Curriculum Vitae

Kirsten E. Coleman (Erger)

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PROFESSIONAL EXPERIENCE

ASSISTANT DIRECTOR, RESEARCH ADMINISTRATION October 2018 – Present
UNIVERSITY OF FLORIDA, POWELL GENE THERAPY CENTER (PGTC) TOXICOLOGY CORE Gainesville, FL
The University of Florida PGTC is a group of researchers investigating the delivery of therapeutic genes to patients with genetic disorders using recombinant adeno-associated viruses. The Toxicology Core follows Good Laboratory Practice (GLP) regulations as set forth by the US FDA to perform IND enabling preclinical studies on gene and cell therapy vectors.

- Facilitate collaborative research efforts between groups within the University of Florida, other academic institutions, and industry partners, including study design and techniques.
- Create study protocols, contributing scientist reports, final reports, audit responses and deviation reports.
- Write, maintain and follow Standard Operating Procedures (SOPs) for all procedures performed.
- Proficient in performing and qualifying laboratory assays including DNA, RNA and protein extraction, QPCR and RT-QPCR analysis on blood and tissue samples and ELISAs.
- Animal husbandry, including blood collection, injections and necropsies in rodents.

RESEARCH AND DEVELOPMENT MANAGER August 2016 – October 2018
UNIVERSITY OF FLORIDA, POWELL GENE THERAPY CENTER TOXICOLOGY CORE Gainesville, FL

RESEARCH PROGRAMS COORDINATOR June 2008 – August 2016
UNIVERSITY OF FLORIDA, POWELL GENE THERAPY CENTER TOXICOLOGY CORE Gainesville, FL

SENIOR BIOSCIENTIST January 2005 – June 2008
UNIVERSITY OF FLORIDA, POWELL GENE THERAPY CENTER TOXICOLOGY CORE Gainesville, FL

LABORATORY TECHNICIAN February 2002 – January 2005
UNIVERSITY OF FLORIDA, DEPARTMENT OF PEDIATRICS Gainesville, FL

EDUCATION

MASTER OF BUSINESS ADMINISTRATION December 2011
UNIVERSITY OF FLORIDA Gainesville, FL

- Elected to Beta Gamma Sigma International Honor Society

BACHELOR OF SCIENCE, MICROBIOLOGY AND CELL SCIENCE May 2004
UNIVERSITY OF FLORIDA Gainesville, FL

- Magna Cum Laude
- Minor in Chemistry and Spanish

ACHIEVEMENTS

TEACHING

GMS6252 – Molecular Therapy II – Disease Targets and Applications (biennial)	2018 – present
IDH3937 – Bench to Market Regenerative Medicine (annual)	2018 – present

PROFESSIONAL MEMBERSHIPS

Association for Academic Women (AAW) at University of Florida	2019 – present
Regulatory Affairs Professionals Society (RAPS)	2018 – 2020
American Society of Gene and Cell Therapy (ASGCT)	2014 – present

CERTIFICATIONS AND CONTINUING EDUCATION

Project Management Professional (PMP)® Certification	Anticipated August 2023
Managing at UF: The Supervisory Challenge	July 2022
Regulatory Affairs Certificate: Pharmaceuticals	June 2019
Empowering Women in Technology Startups (eWiTS)	January – April 2015
Core Concepts in Biomanufacturing and Quality Systems: cGMPs	February 17-20, 2014

PEER REVIEWED PUBLICATION

1. Yan B, Viswanathan S, Brodie SE, Deng W, **Coleman KE**, Hauswirth WW, Nirenberg S. A clinically viable approach to restoring visual function using optogenetic gene therapy. Mol Ther Methods Clin Dev. 2023 May 9;29:406-417. PMID: 37251979 PMCID: PMC10213293
2. Birch SM, Lawlor MW, Conlon TJ, Guo LJ, Crudele JM, Hawkins EC, Nghiem PP, Ahn M, Meng H, Beatka MJ, Fickau BA, Prieto JC, Styner MA, Struharik MJ, Shanks C, Brown KJ, Golebiowski D, Bettis AK, Balog-Alvarez CJ, Clement N, **Coleman KE**, Corti M, Pan X, Hauschka SD, Gonzalez JP, Morris CA, Schneider JS, Duan D, Chamberlain JS, Byrne BB, Kornegay JN. Assessment of systemic AAV-microdystrophin gene therapy in the GRMD model of Duchenne muscular dystrophy. Sci Transl Med. 2023 Jan 4;15(677). PMID: 36599002.
3. Daniel HDJ, Kumar S, Kannangai R, Farzana J, Joel JN, Abraham A, Lakshmi KM, Agbandje-McKenna M, **Coleman KE**, Srivastava A, Srivastava A, Abraham AM. Age-stratified adeno-associated virus serotype 3 neutralizing and total antibody prevalence in hemophilia A patients from India. J Med Virol. 2022 Sep;94(9):4542-4547. PMID: 35577570
4. Kondratov O, Kondratova L, Mandell RJ, **Coleman K**, Savage MA, Gray-Edwards HL, Ness TJ, Rodriguez-Lebron E, Bell RD, Rabinowitz J, Gamlin PD, Zolotukhin S. A comprehensive study of a 29 capsid AAV library in non-human primate central nervous system. Mol Ther. 2021 Jul 20;S1525-0016(21)00364-6. PMID: 34298128. PMCID: PMC8417503.
5. Daniel HDJ, Kumar S, Kannangai R, Lakshmi KM, Agbandje-McKenna M, **Coleman KE**, Srivastava A, Srivastava A, Abraham AM. Prevalence of AAV3 capsid binding and neutralizing antibodies in healthy and individuals with hemophilia B from India. Hum Gene Ther. 2021 May 17. 32:9-10, 451-457. PMID: 33207962.
6. Hamm SE, Fathalikhani DD, Bukovec KE, Addington AK, Zhang H, Perry JB, McMillan RP, Lawlor MW, Prom M, van den Avond M, Kumar S, **Coleman K**, Brown DA, Morris CA, Gonzalez JP, Grange RW. Voluntary wheel running complements microdystrophin gene therapy to improve muscle function in mdx mice. Mol Ther Methods Clin Dev. 2021 Mar 3;21:144-160. PMID: 33850950. PMCID: PMC8020351.

7. Byrne BJ, Fuller DD, Smith BK, Clement N, **Coleman K**, Cleaver B, Vaught L, Falk DJ, McCall A, Corti M. Pompe disease gene therapy: neural manifestations require consideration of CNS directed therapy. Ann Transl Med. 2019 Jul;7(13):290. PMID: 31392202 PMCID: PMC6642929.
8. Gruntman A, Gernoux G, Tang Q, Ye G, Knop D, Benson J, Wang G, **Coleman KE**, Keeler A, Mueller C, Chicoine L, Chulay J, and Flotte T. Bridging from intramuscular to limb perfusion delivery of rAAV: Optimization in a non-human primate study. Mol Ther Methods Clin Dev. 2019 Feb 2;13:233-242. PMID: 30828586. PMCID: PMC6383191.
9. Song C, Conlon T, Deng W, **Coleman K**, Zhu P, Plummer C, Mandapati S, Hoosear M, Green K, Sonnentag P, Sharma A, Timmers A, Robinson P, Knop D, Hauswirth W, Chulay J, Shearman M, Ye G. Toxicology and pharmacology of an AAV vector expressing codon-optimized RPGR in RPGR-deficient Rd9 mice. Hum Gene Ther. 2018 Oct 3. PMID: 30280954. PMCID: PMC6421992.
10. Mondo E, Moser R, Gao G, Mueller C, Sena-Esteves M, Sapp E, Pfister E, O'Connell D, Takle K, **Erger KE**, Liu W, Conlon TJ, DiFiglia M, Gounis MJ, Aronin N. Selective neuronal uptake and distribution of AAVrh8, AAV9, and AAVrh10 in sheep after intra-striatal administration. J Huntingtons Dis. 2018 Sep 29. PMID: 30320596.
11. Lee YM, Conlon TJ, Specht A, **Coleman KE**, Brown LM, Estrella AM, Dambska M, Dahlberg KR, Weinstein DA. Long-term safety and efficacy of AAV gene therapy in the canine model of glycogen storage disease type Ia. J Inherit Metab Dis. 2018 May 25. PMID: 29802554.
12. Corti M, Liberati C, Smith BK, Lawson LA, Tuna IS, Conlon TJ, **Coleman KE**, Islam S, Herzog RW, Fuller DD, Collins SW, Byrne BJ. Safety of intradiaphragmatic delivery of adeno-associated virus-mediated alpha-glucosidase (rAAV1-CMV-hGAA) gene therapy in children affected by pompe disease. Hum Gene Ther Clin Dev. 2017 Dec;28(4):208-218. PMID: 29160099. PMCID: PMC5733674.
13. Conlon TJ, Mah CS, Pacak CA, Rucker Henninger MB, **Erger KE**, Jorgensen ML, Lee CC, Tarantal AF, Byrne BJ. Transfer of therapeutic genes into fetal rhesus monkeys using recombinant adeno-associated type I viral vectors. Hum Gene Ther Clin Dev. 2016 Dec;27(4):152-159. PMID: 27855487. PMCID: PMC5310237.
14. Boyd R, Boye S, Conlon T, **Erger K**, Sledge D, Langohr I, Hauswirth W, Komaromy A, Boye S, Petersen-Jones S, Bartoe J. Reduced retinal transduction and enhanced transgene-directed immunogenicity with intravitreal delivery of rAAV following posterior vitrectomy in dogs. Gene Ther. 2016 Jun;23(6):548-56. PMID: 27052802. PMCID: PMC4891289.
15. Ghazi NG, Abboud EB, Nowilaty SR, Alkuraya H, Alhommadi A, Cai H, Hou R, Deng WT, Boye SL, Almaghamsi A, Al Saikhan F, Al-Dhibi H, Birch D, Chung C, Colak D, LaVail MM, Vollrath D, **Erger K**, Wang W, Conlon T, Zhang K, Hauswirth W, Alkuraya FS. Treatment of retinitis pigmentosa due to MERTK mutations by ocular subretinal injection of adeno-associated virus gene vector: results of a phase I trial. Hum Genet. 2016 Mar;135(3):327-43. PMID: 26825853.
16. Sosa I, Estrada AH, Mincey BD, **Erger KE**, Conlon TJ. In vitro evaluation of mitochondrial dysfunction and treatment with adeno-associated virus vector in fibroblasts from Doberman Pinschers with dilated cardiomyopathy and a pyruvate dehydrogenase kinase 4 mutation. Am J Vet Res. 2016 Feb; 77(2):156-161. PMID: 27027709.
17. Ye G, Conlon T, **Erger K**, Sonnentag P, Sharma AK, Howard K, Knop DR, Chulay JD. Safety and biodistribution evaluation of rAAV2tYF-CB-hRS1, a recombinant AAV vector expressing retinoschisin, in RS1-deficient mice. Hum Gene Ther Clin Dev. 2015 Sep;26(3):177-84. PMID: 26390091.
18. Deng WT, Dyka FM, Dinculescu A, Li J, Zhu P, Chiodo V, Boye SL, Conlon TJ, **Erger KE**, Cossette T, Hauswirth WW. Stability and safety of an AAV vector for treating RPGR-ORF15 X-linked retinitis pigmentosa. Hum Gene Ther. 2015 Sep;26(9):593-602. PMID: 26076799.

19. Koilkonda R, Hong Y, Talla V, Porciatti V, Feurer W, Hauswirth W, Chiodo V, **Erger K**, Boye S, Lewin A, Conlon T, Renner L, Neuringer M, Detrisac C, Guy J. LHON gene therapy vector prevents visual loss and optic neuropathy induced by mutated G11778A mitochondrial DNA: biodistribution and toxicology profile. Invest Ophthalmol Vis Sci. 2014 Oct 23;55(12):7739-53. PMID: 25342621. PMCID: PMC4249950.
20. Corti M, Elder M, Falk D, Lawson LA, Smith B, Nayak S, Conlon T, Clement N, **Erger K**, Lavassani E, Green M, Doerfler P, Herzog R and Byrne B,. B-Cell depletion is protective against anti-AAV capsid immune response: A human subject case study. Mol Ther Methods Clin Dev. 2014; 1: 14033. PMCID: PMC4275004
21. Chen M, Lu Y, Hamazaki T, Tsai H, **Erger K**, Conlon T, Elshikha A, Li H, Srivastava A, Yao C, Brantly M, Chiodo V, Hauswirth W, Terada N, Song S. Reprogramming adipose tissue derived mesenchymal stem cells (AT-MSCs) into pluripotent stem cells by a mutant AAV vector. Hum Gene Ther Methods. 2014 Feb;25(1):72-82. PMID: 24191859.
22. **Erger K**, Conlon TJ, Porvasnik S, Cossette T, Roberts C, Combee L, Islam S, Kelley J, Clement N, Abernathy C, Byrne BL. Preclinical toxicology and biodistribution studies of recombinant adeno-associated virus 1 (rAVV1) human acid α-glucosidase (GAA) in NZW rabbits by intrathoracic direct and thorascopic guided injection in the diaphragm. Hum Gene Ther Clin Dev. 2013 Sep;24(3):127-33. PMID: 24021025.
23. Pogue B, Estrada AH, Sosa-Samper I, Maisenbacher HW, Lamb KE, Mincey BD, **Erger KE**, Conlon TJ. Stem cell therapy for Doberman Pinschers with dilated cardiomyopathy: A pilot study evaluating retrograde coronary venous delivery. J Small Anim Pract. 2013 Jul;54(7):361-6. PMID:23731226.
24. Conlon TJ, Deng WT, **Erger K**, Cossette T, Pang JJ, Ryals R, Clement N, Cleaver B, McDoom I, Boye SE, Peden MC, Sherwood MB, Abernathy CR, Alkuraya FS, Boye SL, Hauswirth WW. Preclinical potency and safety studies of an AAV2-mediated gene therapy vector for the treatment of MERTK associated retinitis pigmentosa. Hum Gene Ther Clin Dev. 2013 Mar;24(1):23-8. PMID: 23692380. PMCID: PMC3856558.
25. Boye SE, Alexander JJ, Boye SL, Witherspoon CD, Sandefer KJ, Conlon TJ, **Erger K**, Sun J, Ryals R, Chiodo VA, Clark ME, Girkin CA, Hauswirth WW, GamlinPD. The human rhodopsin kinase promoter in an AAV5 vector confers rod and cone specific expression in the primate retina. Hum Gene Ther. 2012 Oct;23(10):1101-15. PMID: 22845794. PMCID: PMC3472519.
26. Lee NC, Falk DF, Byrne BJ, Conlon TJ, Clement N, Porvasnik S, Jorgensen MJ, Potter M, **Erger KE**, Watson R, Ghivizzani S, Chiou HC, Chien YH, Hwu WL. An acidic oligopeptide displayed on AAV2 improves axial muscle tropism on systemic delivery. Genet Vaccines Ther. 2012 Jun 18;10(1):3 PMID: 22709483. PMCID: PMC3416570.
27. Keeler AM, Conlon T, Walter G, Zeng H, Shaffer S, Dungtao F, **Erger K**, Cossette T, Tang Q, Mueller C, Flotte TR. Long term correction of very long chain acyl-CoA dehydrogenase deficiency using AAV9 gene therapy. Mol Ther. 2012 Jun;20(6):1131-8. PMID: 22395529. PMCID: PMC3370259.
28. Polyak S, Mach A, Porvasnik S, Dixon L, Conlon T, **Erger KE**, Acosta A, Wright A, Campbell-Thompson M, Zolotukhin I, Wasserfall C, Mah C. Identification of adeno-associated viral vectors suitable for intestinal gene delivery and modulation of experimental colitis. Am J Physiol Gastrointest Liver Physiol. 2012 Feb;302(3):G296-308. PMID: 22114116.
29. Jacobson SG, Cideciyan AV, Ratnakaram R, Heon E, Schwartz SB, Roman AJ, Peden MC, Aleman TS, Boye SL, Sumaroka A, Conlon TJ, Calcedo R, Pang JJ, **Erger KE**, Olivares MB, Mullins CL, Swider M, Kaushal S, Feuer WJ, Iannaccone A, Fishman GA, Stone EM, Byrne BJ, Hauswirth WW. Gene therapy for Leber Congenital Amaurosis caused by *RPE65* mutations: Safety and efficacy in

fifteen children and adults followed up to three years. Arch Ophthalmol. 2012 Jan;130(1):9-24. PMID: 21911650.
30. Boye SL, Conlon T, **Erger K**, Ryals R, Neeley A, Cossette T, Pang J, Dyka FM, Hauswirth WW, Boye SE. Long term preservation of cone photoreceptors and restoration of cone function by gene therapy in the guanylate cyclase-1 knockout (GC1KO) mouse. Invest Ophthalmol Vis Sci. 2011 Sep 9;52(10):7098-108. PMID: 21778276. PMCID: PMC3207713
31. Specht A, Fiske L, **Erger K**, Cossette T, Verstegen JP, Campbell-Thompson M, Struck MB, Lee YM, Chou JY, Byrne BJ, Correia C, Mah CS, Weinstein DA, Conlon T. Glycogen storage disease type Ia in canines: A model for human metabolic and genetic liver disease. J Biomed Biotechnol. 2011; 2011:646257. PMID: 21318173. PMCID: PMC3027000
32. **Erger K**, Conlon TJ, Leal N, Zori R, Bobik T, Flotte TR. *In vivo* expression of human ATP:cob(I)alamin adenosyltransferase (ATR) using recombinant adeno-associated virus (rAAV) serotypes 2 and 8. J Gene Med. 2007 Jun; 9(6):462-9. PMID: 17471589.
33. Conlon TJ, Walter G, Owen R, Cossette T, **Erger K**, Gutierrez G, Goetzman E, Matern D, Vockley J, Flotte TR. Systemic correction of a fatty acid oxidation defect by intramuscular injection of a recombinant adeno-associated virus vector. Hum Gene Ther. 2006 Jan;17(1):71-80. PMID: 16409126.
34. Conlon TJ, Cossette T, **Erger K**, Choi YK, Clarke T, Scott-Jorgensen M, Song S, Campbell-Thompson M, Crawford J, Flotte TR. Efficient hepatic delivery and expression from a recombinant adeno-associated virus 8 (rAAV8) pseudotyped alpha-1 antitrypsin vector. Mol Ther. 2005 Nov; 12(5):867-75. PMID: 16085464.

INVITED SPEAKER

1. "A Practical Guide to Preclinical Study Design". Clinical Trials Training I. American Society of Gene & Cell Therapy 23rd Annual Meeting, Virtual, May 11, 2020.

SELECTED ABSTRACTS

1. Pope MK, **Coleman K**, et al. Assessment of Gene Therapy Treatment in the Pompe Disease Canine Model. American Society of Gene & Cell Therapy 25th Annual Meeting, Washington, D.C. May 16-19, 2022.
2. D'Souza P, **Coleman KE**, et al. Immune Modulation Preceding AAV9-GLB1 Gene Therapy Preserves the Possibility for Re-Dosing in Children with GM1 Gangliosidosis. American Society of Gene & Cell Therapy 24th Annual Meeting, Virtual, May 11-14, 2021.
3. Salabarria SM, Norman SL, Berthy JA, Elder ME, **Coleman KE**, et al. Systemic AAV Delivery Activates the Classical Complement Pathway Leading to Thrombotic Microangiopathy. American Society of Gene & Cell Therapy 24th Annual Meeting, Virtual, May 11-14, 2021.
4. Cravey L, **Coleman KE**, et al. Assessment of Preexisting Humoral Immunity to AAV9 in a Population of Mothers and their Newborn Infants. 38th Annual Pediatric Science Day, University of Florida. June 4, 2020.
5. Shutterly A, Subramony S, Norman S, Sharot B, Perez B, **Coleman K**, et al. Dual Routes of Administration in Clinical Trial Design for the Treatment of Friedreich's Ataxia. American Society of Gene & Cell Therapy 23rd Annual Meeting, Virtual, May 12-15, 2020.

6. Perez BA, **Coleman KE**, et al. Management of Preexisting Immunity to AAV9 in Freidreich's Ataxia. American Society of Gene & Cell Therapy 22nd Annual Meeting, Washington, DC, April 29-May 2, 2019.
7. Nair J, Freeman D, Davis I, McParland T, Pope M, Perez B, Rodriguez-Lebron E, **Coleman K**, et al. AAV9 Bio-Distribution with Different Routes of CNS Administration in Rodents. American Society of Gene & Cell Therapy 22nd Annual Meeting, Washington, DC, April 29-May 2, 2019.
8. Fathalikhani DD, Bukovec KE, Hamm SE, Addington AK, Zhang H, Perry JB, Mansueto A, McMillan RP, Lawlor MW, **Coleman K**, et al. Effects of exercise on the efficacy of microdystrophin gene therapy. New Directions Conference, New Orleans, LA, June 25-28, 2018.
9. Levings R, **Coleman K**, et al. Safety and biodistribution assessment of scAAV2.5.eqIL-Ra gene transfer to a large mammalian joint. American Society of Gene & Cell Therapy 21st Annual Meeting, Chicago, IL, May 16-19, 2018.
10. Corti M, Gessler D, Norman S, **Coleman K**, et al. Immune blockade in CNS gene therapy improves safety and clinical outcome. American Society of Gene & Cell Therapy 21st Annual Meeting, Chicago, IL, May 16-19, 2018.
11. Song C, Conlon T, Deng W, **Coleman K**, et al. Evaluating safety and potency of the AAV2tYF-GRK1-RPGRco vector in RPGR-deficient Rd9 mice. American Society of Gene & Cell Therapy 21st Annual Meeting, Chicago, IL, May 16-19, 2018.
12. Golebiowski D**, Coleman KE**, et al. Profile of circulating and neutralizing antibody titers towards AAV8 & AAV9 in cynomolgus macaques. American Society of Gene & Cell Therapy 20th Annual Meeting, Washington, DC, May 10-13, 2017.
13. **Erger K**, Mandel R, et al. Biodistribution and toxicology studies of AAV2/rh8-CBA-β-hexosaminidase after intracranial delivery in C57/Bl6 mice. American Society of Gene & Cell Therapy 16th Annual Meeting, Salt Lake City, UT, May 15-18, 2013.
14. Conlon TJ, Ryals R, **Erger K**, et al. MERTK. American Society of Gene & Cell Therapy 16th Annual Meeting, Salt Lake City, UT, May 15-18, 2013. [Oral Presentation]
15. Sosa I, Winter MD, **Erger K**, et al. Biodistribution of retrograde coronary sinus delivery of mesenchymal stem cells: a pilot study. Third Annual North American Veterinary Regenerative Medicine Association Conference. Savannah, GA, November 8-10, 2012. [Oral Presentation]
16. Keeler AM, Conlon T, **Erger K**, et al. Sex-Dependent expression of very long chain acyl-CoA dehydrogenase (VLCAD) transgene and the role of the liver in biochemical and phenotypic correction of VLCAD deficiency. American Society of Gene & Cell Therapy 15th Annual Meeting, Philadelphia, PA, May 16-19, 2012.
17. Keeler AM, Esteves MS, Conlon T, **Erger K**, et al. Transduction and expression in brain after systemic delivery of single stranded AAV9 in adult mice. American Society of Gene & Cell Therapy 15th Annual Meeting, Philadelphia, PA, May 16-19, 2012.
18. Fiske L, Specht A, **Erger K**, et al. Evidence of Abnormal Growth and Metabolic Profiles in Heterozygotes in the Canine Model of Glycogen Storage Disease Type Ia. 30th Annual Pediatric Science Day, University of Florida. March 8, 2012. [Oral presentation]
19. Specht A, Fiske L, **Erger K**, et al. Clinical manifestations of heterozygous expression of a mutation that causes glycogen storage disease type Ia in dogs. American College of Veterinary Internal Medicine Forum, Denver, CO, June 15-18, 2011. [Oral presentation]
20. Boye SL, Conlon T, **Erger K**, et al. Long-term persistace of AAV mediated gene therapy for Leber Congenital Amaurosis Type 1 (LCA 1). American Society of Gene & Cell Therapy 14th Annual Meeting, Seattle, WA, May 18-21, 2011.
21. Falk DJ, Mah CS, Soustek MS, Lee K, Cloutier DA, Elmallah MK, **Erger KE**, et al. Intrathoracic administration of AAV9 reverses neural and cardiorespiratory dysfunction in Pompe disease.

American Society of Gene & Cell Therapy 14th Annual Meeting, Seattle, WA, May 18-21, 2011. [Oral Presentation]

22. Boye SE, Boye SL, Conlon T, **Erger K**, et al. Gene therapy for *Gucy2d* Leber congenital amaurosis (LCA1). The Association for Research in Vision and Ophthalmology Annual Meeting, Ft. Lauderdale, FL. May 1-5, 2011.
23. Falk D, **Erger K**, et al. Next generation treatment of Pompe disease using systemic gene transfer with AAV9. World Lysosomal Disease Network, Las Vegas, NV. February 15-19, 2011.
24. **Erger K**, Porvasnik S, et al. Preclinical toxicology and biodistribution studies of recombinant adeno-associated virus 1 (rAAV1) human acid α-glucosidase (GAA) in NZW rabbits by intrathoracic direct injection in the diaphragm. Molecular Therapy 17:1 S822, 2009.
25. **Erger K**, Conlon TJ, et al. Toxicology, biodistribution and long term safety study of a recombinant AAV1 vector for limb girdle muscular dystrophy (LGMD) type-2D. 26th Annual Pediatric Science Day, University of Florida. March 13, 2008. [Oral presentation]
26. **Erger K**, Conlon TJ, Cossette T, Bobik T, Flotte TR. In vivo expression of human cob(I)alamin adenosyltransferase using rAAV serotypes 1, 2 and 8, an approach to gene therapy for methylmalonic aciduria (MMA). Molecular Therapy 11:1 S47, 2005.
27. Poirier A, Conlon TJ, Combee L, **Erger K**, Flotte TR. Toxicology and biodistribution studies of a recombinant adeno-associated virus 1 (rAAV1) alpha-1 antitrypsin (AAT) vector. Molecular Therapy 11:1 S54, 2005.
28. **Erger K**, Conlon TJ, Matern D, Bobik T, Flotte TR. Recombinant adeno-associated virus gene therapy for cob(I)alamin adenosyltransferase deficiency. Molecular Therapy 9:1 S166, 2004.