Curriculum Vitae

PERSONAL DATA

Name: Kent Lai

Birthplace: Hong Kong Citizenship: U.S.A.

EDUCATION

Years	Degree/ Diploma	Institution (Area of Study)
1984 - 1986	Diploma	Hong Kong Polytechnic (Medical Laboratory Sciences), Hong Kong
		(Diploma also awarded by British Technical and Education Council)
1986 - 1988	B.Sc. (Hons.)	University of Bradford (Biomedical Sciences)
	Upper Second Class	Bradford, United Kingdom
1988 - 1994	Ph.D.	University of Maryland (Molecular & Cell Biology)
		Baltimore, Maryland, U.S.A.
1994 - 1997	Postdoctoral Training	Emory University School of Medicine (Biochemical Genetics)
		Atlanta, Georgia, U.S.A.
1997 - 2000	MBA, MHA	Georgia State University (Business/ Health Administration)
		Atlanta, Georgia, U.S.A.

BOARD CERTIFICATIONS

LICENSES/CERTIFICATIONS

British Technical & Education Council (BTEC) Diploma in Medical Laboratory Sciences

ACADEMIC HISTORY

Pediatrics (Pediatric Genetics)

03/01/2009 Hire, Tenure Track, Associate Professor

PROFESSIONAL EXPERIENCE

Full Time Positions

1997 - 1999	Instructor, Emory University School of Medicine, Department of Pediatrics, Division of Medical
	Genetics, Atlanta, Georgia, U.S.A.
1999 - 2002	Research Assistant Professor, Emory University School of Medicine, Department of Pediatrics,
	Division of Medical Genetics, Atlanta, Georgia, U.S.A.
2002 - 2008	Assistant Professor, University of Miami School of Medicine, Department of Pediatrics, Division of
	Medical Genetics, Miami, Florida, U.S.A.
2008 - 2009	Associate Professor, University of Miami School of Medicine, Department of Pediatrics, Division of
	Medical Genetics, Miami, Florida, U.S.A.
2009 - 2014	Associate Professor, University of Utah School of Medicine, Department of Pediatrics, Division of
	Medical Genetics, Salt Lake City, Utah, U.S.A.
2014 - Present	Professor (Tenured), University of Utah School of Medicine, Department of Pediatrics, Division of
	Medical Genetics, Salt Lake City, Utah, U.S.A.
2018 - Present	Adjunct Professor, University of Utah College of Health Sciences, Department of Nutrition and
	Integrative Physiology, Salt Lake City, Utah, U.S.A.

Part Time Positions

Last Updated: 10/10/2023

Editorial Experience

2011 - 2018	Associate Editor for Open Journal of Preventive Medicine
2012 - 2017	Associate Editor for <i>Dataset Papers in Biology</i>
2016 - 2018	Editorial Board Member for Heliyon
2017 2020	Associate Editor for RMC Medical Constics

2017 - 2020 Associate Editor for *BMC Medical Genetics* 2020 - present Associate Editor for *BMC Medical Genomics*

2023 - present Academic Editor for *PLOS ONE*

Reviewer Experience

Ad hoc Reviewer for African Journal of Microbiological Research

Ad hoc Reviewer for Annals of Human Genetics

Ad hoc Reviewer for Antioxidants and Redox Signaling

Ad hoc Reviewer for Applied Biochemistry and Biotechnology

Ad hoc Reviewer for BAOJ Pediatrics

Ad hoc Reviewer for BBA Molecular Basis of Disease

Ad hoc Reviewer for BBA Proteins & Proteomics

Ad hoc Reviewer for Biochemistry

Ad hoc Reviewer for Biochimie

Ad hoc Reviewer for Brain and Behaviour Research

Ad hoc Reviewer for Bioorganic Chemistry

Ad hoc Reviewer for Bioorganic Medicinal Chemistry Letters

Ad hoc Reviewer for Biotechniques

Ad hoc Reviewer for BMC Medical Genetics

Ad hoc Reviewer for Brain Communications

Ad hoc Reviewer for Current Aging Science

Ad hoc Reviewer for Disease Models and Mechanisms

Ad hoc Reviewer for European Journal of Medicinal Chemistry

Ad hoc Reviewer for Gene

Ad hoc Reviewer for Genetics in Medicine

Ad hoc Reviewer for Human Mutation

Ad hoc Reviewer for International Journal of Molecular Sciences

Ad hoc Reviewer for *IUBMB Life*

Ad hoc Reviewer for Journal of Advanced Research

Ad hoc Reviewer for Journal of Inherited Metabolic Diseases

Ad hoc Reviewer for Journal of Inherited Metabolic Diseases Reports

Ad hoc Reviewer for Journal of Molecular Modeling

Ad hoc Reviewer for Journal of Pediatrics

Ad hoc Reviewer for JSM Neurosurgery and Spine

Ad hoc Reviewer for Mini Reviews in Medicinal Chemistry

Ad hoc Reviewer for Military Medical Research

Ad hoc Reviewer for Molecular and Genomic Medicine

Ad hoc Reviewer for Molecular Genetics and Metabolism

Ad hoc Reviewer for Molecular Genetics and Metabolism Reports

Ad hoc Reviewer for Open Journal of Preventive Medicine

Ad hoc Reviewer for *Pediatric Genetics*

Ad hoc Reviewer for Pediatric Research

Ad hoc Reviewer for PLOS One

Ad hoc Reviewer for The Journal of Biological Chemistry

Ad hoc Reviewer for The Journal of Virology and Antiviral Research

SCHOLASTIC HONORS

Travel Award for Poster Presentation at the Annual Yeast Genetics & Molecular Biology Meeting, Madison, Wisconsin, U.S.A.

2001	Travel Award for Research Presentation at the Annual Meeting of The Society for Inborn Errors of
	Metabolism, Miami, Florida, U.S.A.
2003	Madelon Ravlin Memorial Award for Research, The Woman's Cancer Association of The University
	of Miami, U.S.A.
2022	Recognized Senior Researcher, Rare Diseases Clinical Research Network (RDCRN), U.S.A.
2022	Finalist, Foundation of NIH Bespoke Gene Therapy Consortium Request for Proposal
2023	Outstanding Alumni Award, Department of Health Technology and Informatics, Hongkong
	Polytechnic University

ADMINISTRATIVE EXPERIENCE

Administrative Duties

2003 - 2006	Member, University Administrative Services Committee, University of Miami School of Medicine
2004 - 2009	Board member, American Heart Association Miami-Dade Community Board
2007 - 2009	Member, Medical School Admissions Committee, University of Miami School of Medicine
2010 - 2013	Member, Faculty Budget and Planning Advisory Committee, University of Utah
2012 - Present	Member, Medical School Admissions Interview Committee, University of Utah School of Medicine
	(120+ interviews conducted to date)

Professional Organization & Scientific Activities

Grant Review Committee/Study Section

2007 - Present Research Grant Reviewer, Research Committee, Galactosemia Foundation (formerly Parents of	
Galactosemic Children, Inc.), Mandeville, Louisiana, U.S.A.	
2008 Project Grant Reviewer, The Wellcome Trust (U.K.)	
2010 - 2017 Member, American Heart Association Western States Peer Review Study Section	
2010 Project Grant Reviewer, National Children's Research Centre (Ireland)	
2010 Project Grant Reviewer, Irish Health Research Board (Ireland)	
Ad hoc Reviewer, National Institutes of Health (NIH) 2012/01 ZRG1 GGG-R (80) R Study Section 2011	on
Ad hoc Reviewer, National Institutes of Health (NIH) Special Emphasis Panel ZRG1 IDM S (02)	
Ad hoc Grant Reviewer, The Croatian Science Foundation (HRZZ) (2014)	
Ad hoc Grant Reviewer, University of Missouri SPINAL CORD INJURIES RESEAR	RCH
PROGRAM (SCIRP)	
Ad hoc Grant Reviewer, NIH SBIR201615	
Ad hoc Grant Reviewer, NIH SBIR201701	
Ad hoc Grant Reviewer, NIH SBIR201705	
Ad hoc Grant Reviewer, NIH SBIR201801	
Ad hoc Grant Reviewer, NIH SBIR201815	
Ad hoc Grant Reviewer, American Heart Association (Lipids-Basic Science Committee)	
Ad hoc Grant Reviewer, American Heart Association (Lipids-Basic Science Committee)	
Ad hoc Grant Reviewer, NIH Special Emphasis Panel for PA-20-207	
Ad hoc Grant Reviewer, University of Utah Office of VP Research Seed Grant	
Ad hoc Grant Reviewer, Swiss National Science Foundation (Switzerland)	
Ad hoc Grant Reviewer, University of Utah Office of VP Research Seed Grant	
Ad hoc Grant Reviewer, Queen's University (Canada) Seed Grant Program	
Ad hoc Grant Reviewer, American Heart Association Scientific Focus Research Network (Chr	onic
Stress and Cardiovascular Diseases)	

Symposium/Meeting Chair/Coordinator

PROFESSIONAL COMMUNITY ACTIVITIES

2016- Present Member, GalNet (Amsterdam, The Netherlands)

UNIVERSITY COMMUNITY ACTIVITIESSERVICE AT AFFILIATED INSTITUTIONS

SERVICE AT PREVIOUS INSTITUTIONS

2001 - 2002	Member, Management Team, Emory Genetics Laboratory, Emory University, U.S.A.
2002 - 2005	Committee Member, Research Committee, The Dr. John T. MacDonald Foundation Center for
	Medical Genetics, University of Miami School of Medicine, U.S.A.
2003 - 2006	Committee Member, University Administrative Services Committee, University of Miami School of
	Medicine, U.S.A.
2007 - 2009	Committee Member, Medical School Admissions Committee, University of Miami School of
	Medicine, U.S.A.

CURRENT/PAST MEMBERSHIPS IN PROFESSIONAL SOCIETIES

American Society of Human Genetics Sigma Xi, The Scientific Research Society American Association for the Advancement of Science Society for Inherited Metabolic Disorders (SIMD) American Heart Association

<u>FUNDING</u> [due to non-disclosure agreements (NDA) set up between various industrial partners and the University of Utah, the names of the industrial partners cannot be disclosed to the public without prior approvals.]

Active Grants/ Contracts

2021-2023	NIH 1R21HD104056-01A1 Advancing a novel mRNA-based therapy for Classic Galactosemia (Role: PI) (US\$420,750 total / 2 years)
2021-2024	Sponsored Research Agreement from Industry. (Role: PI) (US\$480,000 total/ 3 year)
2021-2024	Sponsored Research Agreement from Industry (Role: PI) (US\$1.36M total/ 3 years)
2022-2024	FCDGC Pilot/Feasibility Grant (Role: Co-PI) (US\$50,000/ 1 year)
2023-2024	Sponsored Research Agreement from Industry (Role: PI) (US128,000 / 2 years)
2023-2023	Sponsored Research Agreement from Industry (Role: PI) (US\$141,250 / 1 year)
2023-2024	Primary Children Foundation Bridge Grant (Role: PI) (US\$54,000/ 1 year)

Pending

R21HD- Optimal Window of Opportunity for Granulosa Cell Gene Therapy in Galactosemia (Pending Council Review. Impact Score: 22; Percentile: 7) (Role: Lead/Contact PI)

R01HL- Pathobiological mechanisms of cardiac disease in PGM1-CDG (Pending Council Review. Impact Score: 35; Percentile: 12) (Role: Lead/Contact PI)

Past Grants/Contracts

04/01/1992-	Grant-in-aid for Research
03/31/1993	Principal Investigator: Kent Lai
	Total Costs: \$200
	Sigma Xi, The Scientific Research Society
07/01/1998-	Molecular analysis of GALT gene mutations in human differentiated cell lines
06/30/2002	Principal Investigator: Louis J. Elsas
	Total Costs: \$325,000
	Nicholas Rochat Research Foundation for Galactosemia
	Role: Co-Investigator
08/01/2001-	Seed Grant, Emory University School of Medicine

07/31/2002 Principal Investigator: Kent Lai Total Costs: \$50,000 Emory-Egleston Children's Research Center Metabolic inhibition of cancer metastasis. Principal Investigator: Kent Lai 01/01/2003-12/31/2003 Total Costs: \$50,000 Madelon Ravlin Memorial Award, The Women's Cancer Association, University of Miami Regulatory Role of Galactose Metabolism in Protein Glycosylation 08/01/2004 -07/31/2007 Principal Investigator: Kent Lai Total Costs: \$240,000 Scientist Development Grant, American Heart Association Greater Southeast Affiliate Regulatory Role of Galactose Metabolism in Epidermal Growth Factor Receptor (EGFR) Expression. 05/01/2004-Principal Investigator: Kent Lai 04/31/2005 Total Costs: \$30,000 Sylvester Comprehensive Cancer Center, University of Miami 06/01/2004-Controlling cancer growth by decreasing cellular UDP-hexose concentrations. Principal Investigator: Kent Lai 05/31/2005 Total Costs: \$25,000 The Woman's Cancer Association, University of Miami 05/15/2007 -NIH 5 RO1 HD054774-6 Innovative Therapies and Clinical Studies for Classic Galactosemia. 06/30/2013 Principal Investigator: Kent Lai Total Costs: \$1.530.000 National Institutes of Health 10/01/2008 -NIH 1 RO3 MH085689-01 Toward Improved Therapy for Classic Galactosemia. 09/30/2009 Principal Investigator: Kent Lai Total Costs: \$25,000 National Institutes of Health NIH 3 R01 HD054744-04S1 Innovative Therapies and Clinical Studies for Classic Galactosemia. 10/01/2009 -Principal Investigator: Kent Lai 09/30/2011 Total Costs: \$168.856 National Institutes of Health (ARRA Stimulus Act Administrative Supplement) 06/01/2012-**Unrestricted Gift** 05/31/2013 Principal Investigator: Kent Lai Total costs: \$20,000 Agios Pharmaceuticals, Inc. (U.S.) Primary Children's Medical Center Foundation Bridge to Success Award 07/01/2012-06/30/2013 Principal Investigator: Kent Lai Total costs: \$50,000 Primary Children's Medical Center Foundation 09/03/2012 -NIH 1 RO 1HD074844-01 Toward Improved Therapy for Classic Galactosemia. 06/30/2015 Principal Investigator: Kent Lai Total Costs: \$928,563 National Institutes of Health 01/01/2013-Targeting Galactose Metabolism in Cancers 12/31/2013 Principal Investigator: Kent Lai Total costs: \$28,974 University of Utah VP for Research Seed Grant Program 05/01/2013 -Characterization of a New Mammalian Animal Model of Classic Galactosemia

04/30/2014 Principal Investigator: Kent Lai

Total Costs: \$33,000 Galactosemia Foundation

Warrior Benefits

Total costs: US\$ 9,000 (unrestricted research gift)

Role: Principal Investigator

2014 Agios Pharmaceuticals Inc.

Total costs: US\$ 30,000 (unrestricted research gift)

Role: Principal Investigator

2015 Warrior Benefits (The Dershem Family)

Total costs: US\$ 5,000 (unrestricted research gift)

Role: Principal Investigator

2015 Galactosemia Foundation Research Grant

Total costs: US\$ 50,000 Role: Principal Investigator

2015 K2R2R Award (Intermountain Healthcare & Dept. of Pediatrics, U. of Utah)

Total costs: US\$ 50,000 Role: Principal Investigator

2016 Race for Jase (The Dershem Family)

Total costs: US\$ 3,000 (unrestricted research gift)

Role: Principal Investigator

2016-2017 K2R2R (Intermountain Healthcare & Dept. of Pediatrics, U. of Utah)

Total Costs: US\$ 49,900 Role: Principal Investigator

2017-2020 NIH R01 "Towards Improved Therapy for Classic Galactosemia"

Total Costs: US\$1,140,000/3 years

NIH

Role: Principal Investigator

2017 Race for Jase (The Dershem Family)

Total costs: US\$ 9,000 (unrestricted research gift)

Role: Principal Investigator

2016-2018 Research Contract with a Boston-based pharmaceutical company (name of the company has to be

withheld due to non-disclosure agreement in place)

Total Costs: US\$369,896 Role: Principal Investigator

K2R2R (Intermountain Healthcare & Dept. of Pediatrics, U. of Utah)

Total Costs: US\$ 45,000 Role: Principal Investigator

Race for Jase (The Dershem Family)

Total costs: US\$ 6,000 (unrestricted research gift)

Role: Principal Investigator

University of Utah Center for Clinical & Translational Science Pilot Grant Program

Targeting Endoplasmic Reticulum Stress in Classic Galactosemia

Total costs: US\$ 30,000/ one year Role: Principal Investigator

2019 Galactosemia Foundation (U.S.A.) Research Grant Program

Total costs: US\$ 49,5000 Role: Principal Investigator

2019 Race for Jase (The Dershem Family)

Total costs: US\$ 6,000 (unrestricted research gift)

Role: Principal Investigator

2019 Consultancy Agreement for Biomarin Pharmaceuticals (2 years)

Total Costs: US\$36,000

Role: Consultant

2019 Atomwise Inc.

Total Costs: US\$20,000/1 year

Role: Co-investigator

2021-2022 University of Utah College of Pharmacy Therapeutics Catalyst Grant

Total Costs: US\$30,000/1 year Role: Principal Investigator

2021-2022 Galactosemia Foundation Research Grant

Total Costs: US\$50,000/ 1 year Role: Principal Investigator

2021-2022 University of Utah Office of VP of Research Incentive Seed Grant Program

Total CostUS\$50,000/ 1 year Role: Principal Investigator

TEACHING RESPONSIBILITIES/ASSIGNMENTS

Education Administration

Course and Curriculum Development

Courses Directed Course Lectures

2013-2016 Lecturer for the MD ID 7350 Course for 2nd Year Medical Students at the University of Utah

Clinical Teaching

2003 - 2004 Invited Faculty Speaker, Clinical Scientist Training Program, University of Miami School of

Medicine (Audience: Department of Pediatrics Residents and Fellows)

Laboratory Teaching Small Group Teaching Trainee Supervision

Post-doctoral Fellows/ MD Residents

2003 - 2004 Supervisor (Research), Deborah Barbouth, M.D., Department of Pediatrics, U. of Miami SOM.

2005 - 2006 2005 - 2006 2010 -2011 2010 -2017 2014-2019	Trainee's Current Career Activities: Associate Professor of Pediatrics, University of Miami. Advisor/Mentor, Avner Ittah, Ph.D., Department of Pediatrics, U. of Miami SOM. Trainee's Current Career Activities: Lecturer of Biochemistry, Miami Dade Community College. Supervisor (Research), Klaas Wierenga, M.D., M.S., Department of Pediatrics, U. of Miami SOM. Trainee's Current Career Activities: Senior Consultant, Mayo Clinic Advisor/Mentor, Sina Odejinmi, Ph.D., Department of Pediatrics, U. of Utah SOM. Trainee's Current Career Activities: Lecturer at the Salt Lake Community College Advisor/Mentor, Manshu Tang, Ph.D., Department of Pediatrics, U. of Utah SOM. Trainee's Current Career Activities: Staff Research Associate, University of Utah. Advisor/Mentor, Bijina Balakrishnan, Ph.D., Department of Pediatrics, U. of Utah SOM. Trainee's Current Career Activities: Research Scientist, University of Utah
<u>Undergraduates</u> 1997	Advisor/Mentor, Amy Catherine Willis, Undergraduate Research (1997) "The 1997 Summer Undergraduate Research Experience" (SURE), a 12 week-long program jointly sponsored by The Howard Hughes Medical Institutes, National Science Foundation and Emory University The research initiated at the summer has been completed and is published. Lai K, Willis AC, & Elsas LJ, 1999. "Biochemical Roles of Glutamine-188 of human galactose-1-phosphate uridyltransferase." The Journal of Biological Chemistry 274: 6559-6566. Trainee's Current Career Activities: Ms. Willis has completed the M.D., Ph.D. program at the Medical College of Georgia, Augusta, GA, and is an Associate Professor of Neurology at University of Alabama at Birmingham, AL.
2004	Advisor/Mentor, Freda Addo, Department of Biomedical Engineering, University of Miami School of Medicine The research initiated at the summer has been completed and is published. Slepak T, Tang M, Addo F, & Lai K, 2005. "Intracellular accumulation of galactose-1-phosphate leads to environmental stress response in a yeast model" Molecular Genetics and Metabolism 86: 360-371. Trainee's Current Career Activities: Dr. Addo was accepted to the M.D. program of University of Texas Medical Center in 2006.
2008	Advisor/Mentor, Yohance Allette, Department of Biological Sciences, University of Maryland and The Leadership Alliance Program Trainee's Current Career Activities: Mr. Allette graduated from the M.D., Ph.D. student at the Indiana University School of Medicine in 2018
2009 - 2010	Advisor/Mentor, Nick Gallegos, University of Utah LEAP program Trainee's Current Career Activities: Mr. Gallegos was accepted to the DDS program at the School of
2010 - 2011	Dentistry at the University of Virginia. Advisor/Mentor, Rafael Rascon, University of Utah LEAP program. Trainee's Current Career Activities: Mr. Rascon was accepted to the DDS program at Marquette University School of Dentistry, WI in 2016.
2012	Advisor/Mentor, Seth Baffoe, University of Utah. University of Utah LEAP program. Trainee's Current Career Activities: Adjunct Assistant Professor at the Department of Health Administration, University of Maryland.
2012 - 2015	Advisor/Mentor, Nisa Fraser, University of Utah Trainee's Current Career Activities: Ms. Fraser was accepted to the DO Program at Rocky Vista University College of Osteopathic Medicine, CO in 2015. She graduated in 2019 and is currently a resident at the School of Medicine at the University of Southern California
2012 - 2016	Advisor/ Mentor, Wyman Chen, University of Utah. Trainee's Current Career Activities: Mr. Chen graduated from the DDS program at the University of Utah School of Dentistry in 2020 and is currently a dentist in private practice.
2014 - 2016	Advisor/Mentor, Rose Caston, Dartmouth College Trainee's Current Career Activities: Ms. Caston was accepted to the MD/PhD program of the University of Utah School of Medicine in Fall, 2017

2015 - 2018	Advisor/ Mentor, Enoabasi Etokidem, University of Utah.
	Trainee's Current Career Activities: Ms. Etokidem was accepted to the MD program of the
	University of California Los Angeles on a full scholarship in Fall, 2018. She graduated in 2022 and
	is currently a medical resident.
2016 - 2017	Advisor/ Mentor, Merry Feng, University of Utah.
2017 2021	Trainee's Current Career Activities: Ms Feng is currently a graduate student at Boston College
2017 - 2021	Advisor/ Mentor, Arielle Lupo, University of Utah
2019 2021	Trainee's Current Career Activities: Ms. Lupo is currently an engineer at Microsoft Inc.
2018 - 2021	Advisor/ Mentor, Annie Li, University of Utah
	Trainee's Current Career Activities: Ms. Li is currently a Pharm.D. student at the University of Utah
2018 - 2021	College of Pharmacy
2016 - 2021	Advisor/ Mentor, Emily Li, University of Utah Train of Compart Career Activities Me. Livis commently on M.D. student at the University of Utah
	Trainee's Current Career Activities: Ms. Li is currently an M.D. student at the University of Utah School of Medicine
2021 - Present	Advisor/ Mentor, Jonathan Hong, University of Utah
PhD/Doctorates	
2004-2010	PhD Advisor/ Mentor, Manshu Tang, Department of Biochemistry and Molecular Biology,
	University of Miami.
	Trainee's Current Career Activities: Dr. Tang is currently a Research Associate at the Department of
	Pediatric Neurology at the University of Utah.
2018- 2022	PhD Advisor/ Mentor for Synneva Hagen-Lillevik, PhD student, Department of Nutrition and
	Integrative Physiology, University of Utah
	Trainee's Current Career Activities: Dr. Hagen-Lillevik is currently a post-doctoral fellow at the
	School of Nursing, University of Utah.

Visiting Faculty

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2010	Research Mentor, Cynthia Gubbels, M.D., Ph.D., Maastricht University, The Netherlands.
	Trainee's Current Career Activities: Dr. Gubbels is currently a Principal Scientist at Biogen Inc.
2015	Research Advisor/ Mentor, Jingxiao Zhang, M.D., Chief Physician (OB/GYN), The Fourth Hospital of Shijiazhuang, Shijiazhuang, Hebei Province 050011, China.

Graduate Student Committees

2004 - 2010	Co-Advisor for Manshu Tang, PhD candidate, Department of Biochemistry and Molecular Biology,
	University of Miami School of Medicine.
2018 - 2022	Advisor for Synneva Hagen-Lillevik, PhD student, Department of Nutrition and Integrative
	Physiology, University of Utah

Educational Lectures

Continuing Education

Other Educational Activities

Feb. 10th, 2005 Invited Speaker, 46th Annual Ruth Self Memorial Education Day, Woman's Cancer Association of the University of Miami (Seminar Title: Galactose Metabolism and Cancer Growth)

PEER-REVIEWED JOURNAL ARTICLES

- 1. **Lai K**, McGraw P. (1994). Dual control of inositol transport in Saccharomyces cerevisiae by irreversible inactivation of permease and regulation of permease synthesis by INO2, INO4, and OPI1. *J Biol Chem*, 269(3), 2245-51.
- 2. **Lai K**, Bolognese CP, Swift S, McGraw P. (1995). Regulation of inositol transport in Saccharomyces cerevisiae involves inositol-induced changes in permease stability and endocytic degradation in the vacuole. *J Biol Chem*, 270(6), 2525-34.
- 3. Lai K, Langley SD, Singh RH, Dembure PP, Hjelm LN, Elsas LJ 2nd. (1996). A prevalent mutation for

- galactosemia among black Americans. J Pediatr, 128(1), 89-95.
- 4. Robinson KS, Lai K, Cannon TA, McGraw P. (1996). Inositol transport in Saccharomyces cerevisiae is regulated by transcriptional and degradative endocytic mechanisms during the growth cycle that are distinct from inositol-induced regulation. *Mol Biol Cell*, 7(1), 81-9.
- 5. Langley SD, **Lai K**, Dembure PP, Hjelm LN, Elsas LJ. (1997). Molecular basis for Duarte and Los Angeles variant galactosemia. *Am J Hum Genet*, 60(2), 366-72.
- Landt M, Ritter D, Lai K, Benke PJ, Elsas LJ, Steiner RD. (1997). Black children deficient in galactose 1phosphate uridyltransferase: correlation of activity and immunoreactive protein in erythrocytes and leukocytes. J Pediatr, 130(6), 972-80.
- 7. **Lai K**, Langley SD, Dembure PP, Hjelm LN, Elsas LJ 2nd. (1998). Duarte allele impairs biostability of galactose-1-phosphate uridyltransferase in human lymphoblasts. *Hum Mutat*, 11(1), 28-38.
- 8. Elsas LJ 2nd, Lai K. (1998). The molecular biology of galactosemia. Genet Med, 1(1), 40-8.
- 9. **Lai K**, Willis AC, Elsas LJ. (1999). The biochemical role of glutamine 188 in human galactose-1-phosphate uridyltransferase. *J Biol Chem*, 274(10), 6559-66.
- 10. **Lai K**, Elsas LJ. (2000). Overexpression of human UDP-glucose pyrophosphorylase rescues galactose-1-phosphate uridyltransferase-deficient yeast. *Biochem Biophys Res Commun*, 271(2), 392-400.
- 11. Elsas LJ, **Lai K**, Saunders CJ, Langley SD. (2001). Functional analysis of the human galactose-1-phosphate uridyltransferase promoter in Duarte and LA variant galactosemia. *Mol Genet Metab*, 72(4), 297-305.
- 12 **Lai K**, Elsas LJ. (2001). Structure-function analyses of a common mutation in blacks with transferase-deficiency galactosemia. *Mol Genet Metab*, 74(1-2), 264-72.
- 13. **Lai K**, Langley SD, Khwaja FW, Schmitt EW, Elsas LJ. (2003). Galactose-1-phosphate uridyltransferase deficiency causes UDP-hexose deficit in human galactosemic cells. *Glycobiology Journal*, *13*, 285-294.
- 14. **Lai K**, Klapa MI. (2004). Alternative pathways of galactose assimilation: Could inverse metabolic engineering provide an alternative to galactosemic patients? *Metab Eng*, 6, 239-244.
- 15 Slepak T, Tang M, Addo F, **Lai K**. (2005). Intracellular galactose-1-phosphate accumulation leads to environmental stress response in yeast model. *Mol Genet Metab*, 86(3), 360-71.
- Barbouth D, Slepak T, Klapper H, Lai K, Elsas LJ. (2006). Prevention of a molecular misdiagnosis in galactosemia. *Genet Med*, 8(3), 178-82
- 17. Slepak TI, Tang M, Slepak VZ, **Lai K**. (2007). Involvement of endoplasmic reticulum stress in a novel Classic Galactosemia model. *Mol Genet Metab*, 92(1-2), 78-87.
- 18. **Lai K**, Tang M, Yin X, Klapper H, Wierenga K, Elsas L. (2008). ARHI: A new target of galactose toxicity in Classic Galactosemia. *Bioscience Hypotheses*, *1*(5), 263-271.
- 19. Siddiqi AM, Li H, Faruque F, Williams W, **Lai K**, Hughson M, Bigler S, Beach J, Johnson W. (2008). Use of hyperspectral imaging to distinguish normal, precancerous, and cancerous cells. *Cancer*, 114(1), 13-21.
- 20. **Lai K**, Wierenga KJ, Buchwald P, Tang M. (2008). High-throughput screening for human galactokinase inhibitors. *J Biomol Screen*, *13*(5), 415-23.
- 21 Syriopoulos C, Panayotarou A, **Lai K**, Klapa MI. (2008). Transcriptomic analysis of Saccharomyces cerevisiae physiology in the context of galactose assimilation perturbations. *Mol Biosyst*, 4(9), 937-49.
- 22 Lai K, Elsas LJ, Wierenga K. (2009). Galactose Toxicity in Animals. *IUBMB Life*, 61(11), 1063-74.
- 23 Tang M, Wierenga K, Elsas LJ, **Lai K**. (2010). Molecular and biochemical characterization of human galactokinase and its small molecule inhibitors. *Chem Biol Interact*, 188(3), 376-85.
- Boxer MB, Shen M, Tanega C, Tang M, **Lai K**, Auld DS. (2011). Toward Improved Therapy for Classic Galactosemia. Probe Reports from the NIH Molecular Libraries Program, Bethesda, MD. *National Center for Biotechnology Information*
- 25. Odejinmi S , Rascon R , Tang M , Vankayalapati H , **Lai K**. (2011). Structure-activity analysis and cell-based optimization of human galactokinase inhibitors. *ACS Med Chem Lett*, 2(9), 667-672.
- 26. Tang M, Odejinmi SI, Allette YM, Vankayalapati H, **Lai K**. (2011). Identification of novel small molecule inhibitors of 4-diphosphocytidyl-2-C-methyl-D-erythritol (CDP-ME) kinase of Gram-negative bacteria. *Bioorg Med Chem*, *19*(19), 5886-95.
- 27. Tang M, Facchiano A, Rachamadugu R, Calderon F, Mao R, Milanesi L, Marabotti A, **Lai K**. (2012). Correlation Assessment among Clinical Phenotypes, Expression Analysis and Molecular Modeling of 14 Novel Mutations in the Human Galactose-1 phosphate Uridylyltransferase Gene. *Hum Mutat*, *33*(7), 1107-1115.

- 28 **Lai K**, Yin X, Tang M, Baffoe S, Johnson B, Bodamer OA. (2012). Galactose-induced lethality and growth retardation in a new galactose-1-phosphate uridyltransferase (GALT) gene-knockout mouse model. *Mol Genet Metab*, 105(3), 330.
- 29. Tang M, Odejinmi S, Vankayalapati H, Wierenga K, **Lai K**. (2012). Innovative Therapy for Classic Galactosemia Tale of Two HTS. *Mol Genet Metab*, 105(1), 44-55.
- 30. Odejinmi SI, Rascon RG, Chen W, **Lai K**. (2012). Formal Synthesis of 4-diphosphocytidyl-2-C-methyl Derythritol From D-(+)-Arabitol. *Tetrahedron*, 68(43), 8937-8941.
- Tang M, Siddiqi A, Witt B, Johnson B, Fraser N, Rascon R, Chen W, Yin X, Bodamer OA, and **Lai K** (2014). Subfertility and growth restriction in a new galactose-1-phosphate uridyltransferase (GALT) gene-trapped mouse model. *European Journal of Human Genetics*, 2014 Feb 19. doi: 10.1038/ejhg.2014.12. [Epub ahead of print]
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- 35 Tang M, Etokidem E, **Lai K**. The Leloir pathway of galactose metabolism a novel therapeutic target for hepatocellular carcinoma. *Anticancer Res.* 2016 Dec;36(12):6265-6271.
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- 37 Balakrishnan B, Nicholas C, Siddiqi A, Chen W, Bales E, Feng M, Johnson J, **Lai K**. Reversal of aberrant PI3K/Akt signaling by Salubrinal in a GalT-deficient mouse model. *Biochim Biophys Acta*.(Molecular Basis of Disease) 2017 Aug 24. pii: S0925-4439(17)30305-8. doi: 10.1016/j.bbadis.2017.08.023. [Epub ahead of print]
- 38. Yuzyuk T, Balakrishnan B, Schwarz EL, De Biase I, Hobert J, Longo N, Mao R, **Lai K**, Pasquali M. Effect of genotype on galactose-1-phosphate in classic galactosemia patients. *Mol Genet Metab*. 2018 Nov;125(3):258-265. doi: 10.1016/j.ymgme.2018.08.012. Epub 2018 Aug 23.
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- 40. Siddiqi A, Webb F, Smotherman C, Shuja S, Mubeen A, **Lai K**. Prevalence of epithelial abnormalities and high-risk human papilloma virus in cervicovaginal Pap smears of population subgroups as a guide toward evidence-based best practice. *Diagn Cytopathol*. 2019 Jul;47(7):648-652. doi: 10.1002/dc.24158. Epub 2019 Feb 23. PubMed PMID: 30801970.
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- 42 Balakrishnan B, Siddiqi A, Mella J, Lupo A, Li E, Hollien J, Johnson J, **Lai K**. Salubrinal enhances eIF2α phosphorylation and improves fertility in a mouse model of Classic Galactosemia *Biochim Biophys Acta Mol Basis Dis.* 2019 Nov 1;1865(11):165516. doi: 10.1016/j.bbadis.2019.07.010. Epub 2019 Jul 27.
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- 45 Yang Y, Vankayalapati H, Tang M, Zheng Y, Li Y, Ma C, Lai K (2020) Discovery of novel inhibitors targeting

- *multi-UDP-hexose* pyrophosphorylases as anticancer agents" Molecules 2020 Feb 3;25(3):645. doi: 10.3390/molecules25030645.
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- 47 Hagen-Lillevik, S., Rushing, J. S., Appiah, L., Longo, N., Andrews, A., **Lai, K**., & Johnson, J. (2021). Pathophysiology and management of classic galactosemic primary ovarian insufficiency, Reproduction and Fertility, 2(3), R67-R84. Retrieved Aug 13, 2021, from https://raf.bioscientifica.com/view/journals/raf/2/3/RAF-21-0014.xml
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- Hagen-Lillevik S, Johnson J, Siddiqi A, Persinger J, Hale G, **Lai K**. Harnessing the Power of Purple Sweet Potato Color and *Myo*-Inositol to Treat Classic Galactosemia. Int J Mol Sci. 2022 Aug 4;23(15):8654. doi: 10.3390/ijms23158654. PMID: 35955788; PMCID: PMC9369367.
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PRE-PRINT ARTICLES

- Balakrishnan B, **Lai K** (2021) Modulation of SARS-CoV-2 Spike-induced Unfolded Protein Response (UPR) in HEK293T cells by selected small chemical molecules *bioRxiv* doi: https://doi.org/10.1101/2021.02.04.429769
- Hagen-Lillevik S, Lai K, Johnson J., Evidence of Fluctuating Integrated Stress Response Activity in Murine Primordial Ovarian Follicles May 2022 DOI: 10.21203/rs.3.rs-1682172/v1

REVIEW ARTICLES

- 1. Elsas LJ 2nd, Lai K. (1998). The molecular biology of galactosemia. Genet Med, 1(1), 40-8.
- 2. Lai K, Elsas LJ, Wierenga K. (2009). Galactose Toxicity in Animals. IUBMB Life, 61(11), 1063-74
- 3. Tang M, Odejinmi S, Vankayalapati H, Wierenga K, **Lai K**. (2012). Innovative Therapy for Classic Galactosemia Tale of Two HTS. *Mol Genet Metab*, 105(1), 44-55.

BOOKS

BOOK CHAPTERS

- 1. Tang M, Wierenga KJ, Lai K. (2013). Use of site-directed mutagenesis (SDM) in the diagnosis, prognosis and treatment of Galactosemia in *Genetic Manipulation of DNA and Protein: Examples from Current Research*, David Figurski, Columbia University (Eds.), Intech Publishers. (ISBN 978-953-51-0994-5)
- 2 Longo L, & Lai K, 2022. Gene Therapy for Inherited Metabolic Diseases in *Nutrition Management of IMD (2nd Edition)*

CONFERENCE PROCEEDINGS

OTHER (Commentary/Letters/Editorials/Case Reports/Video/Film)

Commentary

Book Reviews

Case Reports

Editorials

Letters

Newspaper

Other

1. **Lai K**. (2003). Copyright holder of the Miami GenCure Diagnostic Laboratory's Logo. US Copyright Office Reference: VAu 592-099.

Patents (including Provisional Patents recently filed)

- 1. **Lai K**. (2008). A set of small molecule inhibitors for human galactokinase. (Role: First Inventor) U.S. Patent No. PCT/US08/73152. Washington, D.C.:U.S. Patent and Trademark Office.
- 2. **Lai K**. (2011). Galactokinase Inhibitors for the Treatment and Prevention of Associated Diseases and Disorders (Role: Co-inventor); International patent filed by National Chemical Genomics Center/National Institutes of Health. U.S. Patent No. PCT/US11/53021. Washington, D.C.:U.S. Patent and Trademark Office.
- 3. **Lai K**. (2012). A Set of Small Molecule Inhibitors for Human Galactokinase (Role: First Inventor).U.S. Patent No. 12/672,347. Washington, D.C.:U.S. Patent and Trademark Office.
- 4. A co-crystallized structure between GALK1 and compound NCGC0023864 was licensed to a pharmaceutical company in California in Feb. 2019.
- 5. Balakrishnan B and **Lai K** (2021) Gene Therapy for Phosphoglucomutase I Deficiency (Patent Application Filed in 2022) (Role: Lead Inventor)
- 6 Zhong ML, Balakrishnan B, and Lai K (2023) AAV9-PMM2 Gene Replacement Therapy (Lead Inventor)

Reports

Video/Film/CD/Web/Podcast

- 1. **Lai K**, Langley SD, Guerrero NV, Elsas LJ. (1998). "GALT gene mutations database", World Wide Website [Web]. Available: http://www.cc.emory.edu/PEDIATRICS/medgen/research/galt.htm.
- 2. **Lai K**, Marabotti A , Mao R. (2012). Molecular Genetics of Galactosaemia- eLS Online Library[Web]. Chichester: John Wiley & Sons Ltd. Available: http://www.els.net [doi: 10.1002/9780470015902.a0024323].
- 3. https://www.rarediseasesnetwork.org/news/2022-06-21-FCDGC-Senior-Researcher-Spotlight

PENDING PUBLICATIONS

RECENTLY PUBLISHED ABSTRACTS (Last 3 Years)

UNPUBLISHED POSTER PRESENTATIONS

- 1. **Lai K**, Belas R. (1990). *Genetic analysis of chemotaxis mutants of Vibrio parahemolyticus*. Poster session presented at American Society for Microbiology 90th Annual Meeting, Anaheim, CA.
- 2. McGraw P, Lai K. (1992). *Inositol transport activity is coordinated to phospholipid biosynthesis in Saccharomyces cerevisiae*. Poster session presented at 16th International Conference on Yeast Genetics and Molecular Biology, Vienna, Austria.
- 3. **Lai K**, McGraw P. (1993). *Regulation of inositol metabolism in Saccharomyces cerevisiae*. Poster session presented at 1993 Yeast Genetics and Molecular Biology Meeting, Madison, WI.
- 4. McGraw P, Bolognese C, Lai K, Swift S. (1994). *Inositol-mediated repression of uptake is accompanied by endocytic degradation of the yeast inositol permease*. Poster session presented at 1994 Yeast Genetics and Molecular Biology Meeting, Seattle, WA.
- 5. **Lai K**, Langley SD, Singh R, Hjelm N, Dembure P, Elsas LJ. (1995). *Impairment of galactose-1-phosphate uridyltransferase (GALT) activity due to S135L mutation in the GALT gene is tissue-specific. Experimental Biology* 95, FASEB. Atlanta, GA.
- 6. **Lai K**, Langley SD, Singh R, Hjelm N, Dembure P, Elsas LJ. (1995). *Molecular characterization of the 'Negro variant' of galactosemia*. Poster session presented at American Society of Human Genetics 45th Annual Meeting,

- Minneapolis, MN.
- 7. **Lai K**, Langley SD, Dembure PP, Hjelm N, Elsas LJ. (1996). *The Duarte allele impairs biostability of human galactose-1-phosphate uridyltransferase in human lymphoblasts*. Poster session presented at American Society of Human Genetics 46th Annual Meeting, San Francisco, CA.
- 8. **Lai K**, Willis AC, Elsas LJ. (1999). *The biochemical role of glutamine-188 in human galactose-1-phosphate uridyltransferase*. Poster session presented at Society for Inherited Metabolic Disorders 1999 Annual Meeting, Lake Lanier Islands, GA.
- 9. **Lai K**, Presley CA, Langley SD, Elsas LJ. (1999). *Tissue-specific regulation of the human galactose-1-phosphate uridyltransferase gene*. Poster session presented at American Society of Human Genetics 49th Annual Meeting, San Francisco, CA.
- 10. Saunders CJ, **Lai K**, Langley SD, Elsas LJ. (2000). Functional analysis of the human galactose-1-phosphate uridyltransferase promoter in Duarte and LA variant galactosemia. Poster session presented at American Society of Human Genetics 50th Annual Meeting, Philadelphia, PA.
- 11. **Lai K**, Langley SD, Khwaja F, Schmitt EW, Elsas LJ. (2002). *Galactose-1-phosphate uridyltransferase deficiency causes decreased UDP-glucose concentrations in human galactosemic cells*. Poster session presented at American Society of Human Genetics 52nd Annual Meeting, Baltimore, MD.
- 12. Slepak T, Lai K. (2003). *Decreased abundance of epidermal growth factor receptor in galactosemic patients*. Poster session presented at American Society for Human Genetics 53rd Annual Meeting, Los Angeles, CA.
- 13. **Lai K**, Elsas LJ, Webb AL, Singh RH, Kennedy MJ. (2003). *Indicadores de dyspraxia verbal en galactosemia*. Poster session presented at IV Congreso Latinoamericano de Errores Innatos del Metabolismo y Pesquisa Neonatal, Cataratas del Iguazu, Argentina.
- 14. Klapa M, Lai K. (2004). Alternate pathways of galactose assimilation: Could Inverse Metabolic Engineering provide an alternative to galactosemic patients. Poster session presented at American Institute of Chemical Engineering 2004 Annual Meeting, San Antonio, Texas.
- 15. Slepak T, Longo N, **Lai K**. (2004). *Decreased glycogen content in galactose-1-phosphate uridyltransferase* (*GALT*) deficient patient cells. Poster session presented at The American Society of Human Genetics 54th Annual Meeting, Toronto, Canada.
- 16. Slepak T, Barbouth D, Klapper H, Lai K, Elsas LJ. (2004). *Pseudo uniparental disomy in galactosemia*. Poster session presented at Southeastern regional genetics group annual meeting, Sandestin, FL.
- 17. Slepak T, **Lai K**. (2005). *Classic Galactosemia: a paradigm for endoplasmic stress response*. Poster session presented at American Society for Human Genetics 55th Annual Meeting, Salt Lake City, UT.
- 18. Lai K, Tang M, Slepak T. (2005). *Intracellular accumulation of galactose-1-phosphate leads to environmental stress response in yeast*. Poster session presented at American Society for Human Genetics 55th Annual Meeting, Salt Lake City, UT.
- 19. Wierenga KJ, Lai K. (2006). Development of a high throughput assay for the identification of small molecule inhibitors of human galactokinase. Poster session presented at American Society of Human Genetics 56th Annual Meeting, New Orleans, LA.
- 20. Syriopoulos CH, **Lai K**, Klapa MI. (2006). *Metabolomic analysis of galactosemia using yeast as the model system.* Poster session presented at Metabolic Engineering 6th Conference, Amsterdam, The Netherlands.
- 21. Siddiqi AM, Li H, Williams W, Faruque F, **Lai K**, Beach J. (2006). *Use of hyperspectral imaging to distinguish normal and dysplastic fibroblasts*. Poster session presented at American Society of Cytology 54th Annual Scientific Meeting, Toronto, Ontario, Canada.
- 22. Wierenga KJ, Lai K. (2007). Development of a high throughput assay for the identification of small molecule inhibitors of human galactokinase. Poster session presented at Annual Meeting for the Society for Inherited Metabolic Disorders, Nashville, TN.
- 23. Tang M, Slepak TI, Elsas LJ, Lai K. (2007). Oxidative and endoplasmic reticulum stress in novel isogenic cell models for classic galactosemia. Poster session presented at 2007 Annual Meeting for the Society for Inherited Metabolic Disorders, Nashville, TN.
- 24. Siddiqi AM, Li H, Williams W, Faruque F, **Lai K**, Baliga M, Flowers R, Beach J. (2007). *Use of hyperspectral imaging in detection of precancerous cells*. Poster session presented at The United States and Canadian Academy of

- Pathology 96th Annual Meeting, San Diego, CA.
- 25. Tang M, Wierenga K, Lai K. (2009). A KFC-based approach for rational drug design to treat genetic diseases. Poster session presented at 59th Annual Meeting for American Society of Human Genetics, Honolulu, HI.
- 26. Tang M, Facchiano A, Rachamadugu R, Calderon F, Mao R, Milanesi L, Marabotti A, **Lai K**. (2011). Correlation Assessment among Clinical Phenotypes, Expression Analysis and Molecular Modeling of 14 Uncharacterized Mutations in the Human Galactose-1 phosphate Uridylyltransferase Gene. Poster session presented at 61st American Society for Human Genetics Annual Meeting, Montreal, Quebec, Canada.
- 27. Odejinmi S, Rascon R, Tang M, Vankayalapati H, **Lai K**. (2011). *Optimization of small molecule inhibitors of human galactokinase*. Poster session presented at American Chemical Society Annual Meeting, Anaheim, CA.
- 28. Lai K, Yin X, Tang M, Baffoe S, Johnson B, Bodamer OA. (2012). *Galactose-induced lethality and growth retardation in a new galactose-1-phosphate uridyltransferase (GALT) gene-knockout mouse model.* Poster session presented at 2012 Annual Meeting for the Society for Inherited Metabolic Disorders, Charlotte, NC.
- 29 **Lai K**, Tang M, Siddiqi A, Witt B, Johnson B, Fraser N, Chen W, Rascon R, Yin X, Goli H, Bodamer OA (2013). Subfertility and growth restriction in a new galactose-1-phosphate uridyltransferase (GALT) deficient mouse model. Poster presented at 2013 ICIEM Meeting, Barcelona, Spain.
- 30 Bijina Balakrishnan, Wyman Chen, Manshu Tang, Xiaoping Huang, Didem Demirbas Cakici, Anwer Siddiqi, Gerard Berry, **Kent Lai** (2016) *Galactose-1 phosphate uridylyltransferase* (*GALT*) *deficiency induces pleiotropic down-regulation of Pi3k/Akt signaling in mouse fibroblasts*. Poster presented at 2016 Annual Meeting of Society of Inherited Metabolic Disorders, Jacksonville, Florida.
- Wyman Chen, Rose Caston, Manshu Tang, Anwer Siddiqi, Kamalpreet Parmar, Merry Feng, **Kent Lai** (2016) *Ataxia phenotype assessment in a new mouse model of galactose-1-phosphate uridylyltransferase (GalT) deficiency.*Poster presented at 2016 Annual Meeting of Society of Inherited Metabolic Disorders, Jacksonville, Florida.
- Balakrishnan B, An J, Nguyen V, Lukacs C, Guey L, Martini P, **Lai K** (2018) *Restoration of hepatic galactose-1* phosphate uridylyltransferase (GALT) activity by a novel mRNA-based therapy decreases hepatic and red blood cell GAL-1P and plasma galactose Poster accepted for presentation at 2018 Annual Meeting of Society of Inherited Metabolic Disorders, San Diego, California.
- Balakrishnan B, Lupo A, Johnson J, **Lai K** (2018) *Salubrinal enhances follicular maturation in a mouse model of galactose-1 phosphate uridylyltransferase (GALT) deficiency* Poster accepted for presentation at 2018 Annual Meeting of Society of Inherited Metabolic Disorders, San Diego, California.
- Balakrishnan B, Verheijen J, Lupo A, Yang Y, Carter KL, Whitehead KJ, Kozicz T, Morava E, **Lai K**. (2019) *A New Phosphoglucomutase 2 Gene Knockout (KO) Mouse Model Reveals Aberrant Glycosylation and Embryonic Lethality* Poster accepted for presentation at 2019 Annual Meeting of Society of Inherited Metabolic Disorders.
- Balakrishnan, B., Llerena Cari, E., Siddiqi, A., Bales, C.E., **Lai, K.**, Johnson, J. (2019) *Rescue of Long-Term Ovarian Function, Fertility, and Fecundity in a Murine Model of Galactosemia by Blocking Endoplasmic Reticulum Stress.* Poster accepted for presentation at SRI 66th Annual Scientific Meeting, Paris, France. March 12-16, 2019.
- Verheijen J, Balakrishnan B, Lupo A, Yang Y, Carter KL, Turgeon C, Raymond K Whitehead KJ, Tamas Kozicz, Kent Lai, Eva Morava-Kozicz (2019) A new phosphoglucomutase 2 gene knockout Mouse model shows disrupted glycosylation partially reflecting PGM1-CDG. Poster accepted for presentation at the 2019 Annual Meeting of SSIEM, Rotterdam, The Netherlands.
- 37 Dhall et al., 2022 Gene Replacement Therapy with JAG101 Reduces Pathogenic Biomarkers in a Mouse Model of Type 1 Galactosemia ASGCT 25th Annual Meeting May 2022, Washington D.C.
- 38 Scott D et al., 2022 Development of BBP-818 for the Treatment of Classic Galactosemia ASGCT 25th Annual Meeting May 2022, Washington D.C
- Balakrishnan B,...., Lai, K 2022 Pioneering AAV Gene Therapy for Congenital Disorders of Glycosylation (CDG): Multi-Faceted Correction of Disease Progression in a Mouse Model of PGM1-CDG ASGCT 25th Annual Meeting May 2022, Washington D.C.
- 40 Balakrishnan B, Yan X, Bellagamba O, Winkler F, Zimmer M, McCue M, Martini P, **Lai, K** 2023 WHOLE-BODY GALACTOSE OXIDATION AS A ROBUST FUNCTIONAL ASSAY TO EVALUATE THE EFFECTIVENESS OF HGALT GENE-BASED THERAPIES IN A MOUSE MODEL OF CLASSIC GALACTOSEMIA 2023 Annual Meeting of Society for Inherited Metabolic Disorders, Salt Lake City, Utah, U.S.A.

ORAL PRESENTATIONS

Keynote/Plenary Lectures

2022

<u>International</u>	
2009	Inhibition of galactokinase as treatment for Galactosemia (Invited Speaker). Segal Symposium, 11th
	International Congress of Inborn Errors of Metabolism, San Diego, CA
2009	A novel hybrid approach for rational drug design to treat Classic Galactosemia. 11th International
	Congress of Inborn Errors of Metabolism, San Diego.
2011	Innovative Therapies for Classic Galactosemia: Tales of Two HTS (Invited Speaker), 2011
	PAS/ASPR Joint Scientific Meeting, Denver, CO.
2017	Multiple Therapeutic Modalities for Classic Galactosemia (Invited Speaker) 2017 GalNet &
	European Society of Galactosemia Meeting, Amsterdam, The Netherlands.
2019	Experimental Nucleic acid-based Therapy for Classic Galactosemia (Invited Speaker) 2019 GalNet &
	European Society of Galactosemia Meeting, Amsterdam, The Netherlands.
2021	Novel Mouse Models and Gene Therapy for Phosphoglucomutase I Deficiency 2021 Scientific CDG
	Symposium 2021, Leuven, Belgium
<u>National</u>	
2008	Identification of Novel Antimicrobials, 2008 Annual Biomedical Research Conference for Minority
	Students (ABRCMS), Orlando, FL.
2018	Invited Speaker, 2018 Newborn Screening Translational Research Network (NBSTRN) Meeting,
	Bethesda, MD.
2020	Invited Speaker, Rare Disease Day, Sanford Children's Health Research Center

Invited Speaker, Rare Disease Day, Sanford Children's Health Research Center

Meeting Presentations (Not Published Abstracts and Not Unpublished Posters)

<u>National</u>	
1997	Invited Seminar Speaker, Lai K, Willis AC & Elsas LJ. "The catalytic role of glutamine-188 in
	human galactose-1-phosphate uridyltransferase". American Society of Human Genetics 47th Annual
	Meeting, Baltimore, MD
2003	Faculty Speaker, 9th Ross Metabolic Conference
2007	Oral Platform Presentation, Tang M; Slepak TI; Elsas LJ; Lai K. Oxidative and endoplasmic
	reticulum stress in novel isogenic cell models for classic galactosemia. Annual Meeting for the
	Society for Inherited Metabolic Disorders, Nashville, TN
2007	Oral Platform Presentation, Wierenga KJ; Lai K. Development of a high throughput assay for the
	identification of small molecule inhibitors of human galactokinase. Annual Meeting for the Society
	for Inherited Metabolic Disorders, Nashville, TN
2007	Invited Seminar Speaker, Lai K, Tang M, Yin X, Klapper H, Wierenga K, Elsas LJ. "Up-regulation
	of ARH1 in Galactose-stressed, Isogenic Human Fibroblasts deficient in Galactose-1-phosphate
	Uridyltransferase". American Society of Human Genetics 57th Annual Meeting, San Diego, CA
2010	Invited Seminar Speaker, Novel Therapies for Classic Galactosemia, 2010 Parents of Galactosemic
	Children Conference, Bloomington, MN.
2010	Invited Seminar Speaker, Optimization of human galactokinase inhibitors and beyond, 2010 Parents
	of Galactosemic Children Conference, Bloomington, MN
2010	Invited Seminar Speaker, ARHI: a novel target of galactose toxicity in Classic Galactosemia? 2010
	Parents of Galactosemic Children Conference, Bloomington, MN.
2012	Invited Seminar Speaker, Targeting Human Galactokinase in treatment of Classic Galactosemia &
	common diseases, 2012 Galactosemia Foundation Conference, Dallas, TX.
2012	Invited Seminar Speaker, New mouse models of Classic Galactosemia, 2012 Galactosemia
	Foundation Conference, Dallas, TX.
2012	Invited Seminar Speaker, Toward improved outcome in Classic Galactosemia, 2012 Galactosemia
	Foundation Conference, Dallas, TX.

2014	Invited Seminar Speaker, Research Briefing, 2014 Galactosemia Foundation Conference, Orlando, FL
2014	Invited Seminar Speaker, Characterization of a New Mammalian Animal Model of Classic Galactosemia. 2014 Galactosemia Foundation Conference, Orlando, FL.
2016	Invited Seminar Speaker, Toward improved outcome in Classic Galactosemia: A Decade of Blood, Sweat and Tears, 2016 Galactosemia Foundation Conference, Altanta, GA.
2016	Invited Seminar Speaker, Novel Insights into the Pathophysiology of Classic Galactosemia, 2016 Galactosemia Foundation Conference, Altanta, GA.
2018	Invited Seminar Speaker, Update on Galactosemia Research, 2018 Galactosemia Foundation Conference, Denver, CO.
2018	Invited Seminar Speaker, Novel Experimental Nucleic Acid-based Therapies for Classic Galactosemia 2018 Galactosemia Foundation Conference, Denver, CO.
2020	Invited Seminar Speaker, Gene-based Therapies for Classic Galactosemia 2020 Galactosemia Foundation Conference
2020	Invited Seminar Speaker, Targeting Integrated Stress Response in Classic Galactosemia 2020 Galactosemia Foundation Conference
2022	Invited Seminar Speaker, Marching towards a Cure for Classic Galactosemia 2020 Galactosemia Foundation Conference (Orlando, U.S.A.)
2022	Invited Speaker, European Galactosemia Society. GalNet Joint Conference (Amsterdam, The Netherlands)
Local/Regional	
2003	Division of Medical Genetics Journal Club. Seminar Title: Glycosylation of Nucleocytoplasmic
2000	Proteins: Signal Transduction and O-GlcNAc
2003	Division of Medical Genetics Journal Club. Seminar Title: Expression patterns of α-2, 3-
	sialyltransferases and α-1,3- fucosyltransferases determine the mode of sialyl Lewis X inhibition by
	disaccharide decoys
2004	Lai K , Barbouth D, Klapper H, Slepak T, & Elsas LJ. "The metabolomics of galactose-1-phosphate uridyltransferase (GALT) deficiency in human diploid fibroblasts". PLATFORM Presentation at the Southeastern regional genetics group annual meeting, San destin, FL
2005	University of Miami Yeast Group Seminar Speaker . Title: Stress Management in yeast and beyond
2005	UM Pediatrics Department Clinical Research Forum Speaker. Seminar Title: Classic Galactosemic:
	Paradigm of ER stress
2005	UM Division of Medical Genetics Journal Club Speaker. Seminar Title: Classic Galactosemic:
	Paradigm of ER stress
2007	UM Mitochondria Club Speaker. Seminar Title: Does oxidative stress play a role in the
	pathophysiology of Classic Galactosemia
2008	Seminar Speaker, Department of Biochemistry & Molecular Biology, University of Miami School of
	Medicine, Innovative therapy and pathophysiology studies for Classic Galactosemia
2009	Seminar Speaker, Division of Medical Genetics, Department of Pediatrics, University of Utah School
	of Medicine, Seminar Title: Research Update on Classic Galactosemia
2010	Seminar Speaker, Division of Medical Genetics, Department of Pediatrics, University of Utah School
	of Medicine. Seminar Title: Targeting GHMP kinases for treating rare and common diseases
2011	Seminar Speaker, Division of Medical Genetics, Department of Pediatrics, University of Utah School
	of Medicine. Seminar Title: Galactose metabolism in health and diseases
2011	Research in Progress Seminar Speaker, Department of Biochemistry, University of Utah School of
	Medicine. Seminar Title: Galactose metabolism in health and diseases
2011	Research in Progress Seminar Speaker, Department of Pediatrics, University of Utah School of
~	Medicine. Seminar Title: Galactose metabolism in health and diseases
2012	Seminar Speaker, Division of Medical Genetics, Department of Pediatrics, University of Utah School
	of Medicine. Seminar Title: Research Update in Classic Galactosemia
2014	Research Seminar Speaker, Department of Medicinal Chemistry, U. of Utah.
2016	Seminar Speaker, Resident Lecture, Department of Pediatrics, U. of Utah School of Medicine
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2017	Seminar Speaker, Research-in-Progress, Department of Pediatrics, U. of Utah School of Medicine
2018a	Seminar Speaker, Research-in-Progress, Department of Pediatrics, U. of Utah School of Medicine
2018b	Seminar Speaker, Research in Progress, Department of Pediatrics, U. of Utah School of Medicine
2022	Seminar Speaker, Research in Progress, Department of Pediatrics, U. of Utah School of Medicine

Invited/Visiting Professor Presentations

2001 Invited Seminar Speaker, Department of Medicine, University of Hong Kong

2008 Invited Seminar Speaker, Department of Applied Biology and Chemical Technology, Hong Kong

Polytechnic University, Hong Kong. (Seminar Title: Innovative therapy and pathophysiology studies

for Classic Galactosemia)

2023 Invited Seminar Speaker, Department of Heath Technology and Informatics, Hong Kong Polytechnici

University

National

2005 Invited Speaker, 46th Annual Ruth Self Memorial Education Day, Woman's Cancer Association of

the UM. Seminar Title: Galactose Metabolism and Cancer Growth

2012 Invited Speaker, Agios Pharmaceuticals Inc.

Research Seminar entitled "New Mouse Model of Classic Galactosemia" given to the Division of 2015

Genetics and Genomics, Department of Pediatrics, Harvard Medical School on November 21st, 2015.

2017 Research Seminar entitled "New Mouse Model of Classic Galactosemia" Moderna Therapeutics.

Local/Regional

2003 - 2004 Invited Faculty Speaker, Clinical Scientist Training Program, University of Miami School of

Medicine

2007 Invited Seminar Speaker, Department of Physiology, Pharmacology and Therapeutics, University of

North Dakota. Seminar Title: Innovative therapy and pathophysiology for Classic Galactosemia

Invited Seminar Speaker, Division of Medical Genetics, Department of Pediatrics, University of 2007

Utah. Seminar Title: Innovative therapy and pathophysiology for Classic Galactosemia

Invited Seminar Speaker, Department of Basic Medical Science, Mercer University School of 2008

Medicine, Seminar Title: Innovative therapy and pathophysiology for Classic Galactosemia

2008 Invited Seminar Speaker, Department of Biochemistry, University of Mississippi Medical Center,

Seminar Title: Innovative therapy and pathophysiology studies for Classic Galactosemia

2009 Invited Seminar Speaker, Department of Pharmacology and Toxicology, University of Utah School of

Medicine, Seminar Title: Innovative therapy and pathophysiology studies for Classic Galactosemia

Grand Rounds Presentations

Outreach Presentations

2003 Faculty Speaker, Abbott Laboratories, 9th Ross Metabolic Conference