Leszek Lisowski, PhD, MBA

Children's Medical Research Institute (CMRI)
Vector and Genome Engineering Facility
Translational Vectorology Research Group
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CITIZENSHIP Polish / U.S. dual citizenship

EDUCATION

Sept. 2013 – Dec. 2015	**Master of Business Administration (MBA)** UC San Diego, Rady School of Management La Jolla, CA 92093-0553
July 2001 – March 2008	**Ph.D. in Molecular Biology and Genetics** Weill Graduate School of Medical Sciences Cornell University, New York, NY USA
Sept. 1997 – May 2001	**B.S. in Biology** University of Bridgeport, Bridgeport, CT USA
Oct. 1996 – Dec. 1998	**Medical School, Honor student, Junior** Academia Medica Gedanensis, Gdansk, Poland

RESEARCH TRAINING / WORK EXPERIENCE

Associate Professor, Vectorology Group **(January 2017 – Present)**
Military Institute of Hygiene and Epidemiology (MIHiE)
The Biological Threats Identification and Countermeasure Centre, Puławy, Poland

Senior Lecturer; Sydney Medical School **(December 2016 – Present)**
University of Sydney, Sydney, NSW Australia

Group Leader; Translational Vectorology Group **(January 2016 – Present)**
Children's Medical Research Institute (CMRI), Westmead, NSW Australia

Manager; Vector and Genome Engineering Facility **(November 2015 – Present)**
Children's Medical Research Institute (CMRI), Westmead, NSW Australia

co-founder and CTO of LogicBio Therapeutics, Inc. **(August 2014 – Present)**
Boston, MA, USA

Scientific co-founder, Bio21GE **(November 2014 – Present)**
Wroclaw, Poland

Director; Gene Transfer, Targeting and Therapeutics Facility **(March 2013 – September 2015)**
Salk Institute for Biological Studies, La Jolla, CA, USA
rAAV, Ad, HC-Ad, Lentiviral, Retroviral, Rabies and VSV viral vector development and production.

Development, optimization and production of novel viral and non-viral gene transfer and targeting reagents. Design and cloning cassettes for use in homologous recombination gene targeting. Providing basic viral production and consultation services to Salk community as well as larger international scientific community.
Responsibilities: Direct supervision and evaluation of staff members, staff recruitment, billing and purchasing, viral vector liaison, overall development of the GT3 core into an internationally competitive facility and staying on top of the current technology.

Postdoctoral Fellow **(June 2008 – March 2013)**
Stanford University School of Medicine, Department of Human Gene Therapy, Stanford, CA, USA
Lab Head: Dr. Mark A. Kay
Human ES cell and *in vivo* gene therapy vector evolution via multispecies interbreeding and retargeting of adeno-associated viruses. RNAi based therapy against HCV using AAV vectors. Novel rAAV vectors capable of non-random integration into the host genomic DNA.
Direct involvement in establishing and serving as a co-director of rAAV Vector Core for Beta Cell Biology Consortium (*BCBC*). As part of this effort, established novel rAAV vector production protocols, which allow for production of high-titer rAAV at GLP quality.

Postdoctoral Fellow **(March 2008 - June 2008)**
Memorial Sloan Kettering Cancer Center, Dept of Human Genetics, New York, NY, USA
Lab head: Dr. Michel Sadelain
Optimization of globin lentiviral vector design for the treatment of β-thalassemia in preparation for Stage I clinical trial. Assessment of vector toxicity and risk of insertional oncogenesis using Th3/+ murine model of thalassemia, human normal and thalassemic CD34+ (PB and BM) as well as human ES cells.

Ph.D. Research **(July 2001 – March 2008)**
Memorial Sloan Kettering Cancer Center, Dept of Human Genetics, New York, NY, USA
Lab head: Dr. Michel Sadelain
Optimization of globin lentiviral vector design for the treatment of β-thalassemia. Lentiviral encoded siRNA expression in hematopoietic system. Globin transgene expression in hES cells.

Research Assistant **(May 2000 – September 2000)**
The University of Texas Medical Branch at Galveston UTMB. Department of Human Biological Chemistry & Genetics & Sealy Center for Structural Biology.
Lab Head: Dr. David G. Gorenstein
Monothiophosphate Aptamer Combinatorial Selection Targeting RNase H Domain of HIV-1 Reverse Transcriptase.

Research Assistant **(January 1999 – May 2001)**
Chemistry Department, University of Bridgeport, Bridgeport, CT
Supervisor: Dr. Angela Santiago
Chemistry Laboratory Assistant. Chemistry tutor for Chemistry 103, 104, 114, 205 and 206. Computer laboratory monitor.

Research Assistant **(May 1999 – September 1999 and January 2000 – May 2000)**
Yale University School of Medicine
Bridgeport Hospital, Bridgeport, CT
Supervisor: Dr. Keith Bradley
Patient history and data collection; statistical analysis of collected data.

Research Assistant **(May 1999 – September 1999)**
Perna canaliculus research program at the University of Bridgeport
University of Bridgeport, Bridgeport, CT
Supervisor: Dr. Roger Kendall
Testing effect of green-lipped mussel dietary supplement on cell viability, proliferation and differentiation using human peripheral blood.

PUBLICATIONS

Primary publications

Paulk NK, Pekrun K, Zhu E, Nygaard S, Li B, Xu J, Chu K, Leborgne C, Dane AP, Haft A, Zhang Y, Zhang F, Morton C, Valentine MB, Davidoff AM, Nathwani AC, Mingozzi F, Grompe M, Alexander IE, **Lisowski L** and Kay MA. *Bioengineered AAV capsids with combined high human liver transduction in vivo and unique humoral seroreactivity*. Molecular Therapy. 2018 Jan 3;26(1):289-303

Winters IP, Chiou SH, Paulk NK, McFarland CD, Lalgudi PV, Ma RK, **Lisowski L**, Connolly AJ, Petrov DA, Kay MA, Winslow MM. *Multiplexed in vivo homology-directed repair and tumor barcoding enables parallel quantification of Kras variant oncogenicity*; Nature Communications. 2017 Dec 12;8(1):2053

Logan GJ, Dane AP, Hallwirth CV, Smyth CM, Wilkie EE, Amaya AK, Zhu E, Khandekar N, Ginn SL, Liao S, Cunningham SC, Sasaki N, Cabanes-Creus M, Tam PPL, Russell DW, **Lisowski L**, Alexander IE. *Identification of liver-specific enhancer-promoter activity in the 3' UTR of AAV2*. Nature Genetics, 2017 Aug;49(8):1267-1273

Valdmanis PN, Gu S, Chu K, Jin L, Zhang F, Munding EM, Zhang Y, Huang Y, Kutay H, Ghoshal K, **Lisowski L**, Kay MA. *RNA interference-induced hepatotoxicity results from loss of the first synthesized isoform of microRNA-122 in mice*. Nature Medicine 2016 May;22(5):557-62

Lisowski L, Tay SS, Alexander IE. *Adeno-associated virus serotypes for gene therapeutics*. Curr Opin Pharmacol. 2015 Aug 17;24:59-67

Sebastiano V, Zhen HH, Haddad B, Bashkirova E, Melo SP, Wang P, Leung TL, Siprashvili Z, Tichy A, Li J, Ameen M, Hawkins J, Lee S, Li L, Schwertschkow A, Bauer G, **Lisowski L**, Kay MA, Kim SK, Lane AT, Wernig M, Oro AE. *Human COL7A1-corrected induced pluripotent stem cells for the treatment of recessive dystrophic epidermolysis bullosa.* Science Translational Medicine. 2014, 6(264):264er8

Diecke S, **Lisowski L**, Kooreman, N. G., and Wu J.C., *Second Generation Codon Optimized Minicircle (CoMiC) for Nonviral Reprogramming of Human Adult Fibroblasts*. Methods in Molecular Biology, 2014, 1181: 1-13

Wang Y, Liang P, Lan F, Wu H, **Lisowski L,** et al., *Genome Editing of Isogenic Human Induced Pluripotent Stem Cells Recapitulates Long QT Phenotype for Drug Testing.* Journal of the American College of Cardiology. 2014 Aug 5;64(5):451-9

Melo SP*, **Lisowski L***, Bashkirova E, *Somatic Correction of Junctional Epidermolysis Bullosa by a Highly Recombinogenic AAV Variant*. Molecular Therapy, 2014, 22(4): 725-33 **(*co-first authors)**

Lisowski L, Dane AP, Chu K, et al., *Selection and evaluation of clinically relevant AAV variants in a xenograft liver model.* Nature, 2014, 506(7488): 382-6

Lisowski L, Elazar M, Chu K, et al., *The anti-genomic (negative) strand of Hepatitis C Virus is not targetable by shRNA*. Nucleic Acid Research, 2013, 41(6): 3688-98.

Wang N, Rajasekaran N, Hou T**, Lisowski L**, Mellins ED. *Comparison of transduction efficiency among various lentiviruses containing GFP reporter in bone marrow hematopoietic stem cell transplantation.* Exp Hematology, 2013, 41(11): 934-43.

Valdmanis PN, **Lisowski L**, Kay MA, *rAAV-mediated tumorigenesis: still unresolved after an AAV assault*. Molecular Therapy, 2012, 20(11): 2014-7.

Lisowski L, Lau A, Wang Z, et al. *Ribosomal DNA integrating rAAV-rDNA vectors allow for stable transgene expression*. Molecular Therapy, 2012, 20(10): 1912-23.

Wang Z, **Lisowski L**, Finegold MJ, et al. *AAV vectors containing rDNA homology display increased chromosomal integration and transgene persistence*. Molecular Therapy, 2012. 20(10): 1902-11

Wang Y, Zhang WY, Hu S, Lan F, Lee AS, Huber B, **Lisowski L**, et al. *Genome editing of human embryonic stem cells and induced pluripotent stem cells with zinc finger nucleases for cellular imaging.* Circulation Research, 2012, 111(12):1494-503.

Sadelain M, **Lisowski L**, Chang A., *Supplying therapeutic proteins from hematopoietic stem cell derived-erythroid and megakaryocytic lineage cells*. Molecular Therapy, 2009; 17(12): 1994-9

Hayakawa J, Ueda T, **Lisowski L**, et al. *Transient in vivo beta-globin production after lentiviral gene transfer to hematopoietic stem cells in the non-human primate*. Human Gene Therapy, 2009; 20(6): 563-72

Sadelain M, Boulad F, **Lisowski L**, et al. *Stem cell engineering for the treatment of severe hemoglobinopathies.* Current Molecular Medicine. 2008; 8(7): 690-697

Chang AH, Stephan MT, **Lisowski L**, Sadelain M. *Erythroid-specific human factor IX delivery from in vivo selected hematopoietic stem cells following nonmyeloablative conditioning in hemophilia B mice.* Molecular Therapy. 2008; 16(10): 1745-52

Lisowski L, Sadelain M. *Current status of globin gene therapy for the treatment of β-thalassemia.* British Journal of Haematology, 2008; 114(3): 335-45

Lisowski L, Sadelain M. *Locus control region elements HS1 and HS4 enhance the therapeutic efficacy of globin gene transfer in β-thalassemic mice*. Blood. 2007; 110(13): 4175-8

Samakoglu S, **Lisowski L**, Budak-Alpdogan T, et al. *A genetic strategy to treat sickle cell anemia by coregulating globin transgene expression and RNA interference.* Nature Biotech. 2006; 24(1): 89-94

Sadelain M, **Lisowski L**, Samakoglu S, et al. *Progress toward the genetic treatment of the beta-thalassemias.* Ann N Y Acad Sci. 2005; 1054: 78-91

Sadelain M, Rivella S, **Lisowski L**, et al. *Globin gene transfer for treatment of the beta-thalassemias and sickle cell disease.* Best Pract Res Clin Haematol. 2004; 17(3): 517-34

Rivella S, **Lisowski L**, Sadelain M. *Globin gene transfer: a paradigm for transgene regulation and vector safety.* Gene Therapy and Regulation 2003; 2(2): 149-175

Book Chapters

Sadelain M, **Lisowski L**, Samakoglu S, Riviere I. *Globin gene transfer for the treatment of β-thalassemia. In Disorders of iron homeostasis, erythrocytes, erythropoiesis*. Beaumont C, et al., Editors. European School of Haematology. The Handbook. 2006. Forum Service Editore: Genoa, Italy. p. 348-362

PATENTS

- U.S. Patent Application No.: 60/993,805 - Globin Lentiviral Vectors for beta-thalassemia. Filing date: Sep 13, 2007. Inventor: Leszek Lisowski, *et al*.

- U.S. Patent Application No.: 13/594,773 – AAV Capsid Proteins for Nucleic Acid Transfer. Filing date: Aug 24, 2011. Inventor: Leszek Lisowski, *et al*.

- Australian Patent Application No.: 2017904908 - Methods, cells and constructs for adeno associated virus production. Filing date: Dec 10, 2017. Inventor: Leszek Lisowski.

- Australian Provisional Patent Application No. 2018900609 - Methods for Codon Optimization. Filing date: Feb 26, 2018. Inventor: Leszek Lisowski, Marti Cabanes-Creus

GRANTS / AWARDS

2018 - National Science Centre, Republic of Poland. OPUS 13: - Lisowski: Advanced methods of selecting novel AAV capsids for medical applications, 2018-2020. AU$470,000

2018 - Ophthalmic Research Institute of Australia. RV Jamieson, L Lisowski, JR Grigg, U Grunert, M Madigan. 2018. AU$50,000

2017 - Department of Science and Higher Education of Ministry of National Defense, Ropublic of Poland. Grant K/10/8047/DNiSW/T-WIHE/3 - Lisowski: Next generation of clinical vectors for liver therapy and wound healing. 2017-2020. AU$ 552,000

2017 - "The Sydney Vision Initiative" University of Sydney. RV Jamieson, I Alexander, L Lisowski, JR Grigg, P Tam, D Schofield, P McCluskey, K Barlow-Stewart, M Simunovic. 2017-2018. AU$50,000

2016 - NHMRC Project Grant APP1108311 – Lisowski, Alexander and Tse: Directed evolution of AAV capsid variants for enhanced targeted genome editing in the human liver, 2016 – 2018. AU$386,012.

2015 - National Institute of Neurological Disorders and Stroke (NINDS) R24 grant. PIs: Edward M Callaway and Leszek Lisowski. Grant Number: 1R24NS092943-01. Project Title: Resources for Studying Neural Circuit Structure and Function with G-Deleted Rabies Viruses. Project Period: 09/01/2015 – 06/30/2019. Grant amount: $575,407 USD.

2011 - American Society of Gene Therapy Travel Grant for 14th annual meeting.

2011 - Top Abstract Award. Stanford Department of Pediatrics Annual Research Retreat.

2010 - American Society of Gene Therapy Travel Grant for 13th annual meeting.

2009 - American Society of Gene Therapy Travel Grant for 12th annual meeting.

2009 - 2010. Child Health Research Program. Lucile Packard Children's Hospital/Stanford University. Pediatric Research Fund Award. "*Novel integrating rAAV vectors as a safe tool for gene therapy"*. $35,000 USD.

2009 - 2011. Walter V. and Idun Berry Fellowship. Annual support of $55,000 USD in stipend/salary, health insurance coverage, and any other fellow related expenses related to health insurance plus $5,000 USD for research-related expenses

HONORS

- American Society of Gene & Cell Therapy Travel Grant for 14th annual meeting 2011.
- American Society of Gene & Cell Therapy Travel Grant for 13th annual meeting 2010.
- American Society of Gene Therapy Travel Grant for 12th annual meeting 2009.
- University of Bridgeport Academic Excellence and Leadership Scholarship 1997-2001.
- Academic Achievement in Biology Award, University of Bridgeport 2000-2001
- Charles Reed Award for the Academic Year 1999-2000, University of Bridgeport.
- First Place Poster Presentation. Infection and Immunology. SURP UTMB 2000.
- Dean's List and President's List (University of Bridgeport) 1997, 1998, 1999.
- All-American Scholars 1998, 1999.
- National Collegiate Natural Science Award 1998, 1999.
- Member of Metropolitan Association of College and University Biologists MACUB.
- Member of Sigma Xi, The Scientific Research Society, UTMB.
- Biology Club President, University of Bridgeport, 1999.
- Scientific Scholarship at the Academia Medica Gedanensis, Poland, 1997.
- 1st Prize in a Regional Chemistry Tournament, Poland, 1992.

PROFESSIONAL MEMBERSHIPS

2002-present	Associate Member, American Society of Gene and Cell Therapy (ASGCT)
2008-2009	Associate Member, American Society of Hematology (ASH)
2011-present	Associate Member, Australasian Gene Therapy Society (AGTS)

INVITED REVIEWER

- Human Gene Therapy, Molecular Therapy,
- Invited abstract reviewer: American Society of Gene & Cell Therapy (ASGCT), European Society of Gene and Cell Therapy (ESGCT).

INVITED TALKS

2017
- Invited speaker at the 10th Biennial Meeting of the Australasian Gene and Cell Therapy Society.

2016
- Invited talk at Max Delbrück Center for Molecular Medicine: "Novel AAV Variants for

Gene Addition and Genome Editing in Preclinical and Clinical Applications" November 21, Berlin, Germany

2013
- The 16th Annual Meeting of the American Society of Gene Therapy, Salt Lake City, UT – "AAV-mediated Direct Somatic Genetic Correction of Epidermolysis bullosa"
- The 16th Annual Meeting of the American Society of Gene Therapy, Salt Lake City, UT - "A Species Restricted rAAV Vector Obtained from Viral Capsid Shuffling"

2011
- The 14th Annual Meeting of the American Society of Gene Therapy, Seattle, WA – "Shuffled AAV library selection on human hepatocytes in vivo"
- Australasian Gene Therapy Society (AGTS) Annual Meeting, Melbourne, Australia. "Killing one bird with two stones - single shRNA targeting both strands of HCV"

2010
- The 13th Annual Meeting of the American Society of Gene Therapy, Washington, DC. "Non-canonical integrating AAV vectors as a safe tool for the future of gene therapy"

2009
- Memorial Sloan Kettering Cancer Center, New York, NY. "Design and evaluation of novel AAV-based vectors for hemophilia and HCV gene therapy"
- The 12th Annual Meeting of the American Society of Gene Therapy, San Diego, CA. "AAV based RNAi therapies to treat and / or prevent HCV infection in animal models"

2007
- The 49th Annual Meeting of American Society of Hematology, Atlanta, GA. "The Importance of the Human β-Globin Locus Control Region HS1 and HS4 Elements for Therapeutic β-Globin Gene Expression in β-Thalassemic Mice "

2005
- The 8th Annual Meeting of the American Society of Gene Therapy, St. Louis, MO. "Optimization of Globin Lentiviral Vector Design for the Treatment of β-Thalassemia"

RESEARCH MENTORSHIP

Current PhD students:

Tayyab Sheikh
Adrian Westhaus
Marti Cabanes Creus
Jason Ward

Current MS students:

Tessy Hick
Matteo Franco

Current Honours Students:

Patrick Wilmott

Past mentorship:

Kate Van Brussel – Honours Student
Elijah Lake – Honours Student
Jason Ward – Honours Student
Adrian Westhaus - MS student
Marti Cabanes Creus - MS student

Ashley Lau – Undergraduate Student
Edwartd Grow – PhD student

CERTIFICATIONS

FACSAria II cell sorter - Stanford certified independent operator

Zeiss LSM 510 Meta Confocal Microscope - Stanford certified independent operator

MEETING PARTICIPATION

- The 20th Annual Meeting of the American Society of Gene & Cell Therapy, Washington, DC, 2017
- 10th Biennial Meeting of the Australasian Gene and Cell Therapy Society, Sydney, 2017
- 25th Annual Meeting of the European Gene and Cell Therapy Society, Berlin, Germany, 2017
- The 25th Annual Meeting of the European Society of Gene & Cell Therapy, Berlin, Germany 2017
- The 19th Annual Meeting of the American Society of Gene & Cell Therapy, Washington, DC, 2016
- The 24th Annual Meeting of the European Society of Gene & Cell Therapy, Florence, Italy 2016
- The 18th Annual Meeting of the American Society of Gene & Cell Therapy, New Orleans, LA, 2015
- The 17th Annual Meeting of the American Society of Gene & Cell Therapy, Washington, DC, 2014
- The 16th Annual Meeting of the American Society of Gene & Cell Therapy, Salt Lake City, UT, 2013 – Two Oral Presentations and one Poster Presentation
- The 15th Annual Meeting of the American Society of Gene & Cell Therapy, Philadelphia, PA, 2012 – Oral Presentation and Poster Presentation
- The 14th Annual Meeting of the American Society of Gene & Cell Therapy, Seattle, WA, 2011 – Oral Presentation and Poster Presentation.
- The 13th Annual Meeting of the American Society of Gene & Cell Therapy, Washington, D.C., 2010 – Oral Presentation
- The 12th Annual Meeting of the American Society of Gene Therapy, San Diego, CA, 2009 – Oral Presentation and Poster Presentation
- 49th Annual Meeting of American Society of Hematology, Atlanta, GA, 2007 – Oral Presentation
- The 10th Annual Meeting of the American Society of Gene Therapy, Seattle, WA, 2007 – Poster Presentation
- IV Conference on Stem Cell Gene Therapy, Thessaloniki, Greece, 2007 – Poster Presentation
- The 9th Annual Meeting of the American Society of Gene Therapy, Baltimore, MD, 2006 – Poster Presentation
- 15th Conference on Hemoglobin Switching, 2006, Oxford, UK – Poster Presentation
- The 8th Annual Meeting of the American Society of Gene Therapy, St. Louis, MO, 2005 – Oral Presentation
- The 7th Annual Meeting of the American Society of Gene Therapy, Minneapolis, MN, 2004 – Poster Presentation
- 14th Conference on Hemoglobin Switching, 2004, Orcas Island, WA
- The 6th Annual Meeting of the American Society of Gene Therapy, Washington, DC, 2003