The McKusick-Nathans Department of Genetic Medicine Presents

Inaugural Advances in Genetic and Genomic Medicine 2025 Conference December 10th 2025

Session 1: Opening Session Start End Title 8:30 AM 8:35 AM Welcome and Conference Goals Na	Speaker
	Speaker
8:30 AM 8:35 AM Welcome and Conference Goals Na	
	ara Sobreira, MD PhD
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8:50 AM 9:20 AM 2025 Morning Keynote Address	
· · · · · · · · · · · · · · · · · · ·	ilary Vernon, MD PhD
9:20 AM 9:30 AM Discussion / Question & Answer An	ndy McCallion, PhD
9:30 AM 9:45 AM Break	
	oderator: Jefferson Doyle
Start End Title	Speaker
9:45 AM 9:55 AM Myelin lipid composition in adrenoleukodystrophy Mentor: Ali Fatemi, MD (KKI)	adav Weinstock, MD PhD
9:55 AM 10:00 AM Discussion / Question & Answer Kri	rista Schatz, MS CGC
10:00 AM 10:10 AM Cell death pathways in lower motor neurons in spinal muscular atrophy Mentor: Charlotte Sumner, MD PhD	adeline Aldridge, BS
10:10 AM 10:15 AM Discussion / Question & Answer Ele	ena Gallo, PhD
10:15 AM 10:25 AM Mitophagy as a developmental pathology in the heart in Barth syndrome Ma Mentor: Hilary Vernon, MD PhD	adison Santizo, BA
10:25 AM 10:30 AM Discussion / Question & Answer Ce	esar Fortes-Lima, PhD
10:30 AM 10:40 AM Uncovering disease mechanisms with long-read and methylation patterns Ga Mentor: Nara Sobreira, MD PhD	abriel Gama, PhD
10:40 AM 10:45 AM Discussion / Question & Answer Hu	ungoo Lee, PhD
10:45 AM 10:55 AM A human iPSC-derived skeletal muscle model of Barth Syndrome Va Mentor: Hilary Vernon, MD PhD	aleria A. Silva
10:55 AM 11:00 AM Discussion / Question & Answer Ta	ao Wang, MD PhD
11:00 AM 11:15 AM Break / Refreshments	
Session 3: Complex Disease Mo	oderator: Nara Sobreira
Start End Title	Speaker
11:15 AM 11:25 AM Uncovering genetic predictors of kidney dysfunction in sickle cell disease Fu Mentor: Ambroise Wonkam, MD PhD	ujr Osman
11:25 AM 11:30 AM Discussion / Question & Answer Ch	hristy Smith, ScM CGC
11:30 AM 11:40 AM Genetic studies of hearing loss in African populations Ab Mentor: Ambroise Wonkam, MD PhD	odoulaye Yalcouye, MD PhD
11:40 AM 11:45 AM Discussion / Question & Answer An	my Patterson, MS CGC
11:45 AM 11:55 AM Uncovering the nuclear genetic basis of mitochondrial DNA heteroplasmy Ra Mentor: Dan Arking, PhD	ama Alhariri
11:55 AM 12:00 PM Discussion / Question & Answer Lis	sa Kratz, PhD
12:00 PM 12:10 PM Clonal hematopoiesis is adaptive and convergent Eri Mentor: Mary Armanios, MD PhD	ric Liu, MS
12:10 PM 12:15 PM Discussion / Question & Answer Ne	eeraj Sharma, DVM PhD
12:15 PM 12:25 PM HIF1-regulated PPME1 expression promotes breast cancer via AKT/βCatenin Ya	ajing Lyu
Mentor: Greg Semeza, MD PhD	
12:25 PM 12:30 PM Discussion / Question & Answer Je	efferson Doyle, MD PhD
12:30 PM 1:30 PM Lunchbreak and Poster Session in Turner Concourse	

Start End Deep learning prediction of transposable element exonization in human genomes and montor: Litilana Florea, PhD Zittong He 1:40 PM 1.45 PM Discussion / Question & Answer Mike Beer, PhD 1:45 PM 1.55 PM CRISPRI/a in zebrafish for systemic biological exploration of genes implicated in disease implicated in disease minimort. Andy McCallion, PhD Nelson Barrientos 1:55 PM 2:00 PM Discussion / Question & Answer Shira Ziegler, MD PhD 2:00 PM 2:10 PM SpliSync: Genomic language model-driven splice correction of long RNA sequencing reads whentor: Liliana Florea, PhD Winston Timp, PhD 2:10 PM 2:15 PM Discussion / Question & Answer Winston Timp, PhD 2:25 PM 2:30 PM Discussion / Question & Answer Winston Timp, PhD 2:25 PM 2:30 PM Discussion / Question & Answer Weiyi Mu, ScM 2:40 PM Mentor: Nerraj Sharma, DVM PhD Weiyi Mu, ScM 2:40 PM Discussion / Question & Answer Karen Raraigh, MGC CGC 2:45 PM 3:00 PM Discussion / Question & Answer Karen Raraigh, MGC CGC 2:45 PM 3:10 PM Allele-specific genome editing Moderator: Nara Sobreting </th <th>12:30 PM</th> <th>1:30 PM</th> <th>Lunchbreak and Poster Session in Turner Concourse</th> <th></th>	12:30 PM	1:30 PM	Lunchbreak and Poster Session in Turner Concourse	
1:30 PM 1:40 PM genomes Mentor: Liliana Florea, PhD Discussion / Question & Answer Mentor Spiliana Florea, PhD Discussion / Question & Answer Mentor: Liliana Florea, PhD Discussion / Question & Answer Mentor: Andy McCallion, PhD Discussion / Question & Answer Spiliana Florea, PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Mentor: Andy McCallion, PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Mentor: Vasiliki Machairaki, MSc PhD Discussion / Question & Answer Mentor: Vasiliki Machairaki, MSc PhD Discussion / Question & Answer Weight Municipal Mentor: Neeraj Sharma, DVM PhD Discussion / Question & Answer Mentor: Neeraj Sharma, DVM PhD Discussion / Question & Answer Mentor: Neeraj Sharma, DVM PhD Discussion / Question & Answer Moderator: Nara Sobreira, Moderator: Nara Sobreira, Mentor: Greg Newby, PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Discussion / Question & Answer Weight Machairaki, MSc PhD Di	Session 4	: Genetic	_	Moderator: Jefferson Doyle
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1:55 PM 2:00 PM Discussion / Question & Answer Shira Ziegler, MD PhD 2:00 PM 2:10 PM SpliSync: Genomic language model-driven splice correction of long RNA Mentor: Liliana Florea, PhD Wui Wang Lui 2:10 PM 2:15 PM Discussion / Question & Answer Winston Timp, PhD 2:15 PM 2:25 PM Novel EV-based biomarkers from IPSC neurons for Alzheimer's disease Mentor: Vasiliki Machairaki, MSc PhD Waqar Ahmed, PhD 2:25 PM 2:30 PM Discussion / Question & Answer Weiyi Mu, ScM 2:30 PM 2:40 PM Mentor: Neeraj Sharma, DVM PhD Ana Topasna 2:40 PM 2:45 PM Discussion / Question & Answer Karen Raraigh, MGC CGC 2:45 PM 3:00 PM Statt End Title Speaker 3:00 PM 3:10 PM Allele-specific genome editing Mentor: Greg Newby, PhD Ana Topasna Moderator: Nara Sobrein Speaker 3:15 PM 3:25 PM Discussion / Question & Answer Vasiliki Machairaki, MSc PJ Julia Johansson 3:25 PM 3:30 PM Discussion / Question & Answer Vasiliki Machairaki, MSc PJ Julia Johansson 3:25 PM 3:30 PM Discussion / Question & Answer Greg Newby, PhD 3:30 PM Discussion	1:45 PM	1:55 PM	implicated in disease	Nelson Barrientos
2:00 PM 2:10 PM 2:10 PM sequencing reads	1:55 PM	2:00 PM	•	Shira Ziegler, MD PhD
2:15 PM 2:15 PM Novel EV-based biomarkers from iPSC neurons for Alzheimer's disease Mentor: Vasiliki Machairaki, MSc PhD Discussion / Question & Answer Weiyi Mu, ScM Ana Topasna And therapeutics Mentor: Neeraj Sharma, DVM PhD Discussion / Question & Answer Karen Raraigh, MGC CGC 2:45 PM 3:00 PM Break / Refreshments Satat End Treatments Moderator: Nara Sobreira Speaker Johannes Dohr, PhD Mentor: Greg Newby, PhD Jiscussion / Question & Answer Vasiliki Machairaki, MSc P Julia Johannsson Wentor: Jefferson Doyle, MD PhD Discussion / Question & Answer Greg Newby, PhD Jiscussion / Question & Answer Julia Johannsson Wentor: Jefferson Doyle, MD PhD Jiscussion / Question & Answer Greg Newby, PhD Jiscussion / Question & Answer Julia Johannsson Wentor: Jefferson Doyle, MD PhD Jiscussion / Question & Answer Greg Newby, PhD PhD Jiscussion / Question & Answer Julia Johannsson Wentor: Nara Sobreira, MD PhD Jiscussion / Question & Answer Julia Johannsson Wentor: Nara Sobreira, MD PhD Jiscussion / Question & Answer Julia Hoover-Fong, MD Noah Workman Wentor: Greg Newby, PhD Base and prime editing methods to correct GNE myopathy Noah Workman Wentor: Greg Newby, PhD Discussion / Question & Answer Julia Hoover-Fong, MD Noah Workman Wentor: Greg Newby, PhD Discussion / Question & Answer Julia Hoover-Fong, MD Noah Workman Wentor: Greg Newby, PhD Discussion / Question & Answer Julia Hoover-Fong, MD PhD Mentor: Bert Vogelstein, MD Mentor: Bert Vogelstein, MD Mary Armanios, MD PhD Mentor: Neeraj Sharma, DVM PhD Mentor: Neeraj Sharma, DVM PhD Mentor: Neeraj Sharma, DVM PhD Discussion / Question & Answer Ada Hamosh, MD Nara Sobreira, MD PhD Discussion / Question & Answer Ada Hamosh, MD Nara Sobreira, MD PhD Discussion / Question & Answer Ada Hamosh, MD Nara Sobreira, MD PhD Discussion / Question & Answer Ada Hamosh, MD Nara Sobreira, MD PhD Discussion / Question & Answer Ada Hamosh, MD Nara Sobreira, MD Ph	2:00 PM	2:10 PM	sequencing reads	-
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2:30 PM 2:45 PM and therapeutics Mentor: Neeraj Sharma, DVM PhD 2:40 PM 2:45 PM Discussion / Question & Answer 2:45 PM 3:00 PM Break / Refreshments Session 5: Genetic Treatments Start End Title Speaker 3:00 PM 3:10 PM Allele-specific genome editing Mentor: Greg Newby, PhD 3:10 PM 3:15 PM Discussion / Question & Answer 3:15 PM 3:25 PM Hepatic targeted OAT normalizes plasma ornithine in gyrate atrophy mice Mentor: Defferson Doyle, MD PhD 3:25 PM 3:30 PM Discussion / Question & Answer 3:40 PM 3:45 PM Discussion / Question & Answer 3:45 PM 3:55 PM Base and prime editing methods to correct GNE myopathy Mentor: Greg Newby, PhD 3:55 PM 4:00 PM Discussion / Question & Answer 4:00 PM 4:10 PM Logic-gated CAR-T cells for the treatment of relapsed AML post-transplant Mentor: Bert Vogelstein, MD 4:10 PM 4:25 PM Small molecule & modulators circumvent NMD of nonsense CFTR variants Mentor: Neeraj Sharma, DVM PhD 4:25 PM 4:30 PM Discussion / Question & Answer 4:30 PM A:30 PM Discussion / Question & Answer 4:30 PM A:30 PM Discussion / Question & Answer 4:30 PM A:30 PM Discussion / Question & Answer 4:30 PM A:30 PM Discussion / Question & Answer 4:30 PM A:30 PM Discussion / Question & Answer 4:30 PM A:30 PM Discussion / Question & Answer 4:30 PM A:30 PM Discussion / Question & Answer 4:30 PM A:30 PM Discussion / Question & Answer 4:30 PM A:30 PM Discussion / Question & Answer 4:30 PM A:30 PM Discussion / Question & Answer 4:30 PM A:30 PM Discussion / Question & Answer 4:30 PM A:30 PM Discussion / Question & Answer 4:30 PM A:30 PM Discussion / Question & Answer 4:40 PM A:30 PM Discussion / Question & Answer 4:40 PM A:30 PM Discussion / Question & Answer 4:40 PM A:30 PM Discussion / Question & Answer 4:40 PM A:30 PM Discussion / Question & Answer 4:40 PM A:30 PM Discussion / Question & Answer 4:40 PM A:30 PM Discussion / Question & Answer 4:40 PM A:30 PM Discussion / Question & Answer 4:40 PM A:30 PM Discussion / Question & Answer 4:40 PM A:30 PM Discussion / Question & Answer 4:40 PM A:30 PM Dis	2:25 PM	2:30 PM		Weiyi Mu, ScM
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2:45 PM 3:00 PM Session 5: Genetic Treatments Session 5: Genetic Treatments Start End Title Speaker 3:00 PM 3:10 PM Allele-specific genome editing Mentor: Greg Newby, PhD 3:10 PM 3:15 PM Discussion / Question & Answer 3:15 PM 3:25 PM Hepatic targeted OAT normalizes plasma ornithine in gyrate atrophy mice Mentor: Jefferson Doyle, MD PhD 3:25 PM 3:30 PM Discussion / Question & Answer 3:30 PM 3:40 PM Effects of HIF1A & VHL variants in Ollier disease and Maffucci syndrome Mentor: Nara Sobreira, MD PhD 3:40 PM 3:45 PM Discussion / Question & Answer 3:40 PM Discussion / Question & Answer 4:400 PM Discussion / Question & Answer	2:40 PM	2:45 PM	· · · · · · · · · · · · · · · · · · ·	Karen Raraigh MGC CGC
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