

THURSDAY August 15, 2019 | NNPDF Family Advisory Working Groups

This session applies only to families who applied and pre-registered for the NNPDF Family Advisory Working Groups

TIME	SESSION	
9:00 AM - 9:45 AM	Family Advisory Working Groups Registration (invitation only)	
9:30 AM - 3:30 PM	Activity Zone for Working Group Participant families	
9:30 AM - 3:30 PM	Quiet Room	
10:00 - 11:30 AM	NPC Family Advisory Working Groups (invitation only)	
	NPC Family Advisory Working Group A	
	NPC Family Advisory Working Group B	
	NPC Family Advisory Working Group C	
11:30 AM - 12:45 PM	Family Advisory Working Group Lunch	
11:00 - 11:45 AM	ASMD Family Advisory Working Group Registration	
1:00 - 2:30 PM	ASMD Working Group (invitation only)	

THURSDAY August 15, 2019 | NNPDF Family Support & Medical Conference

The Woodlands Room will be available all weekend for those that need to take a moment

TIME	SESSION	
3:00 - 7:00 PM	NNPDF Family Support & Medical Conference Registration	
5:00 - 5:45 PM	First Time Attendees & Mentors	
5:45 - 6:45 PM	CONFERENCE BEGINS: DINNER & MINGLE	
6:30 - 6:45 PM	Opening Remarks	
6:45 - 9:00 PM	Activity Zone Open	
7:00 - 8:30 PM	Families-to-Families Break-out Groups	
	NPC Adult Onset	
	NPC Juvenile	
	ASMD	
	Siblings	
	Bereaved Families	
8:45 - 9:00 pm	Celebration of Hope	

FRIDAY MORNING August 16, 2019

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TIME	SESSION	
6:30 - 8:30 AM	Breakfast	
7:00 AM	Conference Registration	
7:30 - 8:30 AM	SAB meeting (invitation only)	
8:30 AM	Activity Zone Open	
9:00 - 11:00 AM	KEYNOTE: Legacy, Innovation, and Hope: Path of Drug Development to Market Patti Engel, President & CEO, Engage Health Inc. Moderator: Joslyn Crowe, NNPfD Executive Director	
10:00 - 11:00 AM	Hope: Facilitated Siblings Support Laurie Turner, Family Services Manager	
10:00 - 11:00 AM	Hope: Facilitated Young Adult Patient to Patient Support	
11:00 - 11:15 AM	Persevere Award	
11:15 - 11:45 AM	Free Time for Networking	
11:45 AM	Activity Zone Closed	
12:00 - 1:15 PM	Lunch	

FRIDAY AFTERNOON August 16, 2019

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TIME	SESSION	
1:15 PM	Activity Zone Open 2:00 pm DNA extraction science experiment	
1:30 - 3:00 PM	Innovation: NPC - FDA Approved Recruiting and Active Clinical Trials Moderators: Justin Hopkin, MD, NNPfD Board Chair Andrew Lieberman, MD, PhD., Professor, Department of Pathology, University of Michigan Medical School IB1001-201: Effects of N-Acetyl-L-Leucine on Niemann-Pick type C Disease (NPC): A multinational, multi-center, open-label, rater-blinded Phase II study Dr. Tatiana Bremova. MD, PhD, Department of Neurology, University Hospital Bern (video presentation) adrabetadex (VTS-270): Intrathecal adrabetadex For The Treatment Of Niemann-Pick Disease, Type C: Current Clinical Experience Elizabeth Berry-Kravis, MD, PhD, Rush University Medical Center	

<p>1:30 - 3:00 PM</p>	<p>arimoclomol: <i>arimoclomol Prospective Study in Patients Diagnosed with Niemann-Pick Disease Type C, Results of the Phase II/III Trial</i></p> <p>Marc Patterson, MD, Professor of Neurology, Pediatrics & Medical Genetics, Mayo Clinic, Principal Investigator of Phase II/III Trial with arimoclomol in NPC</p> <p>Trappsol® Cyclo™: <i>Update on CTD's Clinical Trials using Trappsol® Cyclo™ by Intravenous Administration in NPC Patients.</i></p> <p>Caroline Hastings, MD, Fellowship Director, Pediatric Hematology Oncology, Children's Hospital & Research Center Oakland</p>	
<p>1:30 - 2:15 PM</p>	<p>Innovation: ASMD - FDA Approved Clinical Trial</p> <p>Olipudase Alfa: <i>ASMD Update on Clinical Trials</i></p> <p>Melissa Wasserstein, MD, Chief, Division of Pediatric Genetic Medicine, The Children's Hospital at Montefiore</p> <p>Moderator: Edward Schuchman, PhD., Director, International Center for Types A and B, Niemann-Pick Disease Mount Sinai School of Medicine</p>	
<p>2:15 - 3:00 PM</p>	<p>Innovation: ASMD Biologics - Development and Manufacture of Therapeutic Proteins</p> <p>Claudia Buser, Ph.D., Sanofi Genzyme</p> <p>Moderator: Edward Schuchman, PhD., Director, International Center for Types A and B, Niemann-Pick Disease Mount Sinai School of Medicine</p>	
<p>3:00 - 3:15 PM</p>	<p>Break</p>	
<p>3:15 - 4:00 PM</p>	<p>NPC: FDA Approved Recruiting and Active Clinical Trials Panel Discussion and Q&A</p> <p><u>MODERATORS:</u> Justin Hopkin, MD, NNPDF Board Chair</p> <p>Andrew Lieberman, MD, PhD., Professor, Department of Pathology, University of Michigan Medical School</p> <p><u>PANELISTS:</u></p> <p>adrabetadex (VTS-270): Elizabeth Berry-Kravis, MD, PhD, Rush University Medical Center</p> <p>Susan Van Meter, Senior Director, Clinical Research, Autoimmune and Rare Disease, Mallinckrodt Pharmaceuticals</p> <p>arimoclomol: Marc Patterson, Professor of Neurology, Pediatrics & Medical Genetics, Mayo Clinic</p> <p>Paul Merrigan, President and Global Chief Commercial Officer, Orphazyme US, Inc.</p> <p>IB1001-201: Taylor Fields, Senior Vice President, IntraBio</p>	

	<p>Trappsol® Cyclo™: Caroline Hastings, MD, Fellowship Director, Pediatric Hematology Oncology, UCSF Benioff Children's Hospital & Research Center Oakland</p> <p>Sharon Hrynkow, PhD, Chief Scientific Officer and Senior Vice President for Medical Affairs at CTD Holdings, Inc.</p>	
3:15 - 4:00 PM	<p>ASMD: ASMD Open Discussion with Family Services Manager and INPDR Registration</p> <p>Laurie Turner, NNPfD Family Services Manager</p> <p>Jackie Imrie, MSc, International Niemann-Pick Disease Registry Consultant</p>	
4:00 - 4:10 PM	Break	
4:10 - 5:15 PM	<p>Hope: Navigating Insurance and Government Benefits</p> <p>Jennifer Shumsky, RN, BSN, CHCO, Director, Payor Relations, Little Hercules Foundation</p> <p>Moderator: Joslyn Crowe, MSW, NNPfD Executive Director</p>	
4:10 - 5:15 PM	<p>Legacy: Life After Loss Support Session <i>Led by a licensed social worker</i></p>	
5:30 PM	Activity Zone CLOSED	
5:30 PM	<p>Conference Program Ends for the Day Dinner on your own and evening to yourself</p>	

SATURDAY MORNING August 17, 2019

The Woodlands will be available all weekend for those that need to take a moment.

TIME	SESSION	
6:30 - 8:30 AM	Breakfast	
7:45 AM	<p>Activity Zone Open</p> <p>9:45am DNA extraction science experiments</p>	
8:00 - 8:30 AM	Hope: NPC Open Discussion with Family Services Manager	
8:30 - 9:00 am	<p>Welcome!</p> <p>Hope: INPDR Patient Registry & US Launch</p> <p>Justin Hopkin, MD, NNPfD Board Chair</p> <p>Jackie Imrie, MSc, International Niemann-Pick Disease Registry Consultant</p> <p>Moderator: Marc Patterson, MD. Professor of Neurology, Pediatrics & Medical Genetics, Mayo Clinic</p>	

9:00 - 9:45 am	<p>Hope: Newborn Screening Allison Rosen, 3D Communications for Firefly Fund</p> <p>Melissa Wasserstein, MD, Chief, Division of Pediatric Genetic Medicine, The Children's Hospital at Montefiore</p> <p>Moderator: Justin Hopkin, MD, NNPfD Board Chair</p>	
9:45 - 10:00 AM	Break	
10:00 - 11:45 AM	<p>Innovation: Future Developments in Niemann-Pick Disease</p> <p>Moderator: Andrew Lieberman, MD, PhD., Professor, Department of Pathology, University of Michigan Medical School</p> <p><i>"Use of Efavirenz to treat brain pathology in Niemann-Pick Type C"</i> Lola Ledesma, PhD, Centro Biologia Molecular Severo Ochoa, Madrid, Spain</p> <p><i>"Enhancing the endocannabinoid system to treat brain pathology in Niemann-Pick Type A "</i> Lola Ledesma, PhD, Centro Biologia Molecular Severo Ochoa, Madrid, Spain</p> <p>Melissa Wasserstein, MD, Chief, Division of Pediatric Genetic Medicine, The Children's Hospital at Montefiore</p> <p><i>"Improved disease amelioration with combination therapy for Niemann-Pick disease type C1"</i> Cristin Davidson, PhD., Researcher, NHGRI / Project Manager, SOAR-NPC</p> <p><i>"Managing geletic cataplexy in NPC"</i> Suresh Kotagal, MD, Professor of Neurology, Mayo Clinic College of Medicine and Science (By video presentation)</p>	
11:45 AM	Activity Zone Closed	
12:00 - 1:00 PM	Lunch	

SATURDAY AFTERNOON August 17, 2019

The Meadows will be available all weekend for those that need to take a moment.

TIME	SESSION	
1:00 PM	Activity Zone Open	
1:15 - 2:15 PM	<p>NNPDF Fellows Scientific Updates</p> <p>Moderator: Andrew Lieberman, MD, PhD., Professor, Department of Pathology, University of Michigan Medical School</p> <p><i>"Searching for the genes that delay the onset of Niemann-Pick type C disease until adulthood: an update from a previous NNPfD Fellow"</i> Andrew Munkacsj, PhD, Victoria University of Wellington School of Biological Sciences, New Zealand</p>	

1:15 - 2:15 PM	<i>“Investigating the modulation of the E3 ligase CHIP in Niemann-Pick disease type C”</i> Adam Kanack, Medical College of Wisconsin, Biochemistry Department	
1:15 - 2:15 PM	Anticipation & Navigating Grief <i>Led by a licensed social worker</i>	
1:15 - 2:15 PM	Hope: DROP IN Discussions with Laurie Turner <i>ALL FAMILIES WELCOME AT ANY STAGE OF THE NPD JOURNEY</i>	
2:15 - 2:30 PM	Break	
2:30 - 4:00 PM	Legacy: Family Support Break Out Groups	
	Moms of All Diseases	
	Dads of All Diseases	
	Teen & Adult Patients	
	Siblings Conversation (ages 12 and older)	
4:00 PM	End of Conference Program	
4:15 PM	Activity Zone CLOSED	
6:00 - Midnight	2019 Family Conference Gala <i>Endurance Award</i> Introduced by Missy and Jim Ward	