Printed posters will be displayed in the Exhibit Hall (Seaport Ballroom), with each presenter assigned a specific presentation day. Poster sessions will feature abstracts from all categories, with daily poster receptions and live Q&A opportunities with the presenters, as outlined below.

Poster Session I will be presented on **Monday, February 1** from **15:30-17:30 PST**

Poster Session II will be presented on **Tuesday, February 2** from **15:30-17:30 PST**

Poster Session III will be presented on **Wednesday, February 3** from **15:30-17:30 PST**

Poster sessions are not organized by abstract category. Presenters selected for Monday, Tuesday, or Wednesday platform presentations will display their posters on the same day as their scheduled platform presentation. All remaining posters, including Contemporary Forum, Rapid-Fire, and Late-Breaking Science abstracts, will be assigned to one of the three poster sessions. Please refer to the Poster List for individual presentation dates and assignments.

ePosters will be available to all registered attendees via the WORLD*Symposium* mobile app beginning at 05:00 PST on Monday, February 1, 2027, and will remain accessible throughout the live meeting. ePosters are also available in the On Demand platform from February 1 through March 12, 2027.

Any poster numbers not listed will not be presented as the author is unable to attend the conference.

It is the policy of WORLD*Symposium* to publish all abstracts with the list of authors exactly as the abstract was submitted to WORLD*Symposium*. The first author of the submitted abstract will be listed as the presenting author on the Preliminary Program, Agenda, and Poster List.

Poster Session 1 — Monday, February 1, 15:30-17:30 PST

#	Author	Title	Kiosk
1	Magy Abdelwahab	A very rare Gaucher disease genotype with an unusual multisystem severe phenotype with 20 years follow up	1-A
2	Magy Abdelwahab	Intrafamilial heterogeneity of Egyptian type III Gaucher disease patients: An observation or evidence based: Long-term follow-up of 12 families	1-B
3	Maria Acosta	Finding hope: A family's path to GM1 gangliosidosis treatment	1-C
4	Heather Adams	Adaptive behavior and cognition in CLN3 disease and relationship to disease stage	1-D
5	Kyung-Min Ahn	Risk-adapted desensitization for hypersensitivity to enzyme replacement therapy in lysosomal diseases: An updated systematic review	2-A
6	George Aldersley	Clinical experience of gastrointestinal and nutritional challenges amongst a neuronal ceroid lipofuscinosis type 2 cohort in a UK treatment centre	2-B
7	Nadia Ali	Healthcare practitioners' experience-based opinions on providing care after a positive newborn screen for Pompe disease - an update on the field	2-C
8	Nadia Ali	Information processing speed in Fabry disease: Preliminary data from the United States	2-D
9	Ian Alix	Gene editing as a therapeutic for GM1 gangliosidosis	3-A
10	Carlos Javier Alméciga-Díaz	In-vitro and in-vivo evaluation of a recombinant N-acetylgalactosamine-6-sulfate sulfatase produced in yeast for mucopolysaccharidosis type IVA	3-B
11	Jose Alvarez Gonzalez	Complex vectors, enzymatic encapsulation, dairy-based nanoparticles	3-C
13	Isidro Arévalo-Vargas	A gene-specific computational framework for interpretation and prioritization of missense variants in lysosomal disorders	3-D
14	Annie Arguello	Correction of sulfamidase deficiency, biomarkers, and cognition in a mucopolysaccharidosis type IIIA (MPS IIIA) mouse model using transferrin receptor-mediated delivery of zafinofusp alfa	4-A
15	Imane Assiri	Identification of a novel SMPD1 exon 6 variant in an infantile case of Niemann-Pick disease type A	4-B
16	Emilly Ataide	Gaucher disease type 3 in Brazil: Clinical, genotypic, and therapeutic characterization	4-C

#	Author	Title	Kiosk
17	Yevgeniya Atiskova	Longitudinal assessment of retinal degeneration in juvenile neuronal ceroid lipofuscinosis type 3 (CLN3)	4-D
18	Jason Babby	Characteristics and disease burden of diagnosed-but-untreated adults with Fabry disease within a US claims database	5-A
19	Jincy Babuji	Role of research nurse specialist in clinical trials in lysosomal disorders	5-B
20	Rafael Badell-Grau	Hematopoietic stem cell gene therapy for mucopolysaccharidosis type IIIC	5-C
21	Daniel Bailey	Increasing physical activity and reducing sedentary behaviour in Fabry disease, a rare genetic condition: Findings from the ACTIVE-FAB feasibility study	5-D
22	Karissa Barthelson	Shared molecular disturbances link lysosomal childhood dementias to adult-onset neurodegeneration	6-A
23	Julia Becerra	Cardiovascular disease progression across treatment modalities in MPS I and MPS IVA	6-B
24	Gretchen Behnke	Recognizing the people advancing rare disease: The Rare Disease Difference Maker program	6-C
25	Gretchen Behnke	Beyond clinical trial endpoints: Complementary real-world evidence approaches to assess treatment impact in rare disease	6-D
26	Gretchen Behnke	Increasing awareness of rare disease clinical trials through the EnCompass database	7-A
27	Laura Bell	Building bridges between rare disease and gastroenterology: The development of the Niemann-Pick type C (NPC) inflammatory bowel disease (IBD) clinical working group	7-B
28	Amy Berger	A phase 1/2, multicenter, open-label study design to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of DNL952 in participants with Pompe disease	7-C
29	Sarah Berger	Comprehensive analysis of brain MRI findings reveals distinctive features in multiple sulfatase deficiency	7-D
30	Elizabeth Berry-Kravis	Long-term disease stability in juvenile-onset Niemann-Pick disease type C treated with adrabetadex in combination with levacetylleucine and miglustat	8-A
31	Elizabeth Berry-Kravis	Lower baseline disease burden is associated with longer survival in adrabetadex-treated participants with infantile-onset Niemann-Pick disease type C	8-B
33	Elizabeth Berry-Kravis	Long-term adrabetadex treatment is associated with neurological stability across multiple functional domains in infantile-onset Niemann-Pick disease type C	8-C
34	Elizabeth Berry-Kravis	Long-term neurological outcomes with intrathecal adrabetadex targeting impaired cholesterol trafficking in juvenile-onset Niemann-Pick disease type C	8-D
35	Hannah Best	Dehydrodolichol diphosphate synthetase and nuclear undecaprenyl pyrophosphate synthase 1 (DHDDS): A combined congenital disorder of glycosylation and lysosomal disorder: Potential for therapy	9-A
36	Maya Bhattiprolu	ERp29, a genome-wide arrayed CRISPR activation screen hit, modifies glucocerebrosidase level and activity in Gaucher disease and Parkinson disease patient-derived iPSC-neurons	9-B
37	Brian Bigger	Heparan sulphate binding peptides, fused to iduronate-2-sulfatase, correct a mouse model of Hunter syndrome	9-C
38	Magnar Bjørås	Nizubaglustat improves disease-associated features in patient-derived retinal organoids modeling CLN3 Batten disease	9-D
39	Sarah Boeke	Systemic rAAV9-hNAGLUop gene replacement therapy for treating MPS IIIB	10-A
40	Shaun Bolton	A global patient-reported and caregiver-reported outcomes resource for Niemann-Pick disease: Characterisation of the INPDR patient reported database	10-B
41	Matthew Bolz-Johnson	Defining Fabry-related fatigue: A qualitative thematic analysis of patient experiences	10-C
42	Victoria Boock	Gys1 targeted substrate reduction therapy with ASOs in combination with enzyme replacement therapy correct glycogen accumulation and autophagy response in skeletal muscles in neonatal Pompe disease mice	10-D
43	Sam Bradbrook	Gallbladder disease in Gaucher disease - an underrecognized co-morbidity	11-A

#	Author	Title	Kiosk
44	Elizabeth Braunlin	Impact of MPS I newborn screening on age of treatment in a high-volume hematopoietic cell transplant center in the US	11-B
45	Nicole Briceno	Two lysosomal genes GBA1 and ATP13A2 interact to regulate glucosylceramide levels in a neuronal model of both Gaucher and Parkinson disease	11-C
46	Bethany Brown	A specialist centre review of premedication utilised for paediatric metabolic patients receiving enzyme replacement therapy and the prevalence of associated infusion-related reactions	11-D
47	Elizabeth Brownnutt	Patient advocacy in a managed access agreement	12-A
48	Laura Buch	Increasing access to genetic testing for lysosomal disorders through artificial intelligence and no-cost genetic testing	12-B
49	Alberto Burlina	Clinical significance of psychosine in newborn screening for Krabbe disease	12-C
50	Barbara Burton	Real-world effectiveness and safety of idursulfase treatment on clinical outcomes in patients with MPS II who initiated treatment before 6 years of age	12-D
51	Barbara Burton	Early initiation of idursulfase treatment improved growth outcomes in the first decade of life in patients with MPS II	13-A
52	Barbara Burton	Early clinical presentation and diagnosis of severe MPS IIIB: Findings from a prospective natural history study	13-B
54	Tamar Caceres	Diagnostic delays following visceral symptom onset in Niemann-Pick type C	13-C
55	Sheridan Campbell	It is hard to plan life around a disease that can be unpredictable: A survey study of daily functioning and psychological distress among patients with Fabry disease	13-D
56	Maria Camprodon-Gomez	Bilateral tibial lengthening with magnetic intramedullary nails in mucopolysaccharidosis type VI: A first case report	14-A
57	Andrew Cao	Engineering allogeneic induced pluripotent stem cells (iPSCs) for microglia-like cellular micropharmacies to deliver systemic and localized enzymes for lysosomal disorders	14-B
58	Sylvaine Cartot-Cotton	Pediatric extrapolation of venglustat in Gaucher disease type 3 (ages 2 to <12 years) supported by population PK (PopPK) and physiologically-based PK (PBPK) modeling	14-C
59	Jessica Casaletto	Rly-1898, the first non-inhibitory, allosteric chaperone of alpha-galactosidase for the treatment of Fabry disease	14-D
60	Krista Casazza.	Expanding the lived experience of Niemann-Pick disease type C: A multilingual international study of patient and caregiver	15-A
61	Krista Casazza.	Incorporating patient and caregiver risk tolerance into therapeutic decision-making in Niemann-Pick disease type C	15-B
62	Krista Casazza.	Toward clinical trial readiness in acid sphingomyelinase deficiency: Current biomarker landscape	15-C
63	Betul Celik	Ex vivo lentiviral engineering of rapidly expanding MSCs enhances GALNS expression, preserves chondrogenic function, and supports HSC recovery in MPS IVA	15-D
64	Betul Celik	A humanized Galns knockout rat model with an AAVS1 knock-in for MPS IVA: rGalnshAAVS1	16-A
65	Samantha Cermignano	Lysosomal disorders in the Plain population	16-B
66	Laurent Chancharme	An open-label phase 1/2 study of miglustat (Batten-1) for the treatment of juvenile Batten disease (CLN3 disease): 18-month clinical results and extended access safety follow-up	16-C
67	Laurent Chancharme	Addressing swallowing-related treatment challenges in Niemann-Pick disease type C: Development of a ready-to-use miglustat oral solution	16-D
68	Prasant Chandran	Design of a phase 2 trial of S-606001 as add-on substrate reduction therapy (SRT) to enzyme replacement therapy (ERT) in late-onset Pompe disease	17-A
69	Christopher Chatham	An integrated biomarker strategy to advance substrate reduction combination therapy in late-onset Pompe disease	17-B

#	Author	Title	Kiosk
71	Yun-Ru Chen	Real-world experience with migalastat in Fabry disease: Clinical and biochemical outcomes from a single center in Taiwan	17-C
72	Tingjen Cheng	Analysis of mannose-rich oligosaccharides through advanced hydrolytic conditions: Potential applications to LDs	17-D
73	Wei-Chieh Cheng	Chemical modification of α-mannosidase to enhance cellular uptake efficiency	18-A
74	Yu-Ting Chiang	Study of natural history among patients with Chinese hotspot late-onset Fabry mutation IVS4+919G>A through family pedigree analysis	18-B
75	Yin-Hsiu Chien	Evaluating the general quality of life using patient-reported outcomes questionnaires in participants with late-onset Pompe disease who switched therapy from alglucosidase alfa to avalglucosidase alfa: A post-hoc analysis from the COMET trial	18-C
76	Selby Chipman	AAV9 gene therapy for infantile GM1 gangliosidosis	18-D
77	Ryan Colburn	Language matters: Pompe is not a neuromuscular disease, nor is there a “late” onset	19-A
78	Tanya Collin-Histed	Beyond reactive diagnosis turning global Gaucher diagnostic challenges into action	19-B
79	Tanya Collin-Histed	Mapping the global LD landscape turning visibility into action	19-C
81	Matthieu Colpaert	Beyond glycogen: Tissue-specific secondary metabolic alterations in the brain and muscle of a Pompe disease mouse model	19-D
82	Stella Cooley	Clinical correlates of psychiatric manifestations in Niemann-Pick disease type C: A retrospective cohort analysis from the International Niemann-Pick Disease Registry	20-A
83	Brittany Cooper	Juvenile idiopathic arthritis in individuals with Gaucher disease	20-B
84	Rebecca Crawford	Beyond muscle weakness: The multidimensional burden of late-onset Pompe disease from the patient perspective	20-C
85	Jordi Cruz	The alpha-mannosidosis journey: An international study of diagnostic challenges and ongoing caregiver burden across 10 countries	20-D
86	Sante Cundari	Patient and caregiver perspectives on meaningful outcomes in Tay-Sachs and Sandhoff (GM2) and GM1 gangliosidosis diseases: Findings from an NTSAD focus group	21-A
87	Precilla D’Souza	Expanded access use of intravenous gene transfer with AAV9-GLB1 in a participant with juvenile GM1 gangliosidosis	21-B
88	Alexis Darling	Gaucher disease and cancer monitoring	21-C
89	Supta Das	Glucocerebrosidase loss alters GPNMB processing in iPSC models of Gaucher disease with Parkinson disease	21-D
90	Magdalena Daurer	From in vitro lysosomal dysfunction to brain pathology in NPC1 preclinical models	22-A
91	Maria Jose De Castro Lopez	Alloimmune membranous nephropathy secondary to sebelipase alfa enzyme replacement therapy in Wolman disease	22-B
93	Patrick Deegan	Measuring what matters consistently: Global monitoring insights to optimize the followME Pompe Journey Registry	22-C
95	Ankit Desai	Anti-drug antibodies in Fabry disease negatively affects clinical response: Is there a role for immune modulation?	22-D
96	Daniel Desalew	Development and clinical cross-validation of an LC-MS/MS assay for lyso-Gb1 and lyso-Gb3 quantification in plasma and dried blood spots	23-A
97	Daniel Desalew	Dose-dependent plasma exposure and food effect of high-dose ambroxol in adults: An LC-MS/MS pharmacokinetic study	23-B
98	Shyeilla Dhuria	Immunogenicity data from a phase 1/2 study of tividenofusp alfa in mucopolysaccharidosis type II (MPS II)	23-C
99	Patricia Dickson	Substrate reduction therapy using siRNA targeting Ext1 and Ext2 reduces lysosomal heparan sulfate burden in brain of Sanfilippo syndrome type B mice	23-D

#	Author	Title	Kiosk
101	Aimee Donald	Measurement characteristics and meaningful change thresholds for mSARA, f-SARA, and RBANS in Gaucher disease type 3 from the phase 3 LEAP2MONO trial	24-A
102	Lucia Dunia Dougherty De Miguel	Diagnostic delay and diagnostic pathways in mucopolysaccharidosis	24-B
104	Christina Dunbar	A recurrent GALC genotype in the South Asian cohort detected by newborn screening: Challenges to navigating the boundary between pseudo deficiency, hypomorphic alleles and late onset Krabbe disease	24-C
105	Sana El Foutat	Implementing biochemical testing for Menkes disease in a Moroccan laboratory: A case report	24-D
106	Sara Elgott	The invisible years: Understanding the diagnostic journey in acid sphingomyelinase deficiency	25-A
107	Alexa Ellis	Navigating concurrent enzyme replacement therapies for dual inherited metabolic disorders: Management considerations for type I Gaucher disease and hypophosphatasia	25-B
109	Kristina Elvidge	Lessons from Australia's collective approach to childhood dementia: A model for other countries	25-C
110	Felicity Emerson	Social work as a critical component of the multidisciplinary care team for lysosomal disorders	25-D
111	Yazmin Enchautegui Colon	Hyperferritinemia is not always hemochromatosis: Type I Gaucher disease diagnosed at age 66	26-A
112	Marlie Enright	Integrated rehabilitation in inherited metabolic disease: Early outcomes of a physiotherapy-led service	26-B
113	Takumi Era	Molecular and histological characterizations reveal two distinct senescent microglia populations in Niemann-Pick disease type C mouse model	26-C
114	Angela Espejo	Preclinical evaluation of a yeast-derived recombinant iduronate-2-sulfatase as a novel enzyme replacement therapy candidate for mucopolysaccharidosis type II	26-D
115	Sabir Es-Said	Complementary HPLC-MS/MS and GC-MS approaches for the biochemical diagnosis of X-linked adrenoleukodystrophy and Zellweger spectrum disorder: A case series in Morocco	27-A
116	Fatih Ezgu	Phase 3 study design: Evaluation of efficacy and safety of zafinofusp alfa (DNL126) in mucopolysaccharidosis type IIIA (MPS IIIA)	27-B
117	Martha Faraguna	Clinically relevant quantification of CNS involvement in classic infantile Pompe disease: External validation and comparison of the Pompe Brain Score and Eichler Score	27-C
118	Sadig Fatullaev	Minimally invasive assessment of liver fibrosis severity in children based on direct biomarkers and calculated indices	27-D
119	Jie Feng	Risk of neuronopathic disease in patients with Gaucher disease identified by comprehensive diagnostic testing: Post hoc analysis of The Lantern Project	28-A
120	Jie Feng	Neurological manifestations relevant to neuronopathic Gaucher disease in the US: A real-world evidence study	28-B
121	Samuela Fernandes	Collateral damage: ERT-associated membranous nephropathy in Pompe disease	28-C
122	Melissa Fetterley	Clinical trials for rare lysosomal diseases	28-D
123	Vianihuini Figueroa-Vazquez	Continuous vs. episodic assessments: How daily eDiary reveals hidden efficacy measurement gaps in pediatric epilepsy trials	29-A
124	Naomi Fisher	High throughput image-based CRISPR screening identifies KDSR as a modifier of glucocerebrosidase protein level and enzyme activity in SH-SY5Y cells	29-B
125	José Francisco Franco	Cerebrovascular manifestations in Fabry disease: From stroke and headache to intracranial aneurysmal changes, a case report	29-C
126	Yasuyuki Fukuhara	Elevated serum lyso-Gb3 levels in patients with mucolipidosis	29-D

#	Author	Title	Kiosk
128	Francesca Fumagalli	Health-related quality of life (HRQoL) outcomes for children with early-onset metachromatic leukodystrophy (MLD) treated with atidarsagene autotemcel (arsa-cel)	30-A
131	Jose Garcia	Population-scale characterization of GBA1 variation in 69,912 Mexican participants of Proyecto oriGen	30-B
132	Timothy Garrett	Lipidomic profiling of peripheral extracellular vesicles as a potential source of biomarkers in Gaucher disease	30-C
133	Dominique Germain	When endpoints fail despite pharmacodynamic activity: Lessons from Fabry disease substrate reduction therapy clinical trials	30-D
134	Dominique Germain	Effect of oral lucerastat monotherapy on kidney Gb3 burden in Fabry disease: Rationale and design of the phase 3 FAB-KLEAR study	31-A
135	Vera Gielen	Wearable technologies in neurological and neuromuscular disorders: Implications for real-world monitoring in late-onset Pompe disease	31-B
136	Léonie Gindre	Development of meaningful digital measures of ambulation and innovative analysis approaches in late-onset Pompe disease	31-C
137	Roberto Giugliani	An integrated clinical-laboratorial guided pathway for the diagnosis of mucopolysaccharidoses: Experience of a Brazilian rare disease laboratory	31-D
139	Roberto Giugliani	Long-term clinical and safety outcomes of nizubaglustat in GM2 gangliosidosis and Niemann-Pick type C diseases: Extended follow-up of the phase 2 RAINBOW study and transition to the open-label PRISMA study	32-A
140	Sarah Goebel	Consensus-based guidelines for the diagnosis and management of free sialic acid storage disorder	32-B
141	Ozlem Goker-Alpan	Early and progressive CNS manifestations in Gaucher disease type 3: A longitudinal natural history study from the ICGG Gaucher registry	32-C
142	Ozlem Goker-Alpan	Management of neuronopathic Gaucher disease: A framework for neurological and systemic monitoring in GD3	32-D
144	Ozlem Goker-Alpan	Abx1100 substrate reduction therapy for patients with late-onset Pompe disease (LOPD): An update on safety and efficacy data from a phase 1b open-label clinical study	33-A
145	Jessica Gold	Delayed treatment for Fabry disease in genotype-negative and seronegative women	33-B
146	Daniel Goldman	Massively-multiplexed functional characterization of lysosomal disease gene variants	33-C
147	Esteban Gonzalez	Age dependent progression of skeletal abnormalities in a murine model of mucopolysaccharidosis type II	33-D
148	Sonia Gonzalez	Preclinical evaluation of an AI-evolved glucocerebrosidase in model of neuronal enzyme deficiency	34-A
149	Sarah Gosling	Co-design of a physical activity and sedentary behaviour intervention for adults with mucopolysaccharidoses	34-B
150	Robert Gotschall	M6P-enhanced acid alpha-glucosidase (GAA) from rapamycin-tolerized T-cell biofactories significantly reduces cardiac, skeletal muscle, and brain glycogen in Pompe disease mice	34-C
151	Calum Grant	Addressing health equity in undiagnosed Fabry disease using case finding in electronic health records	34-D
152	Christina Grant	Vestibular schwannoma in a long-term survivor of infantile onset Pompe disease	35-A
153	Nathan Grant	The needs of patients and families with mucopolysaccharidosis (MPS) during the transition from pediatric to adult health care	35-B
154	Nathalie Guffon	Thirteen-year multisystemic outcomes of elosulfase alfa in mucopolysaccharidosis type IVA: A prospective real-world cohort from a single French reference center	35-C
155	Nathalie Guffon	Sustained multisystem benefits of long-term laronidase monotherapy in severe and attenuated mucopolysaccharidosis type I	35-D

#	Author	Title	Kiosk
156	Nathalie Guffon	Multidomain natural history of MPS IIIC: Baseline neuroimaging and one-year neurodevelopmental trajectories in 10 French patients	36-A
158	Kristen Hagarty-Waite	Plasma glial fibrillary acidic protein is an early indicator of central nervous system involvement in cross-reactive immunologic material-negative patients with infantile-onset Pompe disease	36-B
159	Leanne Hagen	Poor disease control in Gaucher disease with substrate reduction therapy: A limited case series	36-C
160	Takashi Hamazaki	Safety and efficacy of approved pabinafusp alfa for the treatment of mucopolysaccharidosis type II (MPS II): A post-marketing clinical study of Japanese patients	36-D
161	Miloud Hammoud	Identification of novel phytosphingosine-based hexosylceramides associated with a novel GALC variant in a Moroccan patient with Krabbe disease	37-A
163	Mollie Harrison	Secondary mitochondrial dysfunction in alpha-mannosidosis: Linking lysosomal dysfunction to bioenergetic impairment and therapeutic targeting	37-B
165	Caroline Hastings	Phase 3 study to evaluate intravenous hydroxy-propyl-beta-cyclodextrin (IV-HPβCD) in pediatric and adult patients with Niemann-Pick disease type C1 (TransportNPC): Safety results	37-C
166	Nadene Henderson	Overlapping diagnoses: Inquisitive care unveils other diagnoses in Fabry disease	37-D
167	Nadene Henderson	Real-world experiences with pegunigalsidase alfa: Disease history, treatment patterns, and patient perspectives from a 100-patient US Fabry disease survey	38-A
169	Julia Holdorp	Switch from alglucosidase alfa to cipaglucosidase alfa plus miglustat in Pompe disease improves cellular uptake but not plasma half-life	38-B
170	Geu-Ru Hong	Patient-reported outcomes and Mainz Severity Score index results from cohort 1 of a phase 1/2 trial of HM15421/GC1134A, a novel, monthly, subcutaneous enzyme replacement therapy for the treatment of Fabry disease	38-C
173	Robert Hopkin	Real-world experiences with pegunigalsidase alfa: Patient perspectives on symptom impact and treatment switching from a 100-respondent survey in Fabry disease	38-D
174	Shih-Chang Hsueh	Enhancing binding affinity of psychosine to α-cyclodextrin and evidence of prenatal efficacy in the murine model of Krabbe disease	39-A
175	Jared Hysinger	Autologous transplantation of genome-edited hematopoietic stem cells combined with AAV for Krabbe disease	39-B
176	Christine I Dali	Open-label, randomized pharmacokinetic interaction study of arimoclomol and miglustat in healthy volunteers	39-C
196	Shih-Hsin Kan	Single-vector AAV delivery enables homology-directed recombination-mediated genome editing for Pompe disease	39-D
204	Virginia Kimonis	Correction of β-mannosidosis with CNS-directed transferrin receptor-targeted enzyme replacement therapy in a *Manba*$^{-/-}$ mouse model	40-A
226	Veronica Lentini	Constitutive lysosomal trafficking defects and stimulus-dependent autophagic and secretory dysfunction in Gaucher disease macrophages	40-B
256	Simona Murko	A novel biomarker for newborn screening of alpha-mannosidosis	40-C
327	Livia Sertori Finoti	Microglia co-culture reduces neuronal glycosaminoglycan accumulation in a human model of multiple sulfatase deficiency	40-D

Poster Session 2

Tuesday, February 2, 15:30-17:30 PST

#	Author	Title	Kiosk
12	Isidro Arévalo-Vargas	Pilot selection of in silico predictors for intronic variants in genes associated with lysosomal disorders	1-A

#	Author	Title	Kiosk
53	Marissa Cabay	Accelerating gene therapy development for ultra-rare diseases: The Accelerating Medicines Partnership Bespoke Gene Therapy Consortium regulatory playbook	1-B
70	Xin Chen	Favorable efficacy and safety in preclinical studies pave the way for AAV9/HGSNAT gene therapy for MPS IIIC	1-C
80	John Collyer	Longitudinal evaluation of spinal pathology through magnetic resonance imaging in mucopolysaccharidosis type II: A ten-year cohort study	1-D
94	Claudia Beatriz Del Toro Runzer	Preclinical safety, efficacy, and GMP-scale manufacture of CRISPR/Cas9-edited stem cells for Gaucher disease type 1	2-A
129	Ellen Fung	Effects of 72 weeks of vosoritide on growth velocity and body proportions in children with MPS IVA and VI: Interim results	2-B
130	Jessica Gambardella	Hypertension unmasks bioenergetic vulnerability, driving exaggerated cardiac hypertrophy in Fabry disease	2-C
162	Paul Harmatz	Tralesinidase alfa treatment is associated with sustained reductions in neurodegeneration biomarkers in mucopolysaccharidosis type IIIB: Long-term data	2-D
168	Wendy Heywood	Plasma NULISA proteomics identifies disease-specific signatures and a shared neurodegenerative profile across lysosomal disorders	3-A
172	Justin Hopkin	Report of N-acetyl-L-leucine (NALL) for the neurologic manifestations of ASMD	3-B
177	Asli Inci	Analysis of transcriptomic data for the etiopathogenesis in neuropathic Gaucher patients	3-C
178	Tatsunori Itabashi	Improvement of left ventricular wall thickening in type II Gaucher disease following enzyme replacement therapy	3-D
179	Margarita Ivanova	Impact of pregnancy on osteopenia and osteoporosis risk in women with Gaucher disease	4-A
180	Margarita Ivanova	Glycosphingolipid accumulation is linked to complement network remodeling and lectin pathway activation in Fabry disease	4-B
181	Maroua Jakani	Severe lysosomal acid lipase deficiency in Morocco: A stepwise biochemical diagnostic approach	4-C
183	Meredith James	The North Star Assessment for limb-girdle type muscular dystrophies (NSAD) as a monitoring tool for Pompe disease: Insights from UK physiotherapists	4-D
184	John Jefferies	Cardiac arrhythmias and conduction blocks as early predictors of left ventricular hypertrophy in Fabry disease	5-A
185	Xuntian Jiang	Discovery of a novel lactosylceramide phosphate as biomarker for Niemann-Pick type C1 disease	5-B
186	Franklin Johnson	Adjusted migalastat dose regimens in participants with Fabry disease and severe renal impairment, or end-stage renal disease with hemodialysis or hemodiafiltration: Pharmacokinetic and safety data from the second interim analysis of the RENEW study	5-C
187	Franklin Johnson	An open-label phase IIIB study (AT1001-033; ASPIRE II) to evaluate the safety, pharmacokinetics, pharmacodynamics and efficacy of migalastat in pediatric patients with Fabry disease: First interim analysis	5-D
188	Tyler Johnson	Patterns of diagnosis in adults with Fabry disease from US claims data	6-A
189	Tyler Johnson	Defining the distinct clinico-genomic phenotype among individuals carrying the A143T GLA variant	6-B
190	Deepthy Joseph	The impact of cannabinoid hyperemesis syndrome in the management of enzyme replacement therapy infusion associated reactions	6-C
191	Deepthy Joseph	Alpha mannosidosis: A multidisciplinary best interests framework for complex surgical decision making in patients with reduced cognitive capacity	6-D
192	Rana Jreich	Leveraging model-based evidence to enrich clinical data for avalglucosidase alfa in infantile-onset Pompe disease	7-A

#	Author	Title	Kiosk
193	Kristina Julich	Case report of siblings with infantile-onset Niemann-Pick type C (I-NPC) initiating adrabetadex at different ages and intervals from disease onset	7-B
194	Staci Kallish	The patient voice: Poems from the Fabry community	7-C
195	Staci Kallish	13-year experience with Fabry disease at the Penn Medicine LSD Clinic	7-D
197	Ilkka Kantola	Pulmonary involvement: A forgotten manifestation of Fabry disease	8-A
198	Max Kappler	Comparative transcriptomic analysis of GBA1-KO zebrafish and mouse brains reveals shared neuroinflammatory pathways in neuronopathic Gaucher disease	8-B
200	Tatsuya Kato	Cytometric analysis of patient plasma biomarkers enables minimally invasive monitoring of MPS I cardiovascular disease	8-C
201	Jon Kenniston	Computationally guided GALNS engineering paired with transferrin receptor-targeted biodistribution in MPS IVA	8-D
202	Kenedy Khatri	Association between triglyceride-to-HDL cholesterol ratio and glycohemoglobin among U.S. adults: A cross-sectional National Health and Nutritional Examination Survey (NHANES) study	9-A
203	Grace Kick	Combined CNS directed and systemic gene therapy treats both the brain and bowel dramatically extending the lifespan of a CLN2 mouse model	9-B
206	Caroline Kistorp	MODERN: A multi-country observational study of the real-world safety and effectiveness of pegunigalsidase alfa in patients with Fabry disease	9-C
207	Evan Kleinboehl	Translational development of engineered T cell micropharmacies for sustained enzyme delivery in lysosomal disorders	9-D
208	Agatha Kliman	Variant-specific generation of GBA1 p.L483P and p.N370S HEK293T cell models to enable optimization of mutation-correction strategies	10-A
209	Rohit Kohli	Acidsphingomylinase deficiency (ASMD) improvement with enzyme replacement as measured by non-invasive liver transient elastography	10-B
210	Anja Köhn	The Sanfilippo Clinical Assessment Score (SANCA-Score): Development and prospective evaluation of a disease-specific clinical score for mucopolysaccharidosis type III	10-C
211	Motomichi Kosuga	Safety and pharmacodynamic effects of JR-446 in mucopolysaccharidosis type IIIB: 12-month follow-up from first 5 participants of an ongoing phase 1/2 study	10-D
213	David Kronn	Understanding challenges and opportunities in the Pompe disease diagnostic journey: A targeted literature review	11-A
214	Hani Kushlaf	Baseline disease burden and gaps in real-world monitoring: Insights from the followME Pompe Journey Registry US sites	11-B
216	Hani Kushlaf	Patient characteristics associated with timing and trajectory of 6-minute walk distance response to cipaglucosidase alfa plus miglustat in late-onset Pompe disease: A post hoc analysis of PROPEL and ATB200-02 using minimal clinically important differences	11-C
217	Sandra Kyosen	Real-world adherence patterns and structural barriers in enzyme replacement therapy for lysosomal diseases: Longitudinal analysis of 80 patients at a Brazilian reference center	11-D
218	Christina Lampe	What matters most in alpha-mannosidosis treatment? An international discrete choice experiment study among clinicians and patients/caregivers	12-A
219	Dawn Laney	Progressive renal, cardiac, and cerebrovascular involvement in untreated adults with Fabry disease: Evidence from the Fabry Registry	12-B
222	Chris Lee	A novel human cell platform accumulating endogenous psychosine for GALC variant profiling and therapeutic testing in Krabbe disease	12-C
223	Danielle Lee	No a-synuclein pathology found at autopsy in two octogenarians with type I Gaucher disease	12-D
224	Seonhwa Lee	Impact of agalsidase alfa therapy on cardiac function in patients with Fabry cardiomyopathy: A prospective multimodality assessment of exercise hemodynamics beyond structural remodeling	13-A

#	Author	Title	Kiosk
225	Malte Lenders	Influence of anti-drug antibodies on the long-term efficacy of enzyme replacement therapy in Fabry disease	13-B
227	Emil Lenzing	Are we using the optimal eGFR equation in Fabry? Creatinine-based equations versus measured GFR in 1,177 paired measurements	13-C
228	Emil Lenzing	Lyso-Gb3 measured in plasma and dried blood spot may not be interchangeable: A comparison of two commercial assays in Fabry disease	13-D
229	Austin Letcher	Living with cystinosis as an adult: A qualitative study of the patient experience	14-A
230	Drew Levy	Association between early treatment initiation and 6-minute walk test outcomes in individuals with classical MPS IVA treated with elosulfase alfa in the Morquio A Registry Study (MARS)	14-B
231	Daniel Lewi	Towards global consensus clinical management guidelines for GM2 gangliosidosis: An international multidisciplinary initiative	14-C
232	Daniel Lewi	When the pieces don't fit: The clinical trial puzzle in rare disease	14-D
234	Billie Lianoglou	Recruitment and enrollment experience in a first-in-human phase 1 trial of in-utero enzyme replacement therapy for lysosomal disorders	15-A
235	Aleš Linhart	Pain and mental health burden in untreated patients with Fabry disease: A longitudinal analysis of patient-reported outcomes from the followME Fabry Pathfinders Registry	15-B
236	Aleš Linhart	Comparative efficacy and tolerability of pegunigalsidase alfa in patients transitioning from agalsidase beta: Insights from the F20/BALANCE and F60/BRILLIANCE trials	15-C
237	Gisela Linthorst	What matters most to patients in clinical development? insights from a community engagement workshop exploring patient and caregiver priorities in Niemann-Pick disease type C during development of the NAVIGATE phase 3 trial	15-D
238	Austin Lobitz	The role of body composition in clinical monitoring of individuals with Pompe disease	16-A
239	Charles Lourenco	Double the trouble: Dual diagnosis in Fabry disease	16-B
240	Yung-Hsiu Lu	Newborn screening-enabled therapeutic window in late-onset Fabry disease: Long-term divergent remodeling trajectories with early-stage treatment	16-C
241	Leslie Lynch	Self-reported quality of life improves in children with MPS IVA and MPS VI after 48 weeks of treatment with vosoritide	16-D
242	Amro Maarouf	Demographic, genotypic and treatment characteristics of a large UK single-centre Fabry disease cohort	17-A
243	Shauna Mangum	Patient voices in Gaucher disease type 1: A qualitative analysis	17-B
244	Thorsten Marquardt	Assessing real-world use of arimoclomol in Niemann-Pick disease type C: Design of the EU Early Access Program	17-C
245	Caroline Marre	Characterizing CLN3 disease progression and burden: Contemporary evidence and remaining gaps	17-D
246	Caroline Marre	Niemann-Pick disease type C in the US and Canada: Persistent disease burden and unmet needs in an evolving treatment landscape	18-A
247	Angela Martin Rios	Prophylactic immune tolerance induction in two patients with neuronopathic mucopolysaccharidosis type II treated with tividenofusp alfa	18-B
248	Saki Matsushima	Translational development of a BBB-penetrating AAV gene therapy for GM1 gangliosidosis	18-C
249	Mackenzie McNeely	Characterizing GAA gene variation and its implications for Pompe disease	18-D
251	Eugen Mengel	Arimoclomol in adolescents and adults with Niemann-Pick disease type C: Clinical trial and US Early Access Program experience	19-A
253	Pramod Mistry	Age-dependent treatment effects of venglustat on neurological endpoints in Gaucher disease type 3: Predictive modeling and pediatric extrapolation	19-B
255	Jasper Mullenders	PLA2G15 inhibition as a novel therapeutic concept for CLN3 Batten disease	19-C

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257	Nicole Muschol	Long-term auditory function outcomes in children with mucopolysaccharidosis type IIIB treated with tralesinidase alfa	19-D
260	Samira Najeh	From hypoglycemia to diagnosis: The combined contribution of GC-MS and acylcarnitine profiling in HMG-CoA lyase deficiency	20-A
261	Leyla Namazova-Baranova	Safety, pharmacokinetics, pharmacodynamics, and efficacy of verenafusp alfa in patients with mucopolysaccharidosis type II: Interim results of a phase 2-3, multicenter, open-label, multicohort study	20-B
262	Joshua Nederveen	Exercise and respiratory training drive functional gains in late-onset Pompe disease, with a multi-ingredient supplement improving lean-to-fat mass ratio	20-C
264	Peter Nordbeck	Comparative effectiveness of migalastat versus enzyme replacement therapy in patients with classic or classic-like Fabry disease: Results from the followME Fabry Pathfinders Registry	20-D
265	Lasair O'Callaghan	Qualitative insights into Gaucher disease type 3: Exit interviews from the LEAP2MONO study	21-A
266	Lasair O'Callaghan	Diagnosed prevalence of Fabry disease in the United States and its stratification by age, sex, and treatment status: An administrative claims analysis	21-B
267	Antonio Ochoa-Ferraro	Enhancing enzyme replacement therapy: Faster infusion rates across the UK	21-C
268	Tsubsasa Oguni	Evaluation of dried blood spot glycosaminoglycans as second-tier test in newborn screening for MPS I and II in Japan	21-D
269	Ilyas Okur	From developmental impairment to functional dependency in severe MPS IIIB: Differential loss of function across childhood	22-A
270	Cara O'Neill	Impact of counselor-facilitated parent support program on psychosocial symptoms in families affected by Sanfilippo syndromes: Early learnings from first cohort	22-B
271	Cara O'Neill	Stakeholder-informed design and implementation of SanfilippoLINK, a global longitudinal research registry for Sanfilippo syndrome type III (mucopolysaccharidosis type III)	22-C
272	Cara O'Neill	Genomic newborn screening identifies Sanfilippo syndrome type A at five weeks despite a normal routine newborn screen: A family-centered case for screening expansion	22-D
275	Sadettin Öztürk	Consanguinity-driven cascade genetic screening in Fabry disease: Identifying heterogeneous age groups for timely treatment initiation	23-A
276	Arjun Panchagatti	Pain dynamics in Fabry disease: Effect of sex and age insights from longitudinal analyses	23-B
277	Amanda Pasqualotto	In silico analyses of the indirect regulation of GBA1 gene expression by circular RNAs (circRNAs): Are circRNAs potential biomarkers for Gaucher disease?	23-C
279	Mary Pavlou	We must do better: A global survey of the experiences of women with Fabry disease	23-D
280	Ana Paula Pedroso Junges	Long-term effectiveness of migalastat for the treatment of Fabry disease: A systematic review and meta-analysis	24-A
282	Magali Pettazzoni	Negative amniotic-fluid screening does not rule out Niemann-Pick disease type C	24-B
283	Magali Pettazzoni	Prenatal recognition of perinatal-lethal Gaucher disease: A 24-year French referral cohort	24-C
284	Nathan Phan	Neurological manifestations and functional impact among mucopolysaccharidosis carriers: Results from a cross-sectional survey	24-D
285	Stacy Pike-Langenfeld	Optimizing a constrained genetics workforce through smart technology-enabled virtual genetics model	25-A
286	Nishitha Pillai	Frequent longitudinal biomarker monitoring supports personalized treatment in a child with infantile-onset Pompe disease	25-B
287	Nishitha Pillai	Biochemical and familial evidence supporting pathogenicity of IDS p.Arg493Cys in an adolescent with an attenuated MPS II phenotype	25-C
288	Nishitha Pillai	Missed newborn diagnosis and the elephant in the room	25-D

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289	Nishitha Pillai	Intrafamilial variation in clinical outcomes associated with treatment initiation in four siblings with Morquio syndrome type A	26-A
291	Sarah Pinkley	The new imperative for frequent biomarkers	26-B
292	Lorenzo Pinto	Lipid profile in patients with Gaucher disease type 1: A retrospective cohort study at a Brazilian reference center	26-C
294	Juan Politei	Redefining pain in Fabry disease	26-D
295	Fabiano Poswar	Evaluation of serum biomarkers after treatment with losartan in patients with mucopolysaccharidosis types IVA and VI	27-A
296	Fabiano Poswar	Development of an artificial intelligence-assisted clinical dashboard for longitudinal monitoring of Fabry disease	27-B
297	Jackson Pountney	Examining the reliability and validity of the Niemann-Pick type C quality of life questionnaire for adults and children	27-C
298	Jackson Pountney	Validation of a revised lay-reported Niemann-Pick disease type C Clinical Severity Scale	27-D
299	Carlos Prada	Myostatin as a candidate biomarker of early muscle-growth dysregulation in children with Pompe disease	28-A
300	Rashi Priya	Transferrin receptor (TfR) targeted acid a-glucosidase (GAA) delivery corrects multi-tissue pathology and restores motor function in a Pompe disease mouse model	28-B
301	Vraj Rabadia	Velmanase alfa reduces oligosaccharide burden more extensively in peripheral than CNS tissues and improves neurobehavioral outcomes in an alpha-mannosidosis mouse model	28-C
302	Uma Ramaswami	Outcomes from the Fabry Outcome Survey (FOS) in patients with late-onset cardiac GLA variants	28-D
303	Mark Roberts	Baseline disease burden and real-world monitoring practices: Global insights from the followME Pompe Journey Registry	29-A
304	Sonia Roca-Esteve	Proteomic profiling for the identification of hepatic fibrosis biomarkers in patients and carriers of lysosomal acid lipase deficiency	29-B
305	David Rogers	Long-term outcomes using intravitreal cerliponase alfa in children with neuronal ceroid lipofuscinosis type 2	29-C
306	Beverly Romero	Understanding outcomes important to families: Conceptual disease models of GM1 gangliosidosis type 2a and type 2b	29-D
307	Aviva Rosenberg	Primary care provider understanding of lysosomal disorder diagnosis and treatment options	30-A
308	Courtney Rouse	Dose dependent responses of a capsid mutated AAV8 codon optimized NAGLU vector for treatment of Sanfilippo syndrome type B using the murine model	30-B
309	Pierantonio Russo	Making the invisible visible: An integrated real-world evidence framework to capture the full burden of mucopolysaccharidosis types II and III and Niemann-Pick disease type C	30-C
310	Anastasia Rykunova	Neuroimaging markers of the severity of mucopolysaccharidoses in children	30-D
311	Lili Salinas	Preliminary biochemical evaluation of AAV9 multimodal gene therapy in a canine MPS I model	31-A
313	Wendy Santos-Modesitt	Comparison of early treatment with UX111 (rebisufligene etisparvovec) and MPS IIIA natural history, a case study	31-B
314	Saikat Santra	Gastrointestinal disease after long-term enzyme replacement therapy in Wolman disease	31-C
315	Carla Sargiotto	Isolated ocular manifestations in a multigenerational family with mucopolysaccharidosis type II	31-D
316	Maurizio Scarpa	Development of a clinical decision support tool for diagnosis and longitudinal management of Sanfilippo syndrome	32-A
317	Maurizio Scarpa	Development of LSD EarlyAlert, a clinical decision support tool for early recognition of lysosomal disorders in children aged 1 month to 5 years	32-B



#	Author	Title	Kiosk
318	Jessica Scherr	Comparing developmental outcomes of children with CLN2 Batten disease treated with cerliponase alfa	32-C
319	Raphael Schiffmann	In silico identification of specific downstream mechanisms in Fabry disease	32-D
321	Matthew Schultz	Urinary sulfatide analysis: Test performance and outcomes	33-A
323	Abinaya Seenivasan	Miglustat treatment in paediatric CLN3 disease: Two-year clinical outcomes from a real-world cohort	33-B
324	Yonatan Serlin	Longitudinal echocardiographic changes in GBA1-associated Parkinson disease	33-C
325	Irene Serrano-Gonzalo	A multiplex LC-MS/MS approach for the comprehensive biochemical profiling of lysosomal disorders	33-D
326	Irene Serrano-Gonzalo	Treatment-associated modulation of systemic inflammatory indices in Gaucher disease: Contrasting patterns with acute systemic inflammation	34-A
328	Raghu Rai Sharma	Extracellular vesicle profiling in individuals carrying GBA1 variants: A biomarker discovery approach	34-B
329	Raghu Rai Sharma	GBA1 variants influence gut microbiome composition in Gaucher disease models	34-C
330	Stefanie Siebert-Krassnig	Broad biomarker profiling across preclinical models of lysosomal disorders	34-D
331	Mordechai Slae	Longitudinal analysis of patients with infantile-onset lysosomal acid lipase deficiency treated with sebelipase alfa enzyme replacement therapy	35-A
332	Grace Smith	Artificial intelligence driving genetic genealogy in our lysosomal world	35-B
333	Lachlan Smith	Markerless motion capture-based gait analysis in mucopolysaccharidosis patients	35-C
335	Barbara Soberon	High scores on a clinical-case questionnaire do not translate into diagnostic suspicion: A clinical simulation study on early diagnosis of lysosomal disorders in pediatrics	35-D
336	Young Bae Sohn	Real-world safety and effectiveness of eliglustat in Korean patients with Gaucher disease: Results from a post-marketing surveillance study	36-A
337	Shreyas Soma	Multimodal imaging characterization of skeletal tissues in mucopolysaccharidosis type II: A cadaveric study	36-B
338	Nattachat Srikongyos	Insights from three new cases of beta-mannosidosis a rare lysosomal disorder	36-C
340	Richard Steet	Hypomorphic IDUA genotypes are associated with retinitis pigmentosa in individuals without syndromic mucopolysaccharidosis type I	36-D
341	Karolina Stepien	Lipidomics analysis: A novel approach to identify patients with lysosomal disease–preliminary results from one metabolic centre	37-A
342	Karolina Stepien	Study in progress: A retrospective and prospective natural history study of patients with fucosidosis	37-B
343	Baodong Sun	Retargeting the cell surface receptor with an engineered IgF2-fusion platform enhances therapeutic enzyme delivery to skeletal muscle in Pompe disease	37-C
345	Hidehiko Suzuki	Development and preclinical evaluation of brain-targeted AAV for neuronal ceroid lipofuscinoses	37-D
346	Leah Marie Svarny	Machine learning-based mechanistic phenotyping in Gaucher disease for precision identification and treatment of osteoporosis	38-A
348	Jennifer Taylor	Impact of newborn screening on clinical outcomes in children with mucopolysaccharidosis type I	38-B
349	Heather Teal	From patient voice to patient navigation: Translating access research into support for the Niemann-Pick disease community	38-C
350	Sophie Thomas	Living with childhood dementia in the UK: Family experiences, unmet needs and priorities for action	38-D

#	Author	Title	Kiosk
351	Sophie Thompson	A call to action: The burden of Fabry-associated clinical event (FACE) and major adverse cardiovascular event (MACE) before diagnosis or first assessment and the potential of algorithmic severity scoring: Data from the STRIDE study	39-A
352	Camilo Toro	Charting the course: A natural history study of sialidosis type 1	39-B
353	Lindsay Torrice	Early initiation of tividenfusp alfa in two newborns with severe MPS II: Contrasting experiences from a single clinic	39-C
362	Raquel van Gool	Mapping the disease network in Niemann-Pick type C: Biomarkers across brain structure, function, and muscle	39-D
379	Slawomir Wantuch	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	40-A
389	Chester Whitley	PS gene-editing of brain-penetrant synthetic hexosaminidase reduces brain gangliosides in GM2-gangliosidosis (Tay-Sachs and Sandhoff diseases)	40-B
399	Hongling Zhu	Pronounced therapeutic benefit of liver-directed AAV gene therapy for CNS delivery of β-hexosaminidase A in Sandhoff disease	40-C
400	Xuling Zhu	Preclinical proof-of-concept of treatment for Fabry disease via precise RNA-mediated insertion of a GLA transgene	40-D

Poster Session 3

Wednesday, February 3, 15:30-17:30 PST

#	Author	Title	Kiosk
32	Elizabeth Berry-Kravis	Intrathecal adrabetadex slows disease progression in participants with infantile-onset Niemann-Pick disease type C	1-A
108	Kristina Elvidge	Uniting lysosomal and other causes of childhood dementia under a shared definition: A global Delphi consensus	1-B
138	Roberto Giugliani	Long-term effect of nizubaglustat on seizure frequency in GM2 gangliosidoses: End-of-study data from the phase 2 RAINBOW study	1-C
143	Ozlem Goker-Alpan	Interim results from a phase 1/2 trial of HM15421/GC1134A, a novel, monthly, subcutaneous enzyme replacement therapy for the treatment of Fabry disease	1-D
164	Caroline Hastings	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	2-A
205	Priya Kishnani	Improved clinical outcomes and safety with avalglucosidase alfa versus alglucosidase alfa in infantile-onset Pompe disease: The Baby-COMET external comparator arm analysis	2-B
212	Motomichi Kosuga	Efficacy and safety of combined hematopoietic stem cell transplantation and intraventricular enzyme replacement therapy for severe mucopolysaccharidosis type II	2-C
215	Hani Kushlaf	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	2-D
220	Heather Lau	Rebisufligene etisparvovec (UX111) led to long-lasting improvements in neurodevelopmental function in children with MPS IIIA	3-A
221	Michael Lawlor	Muscle histopathology following AT845 gene replacement therapy in adults with late-onset Pompe disease: Findings from the phase 1/2 FORTIS trial	3-B
252	Pramod Mistry	Venglustat demonstrates consistent clinical benefits in neurological manifestations of Gaucher disease type 3 (GD3) in the LEAP2MONO study	3-C
254	Sara Mitchell	An AI-driven multilayer model of lung involvement in neuronopathic Gaucher disease (nGD): Validation in a 45-patient cohort and implications for management	3-D

#	Author	Title	Kiosk
258	Nicole Muschol	Long-term outcomes of tralesinidase alfa enzyme replacement therapy in patients with mucopolysaccharidosis type IIIB	4-A
263	Peter Nordbeck	Lucerastat effect on cardiac function in patients with Fabry disease: Results from the open label-extension (OLE) of the phase 3 clinical program	4-B
273	Cara O'Neill	Quality of life and family impact in severe Sanfilippo syndrome type B (MPS IIIB): Longitudinal findings from a prospective natural history study	4-C
274	Paul Orchard	Extended laronidase enzyme activity after hematopoietic cell transplantation in children with Hurler syndrome	4-D
278	Marc Patterson	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	5-A
322	Angela Schulz	Cerliponase alfa for the treatment of CLN2 disease: Combined results from two ongoing observational studies	5-B
334	Barbara Soberon	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)	5-C
339	Orna Staretz-Chacham	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	5-D
354	Antonio Toscano	Improvement in outcomes following switch from alglucosidase alfa to avalglucosidase alfa in late-onset Pompe disease: Pompe Registry analysis	6-A
356	Assel Tulebayeva	Birth prevalence and disease-causing genetic variants of mucopolysaccharidoses in Kazakhstan	6-B
357	Eloise Tysoe	A single centre cohort review of complications associated with gastrostomy insertion in patients with mucopolysaccharidosis type III	6-C
358	Giuseppe Uras	Serum proteomic profiling reveals a coordinated interferon-inflammatory and extracellular matrix signature in Fabry disease	6-D
359	Leslie Urdaneta	Beyond the positive screen: Patient advocacy and clinical perspectives on follow-up gaps after newborn screening for MPS I and MPS II	7-A
360	Johanna M.P. van den Hout	Baseline characteristics of participants with mucopolysaccharidosis type II enrolled in the phase 2/3 COMPASS study of tividenofusp alfa	7-B
361	Raquel van Gool	Circulating immune-metabolic and neurotrophic signatures reflect motor dysfunction and muscle integrity in Pompe disease	7-C
363	Amber Van Oudtshoorn	A proactive family screening pathway for earlier Fabry disease diagnosis	7-D
364	Delphine Vandame	Miglustat and swallowing outcomes in Niemann-Pick disease type C: A comprehensive review and potential relationship with survival	8-A
365	Nirubhan Veeraraghavan	Managing IgE-mediated hypersensitivity to olipudase alfa: Omalizumab-supported tailored desensitisation and intensive premedication	8-B
366	Dana Velasquez Rivas	Lyso-Gb1 first: Time to rethink the diagnostic algorithm for Gaucher disease	8-C
367	Biliana Veleva-Rotse	Multisystem correlations and functional capacity in Fabry disease: Insights from the STRIDE study	8-D
368	Shrijay Vijayan	Development of PLX-300 (cinnamic acid), as an orally administered therapy for juvenile neuronal ceroid lipofuscinosis (JNCL)	9-A
369	Jesús Villarrubia	Evaluation of PROMs and PREMs questionnaire for ASMD care	9-B
370	Daniel Virga	Preclinical evaluation of a durable, tissue-targeted gene therapy for Pompe disease	9-C
371	Adam Vogel	Communication phenotypes as markers of neurological involvement in lysosomal diseases	9-D
372	Adam Vogel	Acoustic, perceptual, and neural signatures of dysarthria in Niemann-Pick disease type C and Pompe disease	10-A

#	Author	Title	Kiosk
373	Adam Vogel	Communication outcomes in Rett syndrome: Evidence gaps and opportunities for therapeutic trials	10-B
374	Erika Vucko	Long-term safety of vestronidase alfa in patients with mucopolysaccharidosis type VII	10-C
375	Susan Waisbren	Adaptive functioning outcomes in mucopolysaccharidosis type IIIB patients receiving tralesinidase alfa enzyme replacement therapy	10-D
376	Helen Waller-Evans	Targeting acid ceramidase for therapeutic modulation of lysosomal sphingolipid pathology	11-A
377	Huaping Wang	Gaucher disease remains substantially underdiagnosed in the United States: Insights from a nationwide closed-claims database	11-B
378	Pei-Yi Wang	Substantial cardiac remodeling and functional exercise capacity improvement in a treatment naive IVS4+919G>A Fabry disease Taiwanese patient treated with pegunigalsidase alfa: 1-year follow-up	11-C
380	Cara Weismann	From data generation to data utility: A lifecycle framework for responsible data sharing in lysosomal diseases	11-D
381	Michael West	Female heterozygotes with classic Fabry disease-how much enzyme replacement therapy? A retrospective analysis from the Canadian Fabry Disease Initiative Registry	12-A
382	Michael West	Tolerability of 1-hour infusions of pegunigalsidase alfa in Fabry disease: Data from the F60/ BRILLIANCE trial	12-B
383	Lena Marie Westermann	Novel extended motor and language rating scales for granular clinical assessment in CLN2 disease	12-C
384	Amy White	KrabbeCURES: A five-year review of initial outcomes from a patient-centered registry	12-D
385	Amy White	Newborn screening can improve clinical outcomes for late-infantile Krabbe disease	13-A
386	Hannah White	The impact of an iterative points-based framework on variant classification outcomes for lysosomal disorders (LDs)	13-B
387	Ineka Whiteman	Caregiver experiences with enzyme replacement therapy, treatment burden, and emerging therapies in CLN2 Batten disease	13-C
388	Chester Whitley	Monitoring CSF heparan sulfate analyses to assess treatment of the heparan sulfate neurodegenerative disorders	13-D
390	Christopher Wingrove	Exploring the lived experience of the Fabry community in South Korea	14-A
391	Melissa Wolf	Impact of rebisufligene etisparvovec (UX111) on neurodevelopmental function and related symptoms of MPS IIIA	14-B
392	Annabel Wong	A multiplex urine glycosaminoglycans LC-MS/MS method with developed ratios that can discriminate between the different mucopolysaccharidoses disorders	14-C
393	Shaun Wood	Characterization of the retinal phenotype in a mouse model of HGSNAT deficiency	14-D
394	Hiroyuki Yamakawa	Development of a Fabry disease screening tool using a health screening support app	15-A
395	Chia-Feng Yang	Improvements in multiple clinical outcomes and airway abnormalities in Pompe disease: Insights from an 18-year clinical cohort and 3 years of experience switching to avalglucosidase alfa	15-B
396	Jordan Zeiger	Early treatment initiation of MPS VII diagnosed prenatally by beta-glucuronidase enzyme activity in amniocytes	15-C
398	Hongling Zhu	Trem2-dependent microglial activation is protective in Sandhoff disease	15-D