

WORLDSymposium™ 2026 Preliminary Program*
Platform Presenters



Monday, February 2, 2026: Patient Voice 2026, Speed Mentoring and Be the Catalyst Event

15:00	The Patient Voice 2026 CE Session Accredited provider: Medical Education Resources (MER)	Why Does Publish or Perish Apply to Patient Advocate Groups? <i>This activity is supported in part by educational grants from JCR Pharmaceuticals and Ultragenyx Pharmaceutical Inc.</i>
16:30	Speed Mentoring Event	
16:00	Industry Expert Theater Sponsored by NS Pharma	Panel Discussion: Challenges and new opportunities in MPS II
18:00	Be the Catalyst Event	3rd Annual Catalyst Award presented to Professor Michael H. Gelb

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Tuesday, February 3, 2026: Basic Science

Co-Chairs: Lalitha Belur, Michael Przybilla, Dan Tagle

06:45	Non-CE Satellite Symposium Sponsored by Amicus Therapeutics	Transforming Ambiguity to Action for Personalized Fabry Care
06:45	Non-CE Satellite Symposium Sponsored by Takeda Pharmaceutical Company Limited	The maturation of evidence: what time teaches us about MPS II
08:00	Chester B. Whitley University of Minnesota Minneapolis, MN, United States	Welcome and Announcements Presentation of 2026 Roscoe O. Brady Award to Peter Marks
	Peter Marks Eli Lilly and Company Washington DC, United States	Roscoe O. Brady Award Presentation <i>Accelerating the Global Pace of Progress for Rare Diseases</i>
08:30	Elizabeth Braunlin University of Minnesota Minneapolis, MN, United States	Creation and characterization of a large animal model of MPS IVA
	Angela Gritti Università Vita-Salute San Raffaele Milano, Italy	Therapeutic benefits of hematopoietic stem cell gene therapy using optimized bicistronic lentiviral vectors in mouse models of GM2 gangliosidosis
	Ewa A. Ziolkowska Washington University St. Louis St. Louis, MO, United States	Neuropathological alterations in TPP1-deficient cynomolgus macaques recapitulate key features of human CLN2 disease
	Martyna Kasprzyk University of Edinburgh Edinburgh, United Kingdom	Production and characterisation of a functional recombinant human palmitoyl-protein thioesterase 1 in <i>K. phaffii</i> for the treatment of CLN1
09:18	Moderated Q&A	<i>Braunlin, Gritti, Ziolkowska, Kasprzyk</i>
09:30	Shiny Nair Yale University New Haven, CT, United States	Spatially resolved mechanisms of liver carcinogenesis in Gaucher disease implicate lipid-driven immunosuppressive niches.
	Xiangli Zhao Yale University New Haven, CT, United States	A progranulin derivative blocks the C5a/C5aR1 signaling and mitigates pathology in Gaucher disease
	Ying Sun Cincinnati Children's Hospital Medical Center Cincinnati, OH, United States	Brain delivery of long-acting enzymes via SapC-DOPS nanocarrier for neuronopathic Gaucher disease
	Pau Sarlé Vallés Universitat Autònoma de Barcelona Bellaterra, Spain	Next-generation enzyme replacement strategies for Gaucher disease <i>*2026 Young Investigator Award Recipient</i>
10:18	Moderated Q&A	<i>Nair, Zhao, Sun, Sarlé Vallés</i>
10:30	Break	
11:00	Matthias Dierick University of Edinburgh Edinburgh, United Kingdom	Engineering low immunogenic enzyme replacement therapies for Fabry disease <i>*2026 Young Investigator Award Recipient</i>

	Stephanie Tannous University of Veterinary Medicine Hannover Hannover, Germany	Lipid-dependent uptake of pegunigalsidase alfa across cell types: Implications for multiorgan treatment
	David Dmitrivich Smerkous University of Washington Seattle, WA, United States	A novel highly sensitive machine learning model for automated measurement of kidney peritubular capillary endothelial cell globotriaosylceramide accumulation
	Jessica Doxey Duke University Durham, NC, United States	The important role of CRIM analysis by western blot in the setting of novel GAA variants
11:48	Moderated Q&A	<i>Dierick, Tannous, Smerkous, Doxey</i>
12:00	Break and Satellite Symposia	
12:15	Non-CE Satellite Symposium Sponsored by Sanofi	Evolving Landscape of LDs in Era of Newborn Screening: Monitoring and Treatment Initiation.
12:15	Non-CE Satellite Symposium Sponsored by Takeda Pharmaceutical Company Limited	Optimizing lifelong therapy in Fabry disease: navigating complexity and maximizing impact
13:30	Maria Athanasopoulos McMaster University Hamilton, ON, Canada	Decoding NEU1 - a molecular switch linking sialidosis, hepatic receptor regulation, and atherosclerosis <i>*2026 Young Investigator Award Recipient</i>
	Mahin Hossain National Human Genome Research Institute, National Institutes of Health Bethesda, MD, United States	Glial cell dysfunction and neurodegeneration in a novel knock-in mouse model of lysosomal free sialic acid storage disorder <i>*2026 Young Investigator Award Recipient</i>
	Jordi Diaz-Manera Newcastle University Newcastle Upon Tyne, United Kingdom	Cartography of transcriptomic changes in muscle biopsies of patients with late-onset Pompe disease
	Shih-Chang Hsueh Columbia University New York, NY, United States	Novel cyclodextrins enhance potency of neutralization and clearance of endogenous psychosine in cellular and <i>in vivo</i> models of Krabbe disease
14:18	Moderated Q&A	<i>Athanasopoulos, Hossain, Diaz-Manera, Hsueh</i>
14:30	Katia Alileche Universite Paul Sabatier Toulouse, France	Investigating microglial heterogeneity in a mouse model of mucopolysaccharidosis type IIIB
	Richard Steet Greenwood Genetic Center Greenwood, SC, United States	MPS I missense variants that cause a gain of glycosylation effect are amenable to treatment with novel glycosylation inhibitors
	Ibrar Siddique University of California, Los Angeles Los Angeles, CA, United States	Blood-based biomarkers for mucopolysaccharidosis type I and III
	Keerthana Iyer University of Pennsylvania Philadelphia, PA, United States	Comparative proteomic analysis of saliva, urine, and serum in mucopolysaccharidosis type I patients

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Co-Chairs: Lalitha Belur, Michael Przybilla, Dan Tagle

15:18	Moderated Q&A	<i>Alileche, Steet, Siddique, Iyer</i>
15:30	Basic Science Poster Session	
15:45	Industry Expert Theater Sponsored by Amicus Therapeutics	
17:45	Non-CE Satellite Symposium Sponsored by Chiesi Global Rare Diseases	Closing the Loop: From Clinical Management to Patient Experience with Elfabrio® (pegunigalsidase alfa-ivxj)
17:45	Non-CE Satellite Symposium Sponsored by Azafaros B.V.	The value of conducting an 18-month placebo-controlled study in Rare Diseases: GM1, GM2 and NPC; the NAVIGATE experience.

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Wednesday, February 4, 2026: Translational Research
Co-Chairs: Tierra Bobo, PJ Brooks, Francyne Kubaski

06:45	Non-CE Satellite Symposium Sponsored by Astellas Pharma Inc	From Diagnosis to Care: Transforming rare diseases with gene therapies and advanced technologies
06:45	CE Satellite Symposium Accredited provider: AffinityCE Jointly Provided by: AffinityCE and Lysosomal & Rare Disorders Research & Treatment Center, Inc (LDRTC)	Shared Mechanisms and Clinical Implications of Secondary GSL Deposition in Lysosomal Disorders
08:00	Chester B. Whitley University of Minnesota Minneapolis, MN, United States	Welcome and Announcements Presentation of 2026 Patient Advocate Leader (PAL) Award to Bob Stevens and 2026 Young Investigator Awards Presentation
08:30	Françoise Piguet Paris Brain Institute Paris, France	Novel intravenous AAV gene therapy for mucopolysaccharidosis type IIIA and IIIB in mouse and canine model of the pathology - towards clinical translation
	Rafael A. Badell-Grau University of California, San Diego La Jolla, CA, United States	Hematopoietic stem cell gene therapy for mucopolysaccharidosis type IIIC
	Sampurna Saikia University of Delaware Newark, DE, United States	The complementary strength of the AAV9 gene therapy in hematopoietic stem cells transplanted into MPS IVA mice <i>*2026 Young Investigator Award Recipient</i>
	Jillian Gallagher University of Massachusetts Worcester, MA, United States	Testing a dual AAV gene therapy vector construct to treat sialidosis and galactosialidosis using small and large animal models
09:18	Moderated Q&A	<i>Piguet, Badell-Grau, Saikia, Gallagher</i>
09:30	Udayanga Wanninayake Saint Louis University St. Louis, MO, United States	Enhanced CIMPR binding and cellular uptake of HP-GALNS (M161): A next-generation enzyme replacement therapy for Morquio syndrome type A <i>*2026 Young Investigator Award Recipient</i>
	Jose Victor Alvarez Gonzalez Instituto de Investigación Sanitaria de Santiago de Compostela Santiago de Compostela, Spain	Advances in the administration of ERT (oral pathway) improving the biodistribution of enzymes <i>*2026 Young Investigator Award Recipient</i>
	Chia-Feng Yang Taipei Veterans General Hospital Taipei, Taiwan	Long-term outcomes of very early treated infantile-onset Pompe disease with the improvement after 24-month switching to avalglucosidase alfa: Real-world experiences based on Taiwan nationwide newborn screening program
	Allan Feng Stanford University Stanford, CA, United States	Therapeutic efficacy of a novel glucocerebrosidase variant in a new preclinical model of neuronopathic Gaucher disease
10:18	Moderated Q&A	<i>Wanninayake, Alvarez Gonzalez, Yang, Feng</i>
10:30	Break and Exhibits	
11:00	Shelly Goomber Duke University Durham, NC, United States	Functional profiling-based evaluation of GAA VUS in Pompe disease using a transient expression system upgraded for capacity and robustness

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Wednesday, February 4, 2026: Translational Research (cont.)
Co-Chairs: Tierra Bobo, PJ Brooks, Francyne Kubaski

	Xuntian Jiang Washington University St. Louis St. Louis, MO, United States	Diagnostic and therapeutic applications of the glycan biomarker H3N2b in GM1 gangliosidosis
	Melissa Greco Virginia Tech Roanoke, VA, United States	Infantile Krabbe disease presenting with intermediate psychosine (2-10 nmol/L) values in dried bloodspots <i>*2026 Young Investigator Award Recipient</i>
	Troy Lund University of Minnesota Minneapolis, MN, United States	GAG endogenous non-reducing ends as a new biomarker in CSF and plasma for Hurler syndrome
11:48	Moderated Q&A	<i>Goomber, Jiang, Greco, Lund</i>
12:00	Break, Exhibits and Satellite Symposia	
12:15	CE Satellite Symposium Accredited provider: Medical Education Resources (MER) Jointly Provided by MER and Saterdalen & Associates LLC Supported by an independent educational grant from Takeda Pharmaceuticals U.S.A., Inc.	Bone Disease in Gaucher Disease: A Deeper Dive into Evaluation / A Case Study Approach
12:15	Non-CE Satellite Symposium Sponsored by Amicus Therapeutics	Optimizing late-onset Pompe disease (LOPD) monitoring in an era of multiple treatment options
13:30	Anna-Maria Wiesinger Paracelsus Medical University Salzburg Salzburg, Austria	A decision analysis framework for individualized immunomodulatory therapy in MPS: Early clinical insights
	Chester B. Whitley University of Minnesota Minneapolis, MN, United States	Long-term outcome of Hurler syndrome following bone marrow transplantation
	Ashwin Roy University of Birmingham Birmingham, United Kingdom	Early atrial remodeling: A driver of arrhythmia in Fabry disease
	Salvatore Recupero San Raffaele Scientific Institute Milan, Italy	Gallbladder abnormalities in metachromatic leukodystrophy: Preliminary analysis in patients treated with atidarsagene autotemcel (autologous hematopoietic stem cell gene therapy) and untreated patients
14:18	Moderated Q&A	<i>Wiesinger, Whitley, Roy, Recupero</i>
14:30	Tae Un Han National Institutes of Health Bethesda, MD, United States	Development of a novel systemic AAV gene therapy for neuronopathic Gaucher disease
	Grace R. Kick Washington University St. Louis St. Louis, MO, United States	Efficacy of AAV-mediated gene therapy in a sheep model of CLN1 disease
	Alex Liu Huang Boston Children's Hospital Boston, MA, United States	Self-amplifying mRNA enhances transamniotic fetal mRNA delivery <i>*2026 Young Investigator Award Recipient</i>

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	Surendra Raj Sharma University of North Carolina Chapel Hill Chapel Hill, NC, United States	Rapid transient antibody depletion using genetically engineered IgG-degrading enzyme allows efficient rAAV9 gene delivery in an α-AAV9-Ab ⁺ rabbit model
15:18	Moderated Q&A	<i>Han, Kick, Huang, Sharma</i>
15:30	Translational Research Poster Session	
15:45	Industry Expert Theater Sponsored by Sanofi	
17:45	Non-CE Satellite Symposium Sponsored Sanofi	Biomarkers in Gaucher Disease: A Critical Dialogue on Patient Care Evolution Through Clinical Cases
17:45	Non-CE Satellite Symposium Sponsored Chiesi Global Rare Diseases	Rational Design Meets Real-World Relevance: Pegunigalsidase Alfa in the Treatment of Fabry Disease

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Thursday, February 5, 2026: Clinical Applications

Co-Chairs: Rebecca Ahrens-Nicklas, Roberto Giugliani, Filippo Vairo

06:45	CE Satellite Symposium Accredited provider: AKH Inc., Advancing Knowledge in Healthcare Jointly provided by AKH Inc., Advancing Knowledge in Healthcare and Catalyst Medical Education, LLC Supported by an independent educational grant from Amicus Therapeutics, Inc.	Homing in on Holistic Management of Late Onset Pompe Disease: Contemporary Insights on Disease Monitoring, Treatment Selection and Switching, and Shared Decision-Making
06:45	Non-CE Satellite Symposium Sponsored by Denali Therapeutics	Transforming Patient Care in MPS II
08:00	Chester B. Whitley University of Minnesota Minneapolis, MN, United States	Welcome and Keynote Speaker Introduction
	Michael H. Gelb University of Washington Seattle, WA, United States	Special Catalyst Award Keynote Address: <i>Biochemical newborn screening for all treatable lysosomal diseases</i>
08:30	John A. Bernat University of Iowa Health Care Iowa City, IA, United States	Isralgagene civaparvovec (ST-920) shows positive mean annualized eGFR slope in adults with Fabry disease: Topline results from the registrational phase 1/2 STAAR gene therapy study and long-term follow-up study
	Michael L. West Dalhousie University Halifax, NS, Canada	Comparison of outcomes of the FACTS lentivirus mediated gene therapy trial in Fabry disease with controls from the Canadian Fabry Disease Initiative Registry (CFDR)
	Dominique P. Germain University of Versailles – University Paris Saclay Montigny, France	Hidden double hits in Fabry disease: A critical confounder in clinical trials
	Peter Nordbeck University Hospital Würzburg Würzburg, Germany	Real-world effectiveness of migalastat versus enzyme replacement therapy in previously treatment-naïve patients with Fabry disease: Analyses of matched populations from the global followME Pathfinders registry
09:18	Moderated Q&A	<i>Bernat, West, Germain, Nordbeck</i>
09:30	Ida Vanessa D. Schwartz HCPA/UFRGS Porto Alegre, Brazil	Two-year follow up of FLT201 AAV gene therapy in adults with type 1 Gaucher disease: Results from GALILEO-1 and GALILEO-2
	Deepa Rajan University of Pittsburgh Pittsburgh, PA, USA	Interim results from the PROVIDE Clinical Trial - A phase I/II Study of LY3884961 (PR001) an AAV9-based gene therapy for type 2 Gaucher disease
	Aimee Donald University of Manchester Manchester, United Kingdom	Lentiviral stem cell gene therapy of neuronopathic Gaucher disease (GD3) achieves prolonged enzyme delivery, substrate reduction and stabilisation of neurologic and somatic disease manifestations
	Pramod K. Mistry Yale University New Haven, CT, United States	Beyond ERT/SRT: A neuro-pulmonary-lymphatic spatial-omics atlas redefines pathology and guides immunotherapy in neuronopathic Gaucher disease
10:18	Moderated Q&A	<i>Schwartz, Donald, Donald, Mistry</i>
10:30	Break and Exhibits	

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Thursday, February 5, 2026: Clinical Applications (cont.)

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11:00	Joseph Muenzer University of North Carolina Chapel Hill, NC, United States	Phase I/II study of intravenous tividnofusp alfa for mucopolysaccharidosis type II
	Can Ficicioglu The Children's Hospital of Philadelphia Philadelphia, PA, United States	Effect of clemidsogene lanparvovec (RGX-121), an investigational gene therapy, on neurodevelopmental outcomes in patients with Hunter syndrome
	Simon A. Jones St. Mary's Hospital Manchester, United Kingdom	Ex-vivo modification of autologous CD34+ HSPCs using a CD11b-directed lentiviral vector encoding ApoEII-tagged human IDS leads to supraphysiological enzyme activity and biochemical correction of neuronopathic MPS II patients
	Elizabeth Jalazo University of North Carolina Chapel Hill Chapel Hill, NC, United States	Preliminary results from phase I/II, first-in-human, open-label study of DNL126 in children with mucopolysaccharidosis type IIIA (MPS IIIA)
11:48	Moderated Q&A	<i>Muenzer, Ficicioglu, Jones, Jalazo</i>
12:00	Break, Exhibits and Satellite Symposia	
12:15	Non-CE Satellite Symposium Sponsored Zevra Therapeutics	Charting a Path in Niemann-Pick Disease Type C: Diagnostic Challenges, Therapeutic Innovations, and Real-World Patient Cases
12:15	Non-CE Satellite Symposium Sponsored Sanofi	From the Kidney and Beyond: Early, Multi-Organ Impacts in Fabry Disease
13:30	Brian Bigger University of Edinburgh Edinburgh, United Kingdom	Sustained biochemical correction and improved neurological outcomes at 36-months post hematopoietic stem cell gene therapy for Sanfilippo syndrome.
	Nicole M. Muschol University Medical Center Hamburg-Eppendorf Hamburg, Germany	Long-term administration of tralesenidase alfa enzyme replacement therapy (TA-ERT) results in profound and durable reduction of heparan sulfate (HS) and stabilization of cognitive function and cortical gray matter volume (CGMV) in patients with Sanfilippo syndrome type B (MPS IIIB)
	Ozlem Goker-Alpan Lysosomal & Rare Disorders Research & Treatment Center, Inc Fairfax, VA, United States	Safety, tolerability and biological activity of ABX1100, a CD71 centyrin siRNA conjugate targeting GYS1 in late-onset Pompe disease patients
	Priya S. Kishnani Duke University Durham, NC, United States	Symptom onset, disease biomarkers, and treatment status in US Pompe disease patients identified by newborn screening
14:18	Moderated Q&A	<i>Bigger, Muschol, Goker-Alpan, Kishnani</i>
14:30	Roberto Giugliani Federal University of Rio Grande do Sul Porto Alegre, Brazil	Long-term data from a phase II study with oral nizubaglustat for late-infantile/ juvenile GM2 and NPC diseases (RAINBOW)
	Barbara MacFee OPEN Health Parsippany, NJ, United States	Outcomes of patients with rapidly progressive lysosomal acid lipase deficiency treated with sebelipase alfa before hematopoietic stem cell transplantation
	Elizabeth Berry-Kravis Rush University Medical Center Chicago, IL, United States	Adrabetadex treatment in individuals with Niemann-Pick disease type C1 re-establishes cholesterol trafficking, resulting in decreased markers of neuronal damage and cell death

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Thursday, February 5, 2026: Clinical Applications (cont.)

Co-Chairs: Rebecca Ahrens-Nicklas, Roberto Giugliani, Filippo Vairo

	Caroline Aimee Hastings UCSF Benioff Children's Hospital Oakland Oakland, CA, United States	Real-world safety and effectiveness of arimoclomol in patients with NPC: Outcomes from the US early access program (EAP) over a 4-year period
15:18	Moderated Q&A	<i>Giugliani, MacFee, Berry-Kravis, Hastings</i>
15:30	Clinical Applications Poster Session	
15:45	Industry Expert Theater Sponsored by BioMarin Pharmaceutical	
17:45	5th Annual Robert J. Gorlin Symposium CE Session Accredited provider: Medical Education Resources (MER)	Global Access to Newborn Screening: A Call to Action! <i>This activity is supported in part by educational grants from Chiesi USA, Inc., Denali Therapeutics, Takeda Pharmaceuticals USA, Inc., and Ultragenyx Pharmaceutical Inc.</i>

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Friday, February 6, 2026: Contemporary Forum and Late-Breaking Science

Co-Chairs: Marc Patterson, Uma Ramaswami, Cyndi Tifft



07:30	Chester B. Whitley University of Minnesota Minneapolis, MN, United States	Welcome and New Treatment Awards
08:00	Heather A. Lau Ultragenyx Pharmaceutical Inc Novato, CA, United States	Treatment with UX111 reduced cerebrospinal fluid (CSF) heparan sulfate (HS) exposure and stabilized or improved functioning across dose, age, and stage of MPS IIIA
	Daniel M. Virga Regeneron Pharmaceuticals Tarrytown, NY, United States	Anti-transferrin receptor 1-targeted AAV9 therapy prevents CNS and visceral pathologies in acid sphingomyelinase deficiency
	Marc C. Patterson IntraBio, Inc. Austin, TX, United States	Long-term findings of N-acetyl-L-leucine for Niemann-Pick disease type C
	Yanmei Lu Sangamo Therapeutics Richmond, CA, United States	Isaralgagene civaparvovec (ST-920) gene therapy for adults with Fabry disease: Pharmacology and immunogenicity outcomes from the phase 1/2 STAAR study and ongoing long-term follow-up
08:48	Moderated Q&A	<i>Lau, Virga, Patterson, Lu</i>
10:00	Break	
10:30	Late-Breaking Abstracts	4 Speakers To Be Announced (TBA) with Moderated Q&A
11:30	Break	

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Friday, February 6, 2026: Rapid Fire Competition

Co-Chairs: Amy Gaviglio, Francyne Kubaski, Marc Patterson

12:30	Karolina M. Stepien Salford Royal Hospital Salford, United Kingdom	Evidence of secondary mitochondrial dysfunction in alpha-mannosidosis
12:40	David Moreno Martínez Cambridge University Hospitals Cambridge, United Kingdom	Neuronal dysfunction beyond lysosomes in Fabry disease: Evidence from iPSC-derived forebrain neurons
12:50	Betul Celik Nemours Children's Health Wilmington, DE, United States	Co-transplantation of hematopoietic stem cells and highly purified rapidly expanding clones (REC) of human mesenchymal stem cells rescued the bone pathology of MPS IVA mice
13:00	Ryunosuke Sanada National Center for Child Health and Development Tokyo, Japan	Development of gene editing technologies to correct a mutation in <i>GNPTAB</i> of mucopolidosis type II/III patients <i>*2026 Young Investigator Award Recipient</i>
13:10	Maria Ester Bernardo San Raffaele Scientific Institute Milan, Italy	Sustained supraphysiological alpha-L-iduronidase (IDUA) activity, reduction of glycosaminoglycans (GAGs), and clinical benefits at 5 years post-treatment with OTL-203, an autologous hematopoietic stem cell gene therapy (HSC-GT), in patients with mucopolysaccharidosis type I (MPS IH) Hurler syndrome
13:20	Tahseen Mozaffar University of California, Irvine Orange, CA, United States	208-week outcomes of cipaglucosidase alfa plus miglustat in patients with late-onset Pompe disease treated from PROPEL baseline: Muscle function and biomarkers
13:30	Kathy E. Meyer Sangamo Therapeutics. Richmond, CA, United States	A combined fertility, embryofetal development, AAV integration and germline transmission risk study in mice with isaralgagene civaparvovec (ST-920) for Fabry disease
13:40	Petra Oliva ARCHIMEDlife GmbH Vienna, Austria	Breaking barriers in lysosomal disorder screening: A novel simultaneous LC-MS/MS approach for Tay-Sachs, Sandhoff, and GM1 gangliosidosis diseases
13:50	Rapid Fire Abstract	4 Speakers To Be Announced (TBA)
14:00	Rapid Fire Abstract	
14:10	Rapid Fire Abstract	
14:20	Rapid Fire Abstract	
14:30	Rapid Fire Abstract Competition Voting	Voting and Awards
14:40	WORLDSymposium 2026 Adjourns	

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