

**National Niemann-Pick Disease Foundation, Inc.
24th Annual Family Support & Medical Conference**

Danvers, Massachusetts

August 11th - August 14th, 2016

Conference Agenda

Thursday, August 11, 2016



Time	Activity and Location	Attendees/Speakers/ Introductions
	NNPDF Board Meeting ~ Gloucester ~	NNPDF Board Members, Committee Chairs and Invited Guest
8:30am ~ 10:00am	"Open Meeting"	Open to NNPFD Family Membership
10:00am ~ 12:00pm	"Closed Board Meeting"	
12:00pm ~ 1:30pm	Luncheon ~ Ipswich~	NNPDF Board, SAB, Research Fellows, INPDA and Invited Guests
1:30pm ~ 4:00pm	Scientific Advisory Board (SAB) Meeting ~ Gloucester ~	SAB Members *Closed Meeting*
1:30pm ~ 4:00pm	NNPDF Board Committee Meetings ~ Oak Board Room ~	
1:30pm ~ 5:00pm	Strategic Plan ~ Consultant Interviews ~ Georgetown Room	
4:00pm ~ 4:15pm	SAB Break	
4:15pm ~ 5:30pm	Research Fellows Presentation ~ Gloucester ~	
5:30pm ~ 6:45pm	SAB Dinner ~ Trade Winds Restaurant ~	
5:00pm ~ 7:00pm	Conference Registration ~ Living Room~	All Conference Attendees ~ Sanofi Genzyme Expressions of Hope Art Display
6:00pm ~ 6:30pm	First-Time Conference Attendees & New Family Orientation ~ Newburyport~	First-Time Conference Attendees, Mentors, CAR Coordinator, NNPFD Board Members Welcome!
6:30pm ~ 7:20pm	Getting to Know You	Join us at one of the following informal sessions to say hello, get to know other family participants specific to your family circumstances, meet NNPFD staff and board members
	ASMD Families, Patients, Siblings	<i>Room ~ Hosted by Elissa Miller-Visoky & Anne O'Connor Smith ~Boxford Room~</i>
	NPC Families, Patients, Siblings	<i>North Shore Ballroom ~ Hosted by Darrile Papier & Lisa Chavez ~North Shore~</i>
	NPC Adult On-set Families, Patients, Siblings	<i>Room ~ Hosted by Wayne & Lori Palmiter ~Marble Foyer~</i>

24th Annual NNPDF Family Support and Medical Conference
Thursday, August 11th, 2016 ~ continued ~

Time	Activity and Location	Attendees/Speakers/ Introductions
7:30pm ~ 11:00pm "Greets & Treats"	<u>Welcome Reception</u> ~ <i>North Shore Ballroom</i> ~	All Conference Attendees
7:30pm ~ 8:30pm	Greetings and Welcome to Danvers, Massachusetts!	Lisa Chavez, Board Chair <u>2016 Local Family CHAMPIONS:</u> Ann, Joel & Melissa (NPC) Bennett, Connor & Rhonda Kehoe (in memory of Graham, NPC) and Holly & Mike Roberts (in memory of Erin, NPC)
	(I) NNPDF Board & Committee Members	(I) Lisa Chavez, NNPDF Board Chair
	(II) Scientific Advisory Board (SAB) & Fellows	(II) Elissa Miller-Visoky, NNPDF Research Committee Chair
	(III) Special Guests	(III) Lisa Chavez & Wayne Palmiter, NNPDF Board Chair & Vice Chair
	(IV) First-Time Attendees, Family Mentors and Key	(IV) Nadine Hill, NNPDF Executive Director
	(V) PERSEVERE Award	(V) Tammy Vaughan, NNPDF Secretary
8:30pm ~ 8:45pm	Break	
8:35pm ~ 9:15pm	Childcare/Young Adult Support Available ~ <i>Newburyport</i> ~ (Open during the "Light of Hope" Ceremony)	Childcare/Young Adult Volunteer Staff
8:45pm ~ 9:00pm	Light of Hope ~ Candle lighting Ceremony ~ <i>North Shore Ballroom</i> ~ A special time to remember those lost to NPD during the past year.	<i>Special thanks to Lisa Chavez, Mom to Breann (NPC), for the handmade memorial candles.</i>
9:00pm ~ 11:00pm	Family Gathering and Fellowship ~ <i>North Shore Ballroom</i> ~ Informal time to greet and catch up with old friends, and to make new acquaintances.	All Conference Attendees

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6:30am ~ 9:30am	Breakfast/Grand Ballroom III & IV	All NNPDF Hotel Guests
7:00am ~ 8:45am	NNPDF Research Committee Meeting with Dr. Andrew Liebermann, SAB Chair <i>*Closed Session* ~ Rockport Boardroom ~</i>	NNPDF Research Committee Members Dr. Andrew Liebermann, SAB Chair; Elissa, Miller-Visoky, NNPDF Research Committee Chair, Nadine Hill, NNPDF Executive Director *Closed Session*
8:30am ~ 8:45am	Volunteer Childcare Staff Training/Orientation ~ Newburyport ~	Jim & Missy Ward ~ CAR Coordinators Volunteer Staff
8:00am ~ 9:00 am	Late Registration & Art Exhibit <i>~ Living Room ~</i>	NNPDF Staff and Conference Volunteers
8:45am ~ 12:00pm	Childcare/Young Adult Activity Room Open ~ Newburyport ~	Volunteer Childcare/Young Adult Staff
9:00am ~ 12:00pm	Reflection/Bereavement ~ Maple Room A quiet and private opportunity for families who to gather, reflect and share.	Any interested conference attendee
9:00am ~ 11:30am	ASMD ~ Medical & Clinical Presentations ~ North Shore C	All ASMD families & guests
9:00am ~ 9:10am	Introduction and Welcome	Elissa Miller-Visoky, Research Chair
9:10am ~ 9:30am	ASMD Clinical Development Program Update	Sharon Tan, Sanofi Genzyme
9:30am ~ 9:50am	ASMD ERT ~ Adult and Pediatric Clinical Trial	Dr. Ana Puga & Amy McCagg, Sanofi Genzyme
9:50am ~ 10:30am	Medical Clinical Updates - Including NNPDF funded ASMD Natural History Study	Dr. Melissa Wasserstein, Montefiore
10:30am ~ 10:50am	BREAK ~ Living Room ~	
10:50am ~ 11:10am	ASMD Patient Reported Outcome I (PRO) Results and Plan for PRO II	Ruzan Avetisyan, Sanofi Genzyme
11:10am ~ 11:30am	ASMD Awareness Video & Educational Programs	Kathleen Coolidge Sanofi Genzyme Patient Advocate

24th Annual NNPfF Family Support and Medical Conference
Friday, August 12th, 2016 ~ continued ~

Time	Activity and Location	Attendees/Speakers/ Introductions
9:00am ~ 11:30am	NPC ~ Medical & Clinical Presentations ~ North Shore A & B	All NPC families & guests
9:00am ~ 9:10am	Introduction and Welcome	Dr. Andy Lieberman, NNPfF SAB Chair
9:10am ~ 9:30am	Update on the Phase 1/2A Vorinostat Trial	Dr. Forbes Porter, NIH
9:30am ~ 9:50am	Phase 1/2A Trial of Intrathecal VTS-270: Safety and Efficacy	Dr. Forbes Porter, NIH
9:50am ~ 10:10am	An Update on the Ongoing VTS270 Phase III Trial	Ben Machielse, Vtesse
10:10am ~ 10:30am	Development of Arimoclomol for Niemann-Pick Type C	Dr. Christine i Dali, Orphazyme
10:30am ~ 10:50am	BREAK ~ Living Room ~	
10:50am ~ 11:10am	Performance on Objective Clinical Efficacy Outcome Measures During Long-Term Cyclodextrin Treatment in NPC	Dr. Elizabeth Berry-Kravis, Rush University
11:10am ~ 11:25am	International Niemann-Pick Disease Registry	Jim Green, INPDA President Toni Mathieson, NP-UK Executive Director
11:25am ~ 12:00pm	Experts Panel Discussion ~ Q & A Explanation of Open House Segment	Dr. Andy Lieberman, NNPfF SAB Chair
12:00pm ~ 1:45pm	LUNCH ~Grand Ballroom III ~	
11:45am ~ 4:30pm	ASMD Medical/Clinical Presentation Framingham Manufacturing Tour	
11:45am ~ 12:00pm	Collect boxed lunches and Board Coach	
12:00pm ~ 1:00pm	Depart Hotel & Travel to Framingham	
1:00pm ~ 2:00pm	Genzyme Welcome & Presentation Session	
2:00pm ~ 3:00pm	Tour Genzyme Manufacturing Facility	
3:00pm ~ 4:15pm	Load Coach and Return to DoubleTree	
1:45pm ~ 4:15pm	Childcare/Young Adult Room OPEN ~ Newburyport ~	Volunteer Childcare/Young Adult Staff
1:45pm ~ 2:15pm	FDA Expanded Access (ie: i-IND) ~North Shore A & B	Kevin Bugin, FDA
2:15pm ~ 2:45pm	Clinical Trials: What you need to know, From the perspective of a parent, clinical investigator, clinician meetings/prep and advocacy group. ~ North Shore A & B	Parent ~ Harry Koujaian Physician ~ Dr. Elizabeth Berry-Kravis Clinician ~ Nicole Farhat Advocacy Group ~ Jean Campbell

24th Annual NNPDF Family Support and Medical Conference

Friday, August 12th, 2016 ~ continued ~

Time	Activity and Location	Attendees/Speakers/ Introductions
<p>More on the Open House Session I: 2:45 - 3:15 Session II: 3:15 - 3:45 Session III: 3:45 - 4:15</p> <p><u>OPEN HOUSE Overviews:</u></p> <p>Vtesse & Orphazyme & Galyatech Your opportunity to learn more about the work, goals and objectives of the different Pharma's.</p> <p>INPDA / International Registry Workshops: All Disease Types Stop by for a friendly "hands-on" step-by-step guided workshop to register your family/patient into the NEW International Niemann-Pick Disease Alliance (INPDA) International Patient Registry. I-pads and laptops will be available to assist you with the individual registration process. *Note: Galyatech & the FDA Expanded Access sessions will ONLY be offered on Friday afternoon.</p> <p>FDA Expanded Access</p>		
2:45pm ~ 4:15pm	OPEN HOUSE	
	Vtesse ~ Ipswich ~	
	Orphazyme ~ Gloucester A	
	Galyatech ~ Gloucester B ~	
	INPDA/Registry Training ~ Essex ~	
	FDA Expanded Access ~ Marblehead ~	
2:45pm ~ 3:15pm	For the Bereaved ~Maple Room	Rhonda Brown-Kehoe & Holly Roberts
2:45pm ~ 3:15pm	Enhancing the life of your young adult with NPC ~ Elm Room~	Ann Bennett
4:45pm ~ 5:00pm	Load Coach for Salem & Depart	
6:00pm ~ 7:25pm	Arrive in Salem & Get Dinner (on your own)	
7:25pm ~ 7:30pm	Head over to Hocus Pocus Tours	176 Essex Street
7:30pm ~ 9:00pm	TOUR with Susan Metzger of Hocus Pocus	
9:00pm	Load Coach to head back to DoubleTree	Coach will be at Liberty Street just off of Charter St. at the Salem Witch Trials Memorial
5:00pm ~ 9:00pm	Family Fun Night at CoCo Key Water Resort *Lifeguards on duty all evening *Various options for dinner & treats	*Purchase tickets directly from hotel *\$20 per person/per day (reg. \$45)

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Time	Activity and Location	Attendees/Speakers/ Introductions
6:30am ~ 9:30am	Breakfast/Grand Ballroom III & IV	All NNPDF Hotel Guests
7:00am ~ 8:45am	NNPDF Board of Directors Meeting ~ <i>Rockport</i> ~ CLOSED MEETING	NNPDF Board of Directors Dr. Andrew Lieberman, SAB Chair
9:00am ~ 5:00pm	Reflection/Bereavement ~ Maple ~ A quiet and private opportunity for families to gather, reflect and share.	Any interested conference attendee
8:00am ~ 9:00 am	Late Registration & Art Exhibit ~ <i>Living Room</i> ~	NNPDF Staff and Conference Volunteers
8:45am ~ 12:00pm	Childcare/Young Adult Activity Room Open ~ <i>Newburyport</i> ~	Volunteer Childcare/Young Adult Staff
8:30am ~ 10:00am	ASMD ~ Research Presentations ~ North Shore C	All ASMD families & guests
8:30am ~ 8:35am	Good Morning Greetings	Anne O'Connor-Smith, NNPDF Co-Treasurer
8:35am ~ 9:00am	2015/2016 ASMD Literature & Research	Dr. Edward Schuchman
9:00am ~ 9:20am	INPDA/Registry Updates	Jim Green, INPDA President Toni Mathieson, NP-UK Executive Director
9:20am ~ 9:50am	FDA Expanded Access	Kevin Bugin, FDA
9:50am ~ 10:00am	Updates on Wylder Nation	Steven Laffoon
9:00am ~ 10:00am	NPC ~ Research Presentations ~ North Shore A & B	All NPC families & guests
9:00am ~ 9:10am	Good Morning Greetings	Dr. Andy Lieberman, SAB Chair
9:10am ~ 9:35am	Characteristics of Liver Disease in NPC	Nicole Farhat, C.R.S.M.
9:35am ~ 9:55am	Research & Science behind Arimoclomol	Thomas Kirkegaard Jensen, Orphazyme
9:55am ~ 10:10am	BREAK ~ Living Room ~	
	Joint Sessions ~ ASMD & NPC ~North Shore A & B ~	
10:10am ~ 10:30am	Update on Therapies in the Cat	Dr. Charles Vite, University of PA
10:30am ~ 10:50am	Understanding and Treating Brain Pathology in NPD Type A	Dr. Lola Ledesma, Universidad Autonoma Nicolas

24th Annual NNPDF Family Support and Medical Conference⁷

Saturday, August 13th, 2016 ~ continued ~

Time	Activity and Location	Attendees/Speakers/ Introductions
	JOINT SESSIONS ~ ASMD & NPC ~North Shore A & B ~	
10:50am ~ 11:00am	Parkinson's Disease and SMPD1 Mutations in NPD Type A & B: Is there a link?	Adina Wise, Medical Student
11:00am ~ 11:20am	Newborn Screening Assay (NPC & ASMD)	Dr. Dan Ory, Washington University
11:20am ~ 11:40am	Genetic Aspects of NPC & ASMD	Dr. Simona Bianconi, NIH
11:40am ~ 12:00pm	Expert Panel ~ Joint Discussion (NPC & ASMD)	
12:00pm ~ 2:00pm	LUNCH ~Grand Ballroom III ~	
1:45am ~ 5:15pm	Childcare/Young Adult Activity Room Open ~ Newburyport ~	Volunteer Childcare/Young Adult Staff
	JOINT SESSIONS ~ ASMD & NPC ~North Shore A & B ~	
2:00pm ~ 2:30pm	Clinical Trials: What you need to know, From the perspective of a parent, clinical investigator, clinician meetings/prep and advocacy group. ~ North Shore A & B	Parent ~ Harry Koujaian Physician ~ Dr. Elizabeth Berry-Kravis Clinician ~ Nicole Farhat Advocacy Group ~ Jean Campbell
2:00pm ~ 2:30pm	Family Support Breakout Sessions	
	Newly Diagnosed Families ~Manchester~ For the Newly Diagnosed. All Types.	Missy Ward & Courtney Hopkin
	Bereaved Family Members ~ Maple ~ Man- aging the day -to-day grief and how to get through tough anniversary dates.	Rhonda Brown-Kehoe & Jill Flinton
	Extended Family & Friends ~ Aspen~ My family member/friend is facing the diagnosis of NPD..what can I do to help?	Andy Schroeder & Deb Heinze
2:30pm ~ 4:00pm	NPC/ASMD OPEN HOUSE	
	Vtesse ~ Ipswich ~	
	Orphazyme ~ Gloucester A ~	
	Genzyme ~ Gloucester B~	
	INPDA/Registry Training ~ Essex ~	
	Genetics Anyone? ~ Manchester ~	

24th Annual NNPfD Family Support and Medical Conference

Saturday, August 13th, 2016 ~ continued ~

Time	Activity and Location	Attendees/Speakers/ Introductions
<p>More on the Open House Session I: 2:30 - 3:00 Session II: 3:00 - 3:30 Session III: 3:30 - 4:00</p> <p>OPEN HOUSE Overviews: <i>Genzyme, Vtesse & Orphazyme</i></p> <p>INPDA / International Registry Workshops: All Disease Types Stop by for a friendly “hands-on” step-by-step guided workshop to register your family/patient into the NEW International Niemann-Pick Disease Alliance (INPDA) International Patient Registry. I-pads and laptops will be available to assist you with the individual registration process.</p> <p><u>Genetics Anyone?</u> Take this opportunity to meet and speak with our guest genetic counselor whose focus is on the NPD specific disease community and discuss the ways in which the new updates around screening and testing for NPD have changed.....how will that affect me, my family and my loved one diagnosed with NPD? Each of the sessions below will have an initial emphasis on the MAIN TOPIC item noted below ~ and ~ as well as general questions as they arise within the discussion time.</p> <p>Session I: 2:30pm – 3:00pm / Main Topic: For the Newly Diagnosed. Any and all questions welcome.</p> <p>Session II: 3:00 – 3:30pm / Main Topic: What does an NPD diagnosis mean for my extended family? How should I share this information and work to educate my extended family members?</p> <p>Session III: 3:30 – 4:00 Main Topic: My sibling has NPD. What does that mean for me? What about when I marry? What should I consider before having children?</p> <p>This session will be geared specifically to the NPD (all types) siblings who are in attendance at the conference. Specifically, would like to encourage the tweens of siblings who are at the conference. This genetic session will</p>		
4:00pm ~ 5:00pm	Family Support Breakouts	
	ASMD ~ Ipswich ~ The trials & tribulations of participating in a clinical trial.	Hosts: Anne O'Connor-Smith & Justin Hopkin
	Mom's Group ~ Georgetown ~	Hosts: Darrile Papier & Liz Heinze
	Dad's Group ~ Rockport ~	Hosts: Jim Ward & Mark Papier
	Young Adult/Late Onset NPC ~ Elm ~ How to navigate difficult family choices when your	Hosts: Holly Roberts & Ann Bennett
	Siblings of All Ages ~ Manchester ~ Finding support from those who walk in your shoes and know your journey.	Host: Serina Heinze
6:00pm ~ Midnight	Gala Extravaganza ~ North Shore Ballroom ~	All Conference Attendees
6:00pm ~ 11:00pm	Cash Bar	
7:15pm ~ 8:15 pm	Banquet Dinner	All Conference Attendees
8:00pm ~ Midnight	Conference Wrap-Up/Entertainment	All Conference Attendees
9:00am ~ 12:00noon	Sunday, August 14th, 2016 NNPfD Board Meeting ~ Rockport ~	

Safe travels home and we hope you enjoyed the conference!
See you next year!