STEVEN JAMES GRAY

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EDUCATION

Postdoctoral Training	University of North Carolina	09/2006-09/2008	Virology and Gene Therapy with Dr. R. Jude Samulski
Ph.D.	Vanderbilt University	08/2000-08/2006	Molecular Biology with Dr. Ellen Fanning
B.Sc.	Auburn University	08/1996-06/2000	Molecular Biology, with honors

PROFESSIONAL EXPERIENCE

Associate Professor	Dept. of Pediatrics, U. Texas Southwestern Dept. of Molecular Biology, UTSW Dept. of Neurology and Neurotherapeutics, UTSW Center for Regenerative Science and Medicine, UTSW McDermott Center for Human Growth and Dev., UTSW	12/2017-present
Director	UTSW Viral Vector Facility	12/2017-present
Adjunct Asst. Prof.	Dept. of Pediatrics, UNC Chapel Hill	12/2017-11/2018
Assistant Professor	Dept. of Ophthalmology, U. of N. Carolina at Chapel Hill	06/2015-11/2017
Research Assistant Professor	Dept. of Ophthalmology, U. of N. Carolina at Chapel Hill	11/2012-06/2015
Research Associate	University of North Carolina at Chapel Hill	10/2008-10/2012

HONORS AND AWARDS

2019	American Society of Gene and Cell Therapy, Outstanding New Investigator Award
2016	Triangle Business Journal's 2016 Health Care Hero Award
2000	Phi Kappa Phi National Honor Society (Auburn chapter)
2000	Golden Key National Honor Society (Auburn chapter)
2000	Summa Cum Laude, with Honors, Auburn University
2000	Dept. of Biological Sciences Undergraduate Teaching Award, Auburn U.
1999-2000	Barry M. Goldwater Scholarship Award
1999	The Outstanding Junior in the Dept. of Botany and Microbiology, Auburn U.
1998, 1999	Excellence in Biological Science Scholarship Award, Auburn University
1997-2000	Dean's Medalist, Dept. of Biological Sciences, Auburn University
1996-1999	Howard Hughes (HHMI) Undergraduate Research Fellow, Auburn University

PATENTS

1. United States patent #9636370 (issued 05/02/2017). *AAV vectors targeted to oligodendrocytes*
2. United States patent application PCT/US2015/061788. *AAV Vectors Targeted to the Central Nervous System*
3. United States Patent application PCT/US2017/037118. *Optimized CLN1 genes and expression cassettes and their use*
4. United States Provisional Patent Application No. 62/582,664. *Optimized AGA genes and expression cassettes and their use*
5. United States Provisional Patent Application No. 62/717,251. *Optimized CLN7 genes and expression cassettes and their use*
6. United States Provisional Patent Application No. 62/691,359. *Longitudinal in vivo monitoring of CNS demonstrates the efficacy of gene therapy in a sheep model of CLN5 Batten disease*
7. International patent application PCT/US2019/048776. *Feedback enabled synthetic genes, target seed match cassettes, and their uses*
8. United States Provisional Patent Application No. 62/755,871. *Optimized FIG4 genes and expression cassettes and their use*

9. United States Provisional Patent Application No. 62/783,856. *Optimized GALC genes and expression cassettes and their use*
10. United States Provisional Patent Application No. 62/840,114. *Optimized SUMF1 genes and expression cassettes and their use*
11. United States Provisional Patent Application No. 62/851,411. *UBE3A genes and expression cassettes and their use*

BIBLIOGRAPHY

Published or Accepted for Publication

1. Gray SJ. (2019) The evolution of adeno-associated virus capsids for CNS gene therapy. *Cell & Gene Therapy Insights*. 5(11), 1359–1366.
2. Armao D, Bouldin TW, Bailey RM, Hooper JE, Barucha DX, **Gray SJ**. (2019) Advancing the pathologic phenotype of giant axonal neuropathy: early involvement of the ocular lens. *Orphanet J Rare Dis.* 14(1):27. PMID: 30709364.
3. Goodspeed K, Harder L, Hughes S, Conger D, Taravella J, **Gray SJ**, Minassian B. (2018) Optical coherence tomography features in brothers with aspartylglucosaminuria. *Ann Clin Transl Neurol.* 5(12):1622-1626. PMID: 30564628.
4. Woodley E, Osmon KJL, Thompson P, Richmond C, Chen Z, **Gray SJ**, Walia JS. (2018) Efficacy of a Bicistronic Vector for Correction of Sandhoff Disease in a Mouse Model. *Mol Ther Methods Clin Dev*. 12:47-57. PMID: 30534578.
5. Mitchell NL, Russell KN, Wellby MP, Wicky HE, Schoderboeck L, Barrell GK, Melzer TR, **Gray SJ**, Hughes SM, Palmer DN. (2018) Longitudinal In Vivo Monitoring of the CNS Demonstrates the Efficacy of Gene Therapy in a Sheep Model of CLN5 Batten Disease. *Mol Ther*. S1525-0016(18)30340-X. PMID: 30078766.
6. Ramsingh AI, **Gray SJ**, Reilly A, Koday M, Bratt D, Koday MT, Murnane R, Smedley J, Hu Y, Messer A, Fuller DH. (2018) Sustained AAV9-mediated expression of a non-self protein in the CNS of non-human primates after immunomodulation. *PLoS One.* 6;13(6):e0198154. PMID: 29874260.
7. Lykken EA, Shyng C, Edwards RJ, Rozenberg A, **Gray SJ***. (2018) Recent progress and considerations for AAV gene therapies targeting the central nervous system. *J Neurodev Disord.* 18;10(1):16. PMID: 29776328. [*corresponding author]
8. Bailey RM, Armao D, Nagabhushan Kalburgi S, **Gray SJ**. (2018) Intrathecal Adeno-Associated Virus 9 Gene Therapy for Giant Axonal Neuropathy. *Mol Ther Meth Clin Dev.* 15;9:160-171. PMID: 29766026. [*corresponding author].
9. Marshall MS, Issa Y, Jakubauskas B, Stoskute M, Elackattu V, Marshall JN, Bogue W, Nguyen D, Hauck Z, Rue E, Karumuthil-Melethil S, Zaric V, Bosland M, van Breemen RB, Givogri MI, **Gray SJ**, Crocker SJ, Bongarzone ER. (2018) Long-Term Improvement of Neurological Signs and Metabolic Dysfunction in a Mouse Model of Krabbe's Disease after Global Gene Therapy. *Mol Ther.* S1525-0016(18)30016-9. PMID: 29433937.
10. Sinnett SE, **Gray SJ*** (2017) Recent endeavors in MECP2 gene transfer for gene therapy of Rett syndrome. *Discovery Medicine.* 24(132):153-159. [*corresponding author]
11. Mandel RJ, Marmion DJ, Kirik D, Chu Y, Heindel C, McCown T, **Gray SJ**, Kordower JH (2017) Novel oligodendroglial alpha synuclein viral vector models of multiple system atrophy: studies in rodents and nonhuman primates. *Acta Neuropathol Commun*, 5(1):47. PMID: 28619074.
12. Sinnett SE, Hector RD, Gadalla KKE, Heindel C, Chen D, Zaric V, Bailey MES, Cobb SR, **Gray SJ*** (2017) Improved MECP2 gene therapy extends the survival of MeCP2-null mice without apparent toxicity after intracisternal delivery. *Mol Ther Meth Clin Dev.*, 5:106. PMID: 28497072. [*corresponding author]

13. Gadalla KKE, Vudhironarit T, Hector RD, Sinnett SE, Bahey N, Bailey MES, **Gray SJ**, Cobb SR (2017) AAV gene therapy in a mouse model of Rett syndrome: dose and vector evaluation. *Mol Ther Meth Clin Dev.*, 5:180. PMID: 28497075.
14. Banning A, König JF, **Gray SJ**, Tikkanen R. (2017) Functional Analysis of the Ser149/Thr149 Variants of Human Aspartylglucosaminidase and Optimization of the Coding Sequence for Protein Production. *Int J Mol Sci*, 18(4):E706. PMID: 28346360.
15. Banning A, Gülec C, Rouvinen J, **Gray SJ**, Tikkanen R (2016) Identification of Small Molecule Compounds for Pharmacological Chaperone Therapy of Aspartylglucosaminuria. *Sci Rep.*, 6:37583. PMID: 27876883.
16. Francis JS, Wojtas I, Markov V, **Gray SJ**, McCown TJ, Samulski RJ, Bilaniuk LT, Wang DJ, De Vivo DC, Janson CG, Leone P. (2016) N-acetylaspartate supports the energetic demands of developmental myelination via oligodendroglial aspartoacylase. *Neurobiol Dis.*, 4;96:323-334. PMID: 27717881.
17. Karumuthil-Melethil S and **Gray SJ*** (2016) Immunological Considerations for Treating Globoid Cell Leukodystrophy. *Journal of Neuroscience Research*, (11):1349-58. PMID: 27638617. [*corresponding author]
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21. Armao D, Bailey RM, Bouldin TW, Kim Y, **Gray SJ*** (2016) Autonomic nervous system involvement in the giant axonal neuropathy (GAN) KO mouse: implications for human disease. *Clin Auton Res*., 26(4):307-13. PMID: 27369358. [*corresponding author]
22. **Gray SJ*** (2016) Timing of Gene Therapy Interventions: The Earlier, the Better. *Mol Ther*., 24(6):1017-8. PMID: 27324445. [*corresponding author]
23. Osmon KJ, Woodley E, Thompson P, Ong K, Karumuthil-Melethil S, Keimel JG, Mark BL, Mahuran D, **Gray SJ**, Walia JS (2016) Systemic Gene Transfer of a Hexosaminidase Variant Using an scAAV9.47 Vector Corrects GM2 Gangliosidosis in Sandhoff Mice. *Hum Gene Ther.*, 27(7):497-508. PMID: 27199088.
24. Karumuthil-Melethil S, Nagabhushan Kalburgi S, Thompson P, Tropak M, Kaytor MD, Keimel JG, Mark BL, Mahuran D, Walia JS, **Gray SJ*** (2016) Novel Vector Design and Hexosaminidase Variant Enabling Self-Complementary Adeno-Associated Virus for the Treatment of Tay-Sachs Disease. *Hum Gene Ther.*, 27(7):509-21. PMID: 27197548. [*corresponding author]
25. Tropak MB, Yonekawa S, Karumuthil-Melethil S, Thompson P, Wakarchuk W, **Gray SJ**, Walia JS, Mark BL, Mahuran D (2016) Construction of a hybrid beta-hexosaminidase subunit capable of forming stable homodimers that hydrolyze GM2 gangliosides in vivo. *Mol Ther Methods Clin Dev*, 2;3:15057. PMID: 26966698.
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30. Powell SK, Rivera-Soto R, **Gray SJ*** (2015) Viral expression cassette elements to enhance transgene target specificity and expression in gene therapy. *Discov Med,* 19(102):49-57. PMID 25636961. [*corresponding author]
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33. Kantor B, McCown T, Leone P, and **Gray SJ*** (2014) Clinical Applications Involving CNS Gene Transfer. In T. Friedmann, J.C. Dunlap, & S.F. Goodwin (Eds), Advances in Genetics. Academic Press, p. 71-124. PMID 25311921. [*corresponding author]
34. Sloniowski S, Fox JC, **Gray SJ*** (2013) Perspectives in using gene therapy for lysosomal storage diseases. *Drugs of the Future,* 38(9): 635. [*corresponding author]
35. Nagabhushan Kalburgi S, Khan NN, **Gray SJ*** (2013) Recent gene therapy advancements for neurological diseases. *Discovery Medicine,* 15(81):111-9. PMID: 23449113. [*corresponding author]
36. Simonato M, Bennett J, Boulis NM, Castro MG, Fink DJ, **Gray SJ**, Lowenstein PR, Tobin AL, Vandenberghe LH, Wolfe JH, and Glorioso JC (2013) Progress in gene therapy for neurological disorders. *Nature Reviews Neuroscience,* 9(5):277-91. PMID: 23670108.
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38. **Gray SJ***, Nagabhushan Kalburgi S, McCown TJ, Samulski RJ (2013) Global CNS Gene Delivery and Evasion of Anti-AAV Neutralizing Antibodies by Intrathecal Vector Administration in Non-Human Primates. *Gene Therapy,* 20(4):450-9. PMID: 23303281. PMCID: 3618620. [*corresponding author]
39. Mussche S, Devreese B, Nagabhushan Kalburgi S, Bachaboina L, Fox JC, Shih HJ, Samulski RJ, Van Coster R, **Gray SJ*** (2013) Restoration of Cytoskeleton Homeostasis After Gigaxonin Gene transfer for Giant Axonal Neuropathy. *Human Gene Therapy*, 24(2):209-19. PMID: 23316953. [*corresponding author]
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41. **Gray SJ** (2013) Gene Therapy and Neurodevelopmental Disorders. *Neuropharmacology,* 68:136-42. PMID: 22750077.
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43. Bowles DE, McPhee SWJ, Li C, **Gray SJ**, Samulski JJ, Camp AS, Li J, Wang B, Monahan PE, Rabinowitz JE, Grieger JC, Govindasamy L, Agbandje-McKenna M, Xiao X, Samulski RJ (2012)

Phase 1 Gene Therapy for Duchenne Muscular Dystrophy using a Designer AAV Vector. *Molecular Therapy*, 20(2):443-55. PMID: 22068425. PMCID: 3277234.
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50. **Gray SJ**, Foti SB, Schwartz JW, Bachaboina L, Taylor-Blake B, Coleman J, Ehlers MD, Zylka MJ, McCown TJ, Samulski RJ (2011) Optimizing promoters for rAAV-mediated gene expression in the peripheral and central nervous system using self-complementary vectors. *Human Gene Therapy*, 22(9):1143-53. PMID: 21476867. PMCID: 3177952.
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52. **Gray SJ**, Woodard KT, Samulski RJ (2010) Viral Vectors and Delivery Strategies for CNS Gene Therapy. *Therapeutic Delivery*, 1(4):517-534. PMID: 22833965.
53. Mendell JR, Campbell K, Rodino-Klapac L, Sahenk Z, Shilling C, Lewis S, Bowles D, **Gray S**, Li C, Galloway G, Malik V, Coley B, Clark KR, Li J, Xiao X, Samulski J, McPhee SW, Samulski RJ, Walker CM. (2010) Dystrophin immunity in Duchenne's muscular dystrophy. *New England Journal of Medicine*, 363(15):1429-37. PMID: 20925545. PMCID: 3014106.
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55. **Gray SJ**, Blake B, Criswell HE, Nicolson SC, Samulski RJ, and McCown TJ (2010) Directed Evolution of a Novel Adeno-associated Virus (AAV) Vector that Crosses the Seizure Compromised Blood-Brain Barrier (BBB). *Molecular Therapy*, 18(3):570-8. PMID: 20040913. PMCID: 2831133.
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57. **Gray SJ** and Samulski RJ (2008) Optimizing Gene Delivery Vectors for the Treatment of Heart Disease. *Expert Opinion on Biological Therapy*, 8(7):911-22. PMID: 18549322.
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59. **Gray SJ**, Liu G, Altman AL, Small LE, and Fanning E (2007) Discrete Functional Elements Required for Initiation Activity of the Chinese Hamster Dihydrofolate Reductase Origin Beta at Ectopic Chromosomal Sites. *Experimental Cell Research*, 313(1):109-20. PMID: 17078947

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PROFESSIONAL PRESENTATIONS (SELECTED)

2019 European Society of Gene and Cellular Therapy Annual Mtg. Barcelona, Spain. Oct 2019.
PLENARY TALK: "Intrathecal AAV9 as a platform approach to treat multiple CNS disorders"

The 8th Annual Symposium on ATP1A3 in Disease. Rekjavik, Iceland. Oct 2019.
INVITED TALK: "Steps toward gene therapy for ATP1A3"

2019 SSIEM Meeting. Rotterdam, Netherlands. Sept 2019.
INVITED TALK: "Evolving gene therapy landscape targeting the CNS"

2019 ISRMD meeting. Atlanta, GA. July 2019.
INVITED TALK: "Preclinical gene therapy with scAAV9/AGA in aspartylglucosaminuria mice provides evidence for clinical translation"

2019 Angelman Syndrome Foundation Mtg. St. Louis, MO. July 2019.
INVITED TALK: "Development of gene therapy to treat Angelman disease"

2019 Global Foundation for Peroxisome Disorders Mtg. Washington, DC. July 2019.
INVITED TALK: "Overview of gene therapy for CNS disorders"

2019 American Society of Gene and Cellular Therapy Annual Mtg. Washington, D.C. May 2019.
INVITED TALK: "Basics of AAV Biology."
OUTSTANDING NEW INVESTIGATOR PLENARY TALK: "On the brink of a treatment revolution for inherited pediatric neurological disorders."

Medical Innovations Collaborative Symposium. Fort Worth, TX. March 2019.
KEYNOTE: "The Forever Fix: How Gene Therapy is Bringing New Hope for Untreatable Diseases"

2nd Asia Pacific Lysosome Storage Disease Conference. Auckland, NZ. Feb 2019.
INVITED SPEAKER: "Gene therapy for lysosomal diseases"

WORLD*Symposium* 2019 on Lysosomal Storage Diseases. Orlando, FL. Feb 2019.
ORAL ABSTRACT "Intrathecal and intravenous combination gene therapy in the mouse model of infantile neuronal ceroid lipofuscinosis extends lifespan and improves behavioral outcomes in moderately affected mice"

AAV Gene Therapy Symposium. Houston, TX. December 2018.
INVITED TALK: "Platform AAV-based gene transfer approaches to treat the nervous system"

7th ATP1A3 in Disease Symposium. Chicago, IL. October 2018.
INVITED TALK: "AAV-based gene transfer to the nervous system"

2018 NIH & FDA Gene Therapy Workshop. Bethesda, MD. August 2018.
INVITED TALK & PANELIST: "Patient advocacy driving forward first-in-human clinical trials"

15th International Symposium on MPS and Related Diseases. San Diego, CA. August 2018.
INVITED TALK: "Translating gene therapy from bench to bedside for neurological disorders"

Gene Therapy for CMT: Opportunities and Challenges
INVITED TALK: "Gene Therapy: Challenges and practical considerations"

New Frontiers in Pediatric Neurology. Dallas, TX. June 2018.
KEYNOTE SPEAKER: "The Forever Fix: How Gene Therapy is Bringing New Hope for Untreatable Diseases"

Women's Health Symposium. Dallas, TX. 2018.
INVITED TALK: "The Forever Fix: How Gene Therapy is Bringing New Hope for Untreatable Diseases"

Family Conference on Pediatric Hereditary Spastic Paraparesis (HSP). Dallas, TX. April 2018.
INVITED TALK: "The Forever Fix: How Gene Therapy is Bringing New Hope for Untreatable Diseases"

2018 American Society of Gene and Cellular Therapy Annual Mtg. Chicago, IL. May 2018.

INVITED TALK "Optimization of AAV-mediated MeCP2 gene transfer for the treatment of Rett Syndrome."

15th Annual World Congress for SBMT. Los Angeles, CA. April 2018.
INVITED TALK AND SESSION CHAIR: "First-in-human intrathecal AAV9 gene transfer for the treatment of nervous system diseases"

WORLD*Symposium* 2018 on Lysosomal Storage Diseases. San Diego, CA. Feb 2018.
ORAL ABSTRACT "Identification of novel AAV capsids for the treatment of lysosomal storage diseases"

American College of Toxicology. Palm Springs, CA. November 2017
PLENARY TALK "Enabling rare disease families to drive the development of transformative new treatments"
INVITED TALK "Initiation of First-in-Human Gene Therapy for Giant Axonal Neuropathy"

Global Genes Annual RARE Patient Advocacy Summit. Irvine, CA. September 2017.
INVITED TALK "Initiation of First-in-Human Gene Therapy for Rare Neurological Diseases"

The Global Foundation for Peroxisome Disorders Symposium. Bethesda, MD. July 2017.
INVITED TALK "Gene Therapy for Giant Axonal Neuropathy"

Angelman Syndrome Foundation Research Symposium. Phoenix, AZ. July 2017.
INVITED TALK "Gene Therapy for Central Nervous System Diseases"

American Society for Cell and Gene Therapy. Washington, D.C. May 2017.
ORAL ABSTRACT "Postmortem Assessment of Vector Biodistribution in the First-in-Human Intrathecal scAAV9 Gene Therapy Trial for Giant Axonal Neuropathy"

ARVO. Baltimore, MD. May 2017.
POSTER ABSTRACT "Retinal Ganglion Cell Gene Transfer Is Achieved Following Intrathecal Administration of AAV9"

New York Academy of Science. New York, NY. April 2017.
INVITED TALK "Strategies and applications for widespread CNS gene transfer using AAV vectors.

UNC Catalyst Symposium. Chapel Hill, NC. March 2017
INVITED TALK "Initiation of first-in-human gene therapy for rare neurological diseases.

UT-Southwestern. Dallas, TX. March 2017.
INVITED TALK "AAV-mediated gene therapy for nervous system disorders"

Queen's University. Kingston, Ontario. November 2016.
INVITED TALK "AAV-mediated gene therapy for nervous system disorders"

University or Toronto. Toronto, Ontario. November 2016.
INVITED TALK "AAV-mediated gene therapy for nervous system disorders"

Pitt-Hopkins Research Foundation Annual Symposium. Dallas, TX. November 2016
INVITED TALK "Gene therapy for nervous system disorders"

Establishing Translational Platforms for H-ABC/TUBB4A-related leukodystrophy. Children's Hospital of Philadelphia, Philadelphia, PA. October, 2016.
INVITED TALK "Lessons from Giant Axonal Neuropathy"

Hereditary Neuropathy Foundation Summit. New York, NY. October 2016.
INVITED TALK "Gene replacement therapy in GAN – a potential therapeutic approach for hereditary neuropathies."

Batten Disease Support and Research Association Annual Meeting. St. Louis, MO. July 2016.
INVITED TALK "Gene Therapy for INCL"

GFPD Scientific Advisory Board Meeting. Baltimore, MD. June 2016.
INVITED TALK "Therapeutic Gene Delivery Vectors for CNS Disorders"

Batten Disease: 2016 Update of Translational Research for Management of INCL/LINCL. Bethesda, MD. March 2016.
INVITED TALK "Gene Therapy for INCL"

KTRN Krabbe Translational Research Network Meeting. Captiva Island, FL. February 2016.

INVITED TALK "Intrathecal administration of AAV/GALC vectors in juvenile twitcher mice improves survival and is enhanced by BMT."

14th Annual Gene Therapy Symposium for Heart, Lung, and Blood Diseases. Sonoma, CA. November 2015.
INVITED TALK "Development of a First-in-Human Intrathecal scAAV9 Gene Therapy for Giant Axonal Neuropathy"

Rett Syndrome Research Trust Meeting. Boston, MA. November 2015.
INVITED TALK "AAV vector design considerations for MeCP2 gene transfer"

Glycoproteinoses: 4th International Conference on Advances in Pathogenesis and Therapy. St. Louis, MO. July 2015.
INVITED TALK "Insights from Aspartylglucosaminuria mice"

Belgian Society of Pediatric Neurology. Gent, Belgium. April 2015.
INVITED TALK: "How far are we from AAV-mediated global CNS gene transfer to treat neurological diseases in clinical practice?"

Rett Syndrome – Pathways to Clinical Trials (Part II). Boston, MA. April 2015.
INVITED TALK "Gene transfer strategies for the treatment of Rett syndrome"

British Neuroscience Association. Edinburg, Scotland. April 2015.
INVITED TALK *and session co-chair* "CNS gene therapy in neuropathy – from basic science to clinical trials"

Rett Syndrome – Pathways to Clinical Trials. Boston, MA. October 2014.
INVITED TALK "Gene transfer strategies for the treatment of Rett syndrome"

University of Florida. Gainesville, FL. September 2014.
INVITED TALK **"**AAV Vector design and application: research, preclinical, and clinical gene transfer to the nervous system."

6th Annual Workshop on Krabbe Disease. Ellicottville, NY. July 2014.
INVITED TALK "Translational approaches for Krabbe disease gene therapy."

Workshop on Translational Research Priorities for Infantile (CLN1) and Late Infantile (CLN2) Forms of Batten's Disease. Bethesda, MD. March 2014.
INVITED TALK "Strategies and practical considerations for AAV-mediated global CNS delivery"

KTRN Krabbe Translational Research Network Meeting. Ft. Lauderdale, FL. March 2014.
INVITED TALK "Translational approaches for Krabbe disease gene therapy."

2013 Workshop on Krabbe Disease. Ellicottville, NY. July 2013.
INVITED TALK "Possible immune complications associated with global CNS gene transfer for Krabbe: What we've learned from the monkeys."

KTRN Krabbe Translational Research Network Meeting. Miami, FL. March 2013.
INVITED TALK "Possible immune complications associated with global CNS gene transfer for Krabbe: What we've learned from the monkeys."

Pfizer. Boston, MA. November 2012.
INVITED TALK "AAV vector design and application: Research, preclinical, and clinical gene transfer"

Rett Syndrome Research Trust Meeting. Tarrytown, NY. November 2012.
INVITED TALK "MeCP2 Gene Therapy for Rett Syndrome: Proof of Concept, Translational Obstacles, and Development of New AAV Vectors"

HHMI Janelia Farm Research Campus. Ashburn, VA. October 2012.
INVITED TALK "Designing AAV capsids for specific research and therapeutic applications"

10th European Congress on Epileptology. London, U.K. October 2012.
INVITED TALK "Novel vectors in epilepsy"

5th Annual Workshop on Krabbe Disease. Ellicottville, NY. August 2012.
INVITED TALK "Global CNS gene delivery platform in non-human primates utilizing self-complementary AAV9 vectors."

Tulane National Primate Research Center. New Orleans, LA. June 2012.

INVITED TALK "Intrathecal AAV9 Gene Delivery: A Powerful New Clinical Option for Spinal Cord and Brain Diseases"

7th World Congress on Rett Syndrome. New Orleans, LA. June 2012.
INVITED TALK "MeCP2 Gene Therapy for Rett Syndrome: Proof of Concept and Translational Obstacles"

2012 American Society of Gene and Cellular Therapy Annual Mtg. Philadelphia, PA. May 2012.
INVITED TALK "Engineered AAV capsids tailored for specific therapeutic applications"

2012 GAN Symposium. Chicago, IL. April 2012.
INVITED TALK "Application of Global CNS Gene Delivery for Giant Axonal Neuropathy"

University of Glasgow. Glasgow, UK. March 2012.
INVITED TALK "Clinical Implications for AAV-mediated Global CNS Gene Delivery in Pigs and Non-Human Primates"

KTRN Krabbe Translational Research Network Meeting. Pittsburg, PA. March 2012.
INVITED TALK "Global CNS Gene Delivery Approaches for Krabbe"

University of Albany Wadsworth Center. Albany, NY. February 2012.
INVITED TALK "Intrathecal AAV9 gene delivery: a powerful new clinical option for brain and spinal cord diseases.

2011 Society for Neuroscience Mtg. Washington, D.C. November 2011.
SHORT COURSE #1 INSTRUCTOR, *Gene Vector Design and Application to Treat Nervous System Disorders.* Title: "Breakthroughs in AAV Vector Design"

2011 GAN Symposium. Columbia University; New York City, NY. March 2011.
Served as the meeting chair.
INVITED TALKS: "GAN Gene Therapy: Overview" and "GAN Preclinical Studies"

Workshop on Translational Research Priorities for Infantile (CLN1) and Late Infantile (CLN2) Forms of Batten's Disease. Bethesda, MD. November 2010.
INVITED TALK "Strategies and practical considerations for AAV-mediated global CNS delivery"

Krabbe Translational Research Network Mtg. Chapel Hill, NC. October 2010.
INVITED TALK "Strategies and practical considerations for AAV-mediated global CNS delivery"

Belgium Society for Pediatric Neurology Semi-Annual Mtg. Ghent, Belgium. April 2010.
INVITED TALK "Global gene delivery to the central nervous system via adeno-associated virus (AAV) vectors: Where are we now?"

2nd Annual Symposium on Giant Axonal Neuropathy. Rockville, VA. December 2009.
INVITED TALK "AAV-mediated Gene Therapy for GAN, Current Project Status"

10th Annual International Rett Syndrome Foundation Symposium. Itasca, IL. June 2009
INVITED TALK "Global Delivery of Adeno-Associated Virus (AAV) to the CNS: Implications for Rett Syndrome Gene Therapy"

1st Annual Symposium on Giant Axonal Neuropathy. Boston, MA. August 2008
INVITED TALK "AAV as a Gene Therapy Vector for the Treatment of GAN"

9th Annual International Rett Syndrome Foundation Symposium. Itasca, IL. June 2008
INVITED TALK "Directed Evolution of Adeno-Associated Virus to Produce an Enhanced Gene Therapy Vector for the Treatment of Rett Syndrome"

TEACHING RECORD

University of Texas Southwestern Medical Center Dallas, TX
Lecturer, Human Biology of Disease II (Spring 2019)
Lecturer, Heritable Neurological Diseases of Mice and Man (Spring 2019)

University of North Carolina at Chapel Hill Chapel Hill, NC
Lecturer, Experimental physiology in human health and disease, PHYI 703 (Spring 2017)

Lecturer, Experimental physiology in human health and disease, PHYI 703 (Spring 2016)
Lecturer, Experimental physiology in human health and disease, PHYI 703 (Spring 2015)
Lecturer, Experimental physiology in human health and disease, CBPH 703 (Spring 2014)
Lecturer, Experimental physiology in human health and disease, PHYI 703 (Spring 2013)
Lecturer, Experimental physiology in human health and disease, PHYI 703 (Spring 2012)
Lecturer, Pathobiology of Cardiovascular Disease 667 (Spring 2008)

PREP Post-baccalaureate Training Program for Under-represented Students at UNC
Mentoring a PREP scholar, Ricardo Rivera-Soto, from July 2014 – June 2015
Mentored a PREP scholar, Keon Wimberly, from June 2013 – June 2014
Mentored a PREP scholar, Nadia Khan, from June 2012 – June 2013

CUBS, Carolina Undergraduate Bioethics Scholars. Chapel Hill, NC. February 2013
Invited Speaker, "Gene therapy: How "the cure" clashes with traditional pharmaceutical business models"

Society for Neuroscience Meeting. Washington, D.C. November 2011.
SHORT COURSE #1 INSTRUCTOR, *Gene Vector Design and Application to Treat Nervous System Disorders.* Title: "Breakthroughs in AAV Vector Design"

HHMI Undergraduate Research Program, Vanderbilt U. Nashville, TN
Mentor of undergraduate researchers (Fall 2002 – Summer 2006)
Mentored a total of 4 undergraduates over 3 summers as part of the Howard Hughes Undergraduate Research Program at Vanderbilt University, and mentored 2 additional undergraduates as long-term researchers in the lab (for 2 and 3 years). Mentoring duties included the development of a research project, instruction on laboratory techniques, and support throughout the project.

Vanderbilt University, Interdisciplinary Graduate Program Nashville, TN
Methodology Class Lecturer (Fall 2003, 2004, 2005)
One-hour interactive class on PCR given to 1st-year biomedical graduate students.

Vanderbilt University Department of Biological Sciences Nashville, TN
Graduate Grader, Biochemistry (Fall 2004)
Graded lecture exams for upper-level undergraduate biochemistry class.
Graduate Teaching Assistant, Genetics lab (Spring 2002)
Mid-level undergraduate genetics lab. Duties included giving introductory lectures, explaining and supervising laboratory benchwork, and grading lab reports.
Graduate Teaching Assistant, Biological Sciences lab (Spring 2001)
Introductory laboratory section for biology-related majors. Duties included explaining and supervising laboratory benchwork as well as grading lab reports, quizzes, and lecture exams.

Auburn University Auburn, AL
- Presented with the Dept. of Biological Sciences 2000 Undergraduate Teaching Award

Undergraduate Teaching Assistant, Recombinant DNA lab (Winter 2000)
Advanced undergraduate laboratory class on recombinant DNA technologies and applications. Duties included the preparation of laboratory experiments, explaining and supervising laboratory benchwork, and grading laboratory reports.
Undergraduate Teaching Assistant, General Biology lab (Spring 1999)
Introductory laboratory section for biology-related majors.
Undergraduate Teaching Assistant, Concepts of Biology lab (Winter 1999)
Introductory laboratory section for non-science majors.

Undergraduate Teaching Assistant, Plant Biology lab (Spring 1998 - Spring 2000)
Introductory laboratory section.

RESEARCH SUPPORT

ACTIVE

Project Grant **Gray, PI** 12/1/18-11/30/19
Cure AHC $102,223
ATP1A3 gene therapy
The goal of this investigation is to investigate the feasibility of doing gene therapy for AHC.

SRA **Gray, PI** 10/1/18-9/30/20
Neurogene
CLN5 Non-clinical study

SRPA **Gray, PI** 8/1/18-9/30/23
Neurogene
IND-enabling studies for Aspartylglucosaminuria (AGU) to support the initiation of an AAV9/AGA gene transfer clinical trial

Project Grant **Gray, PI** 1/1/18-12/31/19
United MSD Foundation $29,000/yr
Multiple Sulfatase Deficiency Gene Therapy Using AAV
The goal of this investigation is to investigate the feasibility of doing gene therapy for MSD.

Project Grant **Gray, PI** 9/1/17-8/31/19
Angelman Syndrome Foundation $100,000/yr
Angelman Syndrome Gene Therapy
The goal of this investigation is to investigate the feasibility of doing gene therapy for Angelman Syndrome.

Project Grant **Gray (PI, 1%)** 5/1/17-4/30/19
Mila's Miracle Foundation to Stop Batten $196,181/yr
CLN7 Gene Therapy
This award is to support the development and evaluation of an AAV vector to potentially treat CLN7.

Project Grant #018 **(Philpot and Gray, co-PI, 1%)** 12/1/16-11/30/19
Pitt-Hopkins Research Foundation $61,960
Gene therapy for Pitt-Hopkins Syndrome
The goal of this investigation is to investigate the feasibility of doing gene therapy for Pitt-Hopkins Syndrome.

R01 NS095867-01 **Gray (PI, 20%)** 9/30/16 – 7/31/21
NIH/NINDS $433,282/yr
Directed Evolution of Novel AAV Capsids for Global CNS Gene Delivery in Rodents and Primates
The goal of this project is to use AAV capsid DNA shuffling and directed evolution to derive novel AAV capsids amenable to global CNS gene transfer, via an intra-CSF route in mice and non-human primates.

R01 **Gray (UNC subcontract PI)** 8/01/16 – 7/31/21
U. Penn/NIH/NINDS $300,499 subcontract total
Combination Therapy, Biomarkers, and Imaging in Canine Krabbe Disease
The UNC subcontract will provide AAV vectors, support for biodistribution studies, advice, and help with data interpretation as a collaboration.

R01 NS087175 **Gray (PI, 25%)** 4/01/14 – 3/31/20
NIH/NINDS $218,750/yr
Giant Axonal Neuropathy Gene Therapy
The goal of this investigation is to optimize an intra-CSF gene therapy approach for the treatment of Giant Axonal Neuropathy, and to identify translatable outcome measures that respond to treatment. The goal of these studies is to enable the proper design of a Phase II clinical trial for GAN.

COMPLETED (past 5 years)

Sponsored Research Agreement **Gray (PI, 5%)** 10/1/16 – 9/30/19
Abeona Therapeutics $828,871 total
Completion of CLN1 Efficacy Studies and Novel CNS Capsid Evaluations

RSRT Consortium Grant **Gray (co-PI, 1 of 4, 5%)** 1/15/14 - 1/14/19
Rett Syndrome Research Trust $165,587/yr
A gene therapy consortium to develop and evaluate gene therapy approaches in Rett syndrome
The goal of this consortium is to explore the potential of MeCP2 gene transfer for the treatment of Rett syndrome, and to optimize the vector design for maximal efficacy with minimal adverse effects in a preclinical setting utilizing RTT mice.

Project Grant **Gray (UNC PI, 1%)** 12/15/16-12/14/18
University of Pennsylvania/International Advocate for Glycoprotein Storage Diseases $26,682/yr
Evaluation of adeno-associated virus gene therapy in the feline model of mucolipidosis II
The goal of the proposed project is to evaluate highly translatable gene therapy approaches in the naturally occurring feline model of mucolipidosis II (ML II).

R01 NS082289 **McCown (MPI) Gray (PI, 20%)** 9/15/13 – 9/14/18
NIH/NINDS $218,750/yr
Development of Intravenous AAV Vectors for Intractable Epilepsy
The role of S. Gray is to provide AAV vector expertise and lead the engineering of novel AAV vectors targeted for epilepsy via capsid DNA shuffling and directed evolution.

Project 19715 **Gray (PI, 5%)** 5/01/16 – 4/30/18
AFM/Telethon with Rare Trait Hope Fund $120,000/yr
Aspartylglucosaminuria gene therapy using AAV vectors to target the CNS
The goal of this investigation is to conduct preclinical studies on translatable gene therapy approaches for the treatment of AGU.

New Hope Research Project Grant **Gray (PI, 1%)** 7/01/11 – 12/31/17
New Hope Research Foundation $357,178 (milestone-driven)
AAV-mediated global gene therapy for the treatment of Tay-Sachs Disease
This study will generate reagents and preliminary data to test a gene replacement approach to treat mice with Tay-Sachs disease.

Project Grant **Gray (PI, 1%)** 1/1/17-12/31/17
Hereditary Neuropathy Foundation $29,185
CMT6 (C12orf65): Supportive Studies to Advance Gene Therapy
This award is to support the development and production of an AAV vector to potentially treat CMT6.

Project Grant **Gray (PI, 1%)** 12/1/16 – 11/30/17
Talia Duff Foundation $30,878
CMT4j: Supportive Studies to Advance Gene Therapy
This award is to support the development and production of an AAV vector to potentially treat CMT4j.

Hannah's Hope Fund Project Grant **Gray (PI, 1%)** 12/01/14-11/30/17
Hannah's Hope Fund $328,117 total
Giant Axonal Neuropathy: Supportive Studies to Advance Gene Therapy
Funding to support various projects related to our development of gene therapy approaches to treat Giant Axonal Neuropathy.

Project Grant **Gray (PI, 5%)** 10/01/15 – 9/30/17
The Legacy of Angels Foundation $114,218/yr
Comparison of intrathecal AAV9, AAVrh10, and AAV-Olig001 in combination with bone marrow transplant.
The goal of this investigation is to conduct preclinical proof-of-concept studies exploring translatable gene therapy approaches for the treatment of Krabbe disease.

Project Grant #09032015 **Gray (PI, 1%)** 10/1/15 – 9/30/17
Batten Disease Support and Research Association $93,000 total
INCL Gene Therapy Using AAV9 Vectors

The goal of this investigation is to conduct preclinical studies on translatable gene therapy approaches for the treatment of INCL.

Project Grant **Gray (PI, 5%)** 5/1/15 – 4/30/16
Taylor's Tale $59,682 total
INCL Gene Therapy Project Support
This grant provides supplemental funding to assess the long-term efficacy of gene therapy for INCL and initiate pilot studies with alternative vector approaches.

NC TraCS $50k Grant #550KR121511 **Gray (PI)** 3/01/16 – 2/28/17
NC TraCS $50,000
Development of translational outcome measures for aspartylglucosaminuria

Rare Trait Hope Fund Project Grant **Gray (PI, 2%)** 9/1/14-4/30/16
Rare Trait Hope Fund $174,949
AGU Gene Therapy

SBIR **Schwartz (PI); Gray (sub PI, 10%)** 10/01/15 – 9/30/16
NIH / Tissuevision, Inc $70,469
Distribution of AAV Serotypes by Whole Organ Ex Vivo Imaging.

IRSF HeART Award **Gray (PI, 5%)** 1/01/14 – 12/31/15
International Rett Syndrome Foundation $68,182/yr
BDNF Gene Transfer for the Treatment of RTT

Hunter's Hope Fund Project Grant **Gray (PI, 5%)** 1/01/14 – 12/31/15
Hunter's Hope Fund $50,000/yr
AAV-Mediated Gene Transfer for Krabbe Disease

Bee for Battens Project Grant **Gray (PI, 10%)** 5/01/13 – 4/30/15
Bee for Battens $150,000/yr
Global gene transfer for the treatment of Batten disease

KTRN Collaborative Project Grant **Escolar (MPI) Gray (PI, 5%)** 12/01/11 – 11/30/14
Legacy of Angels Foundation via Children's Hospital of UPMC $50,000/yr

Hannah's Hope Fund Project Grant **Gray (PI, varied%)** 10/01/08 – 9/30/14
Hannah's Hope Fund $1,377,461 total
Investigation of Gene Therapy Approaches for the Treatment of Giant Axonal Neuropathy

Undergraduate and Pre-doctoral Grants

Dissertation Enhancement Grant (2006)	• Vanderbilt University
Honorable Mention, Grad. Res. Fellowship (2002)	• National Science Foundation
Predoctoral NRSIA Training Grant (2001-2003)	• NIH: Virology, Nucl. Acids and Cancer
University Graduate Fellowship (2000-2004)	• Vanderbilt University
Undergraduate Research Fellowship (1999-2000)	• Auburn University
Truelove Research Award & Grant (1997 & 1998)	• Auburn University
Undergraduate Research Award Grant (1998)	• Weed Science Society of America
Future Life Science Scholar Fellowship (1996-98)	• Howard Hughes Medical Institute

PROFESSIONAL SERVICE

Ad hoc journal reviewer	Science Translational Medicine, Molecular Therapy, Gene Therapy, Human Molecular Genetics, Human Gene Therapy, Neurosurgery, Expert Review of Neurotherapeutics, Journal of Inherited Metabolic Disease, PLOSone, Nature Biotechnology, Frontiers in Molecular Neuroscience, Journal of Neuroscience Research, BMC Medical Genetics, Acta Neurobiologiae Experimentalis, Neuron, Brain Sciences
Ad hoc grant reviewer	CDMRP/PRMRP; Vaincre Les Maladies Lysosomales; The Research Foundation – Flanders (FWO); JPND Research; ELA

		Foundation; BDSRA; Action Medical Research; Motor Neuron Disease Association; CMT Association
Committee member	2014-2020	Neurologic & Ophthalmic Gene & Cell Therapy, American Society for Cell and Gene Therapy
Associate Editor	2014-2017	NEUROSURGERY (neuroscience section)
Scientific Advisory Board	2019- present	Vertex Therapeutics
Scientific Advisory Board	2019- present	Opsin Therapeutics
Scientific Advisory Board	2019- present	Lysogene
Scientific Advisory Board	2018- present	CMT Association STAR Advisory Board
Scientific Advisory Board	2017- present	CDKL5 Research Collaborative
Scientific Advisory Board	2017- present	FOXG1 Foundation Scientific Advisory Board
Scientific Advisory Board	2016- present	Cure SPG47
Scientific Advisory Board	2016- present	Foundation to Fight H-ABC
Scientific Advisory Board	2016- present	Galyatech, LLC
Scientific Advisory Board	2015- present	Hunter's Hope Foundation
Scientific Advisory Board	2014- present	Hereditary Neuropathy Foundation
Scientific Review Board	2011- present	International Rett Syndrome Foundation
Investigator	2011- 2017	UNC IDRRC (Intellectual and Develop. Disabilities Res. Centers)
President	1999-2000	Beta Beta Beta, National Biological Hon. Soc. (Auburn chapter)
President	1999-2000	Phi Lambda Upsilon, Natl. Chemistry Hon. Soc. (Auburn chapter)

REFLECTIVE STATEMENT

My core expertise is in AAV gene therapy vector engineering, followed by optimizing approaches to deliver a gene to the central and peripheral nervous system. A major focus of my AAV vector development is to create vectors tailored to serve specific clinical and research applications involving the nervous system. These include the development of novel AAV capsids amenable to widespread CNS gene transfer after intra-CSF administration. These also include development of vectors targeted to specific cell types such as neurons, oligodendrocytes, and astrocytes.

As reagents have been developed to achieve global, efficient, and in some cases cell-type specific CNS gene delivery, my research focus has also included preclinical studies to apply these reagents toward the development of treatments for neurological diseases. Currently these primarily include Rett Syndrome, Giant Axonal Neuropathy, Tay-Sachs, Krabbe, Aspartylglucosaminuria, Multiple Sulfatase Deficiency, Charcot-Marie-Tooth disease type 4J, and Batten Disease. My future directions include 1) continued development and optimization of AAV vectors specifically tailored toward CNS and PNS disorders, 2) testing novel gene therapy approaches for applicable diseases that could benefit from these vectors, and 3) facilitating the translation of these approaches from bench to clinic.

I have successfully partnered with several foundations and secured federal and industry funding to support my goals. I have independently developed a gene therapy approach to treat Giant Axonal Neuropathy, which has culminated in a Phase I clinical trial at the NIH Clinical Center (https://clinicaltrials.gov/ct2/show/NCT02362438) that began in 2015. I am actively pursuing the start of additional first-in-human gene therapy clinical studies using the same general approach for multiple other inherited neurological disorders, as well as training a new cadre of scientists to conduct similar work.