

CURRICULUM VITAE

Richard Jude Samulski, Ph.D.

PERSONAL

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EDUCATION

1982-1986	Post-Doctoral Research Laboratory of Dr. Thomas Shenk Princeton University
1978-1982	Ph.D., Molecular Biology Laboratory of Dr. Nicholas Muzyczka University of Florida, Gainesville, FL
1972-1976	B.S., Microbiology Clemson University, Clemson, SC

PROFESSIONAL EXPERIENCE

04/2024 - Present	Co-Founder, Chief Scientific Officer M34, Inc. 870 Martin Luther King Jr Blvd Chapel Hill, NC
09/2001- 03/2024	Founder, President & Former Chief Scientific Officer Asklepios BioPharmaceutical, Inc. (AskBio), A Wholly Owned Subsidiary of Bayer Pharmaceuticals Research Triangle Park, NC
11/2019-Present	Member, Gene Therapy for Rare Disease Consortium Foundation of National Institutes of Health Washington, DC
04/2018-Present	Founder & Trustee Columbus Children's Foundation Chapel Hill, NC
03/2018-Present	Professor Department of Pharmacology University of North Carolina Chapel Hill, NC

PROFESSIONAL EXPERIENCE (cont'd)

08/2016-02/2018	Vice President, Gene Therapy Bamboo Inc., A Wholly Owned Subsidiary of Pfizer, Inc Morrisville, NC
06/1999-08/2016	Professor Department of Pharmacology University of North Carolina Chapel Hill, NC
06/1993-06/2016	Director of Gene Therapy Center School of Medicine, University of North Carolina Chapel Hill, NC
06/1993-1999	Associate Professor Department of Pharmacology University of North Carolina Chapel Hill, NC
09/1991-06/1993	Associate Professor Department of Biological Sciences University of Pittsburgh, Pittsburgh, PA
09/1986-09/1991	Assistant Professor Department of Biological Sciences University of Pittsburgh, Pittsburgh, PA
1984-1986	Postdoctoral Research Fellow Laboratory of Dr. Thomas Shenk Princeton University, Princeton, NJ
1982-1984	Postdoctoral Research Fellow Laboratory of Dr. Thomas Shenk Microbiology Department, SUNY, Stony Brook

ACADEMIC AND PROFESSIONAL HONORS

1. Life Sciences Award Winner, 2024
2. ESGCT Founder's Award, 2022 (Inaugural award for the society)
3. Columbus Hero Award, Columbus Venture Partners, 2022
4. Nominated for National Academy of Science, 2022
5. BBVA Foundation, Frontiers of Knowledge Award nominee, 2022
6. North Carolina Science Award nominee, 2022
7. Top 20 Most Influential R&D Executives, Endpoints News, August 2021
8. Businessperson of the Year, Triangle Business Journal, 2020
9. Life Sciences Lifetime Achievement Award, Triangle Business Journal, July 2020
10. PharmaVoice 2020 - 100 Most Inspiring People in the Life Sciences Industry, May 2020
11. First non-MD to be placed on the University of Florida's "Wall of Fame", 2016

12. Audience with the Pope for Advancement of Gene Therapy for Genetic Diseases, 2015
13. 1000 Talents for China Award, 2014
14. SCBA Award in China, 2013

ACADEMIC AND PROFESSIONAL HONORS (cont'd)

15. Top 12 Pioneer Awards for Seminal Work in Gene and Cell Therapy, 2013
16. China Thousand Talent Plan Scholar, 2012
17. Honorary Professor, Peking Union Medical College, 2009
18. Honorary Professor, Institute of Hematology, Chinese Academy of Medical Sciences, 2009
19. Meeting with the Minister of Health, China, 2009
20. Senator Paul B. Wellstone Muscular Dystrophy Cooperative Research Center Award, October 2008
21. ASGCT Outstanding Achievement Award, 2008 (Inaugural award for the society)
22. Researcher/Innovator Healthcare Hero Award, Triangle Business Journal, NC, 2005
23. National Hemophilia Foundation Researcher of the Year, 1999
24. William C. Friday Fellowship for Human Relations Nominee, 1995
25. American Heart Association Established Investigator, 1991-1996
26. University of Pittsburgh, President's Distinguished Research Award, 1991
27. Outstanding Young Men of America Award, 1990
28. Medical Guild Graduate Student Research Award, Third place, 1982
29. South Florida Branch ASM Student Research Award, First place, 1981

PROFESSIONAL ACTIVITIES

1. Invited Key Note: Lund Spring Symposium, Lund, Sweden, May 2023
2. Invited European Society of Gene & Cell Therapy Educational Lecturer, Edinburgh, UK, 2022
3. Invited European Society of Gene & Cell Therapy AAV Workshop, San Sebastian, Spain 2022
4. El Pais Newspaper-Rare Diseases Should Be Treated For Free; If We Do, We Will Understand The Rest-Daniel Mediavilla, May 2022
5. Spanish Scientific Council, Madrid, Spain, 2022
6. Neurodegeneration Symposium, Fundacion Joaquin del Pino, Madrid, Spain, 2022
7. The National Institute for Innovation in Manufacturing Biopharmaceuticals (NIIMBL) Workshop: AAV Analytics, April 2022
8. Bayer Pharmaceuticals Breakthrough Innovation Forum, April 2022
9. Science News_Century of Science Series: How Gene Therapy Overcame High-profile Failures, March 2022
10. Carolina Alumni Review-From The Ashes, A Cure-Mark Derewicz, January 2022
11. Nature Reviews Drug Discovery-'Bespoke Gene Therapy Consortium' Sets Out To Enable Gene Therapies For Ultra-rare Diseases, November 2021
12. Gene Therapy and Genome Editing Summit, Session Chair, November 2021
13. Member, Gene Therapy for Rare Diseases Consortium Foundation, October 2021– present
14. NC Bio Annual Meeting_Keynote Speaker – October 2021
15. Gene Therapy 2.1 RESTORE Consortium, September 2021
16. GENEWIZ & Brooks Life Sciences Virtual Event, April 2021
17. BioPharma Dive_Gene Therapy Webinar, April 2021
18. Human Gene Therapy-AAV Jude: An Interview with R. Jude Samulski-Kevin Davies, January 18, 2021

19. BioPharma Dive-How 2 Scientific Pioneers Teamed Up to Run AskBio, Bayer's New Gene Therapy Division-Sarah de Crescenzo, January 21, 2021
20. 20th Gordon L. Snider Critical Issues Workshop-The Rationale for Gene Therapy, Gene Silencing and Gene Editing for Rare Diseases, December 12, 2020

PROFESSIONAL ACTIVITIES (cont'd)

21. Gene Therapy for Neurological Disorders U.S.-Lesson From the Clinic & Why AAV Biology Still Matters, December 10, 2020
22. Global Genes RAREcast-Danny Levine, November 2020
23. OpenGT, Advisor, November 2020 – present
24. News & Observer, Raleigh, NC Interview-Jude Samulski's 40-year Mission in Gene Therapy Leads to \$4B Buy-out - Zachery Eanes, October 27, 2020
25. Triangle Business Journal Interview-How Potential \$4 Billion Deal Lets AskBio Focus on the Science-Seth Gullledge, October 26, 2020
26. Endpoints News-Bayer Buys a Biotech "Race Horse" With a \$4B Deal Aimed at Going Big Into Gene Therapy-John Carroll, October 26, 2020
27. STAT News Interview-Bayer to Buy Gene Therapy Company AskBio For as Much as \$4 Billion-Matthew Herper, October 26, 2020
28. MDA Telethon, MDA Overtime Show, "Genetic Medicine", October 2020
29. Eli Lilly and Company Global Grand Rounds, "AAV Gene Therapy for Neurological Disorders, Personal Experiences", October 2020
30. Chardan 4th Annual Genetic Medicines Conference
31. FNIH Accelerating Medicines Partnership - Gene Therapy Program and Partnering Update Working Group, August 26, 2020
32. Blog Talk Radio, PharmaTalk Podcast, PharmaTalk_How a Gene Therapy for Duchenne Muscular Dystrophy Came to Be, August 25, 2020
33. Cystic Fibrosis Foundation, Gene Therapy On-line Seminar, August 24, 2020
34. ASGCT "Vectors 101" Educational Series featured speaker, August 12, 2020
35. The Conference Forum, Gene Therapy DMD Life Science Podcast, August 5, 2020
36. National Public Radio feature, "Researchers hope experimental gene therapy is an answer to a fatal genetic disorder: Researchers believe gene therapy is poised to change the lives of thousands with the fatal genetic disorder, Duchenne muscular dystrophy – thanks to over 40 years of scientist Jude Samulski's work.", All Things Considered, July 20, 2020
37. Awarded international patent application from World Intellectual Property Organization for regulated gene editing system, June 2020
38. Adjunct Professor, Department of Otolaryngology, University of Iowa, 2020 – present
39. Freeline Therapeutics, Expert Clinical Advisory Panel – Impact of Covid19 Pandemic on Gene Therapy Trials, June 2020
40. Member, Advisory Board, Freeline Therapeutics, 2020 – present
41. Accelerating Medicines Partnership in Gene Therapy – Manufacturing Testing and FDA Interactions, June 2020
42. Member, Scientific Advisory Board, Apic Bio, June 2020 – present
43. FNIH, Accelerating Medicines Partnership in Gene Therapy – Manufacturing/Analytics Working Group, June 2020
44. National Gene Vector Biorepository External Advisory Board – SARS-CoV-2 Spike-Pseudo-typed Lentiviral Vectors for Screening Neutralizing Antibodies and Antivirals, May 2020

45. Expert Guest, Gene Therapy Podcast Series sponsored by BioMarin, May 2020
46. Cell & Gene Therapy Insights, Interview: “A Return to Rational Capsid Design? Predicting the Future of AAV Vector R&D”, May 2020
47. Spectrum News In Focus – “Impact of Rare Disease Research in NC”, March 2020

PROFESSIONAL ACTIVITIES (cont’d)

48. FNIH, Bespoke Gene Therapy: Manufacturing & Analytics Group, Advisor, February 2020 – present
49. Workshop on Expanding AAV Manufacturing Capacity for Rare Disease Gene Therapies, FDA/NIH, January 2020
50. First patient treated in Phase I trial using AAV gene therapy for Congestive Heart Failure, AskBio, January 2020
51. Foundation for the National Institutes of Health, Individualized Gene Therapy Consortium, December 2019
52. PharmaExec-R. Jude Samulski: Gene Therapy’s Guiding Force-Elaine Quilici, November 9, 2019
53. I Symposium on Gene Therapy Manufacturing – The New Era in Manufacturing of Gene Therapy, “An AAV Retrospective”, San Sebastian, Spain, October 2019
54. The University of Iowa, Neurogenetics Symposium, October 2019
55. Chardan 3rd Annual Genetic Medicines Conference, “The Pioneers of Gene Therapy”, October 2019
56. North Carolina Biotechnology Center, Gene and Cell Therapy Industry Consortium, September 2019
57. North Carolina Biotechnology Center, Translational Research Grant – End User, “Adenovirus E4orf6 Variants to Enhance the Production of AAV Viral Vectors”, David Ornelles, PhD, 2019
58. Gene Therapy Fellowship Program - Mentor, “Directed Evolution of Chimeric AAVs for Modification of Targeted Neural Circuits in the Primate Brain”, Martin Bohlen, PhD, 2019
59. CIMA Universidad de Navarra, Gene Therapy Project – Testing New Promoters, 2019
60. Phoenix Nest, MPS IIIC Program, 2019
61. National Institutes of Health / National Institute of Neurological Disorders and Stroke (NINDS), Next Generation Strategies for Gene Targeted Therapies of Central Nervous System Disorders Workshop, September 2019
62. North Carolina Biotechnology Center, Germfree and CRB – GMP Manufacturing Capacity in Research Triangle Park, North Carolina, July 2019
63. North Carolina General Assembly, Genomic Medications Briefing, June 2019
64. Parent Project Muscular Dystrophy Annual Conference, “Overcoming Neutralizing Antibodies for Broader Inclusion”, June 2019
65. National Center for Advancing Translational Sciences, CNS Immunogenicity Considerations for AAV-mediated Gene Therapy, June 2019
66. NF2 BioSolutions Group, Accelerating Gene Therapy Research for Neurofibromatosis Type 2, March 2019
67. University of North Carolina, National Institute for Innovation in Manufacturing Biopharmaceuticals, March 2019
68. Alliance for Regenerative Medicine, Educational On-Camera Interview, “Gene Therapy: The History, The Promise and The Challenges”, March 2019

69. Cystic Fibrosis Foundation, Overcoming Obstacles for Delivery of Molecular Therapies Workshop, February 2019
70. Duke University Pediatric Grand Rounds, “Has DMD Gone Viral? AAV-based Treatment of Duchenne Muscular Dystrophy”, January 2019

PROFESSIONAL ACTIVITIES (cont’d)

71. First patient treated in Phase I/II trial using AAV gene therapy for Pompe disease, AskBio, January 2019
72. FDA/NIH Workshop, “The Growing Promise of Gene Therapy Approaches; Update on the Latest Developments in Viral and Non-Viral Vector Manufacturing; Quality and Manufacturing”, Session Chair, August 2018
73. Initiation of the AAV DMD Clinical Trial, February 2018
74. National War College, National Defense University, Genetic Medicine Teaching Assignment, 2017
75. Member, American Society of Hematology, 2016
76. North Carolina Rare Disease Advisory Council, 2015 – present
77. Guest Editor, Cell & Gene Therapy Insights, “Translation and Application of Gene Editing, Vector Characterization & Validation”, October 2015
78. Science Friday Radio, “Could Gene Therapy Be One Step Closer to the Clinic?”, October 9, 2015
79. National Eye Institute Support, XLR2 Data and Safety Monitoring Committee, 2015 –present
80. AFM/Genethon Advisory Board, 2014
81. Wellstone Center Advisory Board, 2013
82. Target 10 Therapeutics-Dr. Jude Samulski_You Tube, September 30, 2011
83. ASGCT President, 2011
84. ASGCT President-elect, 2010
85. Co-Director Chinese Institute of Gene Therapy, Tianjin, 2009
86. Gene Therapy China Initiative, established MOU with China Institute of Hematology and the UNC Gene Therapy Center, September 2009
87. Traveled with Governor’s Delegation to Beijing and Shanghai, 2009
88. ASGCT Vice President, 2009
89. The National Gene Vector Biorepository (NGVB) External Advisory Board, 2008 – present
90. Advisory Council, American Society for Gene Therapy, August 4, 2006 – 2016
91. FDA Advisory Board for Gene and Cell Therapy, 2005
92. ASGCT Strategic Planning Committee member, ASGCT Board of Directors, 2005
93. Scientific Advisory Committee, Muscular Dystrophy Cooperative Research Center Grant, University of Pittsburgh, June 17, 2005
94. Board of Directors, American Society of Gene Therapy, 2003 – 2016
95. Indiana University School of Medicine Site Visit, NIH/NHLBI PPG Review. “Gene Replacement Therapy in Hematopoietic Stem Cells”, February 19-20, 2003
96. Member – European Society of Gene Therapy Scientific Committee, 2003
97. NHF Research Working Group, NY, NY, July 2002 – 2008
98. NINDS Committee Site Visit, Camden, NJ, June 10, 2002
99. Alcohol Center Site Visit, NIAAA, Bethesda, MD, May 14, 2002
100. Biological Response Modifiers Advisory Meeting, Gaithersburg, MD. “AAV Vector

- Biology”, May 10, 2002
101. Member, Carolina Cardiovascular Biology Center, University of North Carolina School of Medicine, May 2002 – present
 102. Committee Member, National Institute of Neurological Disorders and Stroke (NINDS), May 2002 – present

PROFESSIONAL ACTIVITIES (cont’d)

103. Special Government Employee to the Center for Biologics Evaluation and Research (CBER) of the Food and Drug Administration (FDA), March 2002 – present
104. Evaluation Committee Member, JDRF, University of Florida and University of Miami, February 2002
105. Member, Medical Biochemistry Study Section, NIH, February 2002
106. Program Committee Member, American Society for Virology, 2002 – 2005
107. Juvenile Diabetes Research Foundation Review Participant, New York, New York, July 30, 2001
108. Discovery Channel Health Science Educational Video, May 2001
109. Chair, ASGCT 2001 Nominating Committee, Seattle, WA, May 2001
110. Panelist, 4th National Gene Transfer Safety Symposium, Co-sponsored by the NIH and FDA, Rockville, MD, March 7, 2001
100. Member, Curriculum in Toxicology, University of North Carolina, Chapel Hill, 1999 – present
101. Member, National Gene Vector Laboratories Panel, October 26-27, 1999
102. Member, American Society of Gene Therapy, 1996 – present
103. Member, Society of Virology, 1996 – present
104. Member, American Association for the Advancement of Science 1996 – present
105. Member, Carolina Speakers Bureau, 1995 – 2000
106. Member, Recombinant DNA Advisory Committee, NIH 1994 – 1997
107. Participation in Gene Therapy Center Video for Health Sciences Media, Todd Wilson, Producer, April 1994
108. Member, International Advisory Committee for the Xth International Congress of Virology Advisory Committee, School of Medicine, member, 1993 – present
109. UNC Center for Gastrointestinal Biology and Disease, member, 1993 – present
110. University of North Carolina Curriculum of Genetics, member, 1993 – present
111. University of North Carolina Lineberger Comprehensive Cancer Center, member, August 1993 – present
112. University of Pittsburgh Technology Transfer Committee, 1992
113. Member, Mammalian Genome Society, 1992
114. NIH Study Section, Virology, October 1992
115. NIH/ADAMHA Consultant, 1990 – 1996
116. Pittsburgh Area Recombinant DNA Committee, 1990
111. Advisor for Minority Research Apprentices Program, Summer Semester 1989 & 1990
112. Pittsburgh Cancer Institute, member, September 1987 – July 1993
113. NIH Post-doctoral Fellow, 1983 – 1986
114. NIH Pre-doctoral Trainee, 1981 – 1982

EDITORIAL BOARDS

1. Deputy Editor, Human Gene Therapy, 2020 – present
2. Editorial Board, PLOS Pathogens, 2015 – present
3. Editorial Board, Stem Cells, 2009 – present
4. Editorial Board, World Journal of Biological Chemistry, 2009 – present
5. Associate Editor, Molecular Therapy, 2003 – 2007
6. Editorial Board, Journal of Pharmaceutical Sciences, 2002 – present
7. Reviewer, Archives of Biochemistry and Biophysics, 2001 – present

EDITORIAL BOARDS (cont'd)

8. Editorial Board, Virus Research, 2000 – 2003
9. Editorial Board, Molecular Therapy, 2000 – present
10. Editorial Board, The Journal of Gene Medicine, 1999 – present
11. Editorial Board, Genetic Medicine 1998 – present
12. Editorial Board, Gene Therapy and Molecular Biology, 1998 – present
13. Editorial Board, Gene Therapy and Regulation, 1998 – present
14. Editorial Board, Journal of Nature Medicine, 1998 – present
15. Scientific Editorial Board, Human Gene Therapy, 1996 – 2000
16. Editorial Board, Brain Research, 1993
17. Editorial Board, Gene Therapy 1993 – present
18. Editorial Board for Virology, 1992 – 1995
19. Editorial Board, Journal of Virology, 1992 – present
20. Reviewer for Journal of Virology, 1987 – present
21. *Ad hoc* reviewer RAC, Bethesda, MD, December 6, 2001
22. *Ad hoc* reviewer for Science, 1991 – present
23. *Ad hoc* reviewer for March of Dimes grant applications
24. *Ad hoc* reviewer for National Science Foundation grant applications

INVITED SEMINARS

1. Speaker at NCLifeSci Annual Meeting, “Past, Present, Future Panel,” Durham, NC, September 2024
2. Speaker at Boston Gene & Cell Therapy Conference, “Development of Viral Delivery for Precision Medicine, Boston, MA, March 2024
3. The Ohio State University & University of North Carolina Charlotte, Conference on Biomotos, Virus Assembly and RNA Nanobiotechnology, Columbus, OH, December 2023
4. Keynote Speaker at MGB Gene & Cell Therapy Institute Symposium, Boston, MA, November 2023
5. Penn Medicine & The Orphan Disease Center, Speaker at In Vivo Gene Therapy and Genome Editing Summit, Miami Florida, October 2023
6. Speaker at Cold Spring Harbor, “History of Recombinant DNA,” Cold Spring Harbor, NY, September 2023
7. University of Sheffield, Coimbra Portugal, Speaker at ARDAT General Assembly, June 2023
8. Delivered the Sune Lund Bergstrom Keynote address at the Spring Symposium, “Novel Therapeutic Modalities for the 21st Century,” May 2023
9. Sorbonne University, Paris, France-Monday Muscle Seminar, “AAV Gene Therapy for Duchenne Muscular Dystrophy-A 25-year Journey”, March 29, 2021

10. University of Mississippi Medical Center Grand Rounds, “Gene Therapy as an Emerging Treatment for Duchenne Muscular Dystrophy”, January 20, 2021
11. Keynote Speaker at American Society of Cell and Gene Therapy, “Golden Age of Gene Therapy”, April 2020
12. European Society of Gene & Cell Therapy International Conference, INV046 “The Road from Canavan to Pompe – a 20-Year Clinical Journey”, Barcelona, Spain, October 2019
13. Accelerating Bio-Innovation Conference, “Promise of Gene Therapy: a 40-Year Journey”, Cambridge, United Kingdom, April 2019
14. Gene Therapy for Rare Disorders Conference, “Maximizing the Value of Platform Technology”, March 2019
15. AAV Gene Therapy Seminar, BioScience Research Collaboration, December 10, 2018
16. Seminal speaker at the Royal Society of Science in London in the Isaac Newton Lecture room on “Delivering Novel Therapeutic in the 21st Century”, October 24, 2018
17. Keynote speaker at European Society of Gene Therapy, October 19, 2018
18. Honorary Plenary Speaker Chinese Society of Gene and Cell Therapy (CSGCT), 2013
19. Federation of American Societies for Experimental Biology Virus Assembly, Saxtons River, VT, June 17-22, 2006. “Rational Design vs. Mother Nature: Understanding AAV Tropism.”
20. Cardiovascular Cell and Gene Therapy Conference III, Cambridge, MA, April 24-26, 2006 “Optimizing Gene Delivery for Heart.”

INVITED SEMINARS (cont’d)

21. National Hemophilia Foundation’s Eighth Workshop on Novel Technologies and Gene Transfer for Hemophilia, Philadelphia, PA, March 31-April 2, 2006
22. Gordon Conference, The Science of Viral Vectors for Gene Therapy, Ventura, CA, March 12-17, 2006. “Molecular Forms of AAV Vectors After in Vivo Delivery.”
23. University of Alabama Advances in Molecular and Cellular Pathology seminar, Birmingham, AL, December 1, 2005. “AAV Persistence: Role of Circles vs. Linear Genomes.”
24. NHLBI 4th Annual Gene Therapy Symposium for Heart, Lung and Blood Diseases, November 16-18, 2005
25. American Society of Gene Therapy 8th Annual Meeting, St. Louis, MO, June 1-4, 2005. “Biological Nanoparticles: The Next Generation of Efficient Delivery Reagents.”
26. New Frontiers in Genomics and Cardiovascular Disease, Philadelphia, PA, April 8-9, 2005
27. Cystic Fibrosis Foundation Gene Therapy Meeting, Washington DC, September 29-30, 2004 “Hurdles to overcome with AAV and lentivirus vectors?”
28. Xth Parvovirus Workshop, St. Pete Beach, FL, September 8-12, 2004
29. The American Society for Virology 23rd Annual Meeting, Montreal, QC, July 10-14, 2004. “Evolution of a Viral Vector for Human Gene Delivery.”

30. ASGT 7th Annual Meeting, Minneapolis, MN, June 2-6, 2004
31. Cardiovascular Cell and Gene Therapy Conference II, Cambridge, MA, April 13-16, 2004. "AAV Vectors for Efficient Gene Delivery."
32. Heart Failure Society of America 7th Annual Scientific Meeting, Las Vegas, NV, September 22-23, 2003. "Advances in Vector Design."
33. 6th Conference on Protein Expression in Animal Cells, Quebec, September 7-10, 2003. "Development of Novel Serotype Specific AAV Vectors for Gene Therapy."
34. ASGT 6th Annual Meeting, Washington DC, June 4-8, 2003. "AAV Tropism and In Vivo Transduction."
35. Eagleson Institute: Viral Vectors and Biosafety, Chapel Hill, NC, May 14, 2003. "AAV as a Gene Therapy Vector."
36. Children's Research Institute, Columbus, OH, February 18-19, 2003. "Evolution of a Viral Vector for Gene Therapy."
37. Pharmacia, St. Louis, MS, January 23-24, 2003. "Evolution of a Viral Vector for Human Gene Therapy."
38. GSK (GlaxoSmithKline), Stevenage, UK, November 12-15, 2002. "The Future of Gene Therapy."
39. National Hemophilia Foundation - Gene Therapy: Medicine of the 21st Century, Lake Buena Vista, FL, October 30-31, 2002. "AAV, Including Serotypes, Nuclear Import."

INVITED SEMINARS (cont'd)

40. Ernst Schering Research Foundation Workshop/Human Gene Therapy: Current Opportunities & Future Trends, San Francisco, CA, October 2-4, 2002. "Viral Vectors for Cardiovascular Gene Delivery".
41. GSK (GlaxoSmithKline), Collegeville, PA, August 27, 2002. AAV Vectors and Successful Gene Therapy.
42. INTRONN, Rockville, MD. August 8, 2002. Overview of available AAV Vectors.
43. Center for Cell and Gene Therapy, Baylor University, Houston, TX. AAV Vectors: The Next Generation.
44. AMT, Amsterdam, May 27th, 2002. AAV Research.
45. CEREGENE, San Diego, CA, May 6th, 2002. AAV Vector Presentation.
46. Cell Culture Engineering VII, Snowmass, CO. "Use of Different AAV serotypes for improving gene delivery. April 4, 2002
47. Speaker, East Chapel Hill High School, December 2001
48. Chair, Session II, 9th Annual UNC-CH Virology Colloquium Session, September 2001

49. Pharmacia Seminar, St. Louis, MO. "AAV Vectors in Successful Gene Therapy the Gap is Closing", November 29, 2001
50. Genzyme Seminar, Framingham, MA, "Rate limiting steps to efficient AAV gene delivery", March 13, 2001
51. Cell Genesys Seminar, Foster City, CA, "AAV Vector Development", February 22-23, 2001
52. East Carolina University School of Medicine Seminar, Greenville, NC, February 8, 2000
53. Cell Genesys Scientific Advisory Board Ad Hoc meeting. Foster City, CA, March 3-4, 2000
54. Nature Biotechnology Conference, "Gene Therapy: Delivering the Medicines of the 21st Century", Washington, DC, November 7-9, 1999
55. The Rockefeller University Hospital, Seminars in Clinical Research. NY, NY, October 6, 1999
56. Children's Hospital Medical Center, Gene Therapy Seminar Series, "AAV and Gene Therapy", Cincinnati, OH, May 10, 1999
57. The Bayard D. Catherwood Memorial Lecture, "AAV Vectors and Successful Gene Therapy, the Gap is Closing", Emory University, Atlanta, GA, May 5, 1999
58. FDA/NIH Workshop on Non-clinical Toxicological Study Design Issues for Development of AAV-based Gene Therapeutics, "Recent advances in studying AAV integration", Bethesda, MD, May 3, 1999
59. Thomas Jefferson University, Department of Neurosurgery, "Gene Therapy and the Future, the Gap is Closing", April 22, 1999
60. Duke Medical Genetics Seminar, Division of Medical Genetics, "Gene Therapy and Factor 9 Hemophilia", March 25, 1999

INVITED SEMINARS (cont'd)

61. Smith-Kline Corporation Joint Collaboration for Development of Vectors for HDL Liver Disorders, October 1998
62. Duke University Medical Center, Department of Ophthalmology, "Gene Therapy: AAV Viral Vectors", October 20, 1998
63. Cystic Fibrosis Foundation, Williamsburg Conference, Vector Entry, Cell Tropism, May 31-June 5, 1998
64. 1998 Retinal Gene Therapy Workshop, Foundation Fighting Blindness, February 21-22, 1998
65. International Symposium on Gene Therapy and Hemophilia, University of North Carolina, North Carolina, Co-Chairman and speaker, "AAV Vectors for Hemophilia." September 4-6, 1997
66. Cystic Fibrosis Foundation, Williamsburg Conference, Gene Therapy Section, Williamsburg, VA, Invited Speaker, "Adeno-associated Virus." June 1-4, 1997
67. Muscular Dystrophy Association MDA/AFM DMD Gene Therapy Workshop, Loews Ventana Canyon Resort, Tucson, AZ, Invited Speaker, "Adeno-associated Virus Vectors", May 29-31,

1997

68. First International Symposium of Neurovirology, Philadelphia, PA, Invited Speaker, "AAV in brain delivery," May 6-7, 1997
69. EMBO Workshop on Viral Vectors in Basic Biology, Heidelberg Germany, Invited Speaker, "Adeno-associated Virus Vectors yield long-term expression and delivery of potentially therapeutic genes into non-dividing neuronal cells," April 26-29, 1997
70. Fifth Annual Biotechnology Symposium, NC Biotechnology Center, Research Triangle Park, NC. Keynote Speaker: Gene Therapy, A Researcher's View, April 17, 1997
71. Culturefest 1997, Chapel Hill High School, Gene Therapy: Medicine for the 21st Century, March 19, 1997
72. Research Triangle Foundation participant in Gore Hybrid Technologies, January, 1997
73. Recombinant DNA Advisory Committee, National Institute of Health, committee member invited to speak "Update on Targeted Genetics Protocol." Washington DC, December 9, 1996
74. Hemophilia 1996: Research for a Cure Workshop, invited speaker, "AAV Vectors in vivo," National Hemophilia Foundation, Washington DC, November 23-24, 1996
75. Cambridge Healthtech Institute, invited speaker in the Artificial Self-Assembling Systems for Gene Delivery. "AAV Vectors in Gene Therapy," Coronado, California, November 17-19, 1996
76. European Working Group on Human Gene Transfer and Therapy, invited speaker on "AAV Vectors for Brain Delivery," Netherlands Institute for Brain Research, Amsterdam, The Netherlands, November 14-17, 1996
77. James Wilson Laboratory, invited speaker on "AAV Vectors for Brain Delivery." University of Pennsylvania Health System. October 27-29, 1996
78. National Institute of Health, National Institute of Environmental health Sciences, Research Triangle Park, NC, invited speaker, In vivo gene therapy using adeno-associated viral vectors, October 4, 1996

INVITED SEMINARS (cont'd)

79. Cold Spring Harbor Laboratory Gene Therapy Conference, invited speaker, Cold Spring Harbor, New York, September 25-29, 1996
80. Duke Institute for Learning in Retirement and the Museum of Life and Science, lecture as part of course on Science and Technology, Duke University September 11, 1996
81. National Institutes of Health Gene Therapy Conference, Breakout Session: Development of new vector systems, July 12, 1996
82. Cystic Fibrosis Foundation, 1996 Williamsburg Conference Gene Therapy Section, Panel: New vector systems to clinical trials, June 5, 1996
83. The Cleveland Clinic Foundation, lecture to Research Institute members, "Gene therapy for Parkinson's disease using AAV vectors", May 21, 1996
84. Genomic Science Series Conference on Gene Therapy, Hilton Head, SC. AAV: A non-pathogenic integrating virus, May 10, 1996

85. University of North Carolina at Chapel Hill, Spring Meeting of Association of Clinical Scientists, Gene Therapy: an update, May 2, 1996
86. University of North Carolina at Chapel Hill, Neurobiology Spring Semester Colloquium. "In Vivo Gene Delivery for the Study of Neurological Disorders", February 16, 1996

PLENARY LECTURES

1. Baxter Hemophilia Conference Dublin, Ireland and Vienna, Austria, March 8-13, 2013
2. JSGT Meeting Beijing, China, December 8-12, 2012
3. Gene Transfer & Rare Diseases Workshop, Rockville, MD, "Panel II Discussion", September 12-16, 2012
4. FDA/CBER/OCTGT Meeting Rockville, MD, January 26, 2012
5. 17th Annual Meeting for JSGT Fukuoka, Tokyo, "Special Lecture IV", July 15-17, 2011
6. ASGCT 14th Annual Meeting Seattle, WA, May 18-21, 2011
7. CliniGene Conference Paris, France, April 7-9, 2011
8. FDA Meeting Rockville, MD, February 9, 2011
9. Cell and Gene Therapy forum 2012, Washington, D.C., January 30-February 1, 2011
10. ASGCT Retreat New Orleans, LA, January 26-28, 2011
11. Treat-NMD Evry, France, "Systemic delivery of AAV for Neuro-Muscular Gene Therapy", December 12-17, 2010
12. MPFI Seminar Florida, November 3-5, 2010
13. ESGCT Annual Meeting, Milan, Italy, "Adeno associated viruses and vectors", October 22-25, 2010
14. Wellstone Meeting, Philadelphia, PA, October 7-8, 2010

PLENARY LECTURES (cont'd)

15. Krabbe Translational Research Network, Chapel Hill, NC, October 6, 2010
16. Viral Vectors In Gene Therapy, Kuopio, Finland, "AAV Vectors for Gene Therapy", August 26-September 4, 2010
17. NIH-Raid Application Update, Bethesda, MD, May 25, 2010
18. ASGCT Annual Meeting 2010, 13th Annual Meeting, Washington, DC, "Designing AAV Vectors for Translational Studies: Update on DMD Phase 1 Trial.", May 17-22, 2010
19. Honored Speaker for the European Society of Gene Therapy, 2010
20. Guest Lecturer for the EMBO Lecture Series, 2010
21. National Hemophilia Foundation's Sixth Workshop on Gene Therapies for Hemophilia, San Diego, CA, "Evolution for a Viral Vector for Gene Therapy", April 25-26, 2003

22. Annual Meeting of Association of Virology, Berlin, Germany, “Evolution for a Viral Vector for Human Gene Therapy”, March 26-29, 2003
23. American Society for Microbiology – Gene Therapy: The Next Five Years, Banff, Alberta, Canada, “Evolution of a Viral Vector for Human Gene Therapy”, February 26- March 2, 2003
24. The 10th Meeting of the European Society of Gene Therapy, Antibes Juan-les-Pins, France. “2nd Generation AAV Vectors”, October 13-16, 2002
25. The Salk Institute for Biological Studies, La Jolla California, “AAV vectors for *in vivo* gene delivery”, February 22, 2002
26. The 5th Gene Delivery & Cellular Protein Expression Conference, Semmering, Austria, “Development of Novel Serotype Specific AAV Vectors for Gene Therapy”, September 9-13, 2001
27. The Eighth Annual Glaucoma Foundation Optic Nerve Rescue and Restoration Think Tank: Gene Therapy, New York City, “Development of Novel Serotype Specific AAV vectors for Gene Therapy”, July 20-21, 2001
28. American Society of Gene Therapy Annual Meeting, Seattle, WA, “Parvovirus Vectors Pros and Cons”, May 30-June 3, 2001
29. American Society for Microbiology General Meeting, Orlando, FL, “Pros and Cons of AAV Vectors”, May 20-24, 2001
30. American Society for Microbiology Conference on Viral Gene Vectors: Molecular Biology, Design, & Application to Gene Therapy, Banff, Alberta, Canada. “Developing Viral Vectors for Hemophilia”, April 26-29, 2001
31. The National Hemophilia Foundation Workshop, La Jolla, CA, “AAV Serotypes and FIX Gene Delivery”, April 20-21, 2001
32. American Association of Medical Colleges Spring Meeting, New Orleans, LA. Keynote Address: “The Genomics Revolution in Biology and Medicine: What Does it Mean for 21st Century Academic Medical Centers?”, March 22-24, 2001

PLENARY LECTURES (cont’d)

33. 4th National Gene transfer Safety Symposium: Safety Considerations in the Use of AAV Vectors in Gene Transfer Clinical Trials. Rockville, MD, March 7, 2001. Panelist
34. 1st International Symposium of Sonderforschungsberich 455: Viral Functions and Immunomodulation, Bavaria, Germany, “Analyzing rate limiting steps of Adeno-associated virus vectors gene transduction”, January 22-24, 2001
35. Keystone Symposia, Gene Therapy 2001: A Gene Odssey (A1), Snowbird, UT, “Adenoviral Vectors for Gene Transfer to Muscle”, January 6-11, 2001
36. Fourteenth Annual North American Cystic Fibrosis Conference, Baltimore, MD, “Strategies for Improving AAV Vectors for Gene Transfer” November 11-12, 2000
37. International Symposium on NeuroVirology, San Francisco, CA “Molecular and Cellular

Therapeutics” September 14-16, 2000

38. VIIIth Parvovirus Workshop, Mont Tremblant, Quebec, Canada, “Self-Complementary rAAV (scAAV) Gene Delivery Vectors”, June 28-July 2, 2000
39. ASGT 3rd Annual Meeting, Denver, CO. “Adeno-Associated Virus”, June 1, 2000
40. The National Hemophilia Foundation’s Third Workshop on Gene Therapies for Hemophilia, “Analysis Rate Limited Steps for AAV Gene Delivery”, Washington DC, March 24-25, 2000
41. Keystone Symposia Gene Therapy: The next Millennium (A1). “Adeno-Associated Virus Vectors; Gene Therapy and Viral Vectors, the Gap is Closing”, Keystone, CO, January 6-12, 2000
42. Cold Spring Harbor Laboratory Eukaryotic DNA Replication, “Characterization of cis and trans sequences required for AAV ch-19 targeted integration using viral mutants and a novel mouse model”. Platform presentation. Cold Spring Harbor, NY, September 15-19, 1999
43. Biochemical Society, Hormone Group, Symposium, University of Keele, Gene Therapy from Bench of Bedside. “Novel AAV Vectors”, Staffordshire, U.K, July 20, 1999
44. Cystic Fibrosis Foundation Williamsburg Conference. “Rate Limiting Step for AAV Transduction”. Kingsmill Conference Center, Williamsburg, VA, June 4-9, 1999
45. Inaugural meeting of the Australian Gene Therapy Society, Melbourne, Australia, February 22-23, 1999
46. American Society for Virology, 17th Annual Meeting, University of British Columbia, Vancouver, Canada, July 11-15, 1998
47. International Centre for Genetic Engineering and Biotechnology, Trieste, Italy, Development of new vectors and transcriptional switches for the gene therapy of human diseases, July 1998
48. The American Society of Gene Therapy, 1st Annual Meeting, Seattle, WA, May 27-30, 1998
49. Hunters Hope Symposium for Krabbe/Leukodystrophy Research, May 8-10, 1998
50. Keystone Symposia on Molecular & Cellular Biology, Keystone, CO, January 19-25, 1998
51. Gordon Conference on Cancer, Salve Regina University, Newport, RI, August 3-5, 1997

PLENARY LECTURES (cont’d)

52. Cold Spring Harbor Laboratory Gene Therapy, AAV: a nonpathogenic integrating vector, September 25-29, 1996
53. 1996 Spring Meeting of the Association of Clinical Scientists. Opening Session Presentation, May 2, 1996
54. International Centre for Genetic Engineering and Biotechnology and the Universities of Di Padova and Loma Linda. Trieste, Italy, April 11-14, 1996
55. International Symposium on Gene Therapy of Cancer, AIDS, and Genetic Disorders, Trieste, Italy, AAV vectors in vivo, April 10-13, 1996
56. American Society of Neurochemistry's Satellite Symposium on Gene Therapy for Neurological

Disorders. March 2, 1996

57. Keystone Symposia, Taos, New Mexico. February 4-10, 1996

DEPARTMENTAL ACTIVITIES

1. UNC PHCO 701, November 11, 2024
2. Seminar / Roundtable, UNC School of Pharmacy, March 20,
3. UNC PHCO 701 Gene Therapy Lecture, November 29, 2021
4. UNC IDDRRC Scientific Retreat, April 21, 2021
5. UNC PHCO 701 Gene Therapy Lecture, November 4, 2020
6. UNC Pathology Department Grand Rounds, Gene Therapy, October 22, 2020
7. Carolina Genetics Interest Group: Talk for 1st Year Medical Students, “Gene Therapy for Muscular Dystrophy”, May 2020
8. UNC Pharmacology Department Academic Seminar Series, November 6, 2018
9. Neurodevelopmental Function in Rare Disorders, Clinical/Research, 2010 -
10. Paul Wellstone Center for Muscular Dystrophy Research, 2008 -
11. UNC Gene Therapy Newest Programmatic Initiatives: Carolina Cardiovascular Biology Center Retreat, May 22, 2003
12. UNC Basic Science Retreat. Aqua Duct, August 26, 2002
13. Chancellor’s Retreat, Friday Center, April 2002
14. Faculty Search Committee, Human Genetics, University of North Carolina, 2001
15. Member, Packer Award Committee, University of North Carolina, August 2001
16. Basic Science Retreat, Aqueduct Conference Center, UNC Chapel Hill, August 2001
17. Search Committee, Associate Vice Chancellor and Director, Office of Technology Development, August 2000
18. Faculty Search Committee, 1996
19. University Selection Committee for the Burroughs Wellcome Foundation Career Awards in the Biomedical Field
20. Graduate Recruiting Committee, 1990

DEPARTMENTAL ACTIVITIES (cont’d)

21. Mellon Fellowship Committee, 1989
22. Pittsburgh Area Recombinant DNA Committee
23. Chair, Faculty Search Committee, 1992
24. Faculty Search Committee, 1988

Thesis Committees:

Brenda Hall

Weidong Xiao

Susan Spence
Hui Chen
Anthony Youlton
Fran Sverdrup
Candace Summerford
Beverly Hamilton
Uma Chandran
Xiaojun Cheng
Forrest Ferrari

Marilee Shelton
Chantal (Sullivan) Wall
Yu Wang
Scott Erme
Karen Erford
Zengfeng Wang
Linda Gorman
Chantal Rivera
Terry Amiss

Thesis Committees (cont'd):

Xiaohong Gu
Deanna Hapke
Peter Sazani
Rebecca Haberman
Nathan Allen
Atsuko Negishi
Nina DiPrimio
Stacey Foti
Michael Washburn
PJ Xiao
Angela Mitchell
Kenton Woodard

Oliva Smutney
Krystle P. Strand
Raj Ra
Vivian Choi
Stephen Snowdy
Jed Ferguson
Jarrod Johnson
Matt Bayer
Mark Johnson
Sarah Nicolson
Kathrine Lang
Jamie Warischalk

Comprehensive Examination Committees:

Mong Hong Lee
Carole Harris-Kerr
Margaret Levin
Todd Pippert
Chad Nesbitt
Peter Rau
Sheritha Lee

Aurelie Gresset
John Bauman
Jamie Alan
Angela Russo

Preliminary Examination Committees:

First year graduate students, Biological Sciences.
1989, 1990, 1991, 1994, 1995, 2001, 2002

PAST TRAINEES:

Name/Degree	Pre/Post Doc	Training Period	Degree/Date Institution	Current Position/ Support Source
Earley, Lauriel	Post-doc	2016-2019	Ph.D. 2016 Oregon Health and Science University	Senior Scientist, Shape Therapeutics
Askew, Charles	Post-doc	2015-Present	Ph.D. 2015 University of Virginia	Post-doc, UNC
Woodard, Kenton	Pre-doc	2010-2016	Ph.D. 2016 UNC-CH	Associate Director, Penn Vector Core, University of

				Pennsylvania
Liang, Katharine	Pre-doc	2010-2016	M.D./Ph.D. UNC-CH	Chief Resident, University of Washington Medical Center
Salganik, Max	Post-doc	2013-2015	Ph.D. 2012 University of Florida	Associate Director, Medical Affairs, Biotheranostics, Inc.
Warischalk, Jayme	Pre-doc	2010-2014	Ph.D. 2015 UNC-CH	Academic Editor, Research Square
Lentz, Thomas	Post-doc	2011-2014	Ph.D. 2010 University of Wisconsin-Madison	Assistant Professor of Biology University of Wisconsin
Nicolson, Sara	Pre-doc	2009-2014	Ph.D. 2014 UNC-CH	RTP Biotech
Xiao, Ping-jie	Pre-doc	2008-2012	Ph.D.	D. Neil Hayes Research Laboratory, Lineberger Cancer Center, UNC
DiPrimio, Nino	Pre-doc	2005-2009	Ph.D. 2009 UNC-CH	Director, Strategic Alliances, StrideBio, Inc.
Asokan, Aravind	Post-doc	2004-2009	Ph.D. 2004 UNC-CH	Director of Gene Therapy, Professor of Surgery, Professor of Molecular Genetics and Microbiology, Duke University
Dismuke, David	Post-doc	2007-2011	Ph.D. 2006 Vanderbilt University	VP of Manufacturing, StrideBio, Inc.
Li, Wuping	Post-doc	2005-2010	Ph.D. 2003 State Key Lab of Molecular Virology and Genetic Engineering	Principal Investigator, Wuhan Institute of Virology, Chinese Academy of Sciences
Foti, Stacey	Post-doc Pre-doc	2008-2009 2002-2008	Ph.D. 2008 UNC-CH	University of British Columbia, Vancouver Post-doc

Name/Degree	Pre/Post Doc	Training Period	Degree/Date Institution	Current Position/ Support Source
Gray, Steve	Post-doc	2006-2008	Ph.D. 2006 Vanderbilt University	Associate Professor, University of Texas Southwestern Medical Center at Dallas
Hirsch, Matthew	Post-doc	2005-2008	Ph.D. 2005 West Virginia University	Associate Professor of Ophthalmology, UNC-CH
Duan, Hongzhe	Post-doc	2002-2006	M.D. 1995 Capital University of	Research Specialist, Vascular Biology Center,

			Medical Sciences, Beijing China	Medical College of Georgia
Grieger, Josh	Pre-doc	2002-2006	Ph.D. 2005 UNC-CH	Chief Technology Officer, Asklepios BioPharmaceutical, Inc.
Wu, Zhijian	Post-doc	2002-2006	Ph.D. 2000 Chinese Academy of Preventive Medicine	Vice President, Gene Therapy Research, PTC Therapeutics, Inc.
Allen, Nathan	Pre-doc	2000-2005	M.S. 2005 UNC-CH	Director, Product Management, Bio-Techne
Choi, Vivian	Pre-doc	2001-2005	Ph.D. 2005 UNC-CH	Head of Global Gene Therapy Research, Takeda
Bowles, Dawn	Post-doc	1999-2004	Ph.D. 1998 LSU Medical Center	Assistant Professor, Duke University Cardiovascular & Thoracic Surgery
Li, Chengwen	Post-doc	2000-2004	M.D. / Ph.D. 1995 Peking Union Medical College	Research Associate Professor, UNC Pediatrics
Amiss, Terry	Pre-doc	1995-2001	Ph.D. 2001 UNC-CH	Senior Scientist, Becton, Dickinson and Company
Carter, Pamela	Post-doc	1998-2001	Ph.D. 1998 UNC-CH	unknown
Haberman, Rebecca	Post-doc Pre-doc	2001-2003 1996-2001	Ph.D. 2001 UNC-CH	Associate Research Scientist, Department of Psychological and Brain Sciences, Johns Hopkins Science of Learning Institute
Gavin, Denise	Post-doc	1996-2000	Ph.D. 1996 Rush Medical College	Chief, Gene Therapy Branch, U.S. Food and Drug Administration
Young, Samuel	Pre-doc	1997-2000	Ph.D. 2000 UNC-CH	Associate Professor, University of Iowa College of Medicine
Name/Degree	Pre/Post Doc	Training Period	Degree/Date Institution	Current Position/ Support Source
Rabinowitz, Joseph	Post-doc	1996-1999	Ph.D. 1995 Case Western Reserve University	Senior Director, Capsid Development, Pfizer
Kroner-Lux, Gabriele	Post-doc	1995-1998	Ph.D. 1995 Inst. Molec. Bio. Medicine, Essen, Germany	Progen Biotechnik, Heidelberg, Germany
Yang, Charles	Post-doc	1994-1998	Ph.D. 1994 Brandeis University	Patent Agent, Stanek Lemon Crouse & Meeks, P.A.

Ferrari, Forrest	Post-doc Pre-doc	1997 1991-1997	Ph.D. 1997 UNC-CH	Vice President, Clinical Services, Mirixa Corporation
Tazelaar, John	Post-doc	1996-1997	M.D. 1991 University of Michigan	Director of Pathology, Myriad Genetics
Ansardi, David	Post-doc	1994-1996	Ph.D. 1993 University of Alabama-Birmingham	Chair, Department of Natural Sciences, Calhoun Community College, Decatur
Bartlett, Jeffrey	Post-doc	1993-1996	Ph.D. 1991 University of Pittsburgh	Senior Director, Gene Therapy Strategic Development CSL Behring
Xiao, Weidong	Pre-doc	1992-1996	Ph.D. 1996 UNC-CH	Professor, Temple University
Rolling, Fabienne	Post-doc	1993-1995	Ph.D. 1992 University of Aix-Marseille II	Principle Investigator, Gene Therapy Lab, Centre Hospitalier Universitaire

AWARDS TO MEMBERS OF THE LABORATORY

1. Matthew Hirsch – ASGCT Outstanding New Investigator Award, University of North Carolina Chapel Hill, 2022
2. Steven J. Gray, Ph.D. – ASGCT Outstanding New Investigator Award, University of Texas Southwestern Medical, Dallas TX, On the Brink of a Treatment Revolution for Inherited Pediatric Neurological Diseases, 2019
3. Aravind Asokan, Ph.D. – ASGCT Outstanding New Investigator Award, University of North Carolina Chapel Hill, A Sweet Side to AAV Biology, 2013
4. Junjiang Sun, MD, PhD – Program Project Grant Animal Model Core Contact Project Leader; Junior Investigators' Travel Award; North Carolina Translational and Clinical Institute Tracs Pilot Grant Award; National Hemophilia Foundation Judith Graham Pool Postdoctoral Research Award
5. Bruce Zhang, MS – UNC-CH Graduated with Distinction; Dean's List 6 out of 8 semesters, 2014-2018
6. Lauriel F. Earley, PhD – American Society of Gene and Cell Therapy inaugural Career Development Grant recipient, 2019; Pfizer-NC Biotech Distinguished Postdoctoral Fellowship, 2017
7. Stephen Snowdy, PhD, MBA – National Society of Hispanic MBAs/Hispanic Scholarship Fund Scholar, 2001 – 2002
8. Jackie Stilwell, PhD – American Society Gene Therapy Travel Grant Award for Best Abstract, May 2001; Virology Training Award, 1999
9. Nathan Allen, PhD – UNC Dean's Award, 2003; Virology Training Award, 2000

AWARDS TO MEMBERS OF THE LABORATORY (cont'd)

10. Rebecca Haberman, PhD – Epilepsy Foundation of America Fellowship, July 1999 – June

2000

11. Denise Gavin, PhD – FDA Commissioner’s Special Citation: Zolgensma BLA Review Team, 2020; CBER Director Group Award: Luxturna BLA Review Team, 2018; FDA Honor Award: Outstanding Service Award, 2015; CBER: Center Director’s Distinguished Service Award, 2013; CBER: Scientific Achievement Award-Excellence in Analytical Science: Potency Guidance Development Group Leader, 2009; Peer Review Promotion: FDA Expert in area of AAV Vectors, 2007; Travel Grant Award for Best Abstract, Keystone Meeting, 1999; UNC On-Campus Dissertation Fellowship 1997 – 1998
12. Candace Summerford, PhD – Graduated with Honors from Pharmacology Ph.D. Program, UNC-CH, 1998; Hoechst-Celanese Corporation/UNC-CH Excellence Award, December 1995
13. Joseph Moliterno, Howard Hughes Undergraduate Research, May 1992
14. Lynne Hunter, Pre-Clinical Fellowship, Sandoz Pharmaceutical Company; Basel, Switzerland, June – August 1990; Ben Franklin Fellowship, 1989

TEACHING EXPERIENCE

1. **Pharmacology 394**, Doctoral Research, Spring 2003.
2. **Pharmacology 202**, Principles of Pharmacology and Toxicology, (Emerging Areas in Pharmacology) 4/26/02; (Basic and Clinical Pharmacology) Spring 2003.
3. **Biochemistry 105**, Molecular Biology, Fall 2001, January 1996, November 1995, December 1994.
4. **Microbiology 130**, Virology, Steve Bachenheimer, October 2001, October 2000, October 1999, October 1998, October 1997, October 1996, November 1995, February 1995, December 1994.
5. **Pharmacology 221F**, Course Director, Graduate Course. “Gene Therapy Medicine for the 21st Century.” Fall 2002, Spring 2001, Fall 2000, Spring 1999, Fall 1998, Spring 1997, Fall 1996, Spring 1995.
6. **Pharmacy 61**, lecture for Moo Cho class, Spring 2001, Spring 2000, Spring 1999, Spring 1998, Spring 1997, Fall 1996.
7. **Microbiology 211**, Advanced Topics in Virology, Lineberger Cancer Center, Graduate course, January-February 2001-1997.
8. **Neurobiology Curriculum Speakers Series**, UNC-CH, Lecture, February 2, 1996.
9. **Pharmacology 205**, The Molecular Pharmacology of Cancer, November 15, 1995.
10. **Molecular Biology Undergraduate Course (Biotechnology and It's Social Impact) - "Gene Therapy-Practice and Caveats"**. Princeton University, February 7, 1995.
11. **Virology**, University of Pittsburgh, B.S. 1730, Winter 1993.
12. **Graduate Virology Course**, Lecturer, G.S.P.H., University of Pittsburgh, Winters, 1992- 1989.
13. **Graduate Core Course**, University of Pittsburgh, B.S. "MCDB", Virus Module, Fall 1992, Fall 1991.
14. **Graduate Student Seminar, B.S. 2450**, University of Pittsburgh, Fall 1991, Fall 1988.
15. **Virology**, University of Pittsburgh, B.S. 1730, Winters 1991-1988.
16. **Bacterial Genetics, B.S. 1280**, University of Pittsburgh, Winter 1987.

INTERNATIONAL TEACHING EXPERIENCE

Gene Delivery and Gene Targeting for Brain Diseases, Step One, The Viral Vector, Neuroscience School of Advanced Studies, Summer 2014.

COMMENTS REQUESTED BY PROFESSIONAL PUBLICATIONS

1. *Science Magazine*, News of the Week. “Real-time Single Molecule Imaging of the Infection Pathway of an Adeno-associated Virus” Christophe Brauch lab, Germany.
2. *Molecular Therapy* “Looking into the Eclipse”.
3. *Nature Biotechnology* “Expanding AAV Packaging”.
4. *Nature Medicine* “AAV Receptors”.

REFEREED PUBLICATIONS

1. Thampi P, Seabaugh KA, Pezzanite LM, Chu CR, Phillips JN, Grieger JC, McIlwraith CW, Samulski RJ, Goodrich LR. A pilot study to determine the optimal dose of scAAVIL-1ra in a large animal model of post-traumatic osteoarthritis. *Gene Ther.* 2023 Dec;30(12):792-800. doi: 10.1038/s41434-023-00420-2. Epub 2023 Sep 11. PMID: 37696981; PMCID: PMC10727982.
2. Kim AY, Duerr FM, Phillips JN, Samulski RJ, Grieger JC, Goodrich LR. Serotype-specific transduction of canine joint tissue explants and cultured monolayers by self-complementary adeno-associated viral vectors. *Gene Ther.* 2023 Apr;30(3-4):398-404. doi: 10.1038/s41434-022-00366-x. Epub 2022 Oct 20. PMID: 36261499.
3. Berns KI, Byrne BJ, Flotte TR, Samulski RJ, Srivastava A. Nicholas Muzyczka, PhD. *Mol Ther.* 2023 Mar 1;31(3):607. doi: 10.1016/j.ymthe.2023.01.027. Epub 2023 Feb 10. PMID: 36764298; PMCID: PMC10014261.
4. Le Guiner C, Xiao X, Larcher T, Lafoux A, Huchet C, Toumaniantz G, Adjali O, Anegon I, Remy S, Grieger J, Li J, Farrokhi V, Neubert H, Owens J, McIntyre M, Moullier P, Samulski RJ. Evaluation of an AAV9-mini-dystrophin gene therapy candidate in a rat model of Duchenne muscular dystrophy. *Mol Ther Methods Clin Dev.* 2023 May 18;30:30-47. doi: 10.1016/j.omtm.2023.05.017. PMID: 37746247; PMCID: PMC10512999.
5. Berns KI, Byrne BJ, Flotte TR, Samulski RJ, Srivastava A. Nicholas Muzyczka, PhD [1947-2023]. *Hum Gene Ther.* 2023 Feb;34(3-4):81-82. doi: 10.1089/hum.2023.29235.nmu. PMID: 36794979.
6. Berns KI, Byrne BJ, Flotte TR, Samulski RJ, Srivastava A. Nicholas Muzyczka, PhD. *Mol Ther.* 2023 Mar 1;31(3):607. doi: 10.1016/j.ymthe.2023.01.027. Epub 2023 Feb 10. PMID: 36764298.
7. Daw TB, El-Nahal HG, Basso MA, Jun EJ, Bautista AR, Samulski RJ, Sommer MA, Bohlen MO. Direct Comparison of Epifluorescence and Immunostaining for Assessing Viral Mediated Gene Expression in the Primate Brain. *Hum Gene Ther.* 2023 Mar;34(5-6):228-246. doi: 10.1089/hum.2022.194. Epub 2023 Mar 7. PMID: 36719771.

8. Thampi P, Samulski RJ, Grieger JC, Phillips JN, McIlwraith CW, Goodrich LR. Corrigendum: Gene therapy approaches for equine osteoarthritis. *Front Vet Sci.* 2023 Jan 4;9:1117776. doi: 10.3389/fvets.2022.1117776. eCollection 2022.PMID: 36686173.
9. Prebor J, Samulski B, Armitano-Lago C, Morrison S. Patterns of Movement Performance and Consistency From Childhood to Old Age. *Motor Control.* 2022 Nov 9;27(2):258-274. doi: 10.1123/mc.2022-0006. PMID: 36351427.
10. Kim AY, Duerr FM, Phillips JN, Samulski RJ, Grieger JC, Goodrich LR. Serotype-specific transduction of canine joint tissue explants and cultured monolayers by self-complementary adeno-associated viral vectors.
11. Thampi P, Samulski RJ, Grieger JC, Phillips JN, McIlwraith CW, Goodrich LR. Gene therapy approaches for equine osteoarthritis. *Front Vet Sci.* 2022 Sep 28;9:962898. doi: 10.3389/fvets.2022.962898. eCollection 2022.PMID: 36246316.
12. Pupo A, Fernández A, Low SH, François A, Suárez-Amarán L, Samulski RJ. AAV Vectors: The Rubik's Cube of Human Gene Therapy. *Mol Ther.* 2022 Oct 5:S1525-0016(22)00569-X. doi: 10.1016/j.ymthe.2022.09.015. Online ahead of print.PMID: 36203359.
13. Arriaga I, Navarro A, Etxabe A, Trigueros C, **Samulski RJ**, Moullier P, François A, Abrescia NGG. Cellular and structural characterisation of VP1 and VP2 knockout mutants of AAV3B serotype and implications for AAV manufacturing. *Hum Gene Ther.* 2022 Sep 9. doi: 10.1089/hum.2022.119. Online ahead of print.PMID: 36082996.
14. Xiao W, **Samulski RJ**. Recombinant adeno-associated virus production, the beginning of the end of uncertainty. *Hum Gene Ther.* 2022 Apr;33(7-8):355-357. doi: 10.1089/hum.2022.29207.wxi.PMID :35442070.
15. Stepp WH, Samulski TD, Hackman TG, Issaeva N, Yarbrough WG, Schrank TP. Development of a Novel Molecular Test for Determining HPV Integration Status in HPV-Positive Oropharynx Cancers. *JAMA Otolaryngol Head Neck Surg.* 2022 Oct 1;148(10):987-989. doi: 10.1001/jamaoto.2022.2441. PMID: 36074493; PMCID: PMC9459908.
16. Kishimoto TK, **Samulski RJ**. Addressing high dose AAV toxicity – ‘one and done’ or ‘slower and lower’? *Expert Opin Biol Ther.* 2022 Apr 3:1-5. doi: 10.1080/14712598.2022.2060737. Online ahead of print.PMID: 35373689.
17. Shao W, Sun J, Chen X, Dobbins A, Merricks EP, **Samulski RJ**, Nichols TC, Li C. Chimeric mice engrafted with canine hepatocytes exhibits similar AAV transduction efficiency to hemophilia B dog. *Front Pharmacol.* 2022 Jan 31;13:815317. doi: 10.3389/fphar.2022.815317. eCollection 2022.PMID: 35173619.
18. Arriaga I, Navarro A, Etxabe A, Trigueros C, Samulski RJ, Moullier P, François A, Abrescia NGA. Cellular and Structural Characterization of VP1 and VP2 Knockout Mutants of AAV3B Serotype and Implications for AAV Manufacturing. *Hum Gene Ther.* 2022 Nov;33(21-22):1142-1156. doi: 10.1089/hum.2022.119. PMID: 36082996.

19. Zhang X, Chai Z, Lee Dobbins A, Itano MS, Askew C, Miao Z, Niu H, **Samulski RJ**, Li C. Customized blood-brain barrier shuttle peptide to increase AAV9 vector crossing the BBB and augment transduction in the brain. *Biomaterials*. 2021 Dec 31;281:121340. doi: 10.1016/j.biomaterials.2021.121340. Online ahead of print.PMID: 34998171.
20. Singleton MP, Thambuluru S, Samulski T, Paraghamian SE, Clark LH. Non-islet cell tumor hypoglycemia in a patient with uterine carcinosarcoma. *Gynecol Oncol Rep*. 2021 Dec 20;39:100912. doi: 10.1016/j.gore.2021.100912. PMID: 35005156; PMCID: PMC8718578.
21. Chai Z, Zhang X, Dobbins AL, Samulski RJ, Merricks E, Nichols TC, Li C. Dexamethasone transiently enhances transgene expression in the liver when administered at late phase post long term AAV transduction. *Hum Gene Ther*. 2021 Oct 7. doi: 10.1089/hum.2021.083. Online ahead of print.
22. Zhang J, Xiangping Y, Herzog RW, Samulski RJ. Flies in the ointment: AAV vector preparations and tumor risk. *Mol Ther*. 2021 Aug 26; 29(9): 2637-2639. doi: 10.1016/j.ymthe.2021.08.016.
23. Zhang J, Yu X, Guo P, Firman J, Pouchnik D, Diao Y, **Samulski RJ**, Xiao W. Satellite subgenomic particles are key regulators of adeno-associated virus life cycle. *Viruses* 2021 June 21; 13(6):1185. Doi: 10.3390/v13061185.
24. Johnson SM, Samulski TD, O'Connor SM, Smith SV, Funkhouser WK, Broaddus RR, Calhoun BC. Clinical and Financial Implications of Second-Opinion Surgical Pathology Review. *Am J Clin Pathol*. 2021 Sep 8;156(4):559-568. doi: 10.1093/ajcp/aqaa263. PMID: 33769453.
25. Zhang J, Guo P, Xu Y, Mulcrone PL, **Samulski RJ**, Xiao W. “D” matters in recombinant AAV DNA packaging. *Mol Ther*. 2021 Jun 2; 29(6):1937-1939. doi: 10.1016/j.ymthe.2021.05.002. Epub 2021 May 19. PMID: 34010593.
26. Byrne BJ, McCall AL, Davidson BL, **Samulski RJ**. Mavis Agbandje-McKenna’s lifelong commitment to teaching and research. *Mol Ther*. 2021 May 5;29(5):1661-1662. doi: 10.1016/j.ymthe.2021.03.007. Epub 2021 Apr 24. PMID: 33894816.
27. Byrne BJ, McCall AL, Davidson BL, **Samulski RJ**. Mavis Agbandje-McKenna’s lifelong commitment to teaching and research. *Hum Gene Ther*. 2021 April: 32(7-8):319-320. doi: 10.1089/hum.2021.29157.alm. PMID: 33891506.
28. Francis JS, Markov V, Wojtas ID, Gray S, McCown T, **Samulski RJ**, Figueroa M, Leone P. Preclinical biodistribution, tropism, and efficacy of oligotropic AAV/Olig001 in a mouse model of congenital white matter disease. *Mol Ther Methods Clin Dev*. 2021 Mar 12;20:520-534. doi: 10.1016/j.omtm.2021.01.009.eCollection 2021 Mar 12.
29. Ramesh SK, Bronstein MG, **Samulski RJ**. Ultra-rare but not forgotten: New drug development paradigms to treat the rarest of diseases. *STAT* 2021 Feb 17.
30. Konkle BA, Walsh C, Escobar MA, Josephson NC, Young G, von Drygalski A, McPhee SWJ, **Samulski RJ**, Bilic I, De La Rosa M, Reipert B, Rottensteiner H, Scheiflinger F, Chapin J, Ewenstein BM, Monahan PE. BAX 335 hemophilia B gene therapy clinical trial results - potential impact of CpG sequences of gene expression. *Blood*. 2021 Feb 11;137(6):763-774. doi: 10.1182/blood.

2019004625. PMID: 3306763.

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PUBLIC SERVICE

North Carolina General Assembly, Genomic Medications Briefing, June 2019

North Carolina Museum of Natural Sciences, Best Fest, "Genes, Genetics and Gene Therapy, The Incredible Journey, April 6, 2013

Chapel Hill High School, "Gene Therapy and Molecular Medicine". December 14, 2001

University of North Carolina-Chapel Hill and University Hospitals, "Mini Medical School", Lecture, April 4, 1995.

Research Support

Grant Research

PENDING

1 R01 NS116019-01A1 (McCown, Samulski)	09/01/2020-08/31/2025	1.2 calendar
NIH	\$388,750	

AAV capsid-promoter interactions determines CNS cell selective gene expression in vivo

In vivo studies will assess those promoter elements that contribute to the interactions, and the specific AAV9 capsid elements that influence the change in in vivo cellular tropism. Given the numerous applications of AAV9 vectors, the findings should significantly advance our understanding of basic capsid-promoter interactions that will prove critical to the future design of AAV9 based gene therapies.

1 R01 HL151348-01A1 (Samulski)	09/01/2020-08/31/2025	1.2 calendar
NIH	\$388,750	

Development of AAV vectors for CF therapy

The results generated from these studies will allow us to develop an effective approach to treat CF patients using AAV vector mediated gene delivery.

Grant Research – Pending (cont'd)

1 R01 AI 155451-01 (Samulski)	09/01/2020 - 08/31/2025	2.4 calendar
NIH	\$517,815.00	

The effect of dsRNA innate response on long-term AAV human liver transduction

The objective of the proposal is to study the late innate immune response activation triggered by dsRNA in AAV transduced human hepatocytes.

ACTIVE

SAMULS19XX0 (Samulski)	07/01/19-06/30/22	2.40 calendar
Cystic Fibrosis Foundation	\$216,000	

AAV vector mediated gene editing in cystic fibrosis

We will design more effective polyploid AAV vectors from capsids of serotypes or mutants with superior efficiency to cross blood-vascular barrier and transduce airway epithelial cells after systemic administration.

5R01AI117408-05(Li, Samulski)	03/01/16-02/28/21	0.60 calendar
NIH/NIAID	\$385,642	

Enhance AAV Liver Transduction with Capsid Immune Evasion

This study will allow us to design safer and more effective strategies for human liver gene therapy using AAV.

1R01NS113917-01 (Samulski, Consortium PI)	09/15/19-06/30/24	0.40 calendar
U. of Cincinnati/NIH	\$12,844	

Gene Therapy Targeting of CNTFR and CLC in Muscle to Treat ALS

This project is designed to identify and optimize AAV gene therapy treatments for ALS. The treatments will enhance CNTFRalpha and CLC RNA expression in skeletal muscle.

EGAN19XX1 (Egan, Samulski)	02/01/20-01/31/22	0.60 calendar
Cystic Fibrosis Foundation	\$280,000	

Ex-vivo lung perfusion to deliver nucleic acids to cells in human lungs

This project brings together three experts in different disciplines to test the hypothesis that engineered adenovirus associated viral vectors (AAVs) derived from a capsid library can effectively insert functioning nucleic acids into airway epithelial cells.

BOUCHE19XX0 (Boucher)	02/01/20-01/31/23	0.48 calendar
Cystic Fibrosis Foundation	\$865,000	

Abrogation of Airway Epithelial Barriers to Transduction

The UNC Collaborative Research Grant is designed to: 1) identify airway regional and cellular targets for CFTR nucleic acid therapy; 2) develop novel systems for their study; and 3) innovate techniques to overcome barriers.

PATENTS

1. **Title:** "AAV Transduction Vectors"

Inventors: Dr. Nicholas Muzyczka, Dr. Paul Hermonat, Dr. Kenneth I. Berns, and Dr. Richard Jude Samulski.

Ownership: The University of Florida

2. **Title:** “Recombinant Viral Vector System”
Inventors: Dr. Richard Jude Samulski, Dr. Xiao Xiao
Ownership: University of Pittsburgh / NIH
3. **Title:** “Helper Virus-free AAV Production”
Inventors: Dr. Richard Jude Samulski, Dr. Xiao Xiao, Dr. Forrest Ferrari
Ownership: University of North Carolina-Chapel Hill
4. **Title:** “Temperature-sensitive Regulation of Viral Vector Production”
Inventors: Dr. Richard Jude Samulski, Dr. Denise Gavin, Dr. Nicholas Muzyczka,
Dr. Corinne Abernathy, Dr. Daniel Pereira
Ownership: University of North Carolina-Chapel Hill

PATENTS (cont’d)

5. **Title:** “Virus Vectors and Methods of Making and Administering the Same”
Inventors: Dr. Richard Jude Samulski, Dr. Joseph Rabinowitz, Dr. Weidong Xiao
Ownership: University of North Carolina-Chapel Hill
6. **Title:** “Duplexed Parvovirus Vectors”
Inventors: Dr. Richard Jude Samulski, Dr. Douglas McCarty
Ownership: University of North Carolina-Chapel Hill
7. **Title:** “Methods and Compounds for Controlled Release of Recombinant Adeno- Associated Virus Vectors”
Inventors: Dr. Richard Jude Samulski, Dr. Paul Monahan, Dr. Joseph Rabinowitz
Ownership: University of North Carolina-Chapel Hill
8. **Title:** “Methods for Increasing the Efficiency of Recombinant AAV Products”
Inventors: Dr. Richard Jude Samulski, Dr. Xiao Xiao, Dr. Richard Snyder
Ownership: University of North Carolina-Chapel Hill
9. **Title:** “Methods and Formulations for Mediating AAV Attachment and Infection”
Inventors: Dr. Richard Jude Samulski, Dr. Candace M. Summerford
Ownership: University of North Carolina-Chapel Hill
10. **Title:** “Helper Virus Free AAV Production”
Inventors: Dr. Richard Jude Samulski, Dr. Forrest Ferrari, Dr. Xiao Xiao
Ownership: University of NC at Chapel Hill
11. **Title:** “Chimeric Vector”
Inventors: Dr. Richard Jude Samulski, Dr. Dawn Bowles, Dr. Joshua Grieger,
Dr. Chengwen Li, Dr. Mavis McKenna, Dr. Joseph Rabinowitz
Ownership: University of NC at Chapel Hill
University of Florida Research Foundation at Gainesville
12. **Title:** “Heparin and Heparan Sulfate Binding Chimeric Vectors”
Inventors: Dr. Aravind Asokan, Dr. Richard Samulski, Dr. Zhijian Wu
Ownership: University of NC at Chapel Hill
13. **Title:** “Modified Virus Vectors and Methods of Making and Using the Same”
Inventors: Richard Samulski, Aravind Asokan

Ownership: University of NC at Chapel Hill

14. **Title:** “Methods for fabricating isolated micro- or nano-structures using soft or imprint lithography”
Inventors: Dr. Joseph DeSimone, Dr. Jason Rolland, Dr. Benjamin Maynor, Dr. Larken Euliss, Dr. Ginger Dennison Rothrock, Dr. Ansley Dennis, Dr. Edward Samulski, Dr. Richard Jude Samulski.
Ownership: University of NC at Chapel Hill
15. **Title:** Recombinant parvovirus vectors and method of making
Inventors: Dr. Joseph Rabinowitz, Dr. Richard Jude Samulski, Dr. Weidong Xiao
Ownership: University of NC at Chapel Hill

PATENTS (cont’d)

16. **Title:** “Helper-Free Stocks of Recombinant Adeno-Associated Virus”
Inventors: Dr. Thomas Shenk and Dr. Richard Jude Samulski
Ownership: DNX
17. **Title:** “Adeno-Associated Virus Vector and CIS-acting Regulatory and Promoter Elements Capable of Expressing at Least One Gene and Method of Using the Same for Gene Therapy”
Inventors: Dr. Richard Jude Samulski, Dr. Arthur Nienhuis, Dr. Chris Walsh.
Ownership: University of Pittsburgh / NIH
18. **Title:** “Targeted Integration of AAV Vectors”
Inventor: Dr. Richard Jude Samulski
Ownership: University of Pittsburgh
19. **Title:** AAV Capsid Vehicles for Molecular Transfer”
Inventors: Dr. Richard Jude Samulski, Dr. Forrest Ferrari
Ownership: University of North Carolina-Chapel Hill
20. **Title:** “AAV Transduction of Myoblasts”
Inventors: Dr. Richard Jude Samulski, Dr. Xiao Xiao
Ownership: University of North Carolina-Chapel Hill
21. **Title:** “Compositions and Methods for Enhanced Parvovirus Transduction”
Inventors: Dr. Paul Monahan and Dr. Richard Jude Samulski
Ownership: University of North Carolina-Chapel Hill
22. **Title:** “Restrictive Inverted Terminal Repeats for Viral Vectors”
Inventors: Dr. Curtis Hewitt and Dr. Richard Jude Samulski
Ownership: University of North Carolina-Chapel Hill
23. **Title:** “Chimeric Viruses for Gene Therapy”
Inventors: Dr. Richard Jude Samulski, Dr. Joseph Rabinowitz
Ownership: University of North Carolina-Chapel Hill
24. **Title:** “Improved Reagents for Producing and Delivering Adeno-Associated Virus (AAV) Vectors”
Inventors: Dr. Richard Jude Samulski, Dr. Denise Gavin

Ownership: University of North Carolina-Chapel Hill

25. **Title:** “Controlled Release of Recombinant Adeno-Associated Virus Vectors”

Inventors: Dr. Richard Jude Samulski, Dr. Paul Monahan

Ownership: University of North Carolina-Chapel Hill

U.S. Patent / Application No.	Title
60/107,840	ADENO-ASSOCIATED VIRUS VECTORS AND METHODS OF MAKING THE SAME
60/123,651	ADENO-ASSOCIATED VIRUS VECTORS AND METHODS OF MAKING THE SAME
6,491,907 / 09/438,268	RECOMBINANT PARVOVIRUS VECTORS AND METHOD OF MAKING
7,172,893 / 10/205,942	VIRUS VECTORS AND METHODS OF MAKING AND ADMINISTERING THE SAME
11/413,819 (2006-0188484)	VIRUS VECTORS AND METHODS OF MAKING AND ADMINISTERING THE SAME
11/414,319 (2006-0188484)	VIRUS VECTORS AND METHODS OF MAKING AND ADMINISTERING THE SAME
60/043,547	METHODS FOR INCREASING THE EFFICIENCY OF RECOMBINANT AAV PRODUCT
6,548,286 / 09/060,102	METHODS FOR INCREASING THE EFFICIENCY OF RECOMBINANT AAV PRODUCT
7,229,823 / 10/261,188	METHODS FOR INCREASING THE EFFICIENCY OF RECOMBINANT AAV PRODUCT
60/636,126	CHIMERIC VECTORS
7,892,809 / 11/793,430	CHIMERIC VECTORS
9,012,224 / 12/983,547	CHIMERIC VECTORS
60/762,774	HEPARIN AND HEPARAN SULFATE BINDING CHIMERIC VECTORS
7,867,484 / 11/698,505	HEPARIN AND HEPARAN SULFATE BINDING CHIMERIC VECTORS
61/151,736	MODIFIED VIRUS VECTORS AND METHODS OF MAKING AND USING THE SAME
8,889,641 / 13/201,154	MODIFIED VIRUS VECTORS AND METHODS OF MAKING AND USING THE SAME
9,475,845 / 14/543,459	MODIFIED VIRUS VECTORS AND METHODS OF MAKING AND USING THE SAME
61/294,181	RESTRICTIVE INVERTED TERMINAL REPEATS FOR VIRAL VECTORS
9,169,494 / 13/521,448	RESTRICTIVE INVERTED TERMINAL REPEATS FOR VIRAL VECTORS
10,233,428 / 14/922,935	RESTRICTIVE INVERTED TERMINAL REPEATS FOR VIRAL VECTORS
16/271,163	RESTRICTIVE INVERTED TERMINAL REPEATS FOR VIRAL VECTORS
61/301,998	COMPOSITIONS AND METHODS FOR ENHANCED PARVOVIRUS TRANSDUCTION
9,169,492 / 13/575,975	COMPOSITIONS AND METHODS FOR ENHANCED PARVOVIRUS TRANSDUCTION
61/552,492	CELL LINE FOR PRODUCTION OF ADENO-ASSOCIATED VIRUS
9,441,206 / 14/354,634	CELL LINE FOR PRODUCTION OF ADENO-ASSOCIATED VIRUS

61/787,374	SYNTHETIC ADENO-ASSOCIATED VIRUS INVERTED TERMINAL REPEATS
9,447,433 / 14/211,927	SYNTHETIC ADENO-ASSOCIATED VIRUS INVERTED TERMINAL REPEATS
61/802,111	METHODS AND COMPOSITIONS FOR DUAL GLYCAN BINDING AAV VECTORS
10,077,291 / 14/777,070	METHODS AND COMPOSITIONS FOR DUAL GLYCAN BINDING AAV VECTORS
16/102,834	METHODS AND COMPOSITIONS FOR DUAL GLYCAN BINDING AAV VECTORS
16/110,773 (2018-0371024)	METHODS AND COMPOSITIONS FOR DUAL GLYCAN BINDING AAV VECTORS
62/516,449	OPTIMIZED MINI-DYSTROPHIN GENES AND EXPRESSION CASSETTES AND THEIR USE
15/628,268 (2017-0368198)	OPTIMIZED MINI-DYSTROPHIN GENES AND EXPRESSION CASSETTES AND THEIR USE
62/375,666	METHODS AND COMPOSITIONS FOR TARGETED GENE TRANSFER
16/326,126	METHODS AND COMPOSITIONS FOR TARGETED GENE TRANSFER
62/471,762	MODIFIED ADENO-ASSOCIATED VIRUS VECTORS AND METHODS OF MAKING AND USING THE SAME
62/520,901	METHODS AND COMPOSITIONS FOR ADENO-ASSOCIATED VIRUS VECTORS BOUND WITH SURFACE PROTEINS WITH ENHANCED TRANSDUCTION
62/630,558	HAPLOID ADENO-ASSOCIATED VIRUS VECTORS AND METHODS OF MAKING AND USING THE SAME
62/668,056	POLYPLOID ADENO-ASSOCIATED VIRUS VECTORS AND METHODS OF MAKING AND USING THE SAME
62/678,675	RATIONAL POLYPLOID ADENO-ASSOCIATED VIRUS VECTORS AND METHODS OF MAKING AND USING THE SAME
16/051,110 (2018-0371496)	RATIONAL POLYPLOID ADENO-ASSOCIATED VIRUS VECTORS AND METHODS OF MAKING AND USING THE SAME
62/619,468	METHODS AND COMPOSITIONS FOR INHIBITION OF INNATE IMMUNE RESPONSE ASSOCIATED WITH AAV TRANSDUCTION
62/607,662	METHODS AND COMPOSITIONS FOR DELIVERY OF VIRAL VECTORS ACROSS THE BLOOD BRAIN BARRIER
60/071,210	METHODS AND FORMULATIONS FOR MEDIATING AAV ATTACHMENT AND INFECTION, AND METHODS FOR PURIFYING AAV
6,410,300 / 09/228,203	METHODS AND FORMULATIONS FOR MEDIATING ADENO-ASSOCIATED VIRUS (AAV) ATTACHMENT AND INFECTION AND METHODS FOR PURIFYING AAV
6,703,237 / 10/102,314	METHODS AND FORMULATIONS FOR MEDIATING ADENO-ASSOCIATED VIRUS (AAV) ATTACHMENT AND INFECTION AND METHODS FOR PURIFYING AAV
60/208,604	DIMERIC PARVOVIRUS VECTORS
7,465,583 / 10/276,356	DUPLEXED PARVOVIRUS VECTORS
7,790,154 / 11/655,520	DUPLEXED PARVOVIRUS VECTORS
8,361,457 / 12/848,756	DUPLEXED PARVOVIRUS VECTORS
8,784,799 / 13/751,819	DUPLEXED PARVOVIRUS VECTORS
60/208,702	CONTROLLED RELEASE OF RECOMBINANT ADENO-ASSOCIATED VIRUS VECTORS
7,201,898 / 10/276,501	METHODS AND COMPOUNDS FOR CONTROLLED RELEASE OF RECOMBINANT PARVOVIRUS VECTORS
7,923,436 / 11/697,194	METHODS AND COMPOUNDS FOR CONTROLLED RELEASE OF RECOMBINANT PARVOVIRUS VECTORS

62/266,941	MODIFIED CAPSID PROTEINS FOR ENHANCED DELIVERY OF PARVOVIRUS VECTORS
16/061,952	MODIFIED CAPSID PROTEINS FOR ENHANCED DELIVERY OF PARVOVIRUS VECTORS
62/743,317	REGULATED GENE EDITING SYSTEM
61/782,450	MODIFIED SOLUBLE VEGF RECEPTOR-1 GENES AND VECTORS FOR GENE THERAPY
14/770,991 (2016-0009786)	MODIFIED SOLUBLE VEGF RECEPTOR-1 GENES AND VECTORS FOR GENE THERAPY
60/805,171	IMPROVED FACTOR IX GENE AND VECTORS FOR GENE THERAPY
60/847,337	MODIFIED FIX GENE, VECTORS, AND TREATMENT METHODS
8,198,421 / 12/305,508	MODIFIED FACTOR VIII AND FACTOR IX GENES AND VECTORS FOR GENE THERAPY
8,632,765 / 13/494,068	MODIFIED FACTOR VIII AND FACTOR IX GENES AND VECTORS FOR GENE THERAPY
9,506,052 / 14/157,657	MODIFIED FACTOR VIII AND FACTOR IX GENES
62/649,691	LIVER TROPIC RECOMBINANT AAV6 VECTORS THAT EVADE NEUTRALIZATION
62/688,982	VECTORS FOR GENE DELIVERY THAT PERSIST WITHIN CELLS
62/689,453	VECTORS FOR GENE DELIVERY THAT PERSIST WITHIN CELLS
62/697,750	VECTORS FOR GENE DELIVERY THAT PERSIST WITHIN CELLS
62/717,310	VECTORS FOR GENE DELIVERY THAT PERSIST WITHIN CELLS

Clinical Validation: Bench to Bedside

Blog Talk Radio, PharmaTalk Podcast, August, 25, 2020

[PharmaTalk_How a Gene Therapy for Duchenne Muscular Dystrophy Came to Be](#)

National Public Radio, All Things Considered, July 20, 2020

[NPR_All Things Considered_Researchers Hope Experimental Gene Therapy is an Answer to a Fatal Genetic Disorder](#)

Journal of Virology ▪ Volume 73, Issue 11, November 1999

[Charge-to-Alanine Mutagenesis of the Adeno-Associated Virus Type 2 Rep78/68 Proteins Yields Temperature-Sensitive and Magnesium-Dependent Variants](#)

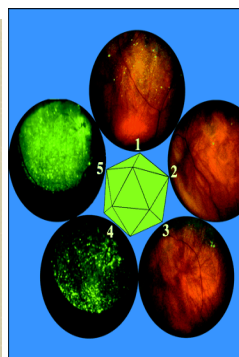
Gavin DK, Young SM Jr, Xiao W, Temple B, Abernathy CR, Pereira DJ, Muzyczka N, **Samulski RJ**



Journal of Virology ▪ Volume 76, Issue 2, January 2002

[Cross-Packaging of a Single Adeno-Associated Virus \(AAV\) Type 2 Vector Genome into Multiple AAV Serotypes Enables Transduction with Broad Specificity](#)

Rabinowitz JE, Rolling F, Li C, Conrath H, Xiao W, Xiao X, **Samulski RJ**

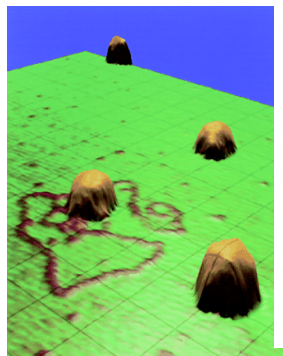


(Copyright © 2002, American Society for Microbiology): Retinal in vivo fluorescence imaging. Wistar rat retinas were imaged by using a Canon UVI retinal camera connected to a digital imaging system (Lhediophwin software). Subretinal injections of recombinant adeno-associated virus serotypes 1 to 5 (rAAV1 to -5), containing the green fluorescent protein (GFP) transgene, were performed via a transscleral transchoroidal approach where the sclera and the choroid were punctured and a 33-gauge needle was then inserted in a tangential direction under an operating microscope. Three microliters of each of the five hybrid rAAV serotypes (5×10^{11} particles/ml) was delivered into the subretinal space. GFP protein expression in live rats at 46 days postinjection is shown. The retina injection with rAAV1 is at the top, and the other serotypes are shown in a clockwise order; these images surround a stylized image of a dodecahedron that represents the AAV virion. (See related article in January 2002: vol. 76, issue 2, p. 791.)

Journal of Virology ▪ Volume 77, Issue 23, December 2003

[Development and Characterization of Novel Empty Adenovirus Capsids and Their Impact on Cellular Gene Expression](#)

Stilwell JL, McCarty DM, Negishi A, Superfine R, **Samulski RJ**



Cover photograph (Copyright © 2004, American Society for Microbiology): Three-dimensionally rendered atomic force microscope image of disrupted and intact adenovirus on native silicon oxide. DNA is uncoiling out of the leftmost virus, while the rightmost virus is intact. (See related article in December 2003: vol. 77, issue 23, p. [12881](#).)

Journal of Virology ▪ Volume 78, Issue 9, May 2004

[Cross-dressing the virion: the transcapsidation of adeno-associated virus serotypes functionally defines subgroups.](#)

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[Surface-Exposed Adeno-Associated Virus Vp1-NLS Capsid Fusion Protein Rescues Infectivity of Noninfectious Wild-Type Vp2/Vp3 and Vp3-Only Capsids but Not That of Fivefold Pore Mutant Virions](#)

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[Enhancement of Adeno-Associated Virus Infection by Mobilizing Capsids into and Out of the Nucleolus](#)

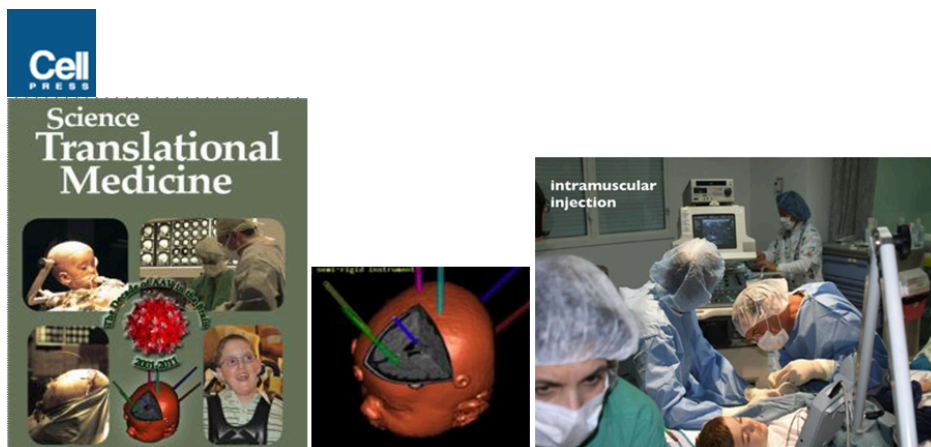
Johnson JS, **Samulski RJ**



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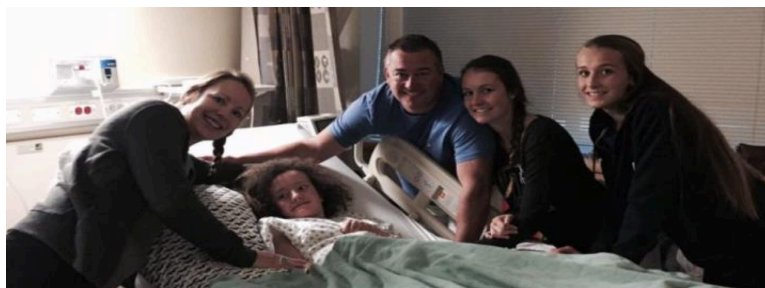
[Phase I Gene Therapy for Duchenne Muscular Dystrophy Using a Translational Optimized AAV Vector](#)

Dawn E Bowles, Scott WJ McPhee, Chengwen Li, Steven J Gray, Jade J Samulski, Angelique S Camp, Juan Li, Bing Wang, Paul E Monahan, Joseph E Rabinowitz, Joshua Grieger, Mavis Agbandje-McKenna, Xiao Xiao, **R Jude Samulski**



Gene Therapy for Giant Axon Neuropathy First Intrathecal delivery of AAV Gene therapy
PLOS Blogs, DNA Science ▪ R Lewis PhD, July 21, 2016

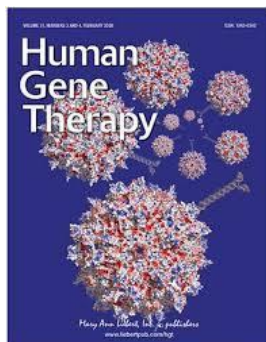
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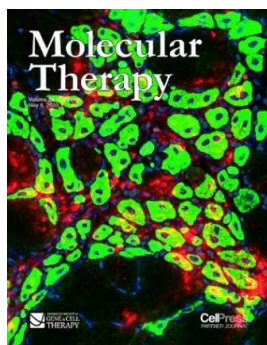
Earley LF, Conatser LM, Lue VM, Dobbins AL, Li C, Hirsch ML, **Samulski RJ**



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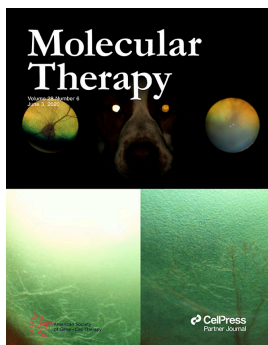
Powell SK, **Samulski RJ**, McCown TJ



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[Intrastromal Gene Therapy Prevents and Reverses Advanced Corneal Clouding in a Canine Model of Mucopolysaccharidosis I](#)

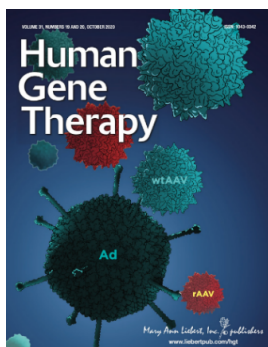
Miyadera K, Conatser L, Llanga TA, Carlin K, O'Donnell P, Bagel J, Song L, Kurtzberg J, **Samulski RJ**, Gilger B, Hirsch ML



Human Gene Therapy ▪ Volume 31, Numbers 19 and 20, October 2020

[Adeno-Associated Virus Vector Mobilization, Risk Versus Reality](#)

Song, L, **Samulski RJ**, Hirsch M



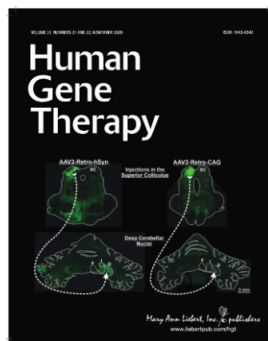
Human Gene Therapy ▪ Volume 31, Numbers 21 and 22, November 2020

- [Adeno-Associated Virus Capsid-Promoter Interactions in the Brain Translate from Rat to the Nonhuman Primate](#)

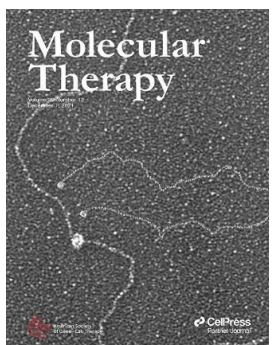
Bohlen MO, McCown TJ, Powell SK, El-Nahal H, Daw T, Basso MA, Sommer MA, **Samulski RJ**

- [Bound Protein- and Peptide-Based Strategies for Adeno-Associated Virus Vector-Mediated Gene Therapy: Where Do We Stand Now?](#)

Zhang X, Chai Z, **Samulski RJ**, Li Chengwen



Molecular Therapy ▪ Volume 29, Number 12, December 2021

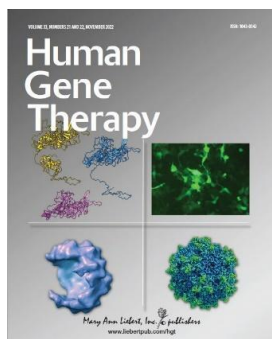
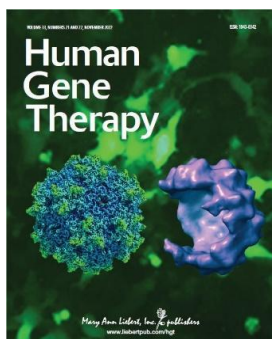


Cover photograph: Electron micrograph of DNA fragments from Chromosome (Ch)19 (larger strand) and AAV vector DNA (smaller strand) complexed with Rep 68kd recombination proteins. Image illustrated Rep binding to site specific integration site (globular structure in middle of fragment) located on q13.4 of Ch 19 and selectively to the AAV.

Human Gene Therapy ▪ Volume 33, Numbers 21 and 22, November 2022

[Cellular and structural characterisation of VP1 and VP2 knockout mutants of AAV3B serotype and implications for AAV manufacturing](#)

Arriaga I, Navarro A, Etxabe A, Trigueros C, **Samulski RJ**, Moullier P, François A, Abrescia NGG



Human Gene Therapy ▪ Volume 33, Numbers 21 and 22, November 2022

Charitable/Philanthropic Activities

[Columbus Children's Foundation](#)

R. Jude Samulski

Founder, Board Chair and Chief Scientific Officer

[Columbus Children's Foundation and CureSPG50 Announce Manufacturing Partnership for AAV Gene Therapy](#)

<https://columbuschildren.org/columbus-childrens-foundation-and-phoenix-nest-bio-partner-to-accelerate-gene-therapy-for-sanfilippo-syndrome-type-c/>

STAT ▪ February 17, 2021

[Ultra-rare But Not Forgotten New Drug Development Paradigms to Treat the Rarest of Diseases](#)

Ramesh SK, Bronstein MG, **Samulski RJ**

Nonprofit biotechnology companies developing treatments for rare diseases

Phoenix Nest <http://www.phoenixnestbiotech.com/>

Rain Bio -- Developing cure for MPS I related blindness <https://rainbio.us/>

Nonprofit organizations

Columbus Children's Foundation <https://columbuschildren.org/>
Founder, Board Chair and Chief Scientific Officer

Muscular Dystrophy Association (MDA) <https://www.mda.org/>

SLC6a1 Connect <https://slc6a1connect.org/>

Cure SPG50 <https://www.curespg50.org/>

Cure Rare Disease <https://cureraredisease.org/>

Rare Trait Hope Fund <http://www.raretrait.com/>

The Progeria Research Foundation <https://www.progeriaresearch.org/our-story/>

Patient Advocacy Letter

November 15, 2020

Dr. Peter Marks
Center for Biologics Evaluation and Research, FDA

Dr. Chris Austin
National Center for Advancing Translational Sciences, NIH

Dr. PJ Brooks
Office of Rare Diseases Research, NCATS, NIH.

Mr. Steve Hoffman
Foundation for the National Institutes of Health

Dear Dr. Marks, Dr. Austin, Dr. Brooks, and Mr. Hoffman,

As parents/family of children diagnosed with devastating genetic rare diseases so rare that they have no known cure, life became focused on what we could do to help our child/family member and all the other children with similar diseases. We are some of the many families who did not take our doctors suggestion that we go home and enjoy our children while we have them. Instead, against the obstacles, we decided to fight for our children and others like them. We have met amazing individuals and institutions along the way. We have also appreciated the openness that you all speak with parents like us in navigating the unknowns as we try to figure out the most efficient pathway to a safe curative gene therapy treatment for our children.

It is our understanding that FDA and FNIH are working on a program that would seek to assist small ultra-rare disease programs that lag behind due to lack of industry investment. This is certainly welcome attention. Most rare diseases and especially ultra-rare diseases suffer commercial non-viability for therapeutic developments and as such, investment in these programs is challenging to secure. As you are aware, the path is unclear and inefficient in many cases and the significant hurdles associated with the cost of manufacturing and lack of scale-up and CMC expertise poses a real challenge for programs like ours. As small disease specific foundations that have promising data and tremendous advisors, but do not have years of expertise and experience that could streamline and speed the development of our desired treatments.

In that light, we want to bring to your attention some amazing work that is being done by the Columbus

Children's Foundation which has built an accelerator leveraging pioneers in the industry combined with access to lower cost manufacturing using a platform approach that is built upon years of experience and expertise to help us to move our programs forward with greater speed and lower costs than we expected. What we needed, they helped us understand and move forward. They have become invaluable partners as we advance our programs. In fact, surprisingly, they have moved 3 ultra-rare programs into manufacturing over the last 2 months alone and have a number of others in the queue followed by a remarkably long pipeline of programs like ours seeking their assistance. It is amazing that this small organization can move with such urgency, action, and capability. They have helped us to leap the significant hurdles we face quickly, professionally, and with empathy and understanding.

We have each had various experiences with industry as we advanced each of our programs. We have had manufacturers with no experience willing to help us, but we are reticent to help them learn on the backs of our children. We have other manufacturers willing to give us a GMP slot should one become available, but these are generally based on very limited capacity availability which makes planning challenging and uncertain. We have manufacturers willing to secure a slot at commercial pricing with no financial consideration for the ultra-rare disease nature of our therapy which makes it unaffordable and impossible in today's fundraising market.

Through the direct support of their CSO and Chairman, Dr. Jude Samulski, their Executive Director, Laura Hameed, and their team of experts; Columbus Children's Foundation stepped in to help us quickly get past all these hurdles with a hands-on facilitation and efficient and cost-effective approach. Our urgency is their urgency.

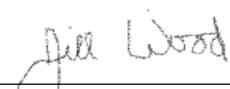
Patient Advocacy Letter (cont'd)

The model for acceleration for programs like ours is out there and it is already working with success. We are thrilled to have created a partnership with CCF that has benefitted our programs and ultimately will benefit the rare and special children for whom all this work is being done. We are writing today to encourage you to support and invest in their efforts so more families can receive the help we have received.

During our travels, attendance at conferences and conversations with other parents and patient organizations, we know CCF is filling a significant market gap and we also know that the degenerative nature of these diseases makes their openness and quick action a key part of saving lives. Anything you can do to support this effort that is already showing results in accelerating treatments for children like our children would be support well placed. Time is life for children that have ultra-rare diseases and these kids -- our kids -- cannot wait.

Thank you for all your efforts!

Yours Sincerely,



Jill Wood
Phoenix Nest



Rich Horgan
Cure Rare Disease



Terry Pirovolakis

Amber N. Freed

Amber Freed
SLC6a1 Connect



Julia Taravella
AGU – Rare Trait Hope Fund