

Tuesday, February 21, 2023

Robert J. Gorlin Symposium and Emerging Trends: State-of-the-art for Experts

1:45 PM	2nd Annual Robert J. Gorlin Symposium	Precision Medicine: A Multidisciplinary Approach (<i>Registration required</i>)
2:00 PM	Jeanine R. Jarnes University of Minnesota Minneapolis, MN, United States	Welcome and Introduction of Speakers and Overview of Precision Medicine
2:15 PM	Filippo Pinto e Vairo Mayo Clinic Rochester, MN, United States	Case Studies of Multi-Omic Approach for the Diagnosis of Lysosomal Diseases
2:35 PM	Jennifer Goldstein UNC-Chapel Hill Chapel Hill, NC, United States	NIH-Funded Resources: ClinGen and ClinVar
2:55 PM	Jeanine R. Jarnes University of Minnesota Minneapolis, MN, United States	Implementation of Pharmacogenomics Programs within Clinical Settings
3:10 PM	Panel Discussion and Audience Q&A	
	Emerging Trends	State-of-the-Art for Experts (<i>Registration required</i>)
4:00 PM	Chester B. Whitley <i>Course Director</i> University of Minnesota Minneapolis, MN, United States	Introduction and Course Overview
4:01 PM	Gregory A. Grabowski Cincinnati Children's Hospital Research Foundation Cincinnati, OH, United States	Lysosomal Function and Pathogenesis
4:15 PM	Marc C. Patterson Mayo Clinic Children's Center Rochester, MN, United States	Clinical Features
4:30 PM	Amy Gaviglio Centers for Disease Control and Prevention Minneapolis, MN, United States	Newborn Screening
4:45 PM	Jeanine R. Jarnes University of Minnesota Minneapolis, MN, United States	Lysosomal Disease Therapies
5:00 PM	Christine Yuen-Yi Hon, PharmD Office of New Drugs CDER FDA Silver Spring, MD, United States	Regulatory Review
5:15 PM	Jennifer Klein National MPS Society Durham, NC, United States	Patient Perspective
5:30 PM	N. Matthew Ellinwood National MPS Society Durham, NC, United States	Rare Disease Research
5:45 PM	Chester B. Whitley <i>Moderator</i> University of Minnesota Minneapolis, MN, United States	Open Q&A
6:00 PM	Be the Catalyst Event	

WORLDSymposium™ 2023 Preliminary Program*

Platform Presenters



Wednesday, February 22, 2023: Basic Science

Moderators: Brian Bigger, Lalitha Belur, and Michael Przybilla

6:15 AM	Satellite Symposia	
7:30 AM	Chester B. Whitley University of Minnesota Minneapolis, MN, United States	Welcome & Announcements Presentation of 2023 Roscoe O. Brady Award to William A. Gahl
	William A. Gahl National Human Genome Research Institute Bethesda, MD United States	Roscoe O. Brady Award Presentation: <i>Pursuing Advances in Rare and Undiagnosed Diseases</i>
8:00 AM	Xiangli Zhao New York University Grossman School of Medicine New York, NY, United States	A brain penetrant progranulin-derived biologic protects against neuronopathic Gaucher disease <i>*2023 Young Investigator Award Recipient</i>
	Yi Lin Cincinnati Children's Hospital Medical Center Cincinnati, OH, United States	Earlier-onset, more severe neurodegeneration in PGRN KO mice with a decreased dose of D409V Gba1
	Zhenting Zhang Cincinnati Children's Hospital Medical Center Cincinnati, OH, United States	A multifaceted evaluation of microgliosis and differential cellular dysregulations of mTOR signaling with fluctuating lysosome function in neuronopathic Gaucher disease <i>*2023 Young Investigator Award Recipient</i>
	Irene Serrano Gonzalo Fundación Española para el Estudio y Terapéutica de la Enfermedad de Gaucher y otras lisosomales Zaragoza, Spain	Study of miRNA expression profiles depending on the severity of bone involvement in patients with Gaucher disease
	Moderated Q&A	<i>Xiangli Zhao, Yi Lin, Zhenting Zhang, and Irene Serrano Gonzalo</i>
9:00 AM	Maria Fuller SA Pathology North Adelaide, Australia	Signature biomarkers for diagnosis, screening, and biochemical monitoring of the mucopolysaccharidoses
	Rebecca C. Ahrens-Nicklas The Children's Hospital of Philadelphia Philadelphia, PA, United States	Biomarkers of disease severity in multiple sulfatase deficiency
	Hannah Best Cardiff University Cardiff, United Kingdom	The Batten disease associated protein CLN3 is required for the efflux of lysosomal K ⁺ <i>*2023 Young Investigator Award Recipient</i>
	Tyler M. Pierson Cedars-Sinai Medical Center Los Angeles, CA, United States	Modeling CLN6 with iPSC-derived neurons and glia
	Moderated Q&A	<i>Maria Fuller, Rebecca C. Ahrens-Nicklas, Hannah Best, and Tyler M. Pierson</i>
10:00 AM	Break	
10:30 AM	Francyne Kubaski Greenwood Genetic Center Greenwood, SC, United States	Sensitivity and specificity of four lysosomal disorder biomarkers in dried blood spots

WORLDSymposium™ 2023 Preliminary Program*
Platform Presenters



Wednesday, February 22, 2023: Basic Science (cont.)
Moderators: Brian Bigger, Lalitha Belur, and Michael Przybilla

	Neil Kasaci Lysosomal and Rare Disorders Research and Treatment Center Fairfax, VA, United States	Caspase inhibitors can counteract inflammasome activation and caspase-1 mediated fibrosis in Fabry disease <i>*2023 Young Investigator Award Recipient</i>
	Saida Ortolano Galicia Sur Health Research Institute Vigo, Spain	PBXs: New pharmacological chaperones to increase α -galactosidase A activity in Fabry disease cellular models
	Efecan Aral University of Massachusetts - Amherst Amherst, MA, United States	Establishing personalized medicine in Fabry disease through functional analysis of disease mutants
	Moderated Q&A	<i>Francyne Kubaski, Neil Kasaci, Saida Ortolano, and Efecan Aral</i>
11:30 AM	Break and Satellite Symposia	
1:00 PM	Behzad Najafian University of Washington Seattle, WA, United States	The spectrum of podocyte injury in later onset (LO) variants of Fabry disease (FD)
	David Smerkous Oregon State University Corvallis, OR, United States	Development of an online cloud-based tool for automatic measurement of foot process width (FPW) using deep learning (DL): Applications in assessment of podocyte injury in Fabry disease (FD) <i>*2023 Young Investigator Award Recipient</i>
	Alex J. Shamoun University of Florida Gainesville, FL, United States	Differences in organ abundance of iduronate 2-sulfatase and intravenous recombinant enzyme delivery: Potential implications for clinical response to ERT in MPS II
	Marta Artola Leiden University Leiden, Netherlands	1,6-epi-cyclophellitol cyclosulfamidate is a new superior lysosomal α -glucosidase stabilizer for the treatment of Pompe disease
	Moderated Q&A	<i>Behzad Najafian, David Smerkous, Alex J. Shamoun, and Marta Artola</i>
2:00 PM	Mahsa Taherzadeh McGill University Montreal, QC, Canada	Severe neuronal demyelination in Sanfilippo disease
	Frederick Ashby University of Florida Gainesville, FL, United States	Bone pathology within Sanfilippo syndrome type B mice as a novel biometric for peripheral disease correction
	Chloé Dias Université Toulouse III Paul Sabatier Toulouse, France	Microglia-derived extracellular vesicles promote neuropathology in Sanfilippo syndrome <i>*2023 Young Investigator Award Recipient</i>
	Angela J. Espejo Pontificia Universidad Javeriana Bogotá D.C., Colombia	Magnetite nanoparticles as a vehicle to transport recombinant hexosaminidase A and B through an in vitro model of the blood-brain barrier
	Moderated Q&A	<i>Mahsa Taherzadeh, Frederick Ashby, Chloé Dias, and Angela J. Espejo</i>
3:00 PM	Exhibit Hall Opens	Poster Session in the Exhibit Hall
5:15 PM	Speed Mentoring Session	

WORLDSymposium™ 2023 Preliminary Program*

Platform Presenters



Thursday, February 23, 2023: Translational Research
Moderators: PJ Brooks, Amy Gaviglio, and Francyne Kubaski

6:15 AM	Satellite Symposia	
7:30 AM	Chester B. Whitley University of Minnesota Minneapolis, MN, United States	Welcome & Announcements Presentation of 2023 Patient Advocate Leader (PAL) Award to Christine Waggoner and 2023 Young Investigator Awards Presentation
8:00 AM	Anna-Maria Wiesinger Paracelsus Medical University Salzburg Salzburg, Austria	A precision medicine tool for high utilization and quality of individual treatment trials with immunomodulatory drugs in mucopolysaccharidosis <i>*2023 Young Investigator Award Recipient</i>
	Barbara K. Burton Northwestern University Feinberg School of Medicine Chicago, IL, United States	Newborn screening for mucopolysaccharidosis type II
	Stuart M. Ellison University of Manchester Manchester, United Kingdom	Validation of a GMP stem cell gene therapy manufacturing process for mucopolysaccharidosis type II (MPS II) in preparation for an approved phase I/II clinical trial
	Anna Luzzi The Lundquist Institute for Biomedical Innovation at Harbor-UCLA Medical Center Torrance, CA, United States	Decreased regulatory T-cells in patients with Sanfilippo syndrome may allow the development of autoimmune disease
	Moderated Q&A	<i>Anna-Maria Wiesinger, Barbara K. Burton, Stuart M. Ellison, and Anna Luzzi</i>
9:00 AM	Kim M. Hemsley Flinders University Bedford Park, Australia	A prohibitin-targeting drug modifies aspects of disease in a mouse model of Sanfilippo syndrome
	Simon Jones St. Mary's Hospital Manchester, United Kingdom	Sustained biochemical engraftment and early clinical outcomes following ex-vivo autologous stem cell gene therapy for mucopolysaccharidosis type IIIA
	Oriana Mandolfo University of Manchester Manchester, United Kingdom	Developing an iPSC-based neural gene therapy approach for MPS IIIA
	Nissrine Ballout Université Toulouse III Paul Sabatier Toulouse, France	Development and validation of a novel adeno-associated viral gene therapy for mucopolysaccharidosis type IIIB (MPS IIIB) <i>*2023 Young Investigator Award Recipient</i>
	Moderated Q&A	<i>Kim M. Hemsley, Simon Jones, Oriana Mandolfo, and Nissrine Ballout</i>
10:00 AM	Break & Exhibits	
10:30 AM	Troy Lund University of Minnesota Minneapolis, MN, United States	Decreases in CSF neuro-inflammatory markers are associated with gain in neurocognitive function after ERT + HCT in Hurler syndrome
	Roselena S. Schuh Universidade Federal do Rio Grande do Sul Porto Alegre, Brazil	Nasal administration of laronidase-loaded liposomes aiming at mucopolysaccharidosis type I treatment <i>*2023 Young Investigator Award Recipient</i>
	Michael J. Przybilla University of Minnesota Minneapolis, MN, United States	Treating murine Hurler syndrome utilizing small-activating RNA following bone marrow transplant

WORLDSymposium™ 2023 Preliminary Program*

Platform Presenters



Thursday, February 23, 2023: Translational Research (cont.)
Moderators: PJ Brooks, Amy Gaviglio, and Francyne Kubaski

	Betul Celik University of Delaware Newark, DE, United States	Lentiviral gene therapy for mucopolysaccharidosis type IVA
	Moderated Q&A	<i>Troy Lund, Roselena S. Schuh, Michael J. Przybilla, and Betul Celik</i>
11:30 AM	Break, Exhibits and Satellite Symposia	
1:00 PM	Leigh Fremuth St. Jude Children's Research Hospital Memphis, TN, United States	AAV-mediated gene therapy for galactosialidosis: A long-term safety and efficacy study
	Sandra Vranic University of Manchester Manchester, United Kingdom	Defect-free graphene enhances enzyme delivery to fibroblasts derived from the patients with lysosomal disorders
	Paul J. Orchard University of Minnesota Minneapolis, MN, United States	Compassionate use of OTL-200 for patients with metachromatic leukodystrophy
	Laura A. Adang Children's Hospital of Philadelphia Philadelphia, PA, United States	Developmental delay can precede neurologic regression in metachromatic leukodystrophy
	Moderated Q&A	<i>Leigh Fremuth, Sandra Vranic, Paul J. Orchard, and Laura A. Adang</i>
2:00 PM	Lars Schlotawa University Medical Center Goettingen Goettingen, Germany	Screening of approved drugs identifies 3rd generation retinoids as in vitro therapeutic agents in multiple sulfatase deficiency
	Aimee Donald University of Manchester Manchester, United Kingdom	Sustained improvement of clinical CNS and somatic features of Gaucher disease type 3 after haematopoietic stem cell (HSC) gene therapy: A first-in-world report
	Andreas Hahn University Hospital Giessen Giessen, Germany	Treatment of CLN1 disease with a blood-brain barrier penetrating lysosomal enzyme AGT-194
	Jason A. Weesner St. Jude Children's Research Hospital Memphis, TN, United States	Preclinical enzyme replacement therapy with a recombinant β -galactosidase-lectin fusion for CNS delivery and treatment of GM1-gangliosidosis <i>*2023 Young Investigator Award Recipient</i>
	Moderated Q&A	<i>Lars Schlotawa, Aimee Donald, Andreas Hahn, and Jason A. Weesner</i>
3:00 PM	Poster Session in the Exhibit Hall	
5:15 PM	Satellite Symposia	

Friday, February 24, 2023: Clinical Applications

Moderators: Lynda Polgreen, Marc Patterson, and Filippo Vairo

6:15 AM **Satellite Symposia**

7:30 AM **Chester B. Whitley**
University of Minnesota
Minneapolis, MN, United States

Welcome and Keynote Speaker Introduction

Peter Marks
Center for Biologics Evaluation and Research
US Food & Drug Administration (FDA)
Silver Spring, MD, United States

Keynote Address:
Taking Gene Therapy to the Next Level

8:00 AM **Francesca Fumagalli**
San Raffaele Telethon Institute for Gene
Therapy, IRCCS San Raffaele Scientific Institute
Milan, Italy

Long-term clinical outcomes of atidarsagene autotemcel (autologous hematopoietic stem cell gene therapy [HSC-GT] for metachromatic leukodystrophy) with up to 11 years follow-up

Maria Jose De Castro Lopez
Hospital Clínico Santiago
Santiago, Spain

Twice weekly dosing with sebelipase alfa rescues severely ill infants with Wolman disease

Robert J. Hopkin
Cincinnati Children's Hospital Medical Center
Cincinnati, OH, United States

STAAR, a phase I/II study of isaralgagene civaparvovec (ST-920) gene therapy in adults with Fabry disease: Dose escalation phase results

Valeria Calbi
San Raffaele Telethon Institute for Gene
Therapy, IRCCS San Raffaele Scientific Institute
Milan, Italy

Lentiviral haematopoietic stem cell gene therapy for metachromatic leukodystrophy: Results in 5 patients treated under nominal compassionate use

Moderated Q&A

Francesca Fumagalli, Maria Jose De Castro Lopez, Robert J. Hopkin, and Valeria Calbi

9:00 AM **Joseph Muenzer**
University of North Carolina Chapel Hill
Chapel Hill, NC, United States

Interim analysis of key clinical outcomes from a phase 1/2 study of weekly intravenous DNL310 (brain-penetrant enzyme replacement therapy) in MPS II

Paul Harmatz
UCSF Benioff Children's Hospital Oakland
Oakland, CA, United States

Interim results of a phase 1/2 study of JR-171 (lepunafusp alfa), a novel brain-penetrant enzyme replacement therapy for MPS I

Raymond Y. Wang
CHOC Children's Specialists
Orange, CA, United States

RGX-111 gene therapy for the treatment of severe mucopolysaccharidosis type I (MPS I): Interim analysis of data from the first in human study

Cara O'Neill
Cure Sanfilippo Foundation
Columbia, SC, United States

Development of consensus guidelines for the clinical care of individuals with Sanfilippo syndrome

Moderated Q&A

Joseph Muenzer, Paul Harmatz, Raymond Y. Wang, and Cara O'Neill

10:00 AM **Break & Exhibits**

10:30 AM **Barry J. Byrne**
University of Florida
Gainesville, FL, United States

Long-term follow-up of cipaglucosidase alfa/miglustat in ambulatory patients with Pompe disease: An open-label phase I/II study (ATB200-02)

WORLDSymposium™ 2023 Preliminary Program*
Platform Presenters



Friday, February 24, 2023: Clinical Applications (cont.)

Moderators: Lynda Polgreen, Marc Patterson, and Filippo Vairo

	Erin Huggins Duke University Durham, NC, United States	Longitudinal follow up uncovers an early emerging phenotype in children with late-onset Pompe disease diagnosed via newborn screening
	Priya S. Kishnani Duke University Medical Center Durham Durham, NC, United States	Efficacy and safety of avalglucosidase alfa in participants with late-onset Pompe disease after 145 weeks of treatment during the COMET trial
	Jordi Diaz Manera Newcastle University Newcastle Upon Tyne, United Kingdom	AT845 gene replacement therapy for late onset Pompe disease: An update on safety and preliminary efficacy data from FORTIS, a phase I/II open-label clinical study
	Moderated Q&A	<i>Barry J. Byrne, Erin Huggins, Priya S. Kishnani, and Jordi Diaz Manera</i>
11:30 AM	Break, Exhibits and Satellite Symposia	
1:00 PM	Eric Wallace University of Alabama Birmingham, AL, United States	First results of a head-to-head trial of pegunigalsidase alfa vs. agalsidase beta in Fabry disease: 2 year results of the phase 3 randomized, double-blind, BALANCE study
	John Bernat University of Iowa Hospitals and Clinics Iowa City, IA, United States	Long-term safety and efficacy of pegunigalsidase alfa administered every 4 weeks in patients with Fabry disease: Two-year interim results from the ongoing phase 3 BRIGHT51 open-label extension study
	Melissa P. Wasserstein Albert Einstein College of Medicine/Children's Hospital at Montefiore Bronx, NY, United States	Plasma lyso-sphingomyelin as a biomarker for acid sphingomyelinase deficiency: Correlations with baseline disease and response to olipudase alfa treatment in clinical trials
	Roberto Giugliani Federal University of Rio Grande do Sul Porto Alegre, RS, Brazil	Long-term catch-up growth in children with acid sphingomyelinase deficiency treated with olipudase alfa enzyme replacement therapy in the ASCEND-Peds trial
	Moderated Q&A	<i>Eric Wallace, John Bernat, Melissa P. Wasserstein, and Roberto Giugliani</i>
2:00 PM	Pramod K. Mistry Yale University School of Medicine New Haven, CT, United States	Changes in hematologic and visceral manifestations over time following imiglucerase initiation in Gaucher disease type 1 and type 3 pediatric patients in the ICGG Gaucher Registry
	Jeanine R. Jarnes University of Minnesota Minneapolis, MN, United States	Updated interim safety, biomarker, and efficacy data from Imagine-1: A phase 1/2 open-label, multicenter study to assess the safety, tolerability, and efficacy of a single dose, intra-cisterna magna (ICM) administration of PBGM01 in subjects with type I (early onset) and type IIA (late onset) infantile GM1 gangliosidosis (GM1)
	Yoshikatsu Eto Institute of Neurological Disease Kawasaki City, Japan	Real-world data of enzyme replacement therapy with pabinafusp alfa for neuropathic MPS-II: Updated clinical data from Japan
	David L. Rogers Nationwide Children's Hospital Columbus, OH, United States	Intravitreal enzyme replacement therapy to prevent retinal disease progression in children with neuronal ceroid lipofuscinosis type 2 (CLN2): Interim safety report
	Moderated Q&A	<i>Pramod K. Mistry, Jeanine R. Jarnes, Yoshikatsu Eto, and David L. Rogers</i>
3:00 PM	Poster Session in the Exhibit Hall	
5:30 PM	Satellite Symposia	

WORLDSymposium™ 2023 Preliminary Program*
Platform Presenters



Saturday, February 25, 2023: Contemporary Forum
Moderators: Nishitha Pillai, Dan Tagle, and Ellen Sidransky

6:15 AM	Satellite Symposia	
7:45 AM	Chester B. Whitley University of Minnesota Minneapolis, MN, United States	Welcome and New Treatment Award
8:00 AM	Shababa T. Masoud Denali Therapeutics South San Francisco, CA, United States	ETV:SGSH, a brain-penetrant enzyme transport vehicle for SGSH, corrects heparan sulfate accumulation, lysosomal lipid storage and inflammation in MPS IIIA mouse brain
	Asuka Inoue JCR Pharmaceuticals Co., Ltd. Kobe, Japan	Nonclinical pharmacodynamics, pharmacokinetics and safety profiles of anti-human transferrin receptor antibody-fused N-sulfoglucosamine sulfohydrolase for mucopolysaccharidosis type IIIA
	Andrew Hedman M6P Therapeutics St. Louis, MO, United States	Novel dual promoter AAV gene therapy platform ensures production of therapeutic soluble lysosomal enzymes with high M6P content to enable broad cellular uptake and cross correction in vivo
	Charu Reddy Codexis San Carlos, CA, United States	An engineered β -galactosidase with improved stability and cross-correction for the potential treatment of GM1 gangliosidosis via AAV gene therapy
	Moderated Q&A	<i>Shababa T. Masoud, Asuka Inoue, Andrew Hedman, and Charu Reddy</i>
9:00 AM	Stephanie Cherqui University of California San Diego La Jolla, CA, United States	Phase 1/2 clinical trial of autologous hematopoietic stem and progenitor cell (HSPC) gene therapy for cystinosis
	Shyam Ramachandran Sanofi Waltham, MA, United States	AAV-ARSA-mediated gene replacement for the treatment of metachromatic leukodystrophy
	Mathews Adera AVROBIO, Inc. Cambridge, MA, United States	The Guard1 clinical trial - A first in-human, phase 1/2 study evaluating AVR-RD-02, a hematopoietic stem cell (HSC) gene therapy for Gaucher disease: Preliminary safety, pharmacodynamic and clinical efficacy results from the subjects observed for up to 24 months post-infusion
	Maria L. Escolar Forge Biologics Grove City, OH, United States	First-in-human phase 1/2 trial of intravenous FBX-101 following hematopoietic stem cell transplantation increases GALC activity, supports brain development, and improves motor function in patients with infantile Krabbe disease: RESKUE clinical trial
	Moderated Q&A	<i>Stephanie Cherqui, Shyam Ramachandran, Mathews Adera, and Maria L. Escolar</i>
10:00 AM	Break & Exhibits	
10:30 AM	Raphael Schiffmann 4D Molecular Therapeutics Emeryville, CA, United States	Cardiac effects of 4D-310 in adults with Fabry disease in a phase 1/2 clinical trial: Functional, quality of life, and imaging endpoints in patients with 12 months of follow up
	Russell Gotschall M6P Therapeutics St. Louis, MO, United States	M021: rhGAA with optimal glycosylation profile containing very high levels of bis-phosphorylated N-glycans clears accumulated glycogen and rapidly normalizes muscle strength in treated Pompe disease mice

WORLDSymposium™ 2023 Preliminary Program*
Platform Presenters



Saturday, February 25, 2023: Contemporary Forum (cont.)

Moderators: Nishitha Pillai, Dan Tagle, and Ellen Sidransky

	Ana C. Puhl Collaborations Pharmaceuticals, Inc. Raleigh, NC, United States	Developing treatments for rare diseases on a shoestring: The Batten disease (CLN1) enzyme replacement therapy experience
	Michael H. Gelb University of Washington Seattle, WA, United States	A glimpse into the feasibility of next generation sequencing for newborn screening of lysosomal and other diseases with second-tier biochemical assays as part of the screening process
	Moderated Q&A	<i>Raphael Schiffmann, Russell Gotschall, Ana C. Puhl, and Michael H. Gelb</i>
11:30 AM	Break, Exhibits and Satellite Symposia	
1:00 PM	Kyle Landskroner Azafaros AG Basel, Switzerland	AZ-3102 significantly increases survival and decreases neuroinflammation in a mouse model of Sandhoff disease
	Yannan Xi Maze Therapeutics South San Francisco, CA, United States	Small molecule inhibition of glycogen synthase 1 restores autophagolysosomal and metabolic pathway dysfunction in a mouse model of Pompe disease
	Shivakumar D. Pattada BioStrategies LC State University, AR, United States	RTB-lectin facilitates the distribution of enzymes across the blood-brain-barrier and correction in the MPS IIIA mouse model
	Michael A. Tortorici Aro Biotherapeutics Berwyn, PA, United States	Centyrin-targeted glycogen synthase-1 siRNA conjugates: A novel therapeutic modality for the treatment of Pompe disease
	Moderated Q&A	<i>Kyle Landskroner, Yannan Xi, Shivakumar D. Pattada, and Michael A. Tortorici</i>
2:00 PM	Nagy Habib MiNA Therapeutics Ltd London, United Kingdom	Drugging transcription factors with small activating RNAs: A novel approach for enhancing bone marrow therapy for monogenic rare diseases
	Meera E. Modi Takeda Cambridge, MA, United States	Building a better translational model of neuropathic Gaucher disease
	Yinyin Huang Sanofi Cambridge, MA, United States	Using single nuclear RNAseq to assess impact of AAV-ARSA gene therapy on oligodendrocyte populations
	Kwi Hye Kim REGENXBIO Inc Rockville, MD, United States	In vitro pharmacology study using retina organoids and retina-on-a-chip of CLN2 patient-derived induced pluripotent stem cells
	Moderated Q&A	<i>Nagy Habib, Meera E. Modi, Yinyin Huang, and Kwi Hye Kim</i>
3:00 PM	Poster Session in the Exhibit Hall	

Sunday, February 26, 2023: Late-Breaking Science

Moderators: Elizabeth Braunlin, Roberto Giugliani, and Rebecca Ahrens-Nicklas

8:00 AM	Li Ou Genemagic Bio Agoura Hills, CA, United States	A meta-analysis of 39 AAV clinical trials for lysosomal diseases: Immunogenicity, toxicity, and durability
	Lucas Tricoli Children's Hospital of Philadelphia Philadelphia, PA, United States	Improved gene therapy for metachromatic leukodystrophy
	Andrés Felipe Leal Pontificia Universidad Javeriana Bogotá D.C., Colombia	Assessment of an iron oxide-coupled CRISPR/nCas9 gene editing in mucopolysaccharidoses type IVA mouse model
	Chester B. Whitley University of Minnesota Minneapolis, MN, United States	The PS Gene-editing (PSG) System for treatment of lysosomal diseases
	Moderated Q&A	<i>Li Ou, Lucas Tricoli, Andres Leal, and Chester B. Whitley</i>
9:00 AM	Can Ficicioglu The Children's Hospital of Philadelphia Philadelphia, PA, United States	RGX-121 gene therapy for the treatment of neuronopathic mucopolysaccharidosis type II (MPS II): interim analysis of data from the first in human study
	Kelly George Sanofi Cambridge, MA, United States	Anti-mouse-TfR-GAA fusion proteins for the treatment of Pompe disease targeting the central nervous system and peripheral tissues
	Julie C. Ullman Maze Therapeutics South San Francisco, CA, United States	Results from a first in human study of MZE001, an orally bioavailable inhibitor of glycogen synthase 1 and potential substrate reduction therapy for Pompe disease
	Benedikt Schoser Ludwig-Maximilians-Universität München Munich, Germany	Long-term efficacy and safety of cipaglucosidase alfa/miglustat in ambulatory patients with Pompe disease: A phase III open-label extension study (ATB200-07)
	Moderated Q&A	<i>Can Ficicioglu, Kelly George, Julie C. Ullman, and Benedikt Schoser</i>
10:00 AM	Break	
10:15 AM	Xiomara Rosales Neurogene Inc. New York, NY, United States	Evidence from a study of CLN5 -/- sheep supporting dose escalation in an ongoing clinical trial of NGN-101 in pediatric patients with CLN5 Batten disease
	Patricia I. Dickson Washington University in St. Louis Saint Louis, MO, United States	Intraventricular recombinant human N-acetylglucosamine-6-sulfatase corrects lysosomal storage in mucopolysaccharidosis type IIID mice
	Eric H. Zanelli Allievex Corporation Boston, MA, United States	Tralesinidase alfa modifies the course of Sanfilippo syndrome type B
	Akos Herzeg Center for Maternal-Fetal Precision Medicine, UCSF San Francisco, CA, United States	A phase 1 clinical trial of in utero enzyme replacement therapy for lysosomal disorders: Interim results
	Moderated Q&A	<i>Xiomara Rosales, Patricia I. Dickson, Eric H. Zanelli, and Akos Herzeg</i>
11:15 AM	WORLDSymposium 2023 Adjourns	

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