm	GETTING TO KNOW YOU BREAKOUT SESSIONS:	
	ASMD: Individuals Diagnosed with ASMD (adults over age 18)	Executive Boardroom 500
	ASMD: Parents and Caregivers of individuals diagnosed with ASMD	Capitol C
	Legacy Families: For families who have experienced the loss of a loved one to Niemann-Pick Disease	Gallivan Boardroom
	NPC: Individuals Diagnosed with NPC (adults over age 18)	Executive Boardroom 600
	NPC: Parents and Caregivers of individuals diagnosed with NPC currently under age 18	Sundance
	NPC: Parents and Caregivers of individuals diagnosed with NPC currently over age 18	Executive Boardroom 300
	Super Siblings: Siblings (age 12+) of individuals diagnosed with Niemann-Pick Disease Facilitator: Serina Heinze	Executive Boardroom 400

FRIDAY July 12

TIME	SESSION	LOCATION
7:00 - 8:30am	NNPDF Scientific Advisory Board <i>(Invitation Only)</i>	Sundance
7:30 - 8:30am	Breakfast	Olympus Ballroom
8:00am	Conference Registration	Amethyst Pre-Function
8:45am - 12:00pm	Activity Zone Open	Amethyst Ballroom
9:00 - 9:30am	Welcome, NNPDA & INPDA Updates Joslyn Crowe, NNPDA Executive Director Becky McGuire, NNPDA Board Chair Sandy Cowie, President, International Niemann-Pick Disease Alliance	Capitol AB
9:30 - 10:20am	Keynote Address: Klaus Romero, MD, MS, FCP, CEO, Critical Path Institute	Capitol AB
10:20 - 10:30am	BREAK	Amethyst Pre-Function
10:30 - 12:00pm	Adult NPD Hangout Space <i>For all diagnosed individuals ages 18+</i>	Sundance
10:30 - 11:15am	Niemann-Pick Diseases: An overview of the diseases for families Ed Schuchman, PhD, Genetic Disease Foundation - Francis Crick, Professor of Genetics & Genomic Sciences, Icahn School of Medicine at Mount Sinai, NNPDA Scientific Advisory Board Caroline Hastings, MD, Director, Pediatric Hematology Oncology Fellowship Program, Children's Hospital & Research Center Oakland, NNPDA Scientific Advisory Board Moderator: Andrew Lieberman, MD, PhD, Professor, University of Michigan Medical School, NNPDA Scientific Advisory Board Chair	Capitol AB
11:15 - 12:00 pm		
NPC PRE-CLINICAL UPDATES Scenic Biotech Guido Hartmann, Head of Translational Research Bloomsbury Genetic Therapies Giulia Leoni, MSc, PhD, Program Management and Leadership Consultant Adrien Lemoine, MSc, Co-Founder & Chief Executive Officer Moderator: Paul Merrigan, NNPDA Board of Directors LOCATION: Capitol AB		ASMD: "MY EXPERIENCE ON TREATMENT" Evren Ayik April Clemenza Sandy Cowie Carol Leon Travis Obermeyer Melissa Wasserstein, MD, Chief, Division of Pediatric Genetic Medicine, Children's Hospital at Montefiore Moderator: Justin Hopkin, MD, NNPDA Scientific Advisory Board LOCATION: Capitol C
12:00 - 1:30pm	LUNCH BREAK	Olympus Ballroom
12:00 - 1:30pm	Super Siblings Lunch Meeting (AGE 16+) <i>Invitation Only</i>	Sundance

NPC CLINICAL UPDATES**nizubaglustat (Azafaros)**

Marc Patterson, MD, Professor of Neurology, Pediatrics & Medical Genetics Chair, Division of Child and Adolescent Neurology, Mayo Clinic

Gisela Linthorst, Head of Patient Engagement and Advocacy, Azafaros

Trappsol Cyclo (Cyclo Therapeutics)

Caroline Hastings, MD, Director, Pediatric Hematology Oncology Fellowship Program, Children's Hospital & Research Center Oakland

Joseph Mejia, MD, Senior Medical Science Liaison, Cyclo Therapeutics

adrabetadex (Mandos Health)

Elizabeth Berry-Kravis, MD, PhD, Rush University Medical Center

Cathy Traz, VP, Patient and Community Engagement, Mandos Health

Moderator: Andrew Lieberman, MD, PhD, NNPfD Scientific Advisory Board Chair

LOCATION: Captiol AB

ASMD RESEARCH**Olipudase Alfa Phase 4 Trial Update**

Josh Baker, DO, Attending Physician, Director of Inborn Error of Metabolism Program, Ann & Robert H. Lurie Children's Hospital of Chicago

ASMD Accelerate

Steven Laffoon, Wylder Nation

Justin Hopkin, MD, Professor of Clinical Medicine, Chief, Division of Hospital Medicine, University of Rochester School of Medicine and Dentistry, NNPfD Scientific Advisory Board

The Future of ASMD Research Update

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

Moderator: Ed Schuchman, PhD, NNPfD Scientific Advisory Board

LOCATION: Captiol C

3:00 - 3:30pm	Break	Amethyst Pre-Function
3:30 - 5:00pm	BREAKOUT SESSIONS:	
	ASMD: Moms of individuals diagnosed with ASMD	Executive Boardroom 300
	DADS: Dads of individuals diagnosed with Niemann-Pick Disease	Olympus Ballroom
	Extended family/grandparents/friends/caregivers of individuals diagnosed with Niemann-Pick Disease	Executive Boardroom 600
	NPC: Moms of children diagnosed with NPC currently under age 15	Executive Boardroom 400
	NPC: Moms of children diagnosed with NPC currently over age 16	Executive Boardroom 500
	Spouses of individuals diagnosed with Niemann-Pick Disease	Gallivan Boardroom
	ADULT HANGOUT ROOM	Sundance
3:30 - 5:00pm	Industry Roundtable (<i>Invitation Only</i>)	Capital C
5:00pm	First Time Attendees Connect with NNPfD (<i>Optional</i>)	Olympus Ballroom
5:00pm	NNPfD Conference Program Ends for the Day Dinner on your own in Salt Lake City - Check out the following options! #1: Salt Lake Bees Baseball Game - <i>NNPfD Special Promotion</i> . For tickets visit t.ly/SaltLakeBees-NNPfD #2: City Creek Center - <i>Salt Lake City Dining & Shopping</i> #3: The Gateway - <i>Salt Lake City's downtown destination for dining, entertainment, and shopping</i>	

SATURDAY July 13

TIME	SESSION	LOCATION
7:00 - 8:15am	Breakfast	Olympus Ballroom
8:15 - 11:30am	Activity Zone Open	Amethyst Ballroom
8:30am	Welcome Joslyn Crowe, NNPDF Executive Director Becky McGuire, NNPDF Board Chair	Capitol AB
8:40 - 9:20am	Digital Health Technologies Update: Possibilities in Supporting Niemann-Pick Disease Research in the Technological Age Cristin Davidson, PhD, National Institutes of Health (NIH) Consortium Solomon Mbua, PhD, International Niemann-Pick Disease Registry (INPDR) Justin Hopkin, MD, Professor of Clinical Medicine, Chief, Division of Hospital Medicine, University of Rochester School of Medicine and Dentistry, NNPDF Scientific Advisory Board Sandy Cowie, INPDA President Moderator: Joslyn Crowe, NNPDF Executive Director	Capitol AB
9:20 - 10:05am	Tips & Tactics for Living with Niemann-Pick Disease: IEPs (Individualized Education Programs) Dawn Stites, Ed.S, Exceptional Student Education Teacher Taylor Sabky, Educator Moderator: Becky McGuire, NNPDF Board Chair	Capitol AB
10:05 - 10:15am	Break	Amethyst Pre-Function
10:15 - 11:00am	NEWBORN SCREENING ASMD Update Justin Hopkin, MD, Professor of Clinical Medicine, Chief, Division of Hospital Medicine, University of Rochester School of Medicine and Dentistry, NNPDF Scientific Advisory Board ScreenPlus Update Melissa Wasserstein, MD, Chief, Division of Pediatric Genetic Medicine, The Children's Hospital at Montefiore Pam Andrews, Firefly Fund Illinois Newborn Screening Update Joshua Baker, DO, Director of Newborn Screening, Lurie Children's Hospital of Chicago & Chair, Illinois Newborn Screening Committee Moderator: Justin Hopkin, MD, NNPDF Scientific Advisory Board	Capitol AB

11:00 - 12:00 pm

NPC CLINICAL/CORPORATE UPDATES

IB-1001 (IntraBio)

Clinical Update: Tatiana Bremova-Ertl, MD, Department of Neurology, University Hospital Bern

Corporate Update: Taylor Fields, Chief Product Development Officer, IntraBio Ltd.

Arimoclomol (Zevra Therapeutics)

Corporate Update: Neil McFarlane, President & CEO

Tara Greene, Sr. Director, US Medical Affairs & Patient Advocacy

Moderator: Joslyn Crowe, NNDPF Executive Director

LOCATION: Capitol AB

ASMD SUPPORTS & SERVICES

Patient Support Services: ASMD: Inheritance and the Importance of Family Screening

Sarah Nabors, Patient Education Liaison

Matt Basham, Patient Education Liaison, Sanofi Genzyme

The Assistance Fund

Stephanie Marshall, Director of Patient Advocacy

Moderator: Taylor Sabky, NNPDP Board of Directors

LOCATION: Capitol C

10:15am - 12:00pm	Adult NPD Hangout Space <i>For all diagnosed individuals ages 18+</i>	Sundance
12:00pm - 1:00pm	Lunch	Olympus Ballroom
12:45 - 4:30pm	Activity Zone Open	Amethyst Ballroom
1:15 - 1:45pm	Research: International Niemann-Pick Disease Registry (INPDR) Shaun Bolton, INPDR Operations Consultant Solomon Mbuja, PhD, INPDR Consultant Harry Koujaian, Ambassador, INPDR Moderator: Moderator: Justin Hopkin, MD, NNPDP Scientific Advisory Board	Capitol AB
1:45 - 2:30pm	Super Siblings Panel Discussion Serina Heinze Jenna Hill Christopher Sousa Samantha Stites Lisa Berry, LGC, Genetic Counselor III, Cincinnati Children's Hospital Moderator: Joslyn Crowe, NNPDP Executive Director	Capitol AB

2:30 - 3:30 pm

Experts Panel Discussion: Continuing the Advancement of Diagnosis and Treatment of Niemann-Pick

Elizabeth Berry-Kravis, MD, PhD, Rush University Medical Center

Sandy Cowie, President, INPDA

Caroline Hastings, MD, Director, Pediatric Hematology Oncology Fellowship Program, Children's Hospital & Research Center Oakland

Super Siblings Session for ages 6 - 12

Facilitators: Serina Heinze, and Laurie Turner, NNPDP Family Services Manager

Justin Hopkin, MD, Professor of Clinical Medicine,
Chief, Division of Hospital Medicine, University
of Rochester School of Medicine and Dentistry

Marc Patterson, MD, FRACP, FANA, FAAN, Chair,
Division of Child and Adolescent Neurology, Mayo Clinic

Forbes (Denny) Porter, MD, PhD, Senior Investigator
and Associate, Scientific Director for Translational
Research, National Institutes of Health

Klaus Romero, MD, MS, FCP, CEO, Critical
Path Institute

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

Moderator: Paul Merrigan, NNPfD Board of Directors

LOCATION: Capitol AB

LOCATION: Executive Boardroom 300

Coffee & Cookies outside main session available

3:30 - 4:30pm	BREAKOUT SESSIONS	
	<p>How to Share Your Diagnosis with Family and Friends</p> <p>Resources:</p> <p>Lisa Berry, LGC, Genetic Counselor III, Cincinnati Children's Hospital</p> <p>Meghann Ferguson, mother of Liam</p> <p>Taylor Sabky, mother of Purnell</p>	Olympus A
	<p>Open Q&A on all things Niemann-Pick: Ask an Expert</p> <p>Melissa Wasserstein, MD, ASMD Expert</p> <p>Caroline Hastings, MD, NPC Expert</p> <p>Justin Hopkin, MD, Families and Caregivers Expert</p> <p>Becky McGuire, NNPfD Expert</p> <p>Kelly Schoenecker, NPC Individual</p> <p>Sandy Cowie, ASMD Individual</p>	Olympus B
	<p>The Impact of Rare Disease on Siblings (for Parents & Caregivers)</p> <p>Joslyn Crowe, NNPfD Executive Director</p> <p>Garland Alvey, father of Abby and Claire</p> <p>Liz Heinze, mother of Tyler, Katie, Faith, and Serina</p> <p>Courtney Hopkin, mother of Avery, Grady, and Garrett</p>	Executive Boardroom 400
	<p>Super Siblings Session for ages 12+</p> <p>Facilitators: Serina Heinze, and Laurie Turner, NNPfD Family Services Manager</p>	Executive Boardroom 300
2:00 - 4:00pm	Adult NPD Hangout Space <i>For all diagnosed individuals ages 18+</i>	Sundance
6:00 - 10:00pm	<p>Gala Dinner</p> <p>Special Presentations:</p> <p>NNPfD Cora Sterling Endurance Award to Charlotte Joy Norris</p> <p>NNPfD Persevere Award to Darrile Papier</p> <p>Special dinner, DJ, dancing, and entertainment from the Activity Zone!</p>	Capitol ABC