


2023 NNPDF Family Support & Medical Conference

*CONTENT SUBJECT TO CHANGE




SESSION COLOR KEY:	■ Family Advisory Working Groups	■ Legacy families	Virtual Conference:  LIVESTREAM
	■ First Time Attendees	■ ASMD	
	■ General Sessions	■ NPC	
	■ Siblings		

THURSDAY July 20 | NNPDF Board of Directors Meeting, Family Advisory Working Groups, Conference Opening

TIME	SESSION	LOCATION		
8:00 - 1:00pm	Board of Directors Meeting <i>(Invitation only)</i>	Journey B		
10:00 - 5:30pm	Conference Registration SSW	Grand Registration Desk		
12:45 - 4:00pm	Activity Zone Open <i>(For Family Advisory Working Group attendees only)</i>	Journey A		
12:30 - 1:00pm	Family Advisory Working Group Registration	Grand Registration Desk		
1:00 - 4:00pm	Family Advisory Working Groups <i>(Pre-registration required)</i>			
	Group A: Azafaros LOCATION: Ponce de Leon IV	Group B: Cyclo Therapeutics LOCATION: Ponce de Leon V	Group C: Kisbee Therapeutics LOCATION: Journey B	Group D: Sanofi LOCATION: Ponce de Leon VII
4:30pm	First Time Attendees Welcome Session	Discovery B		
5:45 - 8:30pm	Activity Zone Open	Journey A		
5:30pm	NNPDF Conference Opening Session and Dinner 5:30 pm Welcome Dinner 6:00pm NNPDF Welcome Joslyn Crowe, NNPDF Executive Director Becky McGuire, NNPDF Board Chair 6:20pm 2022-2023 Memorial Video: We Remember Them <i>To honor and remember the NPD community members we lost in the past year.</i> Debbie Kflowitz, Mother of Rachael Anthony Leoni, Father of Jessica Tim and Liz Heinze, Father and Mother of Tyler, Katie, and Faith	Ponce de Leon I, II, III		

6:45 - 8:30pm	Getting to Know You Breakout Sessions:	
	ASMD Caregivers <i>Facilitators: Michael Smith, Justin Hopkin</i>	Ponce de Leon IV
	NPC Juvenile Caregivers of diagnosed individuals, current age under 18 <i>Facilitator: Meghann Ferguson</i>	Ponce de Leon V
	NPC Adult Caregivers of diagnosed individuals, current age 18+ <i>Facilitators: Gail Koujaian, Barbara Lazarus</i>	Ponce de Leon VI
	Unaffected Siblings *Unaffected siblings group is for ages 12+. Registration required. <i>Facilitator: Serina Heinze</i>	Discovery A
	Individuals Diagnosed with ASMD <i>Facilitator: Anne OConnor Smith</i>	Ponce de Leon VII
	Individuals Diagnosed with NPC (adults 18+ only) <i>Facilitators: Cara Gilmore, Laurie Turner</i>	Journey B
	Legacy Families <i>Facilitators: Anthony Leoni, Becky McGuire, Deb Kflowitz</i>	Discovery B

FRIDAY July 21 | Scientific Advisory Board Meeting, Clinical Updates, Corporate Updates, Research Sessions


TIME	SESSION	LOCATION
7:00 - 8:30am	NNPDF Scientific Advisory Board Meeting (<i>Invitation Only</i>)	Journey B
7:30 - 8:30am	BREAKFAST	Tesoro Cove
7:30am - 8:30am	First Time Attendees Family Breakfast	Tesoro Cove
8:00am	Conference Registration	Grand Registration Desk
8:45 - 12:00pm	Activity Zone Open	Journey A
9:00 - 9:30am	Welcome, NNPDF Annual Update Joslyn Crowe, NNPDF Executive Director Becky McGuire, NNPDF Board Chair  LIVESTREAM	Ponce de Leon I, II, III
9:30 - 10:15am	KEYNOTE ADDRESS: Overcoming Obstacles in Rare Disease Klaus Romero, MD, MS, Chief Scientific Officer; Executive Director of Clinical Pharmacology, Critical Path Institute  LIVESTREAM	Ponce de Leon I, II, III
10:15am	Family Journey  LIVESTREAM	Ponce de Leon I, II, III
10:20 - 10:30am	Break	
10:30 - 12:00pm	Adult NPD Hangout Space <i>For all diagnosed individuals ages 18+</i>	Discovery A




10:30 - 11:00am		10:30 - 11:30am	
<p>RESEARCH (NPC): NIH Genomic Project</p> <p>Dr. Denny Porter, Senior Investigator and Associate Scientific Director for Translational Research, National Institutes of Health</p> <p>Moderator: Dr. Andrew Lieberman, MD, PhD, Chair, NNPfD Scientific Advisory Board</p> <p>LOCATION: Ponce de Leon I, II, III</p> <p> LIVESTREAM</p>		<p>ASMD: The Power of Partnership</p> <p>Duane Clarke, General Manager, Rare Disease, Sanofi</p> <p>Joslyn Crowe, NNPfD Executive Director</p> <p>Moderator: Justin Hopkin, MD, NNPfD Scientific Advisory Board</p> <p>LOCATION: Ponce de Leon IV - VII</p>	
11:00 - 11:30am			
<p>CLINICAL UPDATE: AZ-3102</p> <p>Dr. Caroline Hastings, Pediatric Hematologist-Oncologist, UCSF Benioff Children’s Hospital, Oakland CA</p> <p>Gisela Linthorst, Head of Patient Engagement and Advocacy, Azafaros</p> <p>Moderator: Dr. Andrew Lieberman, MD, PhD, Chair, NNPfD Scientific Advisory Board</p> <p>LOCATION: Ponce de Leon I, II, III</p> <p> LIVESTREAM</p>			
11:30am - 12:00pm		11:30am - 12:00pm	
<p>CLINICAL UPDATE: IB1001</p> <p>Dr. Tatiana Bremova-Ertl, Department of Neurology, University Hospital Bern</p> <p>Taylor Fields, Chief Product Development Officer at IntraBio Ltd.</p> <p>Moderator: Dr. Andrew Lieberman, MD, PhD, Chair, NNPfD Scientific Advisory Board</p> <p>LOCATION: Ponce de Leon I, II, III</p> <p> LIVESTREAM</p>		<p>ASMD: Sanofi Patient Support Services (PSS)</p> <p>Phoebe Ramsay, Sanofi CareConnect PSS Case Manger</p> <p>Andrew Rolla, Sanofi CareConnect PSS Case Manager</p> <p>Moderator: Laurie Turner, NNPfD Family Services Manager</p> <p>LOCATION: Ponce de Leon IV - VII</p> <p> LIVESTREAM</p>	
12:00 - 1:00pm	NIH Genomic Project Blood Draws		Discovery B
12:00 - 1:00pm	Lunch		Tesoro Cove
12:45 - 5:00pm	Activity Zone Open		Journey A
1:00 - 3:00pm	Adult NPD Hangout Space <i>For all diagnosed individuals ages 18+</i>		Discovery A


1:00 - 1:30pm		1:00 - 1:30pm	
<p>CLINICAL UPDATE: Trappsol Cyclo</p> <p>Dr. Caroline Hastings, Pediatric Hematologist-Oncologist, UCSF Benioff Children’s Hospital, Oakland CA</p> <p>Dr. Joseph Mejia, Medical Science Liaison, Cyclo Therapeutics</p> <p>Moderator: Paul Merrigan, NNPfD Board Member, Principal, Merrigan Biopharma Consulting</p> <p>LOCATION: Ponce de Leon I, II, III</p> <p> LIVESTREAM</p>		<p>ASMD for Families: Know Your Healthcare System</p> <p>Phoebe Ramsay, Sanofi CareConnect PSS Case Manger</p> <p>Andrew Rolla, Sanofi CareConnect PSS Case Manager</p> <p>Lisa Berry, LGC, Division of Human Genetics, Cincinnati Children’s Hospital</p> <p>Laurie Turner, NNPfD Family Services Manager</p> <p>Moderator: Chrystelle Bougault, NNPfD Scientific Advisory Board</p> <p>LOCATION: Ponce de Leon IV - VII</p> <p> LIVESTREAM</p>	
1:30 - 2:30pm		1:30 - 2:00pm	
<p>CORPORATE UPDATES:</p> <p>1:30 - 1:50pm Kisbee Therapeutics Doug Feltner, Chief Medical Officer</p> <p>1:50 - 2:10pm Mandos Health Cathy Traz, VP, Patient and Community Engagement, Mandos Health</p> <p>2:10 - 2:30pm Zevra Therapeutics Dan Gallo, PhD, SVP, Medical Affairs and Advocacy</p> <p>Tara Greene, Sr. Director, US Medical Affairs and Patient Advocacy</p> <p>Moderator: Paul Merrigan, NNPfD Board Member, Principal, Merrigan Biopharma Consulting</p> <p>LOCATION: Ponce de Leon I, II, III</p> <p> LIVESTREAM</p>		<p>The Assistance Fund</p> <p>Jan Wigington, Senior Director of Operations, The Assistance Fund</p> <p>Moderator: Chrystelle Bougault, NNPfD Scientific Advisory Board</p> <p>LOCATION: Ponce de Leon IV - VII</p> <p> LIVESTREAM</p>	
		2:00 - 3:00pm	
		<p>The Future of ASMD Research</p> <p>2:00 - 2:20pm ASMD Pediatric and Adult Patient Reported Outcomes Surveys</p> <p>Justin Hopkin, NNPfD Scientific Advisory Board</p> <p>Solomon Mbuja, International Niemann-Pick Disease Registry</p> <p>2:20 - 3:00pm ASMD Research Update</p> <p>Steven Laffoon, Wylder Nation</p> <p>Dr. Lola Ledesma, PhD, Centro Biologia Molecular Severo Ochoa, Madrid, Spain</p> <p>Edward Schuchman, PhD, Director, International Center for Types A and B Niemann-Pick Disease, Mount Sinai School of Medicine, NNPfD Scientific Advisory Board</p> <p>Moderator: Justin Hopkin, MD, NNPfD Scientific Advisory Board</p> <p>LOCATION: Ponce de Leon IV - VII</p> <p> LIVESTREAM</p>	
2:30 - 3:30pm	NIH Genomic Project Blood Draws		Discovery B
3:00 - 3:30pm	Break		

3:30 - 5:00pm	Break Out Sessions:	
	Moms of Young Children (<i>currently under age 18</i>) <i>Facilitators: Meghann Ferguson, Liz Heinze</i>	Journey B
	Moms of Older Children (<i>currently over age 18</i>) <i>Facilitators: Gail Koujaian, Barbara Lazarus</i>	Ponce de Leon V
	ASMD Moms <i>Facilitator: Taylor Sabky</i>	Ponce de Leon VI
	Dads <i>Facilitators: Anthony Leoni, Justin Hopkin</i>	Stairwell Nook
	Spouses of NPD individuals <i>Facilitators: Michael Smith, Dan Reynolds</i>	Ponce de Leon IV
	Extended family/grandparents/friends/caregivers <i>Facilitators: Wendy Hopkin, Laurie Turner</i>	Discovery A
5:00pm	First Time Attendees Connect with NNPDF (<i>Optional</i>)	Grand Ballroom Foyer
5:00pm	NNPDF Conference Program Ends for the Day <i>Dinner on your own in Orlando - Check out the following options!</i> #1: Hotel Shuttle bus to theme parks #2: Hotel Shuttle bus to Disney Springs #3: Check out all the resort has to offer... 5 swimming pools, 2 lazy rivers, mini golf, multiple restaurants to choose from and fireworks from 9:00 - 9:30pm!	

SATURDAY July 22nd | Family Services Oriented Sessions, Family Services Breakout Sessions, Research and Gala Dinner

TIME	SESSION	LOCATION
7:00 - 8:00am	NIH Genomic Project Blood Draws	Discovery B
7:00 - 8:15am	BREAKFAST	Tesoro Cove
7:00 - 8:15am	BREAKFAST with First Time attendees (optional)	Tesoro Cove
7:00 - 8:30am	NPC Digital Health Technology Working Group meeting (<i>invitation only</i>)	Journey B
8:15 - 11:30am	Activity Zone Open	Journey A
8:30 am	Welcome Joslyn Crowe, NNPDF Executive Director Becky McGuire, NNPDF Board Chair 	Ponce de Leon I, II, III

8:40 - 9:10am	<p>CLINICAL UPDATE: Adrabetadex</p> <p>Dr. Elizabeth Berry-Kravis, Rush University Medical Center</p> <p>Moderator: Dr. Andrew Lieberman, MD, PhD, Chair, NNPfD Scientific Advisory Board</p> <p> LIVESTREAM</p>	Ponce de Leon I, II, III
9:10am	<p>Family Journey</p>	Ponce de Leon I, II, III
9:15 - 10:05am	<p>Main Session: Advocacy Hot Topics - Round table discussion with our experts</p> <p>Dr. Elizabeth Berry-Kravis, Director, RUSH Pediatric Neurosciences F.A.S.T. Center for Translational Research, Rush University Medical Center</p> <p>Sandy Cowie, President, International Niemann-Pick Disease Alliance</p> <p>Dr. Caroline Hastings, Pediatric Hematologist-Oncologist, UCSF Benioff Children’s Hospital, Oakland</p> <p>Dr. Andrew Lieberman, MD, PhD, Chair, NNPfD Scientific Advisory Board</p> <p>Paul Merrigan, NNPfD Board Member, Principal, Merrigan Biopharma Consulting</p> <p>Dr. Denny Porter, Senior Investigator and Associate Scientific Director for Translational Research, National Institutes of Health</p> <p>Dr. Klaus Romero, Chief Science Officer, Critical Path Institute (C-Path)</p> <p>Dr. Edward Schuchman, PhD, Director, International Center for Types A and B Niemann-Pick Disease, Mount Sinai School of Medicine</p> <p>Dr. Melissa Wasserstein, Chief, Division of Pediatric Genetic Medicine, The Children’s Hospital at Montefiore</p> <p>Moderator: Justin Hopkin, MD, NNPfD Scientific Advisory Board</p> <p> LIVESTREAM</p>	Ponce de Leon I, II, III
10:05 - 10:15am	Break	
10:15 - 10:45am	<p>Siblings Panel Discussion</p> <p>Erol Ayik Serina Heinze Mollie Honsinger Christopher Sousa Lisa Berry, LGC, Genetic Counselor III, Cincinnati Children’s Hospital</p> <p>Moderator: Joslyn Crowe, NNPfD Executive Director</p> <p> LIVESTREAM</p>	Ponce de Leon I, II, III

10:00 - 11:30am	Adult NPD Hangout Space <i>For all diagnosed individuals ages 18+</i>		Discovery A
10:50 - 11:30am	NNPDF Research Fellowship Updates 10:50 - 11:10am Bilal Abdul Moiz NNPDF Peter G. Penchev NPC Research Fellowship recipient <i>"Assessing the impact of membrane cholesterol deficiency in Niemann-Pick Disease Type C on brain endothelial-mediated glucose supply to the brain"</i> 11:10 - 11:30am Sara Naya Forcano NNPDF Edward H. Schuchman ASMD Research Fellowship recipient <i>"Intranasal delivery of chitosan-encapsulated hydrocortisone to treat brain pathology in ASMD"</i> Moderator: Edward Schuchman, PhD, Director, International Center for Types A and B Niemann-Pick Disease, Mount Sinai School of Medicine, NNPDF Scientific Advisory Board 		Ponce de Leon I, II, III
11:30 - 12:30pm	NIH Genomic Project Blood Draws		Discovery B
11:30 - 12:30pm	LUNCH		Tesoro Cove
12:15 - 4:30pm	Activity Zone Open		Journey A
12:30 - 1:15pm	Breakout Sessions		
	How to share your diagnosis with family and friends <u>Facilitators:</u> Kelly and Aaron Shoenecker Cara Gilmore <u>Expert Resources:</u> Dr. Caroline Hastings, Pediatric Hematologist-Oncologist, UCSF Benioff Children's Hospital, Oakland Lisa Berry, LGC, Genetic Counselor III, Cincinnati Children's Hospital LOCATION: Ponce de Leon IV-VI	Managing your own healthcare as an adult <u>Facilitators:</u> Alec Koujaian Barbara Lazarus <u>Expert Resources:</u> Dr. Elizabeth Berry-Kravis, Rush University Medical Center Dr. Melissa Wasserstein, The Children's Hospital at Montefiore LOCATION: Journey B	Younger Sibling Session (Ages 6 – 12) Serina Heinze Laurie Turner, NNPDF Family Services Manager LOCATION: Ponce de Leon VII

1:15 - 2:00pm	Breakout Sessions		
	Empowering the Patient Voice: How to tell your NPD story and raise awareness <u>Facilitators:</u> Cara Gilmore Kari Lato Taylor Sabky LOCATION: Ponce de Leon IV-VI	How to Advocate for what you need medically: How to navigate insurance issues and access specialists <u>Facilitators</u> Jillian Arnold Meghann Ferguson Gail Koujaian <u>Expert Resource:</u> Lisa Berry, LGC, Genetic Counselor III, Cincinnati Children's Hospital LOCATION: Journey B	Older Sibling Session (Ages 12+) Serina Heinze Laurie Turner, NNPDF Family Services Manager LOCATION: Ponce de Leon VII
2:00 - 2:15pm	Break		
2:00 - 4:00pm	Adult NPD Hangout Space <i>For all diagnosed individuals ages 18+</i>		Discovery A
2:15 - 2:45 pm	Research: Gene Therapy Cristin Davidson - NIH consortium Moderator: Dr. Andrew Lieberman, MD, PhD, Chair, NNPDF Scientific Advisory Board  LIVESTREAM		Ponce de Leon I, II, III
2:45 - 3:15 pm	Research: Efavirenz Dr. Lola Ledesma, PhD, Centro Biologia Molecular Severo Ochoa, Madrid, Spain Moderator: Dr. Andrew Lieberman, MD, PhD, Chair, NNPDF Scientific Advisory Board  LIVESTREAM		Ponce de Leon I, II, III
3:15 - 3:45 pm	Research: International Niemann-Pick Disease Registry (INPDR) Jackie Imrie, Clinical Research Manager, International Niemann-Pick Disease Registry (INPDR) Solomon Mbua, PhD, International Niemann-Pick Disease Registry Toni Mathieson, Director, INPDR and Gateway Ltd Moderator: Justin Hopkin, MD, NNPDF Board Chair Emeritus  LIVESTREAM		Ponce de Leon I, II, III
3:45 - 4:45pm	NPC Community Feedback Session: Use of Technology for Collecting Patient Data (NPC Family Members/ Caregivers only) Facilitators: Cristin Davidson, Justin Hopkin, Solomon Mbua		Ponce de Leon I, II, III
6:00 - 10:00pm	Gala Dinner Special Presentations: NNPDF Cora Sterling Endurance Award NNPDF Persevere Award NNPDF Breakthrough Award Special skit and entertainment from the Activity Zone!		Ponce de Leon I, II, III
End of NNPDF Family Support & Medical Conference			