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

Professor	Dept. of Pediatrics, U. Texas Southwestern Dept. of Molecular Biology, UTSW Dept. of Neurology, UTSW McDermott Center for Human Growth and Dev., UTSW	08/2024-present
Director	UTSW Gene Therapy Program	04/2024-present
Associate Professor	Dept. of Pediatrics, U. Texas Southwestern Dept. of Molecular Biology, UTSW Dept. of Neurology, UTSW Center for Regenerative Science and Medicine, UTSW McDermott Center for Human Growth and Dev., UTSW	12/2017-08/2024
Director	UTSW Translational Gene Therapy Core	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

2022	Thomas Dierks Award for remarkable contributions to Multiple Sulfatase Deficiency research
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 #9,636,370 (issued 05/02/2017). *AAV vectors targeted to oligodendrocytes*
2. United States patent #10,532,110 (issued 01/14/2020). *AAV Vectors Targeted to the Central Nervous System*
3. United States patent # 11,504,435 (issued 11/22/2022). *Optimized CLN1 genes and expression cassettes and their use*
4. United States patent #11,491,241 (issued 11/08/2022). *Optimized AGA genes and expression cassettes and their use*

5. International Patent application PCT/US19/045911. *Optimized CLN7 genes and expression cassettes and their use*
6. International Patent application PCT/US19/039458. *Optimized CLN5 genes and expression cassettes and their use*
7. International patent application PCT/US19/048776. *Feedback enabled synthetic genes, target seed match cassettes, and their uses*
8. International patent application PCT/US19/59752. *Optimized FIG4 genes and expression cassettes and their use*
9. International patent application PCT/US19/67727. *Optimized GALC genes and expression cassettes and their use*
10. International patent application PCT/US20/30236. *Optimized SUMF1 genes and expression cassettes and their use*
11. International Patent Application No. PCT/US20/030427. *Intrathecal and Intravenous combination gene therapy for the treatment of infantile batten disease*
12. International Patent Application No. PCT/US2020/059570. *Recombinant adeno-associated viral vector for gene delivery.* [specific to the SURF1 gene]
13. United States Provisional Patent Application No. 62/851,411. *UBE3A genes and expression cassettes and their use*
14. United States patent #12,077,772 (issued 9/3/2024). *Transgene Cassettes, AAV Vectors and AAV Viral Vectors for the Expression of Human Codon-Optimized Slc6a1*.
15. United States patent #11,753,655 (issued 9/12/2023). *Compositions and methods for treatment of neurological disorders*.
16. International Patent Application No. PCT/US2020/063300. *Transgene cassettes designed to express a human MeCP2 gene*.
17. International Patent Application No. PCT/US2022/014926. *Gene therapy for Angelman syndrome*.
18. International Patent Application No. PCT/US2022/025749. *Optimized AP4M1 polynucleotides and expression cassettes and their use*.
19. United States Provisional Patent Application No. 63/159,697. *Transgene cassettes designed to express the human codon-optimized gene EPM2A or NHLRC1*.
20. International Patent Application No. PCT/US2022/080625. *Vector genome design to express optimized CLN7 transgene*.
21. International Patent Application No. PCT/US2022/080805. *GNAO1 Gene therapy vectors and uses thereof*.
22. International Patent Application No. PCT/US2023/061459. *Transgene cassette designed to express the human codon-optimized gene FMR1*.

BIBLIOGRAPHY

1. Dowling JJ, Pirovolakis T, Devakandan K, Stosic A, Pidsadny M, Nigro E, Sahin M, Ebrahimi-Fakhari D, Messahel S, Varadarajan G, Greenberg BM, Chen X, Minassian BA, Cohn R, Bonnemann CG, **Gray SJ.** (2024) AAV gene therapy for hereditary spastic paraplegia type 50: a phase 1 trial in a single patient. *Nat Med.* Online ahead of print. PMID: 38942994
2. Bharucha-Goebel DX, Todd JJ, Saade D, Norato G, Jain M, Lehky T, Bailey RM, Chichester JA, Calcedo R, Armao D, Foley AR, Mohassel P, Tesfaye E, Carlin BP, Seremula B, Waite M, Zein WM, Huryn L, Crawford TO, Sumner CJ, Hoke A, Heiss JD, Charnas L, Hooper JE, Bouldin TW, Kang EM, Rybin D, **Gray SJ**, Bönnemann CG. (2024) Intrathecal gene therapy for giant axonal neuropathy. *NEJM,* 390(12): 1092-1104, PMID: 38507752.
3. Ling Q, Boitnott A, Garza I, Casy W, Shaffo FC, Sinnett SE, and **Gray SJ**. Adeno-associated virus-mediated gene therapy in central nervous system genetic disorders. (2024) In R.N. Rosenberg and J.M. Pascual (Eds), *Rosenberg's Molecular and Genetic Basis of Neurological and Psychiatric Disease, 7th edition*. *Accepted.*

4. Goodspeed K, Armstrong D, Boitnott A, Dolce A, Ling Q, and **Gray SJ**. (2024) Gene therapy for epilepsy. In J.F Noebels, M. Avoli, M.A. Rogawski, A Vezzani, and A.V. Delgado-Escueta (Eds), *Jasper's basic mechanisms of the epilepsies, fifth edition*. Oxford university press, *Publication pending.*
5. Sadhu C, Lyons C, Oh J, Jagadeeswaran I, **Gray SJ**, Sinnett SE (2023) The Efficacy of a Human-Ready miniMECP2 Gene Therapy in a Pre-Clinical Model of Rett Syndrome. *Genes*. 15(1):31. PMID: 38254921.
6. Ryckman AE, Deschenes NM, Quinville BM, Osmon KJL, Mitchell M, Chen Z, **Gray SJ**, Walia JS (2023) Intrathecal Delivery of a Bicistronic Adeno-associated Vector Serotype 9 Expressing Hexosaminidase Corrects Sandhoff Disease in a Murine Model in a Dose Responsive Manner. *Mol Ther Meth Clin Dev, accepted.*
7. Gumusgoz E, Kasiri S, Verma M, Wu J, Acha DV, Marriam U, Fyffe-Maricich S, Lin A, Chen X, **Gray SJ**, and Minassian BA (2023) CSTB gene replacement improves neuroinflammation, neurodegeneration and ataxia in murine Type 1 Progressive Myoclonus Epilepsy. *Gene Therapy, accepted*. PMID: 38135787.
8. Murray SJ, Wellby MP, Barrell GK, Russell KN, Deane AR, Wynyard JR, **Gray SJ**, Palmer DN, Mitchell NL. (2023) Efficacy of dual intracerebroventricular and intravitreal CLN5 gene therapy in sheep prompts the first clinical trial to treat CLN5 Batten disease. *Frontiers in Pharmacology, accepted*.
9. Vyas M, Deschenes NM, Osmon KJL, Chen Z, Ahmad I, Kot S, Thompson P, Richmond C, **Gray SJ**, Walia JS (2023) Efficacy of Adeno-Associated Virus Serotype 9-Mediated Gene Therapy for AB-Variant GM2 Gangliosidosis. *Int J of Mol Sci, accepted*.
10. Casy W, Garza IT, Chen X, Dong T, Hu Y, Kanchwala M, Trygg CB, Shyng C, Xing C, Bunnell BA, Braun SE, **Gray SJ*** (2023) SMRT Sequencing Enables High-Throughput Identification of Novel AAVs from Capsid Shuffling and Directed Evolution. *Genes.* 14(8):1660. PMID: 37628711. [*corresponding author]
11. Mitchell NL, Murray SJ, Wellby MP, Barrell GK, Russell KN, Deane AR, Wynyard JR, Palmer MJ, Pulickan A, Prendergast PM, Casy W, **Gray SJ**, Palmer DN. (2023) Long-term safety and dose escalation of intracerebroventricular CLN5 gene therapy in sheep supports clinical translation for CLN5 Batten disease. *Front Genet.* 14:1212228. PMID: 37614821.
12. Deschenes NM, Cheng C, Ryckman AE, Quinville BM, Khanal P, Mitchell M, Chen Z, Sangrar W, **Gray SJ**, and Walia JS. (2023) Biochemical Correction of GM2 Ganglioside Accumulation in AB-Variant GM2 Gangliosidosis. *Int. J. Mol. Sci.,* 24(11): 9217.
13. Wong H, Hooper AWM, Kang HR, Lee SJ, Zhao J, Sadhu C, Rawat S, **Gray SJ**, and Hampson DR. (2023) Gene Therapy using a CNS Dominant Human FMRP Isoform Rescues Seizures, Fear Memory, and Sleep Abnormalities in Fmr1 KO Mice. *JCI Insights*, 8(11):e169650. PMID: 37288657.
14. Ling Q, Herstine J, Bradbury A, and **Gray SJ***. (2023) Gene therapy for neurological disorders. *Nature Reviews Drug Discovery, accepted*. PMID: 37658167. [*corresponding author]
15. Chen X, Dong T, Hu Y, De Pace R, Mattera R, Eberhardt K, Ziegler M, Pirovolakis T, Sahin M, Bonifacino JS, Ebrahimi-Fakhari D, **Gray SJ***. (2023) Intrathecal AAV9/AP4M1 gene therapy for hereditary spastic paraplegia 50 shows safety and efficacy in preclinical studies. *J Clin Invest,* 133(10):e164575. PMID: 36951961. [*corresponding author]
16. Chen X, Lim DA, Lawlor MW, Dimmock D, Vite C, Lester T, Tavakkoli F, Sadhu C, Prasad S, **Gray SJ*.** (2023) Biodistribution of adeno-associated virus gene therapy following CSF-directed administration. *Hum Gene Ther.* 34(3-4):94-111. PMID: 36606687. [*corresponding author]
17. Rowe AA, Chen X, Nettesheim ER, Issioui Y, Dong T, Hu Y, Messahel S, Kayani SN, **Gray SJ**, Wert KJ. (2022) Long-term progression of retinal degeneration in a preclinical model of CLN7 Batten disease as a baseline for testing clinical therapeutics. *EBioMedicine*. 85:104314. PMID: 36374771.
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20. Osmon KJ, Thompson P, Woodley E, Karumuthil-Melethil S, Heindel C, Keimel JG, Kaemmerer WF, **Gray SJ**, Walia JS. (2022) Treatment of GM2 Gangliosidosis in Adult Sandhoff Mice using an Intravenous Self-Complementary Hexosaminidase Vector. *Curr Gene Ther*. 22(3):6751. PMID: 34530708
21. Ling Q, Rioux M, Hu Y, Lee M, **Gray SJ***. (2021) Adeno-associated viral vector serotype 9-based gene replacement therapy for SURF1-related Leigh syndrome. *Mol Ther Methods Clin Dev*. 23:158-168. PMID: 34703839. [*corresponding author]
22. Judson MC, Shyng C, Simon JM, Davis CR, Punt AM, Salmon MT, Miller NW, Ritola KD, Elgersma Y, Amaral DG, **Gray SJ**, Philpot BD. (2021) Dual-isoform hUBE3A gene transfer improves behavioral and seizure outcomes in Angelman syndrome model mice. *JCI Insight*. 6(20):e144712. PMID: 34676830
23. Kot S, Karumuthil-Melethil S, Woodley E, Zaric V, Thompson P, Chen Z, Lykken E, Keimel JG, Kaemmerer WF, **Gray SJ***, Walia JS. (2021) Investigating Immune Responses to the scAAV9-HEXM Gene Therapy Treatment in Tay–Sachs Disease and Sandhoff Disease Mouse Models. *Int. J. Mol. Sci.* 22:6751. [*co-corresponding author]
24. Bharucha-Goebel DX, Norato G, Saade D, Paredes E, Biancavilla V, Donkervoort S, Kaur R, Lehky T, Fink M, Armao D, **Gray SJ**, Waite M, Debs S, Averion G, Hu Y, Zein WM, Foley AR, Jain M, Bönnemann CG. (2021) Giant axonal neuropathy: cross sectional analysis of a large natural history cohort. *Brain.* 144(10):3239-3250. PMID: 34114613
25. Banning A, Zakrzewicz A, Chen X, **Gray SJ**, and Tikkanen R. (2021) Knockout of the CMP–Sialic Acid Transporter SLC35A1 in Human Cell Lines Increases Transduction Efficiency of Adeno-Associ ated Virus 9: Implications for Gene Therapy Potency Assays. *Cells.* 10(5):1259. PMID: 34069698.
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28. Sinnett SE, Boyle E, Lyons C, **Gray SJ*.** (2021) Engineered microRNA-based regulatory element permits safe high-dose miniMECP2 gene therapy in Rett mice. *Brain.* 144(10):3005-3019. PMID: 33950254. [*corresponding author].
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38. **Gray SJ***. (2019) The evolution of adeno-associated virus capsids for CNS gene therapy. *Cell & Gene Therapy Insights*. 5(11), 1359–1366. [*corresponding author].
39. 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. [*corresponding author].
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motor neurons derived from giant axonal neuropathy iPSCs rescued by restoration of gigaxonin. *Human Mol Genet, in press*. PMID: 25398950.
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PROFESSIONAL PRESENTATIONS (SELECTED)

N of 1 Collaborative Seminar. Virtual. June 2024.
INVITED SEMINAR: "Rapid development of an n-of-few gene therapy for SPG50 through dauntless collaboration"

2024 Global Rare Disease Research Symposium. Shanghai, China. May 2024.
INVITED TALK and PANELIST: "Expanding gene therapy for neurological disorders"

Gordon Conference on Fragile X and Autism-Related Disorders. Tuscany, Italy. May 2024.
INVITED TALK: "Gene Therapy for Fragile X Syndrome and Other Neurodevelopmental Disorders"

ASENT 2024 Global Neurotherapeutics Conference. Bethesda, MD. March 2024.
INVITED TALK and PANELIST: "Using gene therapy platforms to enable "grass roots" rare disease treatments"

American Epilepsy Society 2023 Annual Meeting. Orlando, FL. December 2023.
INVITED TALK: "Engineered feedback regulation of a dose-sensitive gene: Rett Syndrome example"

SLC6A1 Connect 2023 Annual Symposium. Orlando, FL. December 2023.

INVITED TALK: "Gene therapy for SLC6A1-related disorders"

Global Forum on Future Medicine. Seoul, South Korea, October 2023
INVITED TALK: "Expanding AAV-mediated gene therapy for rare neurological disorders"

University of North Carolina at Chapel Hill. Chapel Hill, NC. July 2023.
INVITED TALK "Expanding AAV-mediated gene therapy for rare neurological disorders"

Gordon Conference on ALS and Related Motor Neuron Diseases. Les Diablerets, Switzerland, July 2023
INVITED TALK: "Standardized Translational Approach to Target Motor and Sensory Neurons Via Intrathecal Administration of AAV9"

Illumina Grand Rounds. Virtual, Apr 2023.
INVITED TALK: "Gene therapy for neurological disorders"

FDA CBER Office of Therapeutic Products (OTP) Advanced Manufacturing and Analytical Technologies (AMAT) for Regenerative Medicine Therapies (RMT) Workshop. Virtual, Mar 2023.
INVITED TALK AND PANELIST: "Overcoming AAV CMC challenges through the use of platform-based approaches to streamline AAV-based drug development"

45th Annual Carrell-Krusen Neuromuscular Symposium. Dallas, TX. Feb 2023.
SPECIAL LECTURE: "Challenges and successes for AAV-mediated gene therapy for neuromuscular disorders"

American Society of Cell and Gene Therapy Insight Series - Immunological Responses to AAV Gene Therapy in Neurological Compartments: Mechanisms and Management. Virtual, November 2022.
INVITED TALK AND PANELIST: "Possible mechanisms (and mitigation strategies) for AAV-mediated DRG toxicity"

Federation of European Biochemical Societies 360 Lysosome Course. Izmir, Turkey. October 2022.
INVITED PLENARY TALK: "AAV-mediated gene therapy for lysosome disorders"

Neurology Grand Rounds. UT Southwestern Medical Center, Dallas, TX. September 2022.
INVITED TALK: "AAV9/AP4M1 gene therapy for spastic paraplegia type 50" (Steven Gray) and "Journey to cure Michael" (Terry Pirovolakis)

American College of Medical Genetics and Genomics Summer Gene Therapy Education Series: Advancement in gene therapy options for rare diseases. Virtual, July 2022.
INVITED TALK: "Gene therapy targets: broadening the scope of therapy"

Garrod Symposium. Calgary, Canada. May 2022.
KEYNOTE ADDRESS: "Approaches to gene therapy for inborn errors of metabolism"

Neurology Grand Rounds. UT Southwestern Medical Center, Dallas, TX. March 2022.
INVITED TALK: "AAV9-mediated gene transfer as a platform approach for treating neurological disorders"

14th International Congress of Inborn Errors of Metabolism 2021. Sydney, Australia. Nov 2021.
KEYNOTE: "CNS Targeted Gene Therapy: Update on Gene Therapy for Lysosomal Diseases"

Lurie Children's Hospital Neuroscience of Disease Series. Chicago (Virtual), Sept 2021.
INVITED TALK: "AAV9-mediated gene transfer as a platform approach for treating neurological disorders."

Société française pour l'étude des erreurs innées du métabolisme (CETL & SFEIM). France (Virtual), June 2021.
KEYNOTE: "Update on gene therapy for lysosomal diseases"

NIH/NINDS Clinical Neuroscience Grand Rounds. Bethesda, MD (Virtual), May 2021.
INVITED TALK: "AAV9-mediated gene transfer as a platform approach for treating neurological disorders."

International Child Neurology Teaching Network – All India Institute of Medical Science Neurogenomics Webinar Series. Virtual, April 2021.
INVITED TALK: "AAV-mediated gene therapy for neurological disorders."

Seaver Seminar Series – Icahn School of Medicine at Mt. Sinai. Mt. Sinai, NY (Virtual), Mar 2021.
INVITED TALK: "AAV-mediated gene therapy for neurological disorders."

2nd Annual Gene Therapy for Neurological Disorders. Virtual. Dec 2020.

INVITED TALK AND WORKSHOP LEADER: "Workshop B: Reducing the translational gap in neurology."

10th Annual Sanford Virtual Rare Disease Symposium. Sioux Falls, ND (Virtual). Oct 2020.
INVITED TALK: "On the brink of a treatment revolution for inherited nervous system disorders.

American Society of Gene and Cell Therapy Virtual. May 2020.
INVITED TALK: "Challenges to starting an academic AAV GMP facility."

Harvard and Boston Children's Hospital Adult and Child Neurology Grand Rounds. Boston, MA. Mar 2020.
INVITED TALK: "AAV-mediated gene therapy for neurological diseases"

Workshop on Expanding AAV Manufacturing Capacity for Rare Disease Gene Therapies. NIH, Bethesda, MD. Jan 2020.
CO-ORGANIZER, INVITED SPEAKER, AND PANELIST: "AAV manufacture via triple transfection in HEK293 cells"

2019 SLC6A1 Connect Symposium. Baltimore, MD. Dec 2019.
INVITED TALK and SESSION CHAIR: "Gene therapy approach for SLC6A1"

2019 Belgian Society of Pediatric Neurology Symposium. Gent, Belgium. Nov 2019.
INVITED TALK: "Intrathecal AAV9 as a platform approach to treat multiple CNS disorders"

2019 Roche Genomic Medicine Seminar Series. Basel, Switzerland. Nov 2019.
INVITED SPEAKER: "On the brink of a treatment revolution for inherited pediatric neurological disorders"

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 Society for the Study of Inborn Errors of Metabolism (SSIEM) International Congress. Rotterdam, Netherlands. Sept 2019.
INVITED TALK: "Evolving gene therapy landscape targeting the CNS"

2019 ISMRD Symposium on Glycoprotein Storage Disorders. 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

Mentored PhD candidate, Andrea Boitnott (2021-current)
Mentored PhD candidate, Irvin Garza (2023-current)
Mentored PhD candidate, Rachel Adams (2024-current)

Mentored postdoctoral trainee, Frances Shaffo (2018-2020) [currently at Roche]
Mentored postdoctoral trainee, Qinglan Ling (2018-2024) [Asst. Professor, U.Mass]
Mentored postdoctoral trainee, Widler Casy (2018-2020) [currently at Aavanti Therapeutics]
Mentored postdoctoral trainee, Mehmet Takar (2020-2022) [Currently at Sanofi]
Mentored postdoctoral trainee, Sumana Venkat (2022-2024)
Mentored postdoctoral trainee, Siyuna Hao (2024-present)

Dissertation Committee member

- Dami Alao (UTSW)

STARS High school summer interns

- Emily Caplan (2019)
- Samatha Belatur (2022)
- Satvik Paduri (2023)
- Siri Gangireddy (2024)

Summer Undergraduate Research Fellows (SURF)

- Harrison Higgs (2023)

Lecturer, Developmental Principles in Regenerative Science and Medicine (Spring 2024)
Lecturer, Frontiers in Medicine class (Spring 2024)
Lecturer, GDD Gene Expression class (Fall 2023)
Lecturer, Responsible Conduct of Research (Fall 2023)
Lecturer, Frontiers in Medicine class (Spring 2023)
Lecturer, Neurotechniques class (Spring 2023)
Lecturer, Heritable Neurological Diseases of Mice and Man (Spring 2023)
Group discussion facilitator, Responsible Conduct of Research (Fall 2022)
Lecturer, Heritable Neurological Diseases of Mice and Man (Spring 2021)
Lecturer, Human Biology of Disease II (Spring 2019)
Lecturer, Heritable Neurological Diseases of Mice and Man (Spring 2019)

University of North Texas Denton, TX
Guest lecturer, Advanced Molecular Biology course (Fall 2023)

Universidad Autonoma de Guerrero. Guerrero, Mexico
Guest lecturer (2 classes), "Gene Therapy: Principles and Applications" course (Fall 2022)

Carroll Senior High School. Southlake, TX
Guest lecturer, biotechnology class (Fall 2022)
Guest lecturer, biotechnology class (Fall 2021)

University of North Carolina at Chapel Hill Chapel Hill, NC

Mentored postdoctoral trainee, Rachel Bailey [currently Asst Prof, UT Southwestern]
Mentored postdoctoral trainee, Slawomir Sloniowski
Mentored postdoctoral trainee, Alejandra Rozenberg
Mentored postdoctoral trainee, Sarah Sinnett [currently Asst Prof, UT Southwestern]
Mentored postdoctoral trainee, Subha Karumuthil Melethil [currently at RegenX Bio]
Mentored postdoctoral trainee, Erik Lykken [currently at Dyno Therapeutics]
Mentored postdoctoral trainee, Sara Powell [currently Asst Prof, UNC Chapel Hill]
Mentored postdoctoral trainee, Barbara Detweiler

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

PENDING

ACTIVE

Sponsored Research Agreement **Gray, PI** 6/01/2024-5/31/2025
Form Bio $25,627
SURF1 Vector Designs
The goal of this project is to evaluate alternative designs of a SURF1 AAV vector.

Sponsored Research Agreement **Gray, PI** 3/01/2024-2/28/2025
Form Bio $65,554
SURF1 Vector Designs
The goal of this project is to evaluate alternative designs of a SURF1 AAV vector.

Sponsored Research Agreement **Gray, PI** 11/01/2023-10/31/2025
Elpida Therapeutics $538,525
IND-enabling support studies for CMT4J
The goal of this project is to conduct and support activities leading to the submission of an IND application to test AAV9/FIG4 as a treatment for CMT4J.

Sponsored Research Agreement **Gray, PI** 11/01/2023-10/31/2024
Cure Mito Foundation $96,273
Gene Therapy for SURF1-related Leigh Syndrome
The goal is to investigate SURF1 gene transfer as a treatment for SURF1-related Leigh Syndrome.

Grant Project **Gray, PI** 11/01/2023-10/31/2024
RTW Foundation $136,545
Gene Therapy for SURF1-related Leigh Syndrome
The goal is to investigate SURF1 gene transfer as a treatment for SURF1-related Leigh Syndrome.

R01GM149949 **Kim, lead PI; Gray, subcontract PI** 09/25/2023 – 08/31/2027
NIH $296,172
Multimodal Label-Free Nanosensor for Single Virus Characterization and Content Analysis
The overall goal of the project is to develop new analytical methods to characterize AAV particles.

1U01NS129339 (UrGENT) **Gray, PI** 09/01/2023 – 08/31/2025
NIH/NINDS $2,202,371
IND-enabling studies for Aspartylglucosaminuria (AGU) to support the initiation of an AAV9/AGA gene transfer clinical trial
The goal of this project is to conduct activities leading to an approved IND for a Phase I/II gene therapy trial for AGU. Upon completion this will transition to a noncompetitive review of a funded clinical trial.

U19NS132304 **Gray, site PI** 05/15/2023 – 05/14/2028
NIH (subcontract from Jackson Laboratories) $7,047,180
Preclinical Genome Editing for Rare Neurological Diseases

In this multi-institutional consortium grant, UTSW leads the Regulatory Core. The responsibilities of this core are to facilitate all necessarily activities to move a lead candidate from the consortium projects to an Investigational New Drug submission.

Project Grant **Chen, PI; Gray, co-PI** 04/01/2023 – 03/31/2025
Hope for PDCD Foundation $344,769
Gene Therapy for PDCD
The goal is to investigate PDHA1 gene transfer as a treatment for PDCD.

Project Grant **Gray, PI; Chen, co-PI** 05/01/2023 – 04/30/2025
Ogman Foundation $403,076
Gene Therapy for TECPR2
The goal is to investigate TECPR2 gene transfer as a treatment for SPG49.

Project Grant **Chen, PI; Gray, co-PI** 04/01/2023 – 03/31/2025
Rare Olive Foundation $399,093
Gene Therapy for LNPK
The goal is to investigate LNPK gene transfer as a treatment for NEDEHCC.

Sponsored Research Agreement **Gray, PI** 12/01/2022-11/30/2024
Hannah's Hope Fund $574,201
GAN Vagus Nerve Translational Studies
The goal of this project is to conduct and support activities leading to the submission of an IND application to test vagus nerve injection of AAV9/GAN to treat autonomic dysfunction in Giant Axonal Neuropathy.

Sponsored Research Agreement **Chen, PI; Gray, co-PI** 12/01/2022-4/30/2025
DDX3X Foundation $355,343
DDX3X Gene Therapy
The goal is to investigate DDX3X gene transfer as a treatment for DDX3X deficiencies.

Sponsored Research Agreement **Gray, PI** 12/01/2022-11/30/2024
Phoenix Nest $169,447
MPS IIIC gene therapy: preclinical in vivo assessment of treatment efficacy
The goal is to an AAV/HGSNAT vector for efficacy in an MPS3C mouse model.

Sponsored Research Agreement **Gray, PI** 04/01/2022-3/31/2024
MOAD Foundation $380,993
MOA-A/B Gene Therapy
The goal is to investigate MAO-A or MAO-B gene transfer as a treatment for MAO deficiencies.

Sponsored Research Agreement **Gray, PI** 04/01/2020 – 09/30/2024
Taysha Gene Therapies $2,098,332
SLC6A1 Gene Therapy
The goal of this is to conclude preclinical studies to support the submission of an Investigational New Drug application to initiate a gene therapy clinical trial for SLC6A1.

1R01NS096087 **Gray (UTSW subcontract PI)** 12/01/2020 – 11/30/2025
U. Penn/NIH/NINDS $747,916 subcontract total
AAV-mediated gene therapy for CNS disease correction in feline NPC1 disease
The UTSW subcontract will provide AAV vectors, support for biodistribution studies, advice, and help with data interpretation as a collaboration.

<u>COMPLETED (past 5 years)</u>

Sponsored Research Agreement **Gray, PI** 12/01/2022-11/31/2023
Form Bio $162,461
Genome Stability and Optimization of AAV Vector Genomes
The goal of this project is to identify parameters that enable optimal packaging of intact AAV vector genomes.

Sponsored Research Agreement **Gray, PI** 04/01/2020 – 09/30/2023
Taysha Gene Therapies $2,184,484
SURF1 Gene Therapy

The goal of this is to conclude preclinical studies to support the submission of an Investigational New Drug application to initiate a gene therapy clinical trial for SURF1 Leigh Syndrome.

Sponsored Research Agreement **Chen, PI; Gray, co-PI** 03/01/2021 – 02/28/2024
Taysha Gene Therapies $803,153

CMT4A Gene Therapy

The goal is to investigate GDAP1 gene transfer as a treatment for CMT4A.

Sponsored Research Agreement **Chen, PI; Gray, co-PI** 1/01/2021 – 12/31/2023
Taysha Gene Therapies $898,996

GNAO1 Gene Therapy

The goal is to investigate GNAO1 gene transfer as a treatment for GNAO1 deficiency syndrome.

Project Grant **Gray, PI** 02/01/2020 – 10/31/2023
Cure SPG50 Foundation $185,125

AP4M1 gene therapy

The goal is to investigate AP4M1 gene transfer as a treatment for SPG50.

R01 NS095867 **Gray, PI** 09/30/16 – 11/31/23
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.

Sponsored Research Agreement **Sinnet, PI; Gray, co-PI** 05/01/2020 – 05/31/2023
Taysha Gene Therapies

Preclinical assessment of a regulated miniMeCP2 vector

The goal of this is to assess the safety and efficacy of a regulated MeCP2 vector to treat Rett Syndrome.

Sponsored Research Agreement **Butler, PI; Gray, co-PI** 04/01/2020 – 03/31/2023
Taysha Gene Therapies

Gene Therapy for Angelman Syndrome

The goal of this is to assess the safety and efficacy of an AAV-mediated shRNA knock-down approach to treat Angelman Syndrome.

Project Grant **Gray, PI** 02/01/2020 – 07/31/2022
Sappani Foundation $359,913

SRD5A3 gene therapy

The goal is to investigate SDR5A3 gene transfer as a treatment for SRD5A3 congenital disorder of glycosylation.

Sponsored Research Agreement **Gray, PI** 09/01/2020 – 08/31/2022
Taysha Gene Therapies $618,446

FMR1 Gene replacement therapy for Fragile X syndrome

The goal of this is to design and produce an adeno-associated virus (AAV) vector carrying human FMR1 gene, which could potentially treat Fragile X syndrome.

Sponsored Research Agreement **Chen, PI; Gray, co-PI** 12/01/2020 – 11/30/2022
Taysha Gene Therapies $798,553

DDX3X Gene Therapy

The goal is to investigate DDX3X gene transfer as a treatment for DDX3X deficiency syndrome.

Sponsored Research Agreement **Gray, PI** 01/01/2022 – 12/31/2022
Taysha Gene Therapies $467,635

SHANK3 minigene evaluation for gene therapy

The goal of this is to investigate the safety and efficacy of 5 SHANK3 minigene designs for gene therapy.

Sponsored Research Agreement **Gray, PI** 01/01/2022 – 12/31/2022
Taysha Gene Therapies $952,020

Intra-CSF Route of Administration Comparison in Non-Human Primates

The goal of this is compared various routes of administration for optimal CNS gene transfer with AAV9.

Sponsored Research Agreement **Gray, PI** 10/01/2021 – 09/30/2022
Taysha Gene Therapies $94,671

CLN7 gene replacement therapy for Batten disease
The goal of this is to create a second-generation version of our published CLN7 vector, and assess the degree of comparability with the original version.
Sponsored Research Agreement **Gray, PI** 07/01/2020 – 6/30/2022
Taysha Gene Therapies $502,952
Vagus Nerve non-human primate proof of concept
The goal of this is to assess the transduction of AAV9/GFP following VN injection, with or without pre-immunization against AAV9.
Sponsored Research Agreement **Sinnet, PI; Gray, co-I** 06/01/2020 – 05/31/2022
Taysha Gene Therapies
Gene therapy for autism and tumor phenotypes related to PTEN Hamartoma Tumor Syndrome
The goal of this is to develop and investigate an AAV vector delivering the PTEN gene as a treatment for PTEN Hamartoma Tumor Syndrome.
Project Grant **Gray, PI** 11/01/19-01/30/22
Hannah's Hope Fund $239,289
GAN vagus nerve injection studies
The goal is to investigate approaches to target the autonomic nervous system in Giant Axonal Neuropathy.
Sponsored Research Agreement **Gray, PI** 11/01/2020 – 10/31/2021
Roche $160,603
Biodistribution analysis of AAV9 from archived Gray lab NHP samples
The goal is to retrospectively and comprehensively evaluate the biodistribution of AAV9 from past specimens collected by the Gray lab.
Project Grant **Gray, PI** 08/01/2020 – 07/31/2021
Drake Rayden Foundation $27,384
NKH (GLDC) Gene Therapy: First Steps
The goal of this project is to develop a GLDC gene transfer vector and conduct initial safety studies of that vector in mice.
7R01NS095867-03 **PI: Scherer, Su; UTSW PI: Gray** 09/01/2019 – 08/31/2021
UCLA/NINDS
Autoimmune Mechanisms in Peripheral Neuropathy
The goal of the subaward to UTSW is to advise the UCLA PI on the use of AAV vectors on the project.
R01 NS096087 **Gray (UNC/UTSW 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/UTSW subcontract will provide AAV vectors, support for biodistribution studies, advice, and help with data interpretation as a collaboration.
R01 NS087175 **Gray** 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.
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.
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**	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.

SRA	**Gray, PI**	10/1/18-12/31/19
Neurogene		

CLN5 Non-clinical study

SRPA	**Gray, PI**	8/1/18-12/31/19
Neurogene		

IND-enabling studies for Aspartylglucosaminuria (AGU) to support the initiation of an AAV9/AGA gene transfer clinical trial

Project Grant #018	**(Philpot and Gray, co-PI)**	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.

Sponsored Research Agreement	**Gray, PI**	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,)**	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.

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	Nature, Nature Biotechnology, Nature Medicine, Nature Communications, Science Translational Medicine, Molecular Therapy, Gene Therapy, Human Molecular Genetics, Human Gene Therapy, Neurosurgery, Expert Review of Neurotherapeutics, Journal of Inherited Metabolic Disease, PLOSone, Frontiers in Molecular Neuroscience, Journal of Neuroscience Research, BMC Medical Genetics, Acta Neurobiologiae Experimentalis, Neuron, Brain Sciences, Pediatric Neurology, Journal of Clinical Investigation (JCI), Brain
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, Medical Research Council (UK), AFM Telethon
Ad hoc NIH Study section		NIH GDD Study Section, NIH NSD Study Section; NIH BPN study section, NIH BRAIN U24 study section
Committee member	2014-2020	Neurologic & Ophthalmic Gene & Cell Therapy, American Society for Cell and Gene Therapy
Abstract review chair	2024	American Society for Gene and Cell Therapy, Neurologic Disease category
Associate Editor	2014-2017	NEUROSURGERY (neuroscience section)
Board of Directors	2023-present	Elpida Therapeutics
Scientific Advisory Board	2023- present	Form Bio
Scientific Advisory Board	2021- present	Nanoscope Therapeutics
Scientific Advisory Board	2021- 2023	Codexis
Scientific Advisory Board	2021- present	Forge Biologics
Chief Scientific Advisor	2020-present	Taysha Gene Therapies
Scientific Advisory Board	2019- present	Sarepta Therapeutics
Scientific Advisory Board	2019- 2021	Vertex Therapeutics
Scientific Advisory Board	2019- present	Opsin Therapeutics
Scientific Advisory Board	2019- 2021	Lysogene
Scientific Advisory Board	2018- present	CMT Association STAR Advisory Board
Scientific Advisory Board	2017- 2022	CDKL5 Research Collaborative
Scientific Advisory Board	2017- 2022	FOXG1 Foundation Scientific Advisory Board
Scientific Advisory Board	2016- 2022	Cure SPG47
Scientific Advisory Board	2016- 2022	Foundation to Fight H-ABC
Scientific Advisory Board	2016- 2022	Galyatech, LLC
Scientific Advisory Board	2015- 2022	Hunter's Hope Foundation
Scientific Advisory Board	2014-2020	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. 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. Phase I trials have also been initiated for CLN7 Batten disease, CLN5 Batten disease, CLN1 Batten disease, Spastic Paraplegia Type 50, Rett Syndrome, and GM2 gangliosidosis stemming from my laboratory's research. 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.