WORLD*Symposium*™ 2027 Preliminary Program*
Platform Presenters

Sunday, January 31, 2027: Patient Voice 2027,
WORLD*Symposium* Connections and Be the Catalyst Event

15:00	**The Patient Voice 2027** CE Session Accredited provider: Haymarket Medical Education (HME)	Small Numbers, Big Impact: Using Real-World Experience and Patient Insights to Create Standards of Care *This activity is supported, in part, by an educational grant from Shionogi Inc.*
16:30	**Industry Expert Theater** Sponsored by Azafaros B.V.	From pathway to patient: What matters in neurological lysosomal diseases
17:45	**WORLD*Symposium* Connections Program**	Check-in and Introductions
18:00	**Be the Catalyst Event**	4th Annual Catalyst Award presented to Karen Pignet

Basic Science, Translational Research, and Clinical Application Sessions are available for CME/CNE/CEU credits. Contemporary Forum and Late-Breaking Science Sessions are not available for CME/CNE credits, however CEUs for GCs may apply.

WORLD*Symposium*™ 2027 Preliminary Program*
Platform Presenters

Monday, February 1, 2027: Basic Science
Co-Chairs: Lalitha Belur, Michael Przybilla, Danilo Tagle

06:45	**Non-CE Satellite Symposium** Sponsored by Amicus Therapeutics	Let's step outside together – Exploring fresh perspectives for people living with LOPD
06:45	**Non-CE Satellite Symposium** Sponsored by Sanofi	Learn About Expanding Your Care Options in Gaucher Type 3
08:00	**Chester B. Whitley** University of Minnesota Minneapolis, MN, United States	Welcome and Announcements Presentation of 2027 Roscoe O. Brady Award to Cynthia J. Tifft
	Cynthia J. Tifft National Institutes of Health Bethesda, MD, United States	Roscoe O. Brady Award Presentation *To Everything There Is a Season*
08:30	**Patricia I. Dickson** Washington University in St. Louis St. Louis, MO, United States	Substrate reduction therapy using siRNA targeting *Ext1* and *Ext2* reduces lysosomal heparan sulfate burden in brain of Sanfilippo syndrome type B mice
	Brian Bigger University of Edinburgh Edinburgh, United Kingdom	Heparan sulphate binding peptides, fused to iduronate-2-sulfatase, correct a mouse model of Hunter syndrome
	Angela Johana Espejo Pontificia Universidad Javeriana Bogotá, Colombia	Preclinical evaluation of a yeast-derived recombinant iduronate-2-sulfatase as a novel enzyme replacement therapy candidate for mucopolysaccharidosis type II
	Betul Celik Nemours Children's Health System Wilmington, DE, United States	*Ex vivo* lentiviral engineering of rapidly expanding MSCs enhances GALNS expression, preserves chondrogenic function, and supports HSC recovery in MPS IVA
	Moderated Q&A	*Dickson, Bigger, Espejo, Celik*
09:30	**Mark L. Schultz** University of Iowa Iowa City, IA, United States	Codon-optimized *Npc1* mRNA corrects Niemann-Pick type C1 disease phenotypes *in vitro* and *in vivo*
	Matthias Dierick University of Edinburgh Edinburgh, United Kingdom	A deimmunised α-galactosidase A as a next-generation enzyme replacement therapy for Fabry disease
	Ying Sun Cincinnati Children's Hospital Medical Center Cincinnati, OH, United States	Prosaposin gene therapy ameliorates glycosphingolipid storage, neuropathology, and survival deficits in a mouse model of prosaposin deficiency
	Shih-Hsin Kan Rady Children's Health Orange, CA, United States	Single-vector AAV delivery enables homology-directed recombination-mediated genome editing for Pompe disease
	Moderated Q&A	*Schultz, Dierick, Sun, Kan*
10:30	**Break**	
11:00	**Simona Murko** University Medical Center Hamburg-Eppendorf Hamburg, Germany	A novel biomarker for newborn screening of alpha-mannosidosis
	Virginia Kimonis University of California Irvine Irvine, CA, United States	Correction of β-mannosidosis with CNS-directed transferrin receptor-targeted enzyme replacement therapy in a *Manba*$^{-/-}$ mouse model
	Livia Sertori Finoti Children's Hospital of Philadelphia Philadelphia, PA, United States	Microglia co-culture reduces neuronal glycosaminoglycan accumulation in a human model of multiple sulfatase deficiency

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Monday, February 1, 2027: Basic Science *(cont.)*
Co-Chairs: Lalitha Belur, Michael Przybilla, Danilo Tagle

	Sarah Berger University Medical Center Göttingen Göttingen, Germany	Comprehensive analysis of brain MRI findings reveals distinctive features in multiple sulfatase deficiency **2027 Young Investigator Award Recipient*
	Moderated Q&A	*Murko, Kimonis, Sertori Finoti, Berger*
12:00	**Break and Satellite Symposia**	
12:15	**Non-CE Satellite Symposium** Sponsored by Amicus Therapeutics	Looking deeper, acting sooner – thinking management in Fabry Disease
12:15	**Non-CE Satellite Symposium** Sponsored by Denali Therapeutics	From Measures to Meaning: Clinical Interpretation of Neurocognitive Outcomes in MPS II
13:30	**Shih-Chang Hsueh** Columbia University Medical Center New York, NY, United States	Enhancing binding affinity of psychosine to α-cyclodextrin and evidence of prenatal efficacy in the murine model of Krabbe disease
	Matthieu Colpaert University of Florida Gainesville, FL, United States	Beyond glycogen: Tissue-specific secondary metabolic alterations in the brain and muscle of a Pompe disease mouse model
	Magnar Bjørås Norwegian University of Science and Technology Trondheim, Norway	Nizubaglustat improves disease-associated features in patient-derived retinal organoids modeling CLN3 Batten disease
	Mark Sands Washington University St. Louis, MO, United States	Convergent human and cross-species evidence associates lysosomal enzyme haploinsufficiency with Alzheimer's disease
	Moderated Q&A	*Hsueh, Colpaert, Bjørås, Sands*
14:30	**Veronica Lentini** University College London London, United Kingdom	Constitutive lysosomal trafficking defects and stimulus-dependent autophagic and secretory dysfunction in Gaucher disease macrophages **2027 Young Investigator Award Recipient*
	Maria Fuller Adelaide University Adelaide, Australia	Inhibition of lysosomal beta glucocerebrosidase activity promotes *de novo* sphingolipid synthesis
	Naomi Fisher National Institutes of Health Bethesda, MD, United States	High throughput image-based CRISPR screening identifies *KDSR* as a modifier of glucocerebrosidase protein level and enzyme activity in SH-SY5Y cells **2027 Young Investigator Award Recipient*
	Shiny Nair Yale University New Haven, CT, United States	A circulating CD63-GPNMB-TREM2-CCL18 module linked to lyso-GL1 and treatment in Gaucher disease
	Moderated Q&A	*Lentini, Fuller, Fisher, Nair*
15:30	**Poster Session I**	Exhibits Open
15:45	**Industry Expert Theater** Sponsored by Denali Therapeutics	
17:45	**Non-CE Satellite Symposium** Sponsored by Chiesi Global Rare Diseases	When AI Meets Clinical Judgment: Navigating AI-Informed Conversations with Fabry Patients
17:45	**Non-CE Satellite Symposium** Sponsored by Sanofi	GD3: CNS Burden — What to Measure, How to Measure It

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Tuesday, February 2, 2027: Translational Research
Co-Chairs: Tierra Bobo, PJ Brooks, Francyne Kubaski

06:45	**Non-CE Satellite Symposium** Sponsored by Spruce Biosciences, Inc.	MPS IIIB: New insights into natural history and evolving therapeutic landscape
06:45	**Non-CE Satellite Symposium** Sponsored by Sanofi	Immunogenicity in ERT: Untangling Challenges Across LDs
08:00	**Chester B. Whitley** University of Minnesota Minneapolis, MN, United States	Welcome and Announcements Presentation of 2027 Patient Advocate Leader (PAL) Award to Daniel Lewi and 2027 Young Investigator Awards Presentation
08:30	**Chester B. Whitley** University of Minnesota Minneapolis, MN, United States	PS gene-editing of brain-penetrant synthetic hexosaminidase reduces brain gangliosides in GM2-gangliosidosis (Tay-Sachs and Sandhoff diseases)
	Claudia Beatriz Del Toro Runzer Stanford University Stanford, CA, United States	Preclinical safety, efficacy, and GMP-scale manufacture of CRISPR/Cas9-edited stem cells for Gaucher disease type 1 **2027 Young Investigator Award Recipient*
	Barbara S. Perez University of California, San Francisco San Francisco, CA, United States	An *in vitro* pipeline for developing patient-specific base editing strategies for patients with lysosomal disorders
	Marissa Cabay Foundation for the National Institutes of Health North Bethesda, MD, United States	Accelerating gene therapy development for ultra-rare diseases: The Accelerating Medicines Partnership Bespoke Gene Therapy Consortium regulatory playbook
	Moderated Q&A	*Whitley, Del Toro Runzer, Perez, Cabay*
09:30	**Amali Karunathilaka** Nemours Children's Hospital Wilmington, DE, United States	Maternal AAV-mediated prenatal delivery of GALNS ameliorates early growth plate pathology in MPS IVA mice **2027 Young Investigator Award Recipient*
	Xin Chen University of Texas Southwestern Medical Center Dallas, TX, United States	Favorable efficacy and safety in preclinical studies pave the way for AAV9/HGSNAT gene therapy for MPS IIIC
	Grace Kick Washington University in St. Louis St. Louis, MO, United States	Combined CNS directed and systemic gene therapy treats both the brain and bowel dramatically extending the lifespan of a CLN2 mouse model
	Hongling Zhu National Institutes of Health Bethesda, MD, United States	Pronounced therapeutic benefit of liver-directed AAV gene therapy for CNS delivery of β-hexosaminidase A in Sandhoff disease
	Moderated Q&A	*Karunathilaka, Chen, Kick, Zhu*
10:30	**Break and Exhibits**	
11:00	**Julia O. De la Garza** McGill University Montreal, QC, Canada	HSPC transplantation ameliorates disease presentation in sialidosis type 2 patient and a mouse model **2027 Young Investigator Award Recipient*
	Longguo Zhang McGill University Montreal, QC, Canada	Lentiviral stem cell gene therapy shows efficacy in MPS IIIC, caused by defects in a lysosomal membrane protein
	Valentina Poletti University of Padova Padova, Italy	A hyperactive human beta-galactosidase for gene therapy of GM1 gangliosidosis and related diseases **2027 Young Investigator Award Recipient*

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Tuesday, February 2, 2027: Translational Research *(cont.)*
Co-Chairs: Tierra Bobo, PJ Brooks, Francyne Kubaski

	Isidro Arévalo-Vargas Fundación Española para el Estudio y Terapéutica de la Enfermedad de Gaucher y Otras Lisosomales Zaragoza, Spain	Pilot selection of in silico predictors for intronic variants in genes associated with lysosomal disorders **2027 Young Investigator Award Recipient*
	Moderated Q&A	*De la Garza, Zhang, Poletti, Arévalo-Vargas*
12:00	**Break, Exhibits and Satellite Symposia**	
12:15	**Non-CE Satellite Symposium** Sponsored by Chiesi Global Rare Diseases	Pegunigalsidase alfa: evolving evidence in Fabry disease *This satellite is open only to registered attendees from outside the United States. International participants only.*
12:15	**Non-CE Satellite Symposium** Sponsored by Sanofi	From First Steps to Next-Gen: Redesigning the Road to Approval in IOPD
13:30	**Wendy E. Heywood** University College London London, United Kingdom	Plasma NULISA proteomics identifies disease-specific signatures and a shared neurodegenerative profile across lysosomal disorders
	Xinying Hong Children's Hospital of Philadelphia Philadelphia, PA, United States	Biochemical characterization of metachromatic leukodystrophy by integrated LC-MS/MS profiling of arylsulfatase A activity and sulfatides
	Raghu Rai Sharma University of Minnesota Minneapolis, MN, United States	Extracellular vesicle profiling in individuals carrying *GBA1* variants: A biomarker discovery approach
	Karolina M. Stepien Salford Royal Hospital Salford, United Kingdom	Lipidomics analysis: A novel approach to identify patients with lysosomal disease–preliminary results from one metabolic centre
	Moderated Q&A	*Heywood, Hong, Sharma, Stepien*
14:30	**Paul Harmatz** UCSF Benioff Children's Hospital Oakland, CA, United States	Tralesinidase alfa treatment is associated with sustained reductions in neurodegeneration biomarkers in mucopolysaccharidosis type IIIB: Long-term data
	Peter Meinke Ludwig-Maximilians-Universität München Munich, Germany	Direct comparison of enzyme replacement therapies in patient-derived late-onset Pompe disease myoblasts reveals a modifiable glycogen clearance bottleneck
	Jessica Gambardella Federico II University of Naples Naples, Italy	Hypertension unmasks bioenergetic vulnerability, driving exaggerated cardiac hypertrophy in Fabry disease **2027 Young Investigator Award Recipient*
	Nishitha R. Pillai University of Minnesota Minneapolis, MN, United States	Untargeted metabolomics identifies metabolic signatures of early disease activity and treatment response in late-onset Pompe disease
	Moderated Q&A	*Harmatz, Meinke, Gambardella, Pillai*
15:30	**Poster Session II**	Exhibit Hall
15:45	**Industry Expert Theater** Sponsored by BioMarin	The Diagnostic Journey: Expert Panel Discussion on Early LD Diagnosis and Improved Outcome
17:45	**Non-CE Satellite Symposium** Sponsored by Lysosomal & Rare Disorders Research & Treatment Center (LDRTC)	Neurodegeneration vs. Neuroinflammation in Lysosomal Diseases: Pathophysiology, Biomarkers, Clinical Assessment, and Treatment Paradigms
17:45	**Non-CE Satellite Symposium** Sponsored by Ultragenyx Pharmaceutical	Changing the Course: A New Era in MPS IIIA

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Wednesday, February 3, 2027: Clinical Applications
Co-Chairs: Roberto Giugliani, Cynthia Tifft, Filippo Vairo

06:45	**Non-CE Satellite Symposium** Sponsored by Beren Therapeutics P.B.C.	A Newly approved therapy for I-NPC
06:45	**Non-CE Satellite Symposium** Sponsored by Takeda Pharmaceutical Company Limited	Redefining disease trajectories: Enhancing patient monitoring to support timely intervention in lysosomal disorders
08:00	**Chester B. Whitley** University of Minnesota Minneapolis, MN, United States	Welcome and New Treatment Awards
08:30	**Elizabeth Berry-Kravis** Rush University Medical Center Chicago, IL, United States	Intrathecal adrabetadex slows disease progression in participants with infantile-onset Niemann-Pick disease type C
	Caroline Aimee Hastings UCSF Benioff Children's Hospital Oakland Oakland, CA, United States	Intravenous hydroxy-propyl-beta-cyclodextrin (IV-HPβCD) demonstrates clinically meaningful results in Niemann-Pick disease type C (NPC): Results from a randomized double-blind placebo controlled, phase 3 trial, TransportNPC
	Orna Staretz-Chacham Soroka Medical Center Be'er Sheva, Israel	Improved overall survival in infantile-onset Niemann-Pick disease type C treated with intravenous hydroxy-propyl-beta-cyclodextrin (IV-HPβCD) in the CTD-TCNPC-301 and CTD-TCNPC-201 trials
	Adam P. Vogel The University of Melbourne Melbourne, Australia	Acoustic, perceptual, and neural signatures of dysarthria in Niemann-Pick disease type C and Pompe disease
	Moderated Q&A	*Berry-Kravis, Hastings, Staretz-Chacham, Vogel*
09:30	**Jessica Doxey** Duke University Durham, NC, United States	Characterizing the early phenotype of late-onset Pompe disease: Insights from longitudinal follow-up of a cohort identified by newborn screening
	Antonio Toscano University of Messina Messina, Italy	Improvement in outcomes following switch from alglucosidase alfa to avalglucosidase alfa in late-onset Pompe disease: Pompe Registry analysis
	Hani Kushlaf University of Cincinnati Cincinnati, OH, United States	Improvements in disease trajectory after switching from alglucosidase alfa to cipaglucosidase alfa plus miglustat: A pre- and post-baseline slope analysis of patients with late-onset Pompe disease in ATB200-02 and PROPEL/PROPEL OLE
	Priya S. Kishnani Duke University Durham, NC, United States	Improved clinical outcomes and safety with avalglucosidase alfa versus alglucosidase alfa in infantile-onset Pompe disease: The Baby-COMET external comparator arm analysis
	Moderated Q&A	*Doxey, Toscano, Kushlaf, Kishnani*
10:30	**Break and Exhibits**	
11:00	**Peter Nordbeck** University of Würzburg Würzburg, Germany	Lucerastat effect on cardiac function in patients with Fabry disease: Results from the open label-extension (OLE) of the phase 3 clinical program
	Mitra Tavakoli University of Exeter Medical School Exeter, United Kingdom	Investigating small fiber neuropathy and pain in Fabry disease: A 24-month study of corneal nerve, immune cell and treatment-related changes
	Ozlem Goker-Alpan Lysosomal & Rare Disorders Research & Treatment Center, Inc Fairfax, VA, United States	Interim results from a phase 1/2 trial of HM15421/GC1134A, a novel, monthly, subcutaneous enzyme replacement therapy for the treatment of Fabry disease

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Wednesday, February 3, 2027: Clinical Applications *(cont.)*
Co-Chairs: Roberto Giugliani, Cynthia Tifft, Filippo Vairo

	Kristina L. Elvidge Childhood Dementia Initiative Brookvale, Australia	Uniting lysosomal and other causes of childhood dementia under a shared definition: A global Delphi consensus
	Moderated Q&A	*Nordbeck, Tavakoli, Goker-Alpan, Elvidge*
12:00	**Break, Exhibits and Satellite Symposia**	
12:15	**Non-CE Satellite Symposium** Sponsored by Chiesi Global Rare Diseases	Shaping the Future for Lysosomal Diseases with CNS Involvement
12:15	**Non-CE Satellite Symposium** Sponsored by Sanofi	What We Have Learned From Over 20 Years of Agalsidase-Beta: A Brief Perspective
13:30	**Susan E. Waisbren** Boston Children's Hospital Boston, MA, United States	Adaptive functioning outcomes in mucopolysaccharidosis type IIIB patients receiving tralesinidase alfa enzyme replacement therapy
	Cara O'Neill Cure Sanfilippo Foundation Columbia, SC, United States	Quality of life and family impact in severe Sanfilippo syndrome type B (MPS IIIB): Longitudinal findings from a prospective natural history study
	Nicole M. Muschol University Medical Center Hamburg-Eppendorf Hamburg, Germany	Long-term outcomes of tralesinidase alfa enzyme replacement therapy in patients with mucopolysaccharidosis type IIIB
	Elizabeth Jalazo University of North Carolina Chapel Hill, NC, United States	Interim results from the phase 1/2, first-in-human, open-label study of zafinofusp alfa in children with mucopolysaccharidosis type IIIA (MPS IIIA)
	Moderated Q&A	*Waisbren, O'Neill, Muschol, Jalazo*
14:30	**Pramod K. Mistry** Yale University New Haven, CT, United States	Venglustat demonstrates consistent clinical benefits in neurological manifestations of Gaucher disease type 3 (GD3) in the LEAP2MONO study
	Sara Mitchell Lysosomal & Rare Disorders Research & Treatment Center Fairfax, VA, United States	An AI-driven multilayer model of lung involvement in neuronopathic Gaucher disease (nGD): Validation in a 45-patient cohort and implications for management
	Barbara C. Soberon Hospital de Ninos Ricardo Gutierrez Caba, Argentina	Gaucher disease type 1 (GD1): Frequency of osteonecrosis in the pediatric age. A study of the Argentine Group for the Diagnosis and Treatment of Gaucher Disease (GADTEG)
	Roberto Giugliani Federal University of Rio Grande do Sul Porto Alegre, Brazil	Long-term effect of nizubaglustat on seizure frequency in GM2 gangliosidoses: End-of-study data from the phase 2 RAINBOW study
	Moderated Q&A	*Mistry, Mitchell, Soberon, Giugliani*
15:30	**Poster Session III**	Exhibit Hall
15:45	**Industry Expert Theater** Sponsored by Chiesi Global Rare Diseases	Improving lysosomal disorder diagnosis: Progress, challenges, and patient community perspectives
17:45	**6th Annual Robert J. Gorlin Symposium**	AI in Our Lysosomal World

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Thursday, February 4, 2027: Contemporary Forum and Late-Breaking Science
Co-Chairs: Maria Fuller, Marc Patterson, Uma Ramaswami

06:45	**CE Satellite Symposium** Accredited provider: Physicians' Education Resource, LLC Supported by an independent educational grant from Beren Therapeutics, P.B.C	Changing the Course of Niemann-Pick Disease Type C – From Earlier Diagnosis to Long-Term Management
06:45	**CE Satellite Symposium** Accredited provider: Answers in CME Supported by an educational grant from Takeda Pharmaceuticals U.S.A., Inc.	Turning Points in Gaucher Disease: Navigating Women's Health Across the Lifespan
08:00	**Peter Marks** Eli Lilly and Company Washington DC, United States	Welcome and Keynote Forum Introduction
	Rebecca Ahrens-Nicklas The Children's Hospital of Philadelphia Philadelphia, PA, United States **Kiran Musunuru** University of Pennsylvania Perelman School of Medicine Philadelphia, PA, United States	Keynote Forum Beyond One Patient: The Future of Personalized Gene Therapies for Lysosomal and Rare Diseases *Moderated by Peter Marks*
08:45	**Xuling Zhu** Addition Therapeutics South San Francisco, CA, United States	Preclinical proof-of-concept of treatment for Fabry disease via precise RNA-mediated insertion of a *GLA* transgene
	Baodong Sun Duke University Durham, NC, United States	Retargeting the cell surface receptor with an engineered IgF2-fusion platform enhances therapeutic enzyme delivery to skeletal muscle in Pompe disease
	Heather Ann Lau Yale University Weston, CT, United States	Rebisufligene etisparvovec (UX111) led to long-lasting improvements in neurodevelopmental function in children with MPS IIIA
	Marc C. Patterson IntraBio Inc. Austin, TX, United States	Long-term efficacy of levacetylleucine at 36 months in Niemann-Pick disease type C: Subdomain analysis of the 5-domain NPC-CSS in adult and pediatric participants
	Moderated Q&A	*Zhu, Sun, Lau, Patterson*
09:45	**Michael W. Lawlor** Medical College of Wisconsin Milwaukee, WI, United States	Muscle histopathology following AT845 gene replacement therapy in adults with late-onset Pompe disease: Findings from the phase 1/2 FORTIS trial
	Late-Breaking Abstracts	3 Speakers To Be Announced (TBA) with Moderated Q&A
12:15	**Break**	

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Thursday, February 4, 2027: Rapid Fire Competition
Co-Chairs: Maria Fuller, Marc Patterson, Uma Ramaswami

13:15	**Jose Victor Alvarez Gonzalez** Instituto de Investigacion Sanitaria de Santiago de Compostela Santiago de Compostela, Spain	Complex vectors, enzymatic encapsulation, dairy-based nanoparticles
13:25	**Slawomir Wantuch** Orchard Therapeutics London, United Kingdom	Transplantation of hematopoietic stem cells expressing an IgF2-tagged GAA leads to full phenotypic correction and reversal of established disease in the Pompe mouse model
13:35	**Evan W. Kleinboehl** University of Minnesota Minneapolis, MN, United States	Translational development of engineered T cell micropharmacies for sustained enzyme delivery in lysosomal disorders **2027 Young Investigator Award Recipient*
13:45	**Ellen B. Fung** University of California, San Francisco Oakland, CA, United States	Effects of 72 weeks of vosoritide on growth velocity and body proportions in children with MPS IVA and VI: Interim results
13:55	**Raquel van Gool** Boston Children's Hospital Boston, MA, United States	Mapping the disease network in Niemann-Pick type C: Biomarkers across brain structure, function, and muscle
14:05	**Justin Hopkin** University of Rochester Rochester, NY, United States	Report of N-acetyl-L-leucine (NALL) for the neurologic manifestations of ASMD
14:15	**John Warren Collyer** Children's Hospital of Pittsburgh Pittsburgh, PA, United States	Longitudinal evaluation of spinal pathology through magnetic resonance imaging in mucopolysaccharidosis type II: A ten-year cohort study
14:25	**Angela Schulz** University Medical Center Hamburg-Eppendorf Hamburg, Germany	Cerliponase alfa for the treatment of CLN2 disease: Combined results from two ongoing observational studies
14:35	**Amy White** Mayo Clinic Rochester, MN, United States	Newborn screening can improve clinical outcomes for late-infantile Krabbe disease
14:45	**Paul J. Orchard** University of Minnesota Minneapolis, MN, United States	Extended laronidase enzyme activity after hematopoietic cell transplantation in children with Hurler syndrome
14:55	**Motomichi Kosuga** National Center for Child Health and Development Tokyo, Japan	Efficacy and safety of combined hematopoietic stem cell transplantation and intraventricular enzyme replacement therapy for severe mucopolysaccharidosis type II
15:05	**Rapid Fire Abstract**	1 Speaker To Be Announced (TBA)
15:15	**Rapid Fire Abstract Competition Voting**	Voting and Awards
15:25	**WORLD*Symposium* 2027 Adjourns**	

**The Preliminary Program is subject to change without notice. Any updates to the program will be posted on the website: worldsymposia.org*

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