Terence R Flotte, M.D.
Celia and Isaac Haidak Professor
Dean, Provost and Executive Deputy Chancellor
Chief Research Officer
Professor, Department of Pediatrics, Horae Gene Therapy Center
and Microbiology and Physiological Systems (MaPS)
University of Massachusetts Chan Medical School
55 N Lake Avenue
Worcester, MA 01655
[redacted]

EDUCATION

M.D., Louisiana State University School of Medicine, New Orleans, LA/USA — 05/17/1986

Awards: Chancellor's Award (rank #1 in class), Alumni Assn. Osler Award, George S. Bel Memorial Award, Russell Holman Memorial Award in Pathology, Garret J. Buddingh Memorial Award in Microbiology, Greater New Orleans Pediatric Society, Member of Alpha Omega Alpha (AOA) Honor Society, Member of Aesculapian Society, Vaughn Memorial Scholarship, Philip H. Jones Memorial Scholarship, Michael McDonald Memorial Microscope Award, Gold probe Award in Medical Gross Anatomy, Carl F. Tucker Award in Sophomore Pathology.

B.S., Summa Cum Laude in Biological Sciences, University of New Orleans, New Orleans, LA/USA — 05/21/1982

Awards: Summa Cum Laude, College of Sciences Dean's Award, Biological Sciences Faculty Award, Phi Beta Kappa Club Award, Phi Kappa Phi Club Award, Decennial Honor Award, Louisiana Land and Exploration Company Scholarship, Biological Sciences Achievement Award, Dean's List in the College of Sciences, Freshman Chemistry Award, National Merit Scholarship.

POSTDOCTORAL TRAINING

Pediatric Internship, The Harriet Lane Pediatric House Staff of the Johns Hopkins Hospital, JHU, Baltimore, MD — 07/01/1986-06/30/1987

Pediatric Residency, The Harriet Lane Pediatric House Staff of the Johns Hopkins Hospital, JHU, Baltimore, MD — 07/01/1987-06/30/1989

Pediatric Pulmonary and Cystic Fibrosis Foundation Fellowship, Eudowood Division of Respiratory Sciences, Johns Hopkins University, Baltimore, MD — 07/01/1989-06/30/1992

Postdoctoral Fellow/Scholar — 07/01/1989-06/30/1992
Supervisor: Barrie Carter Ph.D.
National Institute of Diabetes and Digestive and Kidney Diseases, NIH, Bethesda, MD

ACADEMIC APPOINTMENTS

Endowed Chair — 2010-present
Celia and Isaac Haidak Professor of Medical Education, University of Massachusetts Chan Medical School, Worcester, MA

Professor with Tenure — 05/10/2007-present
Department of Pediatrics and Microbiology and Physiological Systems
T.H. Chan School of Medicine, UMass Chan Medical School, Worcester, MA

Updated: 12/20/2021

Professor and Chair Nemours Eminent Scholar, Department of Pediatrics and Department of Molecular Genetics and Microbiology, College of Medicine, University of Florida, Gainesville, FL	07/01/2002- 04/14/2007
Associate Professor Pediatrics and Molecular Genetics and Microbiology, University of Florida, College of Medicine, University of Florida, Gainesville, FL	08/06/1998- 06/30/2001
Assistant Professor Pediatrics and Molecular Genetics and Microbiology, College of Medicine, University of Florida, Gainesville, FL	07/01/1996- 08/06/1998
Assistant Professor Pediatrics, Johns Hopkins University, School of Medicine, JHU Hospital, Baltimore, MD	07/01/1993- 06/30/1996
Instructor Pediatrics, Johns Hopkins University, School of Medicine, JHU Hospital, Baltimore, MD	07/01/1992- 06/30/1993

MAJOR INSTITUTIONAL LEADERSHIP POSTIONS

Dean Provost & Executive Deputy Chancellor T.H. Chan School of Medicine, University of Massachusetts Chan Medical School, Worcester, MA	05/10/2007- present
Chief Research Officer University of Massachusetts Chan Medical School, Worcester, MA	07/01/2010- present
Chair Department of Pediatrics, College of Medicine, University of Florida, Gainesville, FL	11/04/2000- 10/01/2002
Director University of Florida Genetics Institute, University of Florida, Gainesville, FL	11/04/2000- 10/01/2002
Director Powell Gene Therapy Center, College of Medicine, University of Florida, Gainesville, FL	11/04/2000- 08/01/2002
Co-Director Powell Gene Therapy Center, College of Medicine, University of Florida, Gainesville, FL	07/01/1996- 11/03/2000
Interim/Founding Director University of Florida Genetics Institute, University of Florida, Gainesville, FL	07/01/1996- 11/03/2000

MAJOR NATIONAL LEADERSHIP POSITIONS

President	2025-2026
President-Elect	2024-2025
Vice President	2023-2024
Secretary American Society of Cell & Gene Therapy (ASGCT)	2019-2023
Council of Deans Administrative Board Member	2021-2025
Council of Deans Liaison to Group on Women in Medicine and Science (GWIMS) The Association of American Medical Colleges (AAMC)	2016-2021
Member Liaison Committee on Medical Education (LCME)	2013-2016

Steering Committee Member 2013-present
Steering Committee Chair 2018-present
NHLBI Gene Therapy Resource Program (GTRP)

Editor-in-Chief 2013-present
Human Gene Therapy

HONORS and AWARDS

1. See the Light Award, Matthew Forbes Romer Foundation 03/16/2019
2. Alumnus of the Year 2017, College of Sciences University of New Orleans 11/02/2017
3. The Worcester District Medical Society 221st Annual Society's Orator 02/08/2017
4. Gold Humanism Honor Society Membership, inducted 01/06/2013
5. Johns Hopkins Society of Scholars, inducted 05/11/2013
6. Massachusetts Society for Medical Research, Investigator Award 2012
7. Celia and Isaac Haidak Professorship (Endowed Chair) 2010-present
8. National Institutes of Health College of CSR Reviewers 2010-2012
9. Society for Pediatric Research E. Mead Johnson Award for Outstanding Scientific Contributions 2005
10. Association of American Physicians Member 2010
11. American Pediatric Society/Society for Pediatric Research Member 2005
12. Permanent Charter Member NIH Study Section: Gene Drug Delivery Systems 2004-2008
13. American Board of Pediatrics, Sub-board on Pulmonology Member 2003-2008
14. Alpha Omega Alpha Honor Medical Society, Elected as Jr Medical Student 1985

EDUCATIONAL ACTIVITIES

Teaching Activities in Programs and Courses

1. Principles of Human Genetics, Applied Genetics, UMass Chan Medical School, School of Medicine
2. Integrated Clinical Experiences, UMass Chan Medical School, School of Medicine
3. Development, Structure, & Function (DSF), Respiratory Physiology, UMass Chan Medical School, School of Medicine
4. Optional Enrichment Elective (OEE): Leadership in Medicine, UMass Chan Medical School, School of Medicine
5. American Board of Pediatrics, Sub-board on Pulmonology

Graduate Students (Trainees under my primary oversight)

PhD Graduate Students

1. Alisha Gruntman DVM, PhD DACVIM (PhD Student, mentee) Assistant Professor, University of Massachusetts Medical School, Pediatrics, Gene Therapy Center, UMass Chan Medical School, Worcester, MA and Assistant Professor, Large Animal Internal Medicine, Tufts University, Cummings School of Veterinary Medicine, N Grafton, MA 2011-2016
2. Allison Keeler-Klunk, PhD (PhD student, mentee) Associate Professor, Pediatrics, Gene Therapy Center, UMass Chan Medical School, Worcester, MA 2008-2012

3. Jared Silver MD PhD (MD-PhD student) Allergy and Immunology, Brigham and Women's Hospital Allergy Clinic, Chestnut Hill, MA 2002-2009
4. Ashley Martino PhD (PhD student) Assistant Professor, Pharmaceutical Sciences, St. John's University, New York NY 2002-2006
5. Kevin Foust PhD (PhD student) Senior Director of R&D, Avexis, San Diego, CA 1999-2007
6. Christian Mueller PhD (PhD student, Post-doc, Parker B Francis Fellowship Program mentee) Associate Professor of Pediatrics/Gene Therapy UMass Medical School, Worcester; Chief Scientific Officer Apic Bio, Inc., Cambridge MA 1999-2006
7. Thomas Conlon, PhD (PhD student and post-doc) Chief Scientific Officer of the Michelson Found Animals Foundation and Chief Executive Officer of CR Scientific and Compliance Consulting (CRSci), Gainesville, FL 1998-2004
8. Jeffrey R Sirninger, PhD, DVM, (PhD Student) Sirninger Cytologic Diagnostics, Cytologic Consultation Service, West Palm Beach, FL 1998-2003
9. Renius Owen, IV PhD (PhD Student) Sr. Scientific Director, Advanced Diagnostics, Quest Diagnostics, Orange County, CA 1996-2001
10. Sandra Afione PhD (Grad Student, then Post-doc) Tenure Track position, NIH (NICDR) – Bethesda MD 1990-1996

MS Graduate Students

1. Heather Loring, BS (post-baccalaureate mentee), BS student, Trinity College, Hartford, CT 2017
2. Sofia A Mueller MS, MBA (MS student) Corporate Alliance Manager - Innovation & Business Development, UMMS Worcester 2004-2005
3. Kirsten Erger Coleman MBA (MBA student) Manager of Research and Development, University of Florida, Gainesville FL 2004
4. Joe Hernandez MBA, MSC Biostatistics (MS/MBA Student) Chairman of the Board, Blue Water Vaccines, Inc., Founder, Microlin Bio Inc, NY, NY, Founder & Chairman of Board Ember Therapeutics, Inc. (Mariel Therapeutics, Inc.), Boston MA, Executive Chairman of Sydys Corp and Founder, Board Member at Prolias Technologies, Inc 1997-1998

Graduate Students (Advisory Committee Member)

1. Caitriona McLean, Respiratory Research Division, Special External Examiner PhD Committee, Royal College of Surgeons in Ireland, National University of Ireland.
2. Linzy Henrickson, GSBS Neuroscience Program, TRAC Committee, University Texas, Institute for Neuroscience,
3. Joanna Chaurette, MD/PhD Neuroscience Program, MD/PhD Program In Biomedical Sciences TRAC Committee
4. Seemin Ahmed, GSBS Interdisciplinary Graduate Program, TRAC Committee
5. Cara Weismann, GSBS Interdisciplinary Graduate Program, TRAC Committee
6. Gabriela Toro, GSBS Interdisciplinary Graduate Program, Chair Qualifying Exam Committee
7. Lorelei Stoica, GSBS, Interdisciplinary Graduate Program, TRAC Committee
8. Leticia Fridman, GSBS, Neurobiology Program, TRAC Committee

9. Abhishek Satishchandran, MD/PhD, MD/PhD Program In Biomedical Sciences TRAC Committee
10. Reka A Haraszti, MD, GSBS Program, TRAC Committee
11. Amanda Mary Dudek, PhD Program in Virology, Harvard University, Dissertation Defense Committee
12. Dominic Gessler MD, Millennium Ph.D. Program, Chair Qualifying Exam Committee
13. Heather Loring, GSBS Biochemistry & Molecular Pharmacology Program, TRAC Committee

Postdoctoral Trainees (Trainees under my primary oversight)

1. Rejean Liqun Wang PhD (post-doc) Senior Scientist Alpha-1 Research Program, University of Florida — 2004
2. Stuart Beattie PhD (Post-doc) Senior Manager, Regulatory Affairs, Maidenhead, Berkshire, UK. — 2003-2008
3. Scott Loiler PhD (Post-doc) Chief Technology Officer Apic Bio, Inc, Cambridge, MA — 1997-2006
4. Sihong Song PhD (Post-doc) Associate Professor, Dept. of Pharmaceutics, University of Florida, Gainsville, FL. — 1996-2001
5. Suzanne Beck MD (Post-doc) Professor of Clinical Pediatrics, Perelman School of Medicine at the University of Pennsylvania/Children's Hospital of Philadelphia, Philadelphia PA. — 1995-1996
6. Carol Conrad MD (Post-doc) Associate Professor of Pediatrics, Stanford University, CA — 1992-1995

K Award Mentoring

1. Mai Elmallah, MBBCh (K-award and Parker B Francis Fellowship Program mentee) Associate Professor of Pediatrics, Duke University School of Medicine, Durham, NC — 2014-2017
2. Michael Stalvey, MD (K-award mentee) Associate Professor in Pediatric Endocrinology, Assoc. Dir. for Research, Fellowship Program Director, UAB — 2007-2010
3. Daniel A. Salmon, PhD (K-award mentee) Director, Institute for Vaccine Safety, Professor, Johns Hopkins Bloomberg School of Public Health, Baltimore MD — 2005-2007
4. Isabel Virella-Lowell, MD (K-award mentee, Fellow) Professor, Co-Director CF Therapeutics Development Network Center, Co-Director Airways Clinical Research Center, Co-Director Pediatric Pulmonary Clinical Research Program, Medical Director of 10Q Pediatric Pulmonary Care Unit, University of Alabama at Birmingham Department of Pediatrics, School of Medicine, Birmingham, AL — 1998-2002

INVESTIGATION

Human Gene Therapy Expanded Access Use Study:

Expanded Access Use Of AAVrh8 Vectors Encoding Hexa And Hexb Delivered Intrathecally For Tay-Sachs Or Sandhoff Disease: *first-in-humans: Primary PI*. The primary objective of this study was to provide compassionate use of virally delivered HEXA and HEXB genes (rAAVrh8-HEXA/B) to one participant with Tay-Sachs disease (patient was dosed 11/2018). Two monocistronic AAVrh8 vectors encoding HEXA and HEXB separately were mixed in a 1:1 formulation and delivered into the cerebral spinal fluid via intrathecal administration under fluoroscopic guidance. Studies in animal models supported the safety and moderate efficacy of this delivery approach.

Phase I-II trial of Axo-AAV-GM2: A Two Stage, Dose-Escalation and Safety & Efficacy Study of Bilateral Intraparenchymal Thalamic and Intracisternal/Intrathecal Administration of AXO-AAV-GM2 in Tay-Sachs or Sandhoff Disease. Nine patients have been enrolled. The vector is given in two consecutive days. On Day 1, participants will receive bilateral intraparenchymal infusions of AXO-AAV-GM2 in the thalamus (BiTh). On Day 2, participants will receive ICM/IT infusion of AXO-AAV-GM2 into the CSF. The infusions are comprised of a 1:1 mixture of rAAVrh8-HEX A and rAAVrh8-HEXB. The primary objective is to assess the safety and tolerability of the treatment and the secondary objective is to identify the optimal dose for treatment. Exploratory objectives are assessment of the impact of the treatment on 1) clinical functions related to development and neuromotor function, 2) biomarkers of disease activity, 3) neurodegenerative and myelination imaging biomarkers and 4) peripheral and central nervous system integrity.

Human Gene Therapy Clinical Trials:

rAAV2-sFLT01 Phase I intravitreal injection: co-PI (first subject enrolled 2010) As Dean at UMMS, I encouraged the initiation of this trial by the R&D division of Genzyme. I have subsequently served as the nominal PI as the primary ophthalmologist (Dr. Kaushal), and surgeon (Dr. Barsamian) left UMMS. I have continued to oversee all of the on-site clinical aspects (recruitment, IRB submissions, Investigational pharmacy, IBC submissions, etc.) for the trial.

rAAV1-AAT Phase IIa intramuscular: Primary PI (first subject enrolled 2010). This was the next phase of development of the product mentioned in #4. This studied served both as a further dose escalation and as a bridge between rAAV1 cGMP material produced by transfection and that produced with a new scalable herpes virus-based system. Funding was again shared between my R01 and AGTC. I was the PI of the clinical protocol and senior author on the resulting publications, including a recent high impact paper in *JCI* release on line November 2013.

rAAV2-rpe65 Phase I subretinal injection: co-PI (first subject enrolled 2007). This was a study in which AGTC was the sponsor, and I assisted at several stages with the preclinical studies, the development of cGMP production, and outline of the clinical trial. The study enrolled subjects both at UF and at UMMS after I moved here in 2007.

rAAV1-AAT Phase I intramuscular: *first-in-humans: Primary PI* (first subject enrolled 2006). This was the first in a series of studies partnered with Applied Genetic Technologies Corporation (AGTC) a company in Alachua, Florida, for which I was one of 5 co-founders (with R. Jude Samulski, Nicholas Muzyczka, Barry Byrne, and William Hauswirth). AGTC was the sponsor of record on the rAAV1-AAT IND, but there was substantial shared funding with my NHLBI R01 (HL69877), and the NHLBI Gene Therapy Resource Program for both the preclinical portions and the clinical trial. I performed portions of the preclinical package, assisted in the IND preparation, and was the PI of the clinical trial and senior author on the *PNAS* paper resulting from the work.

rAAV2-AAT Phase I intramuscular: *first-in-humans: Primary PI and Sponsor* through University for physician IND (first subject enrolled 2003). A major portion of the work was funded through another NHLBI P01 subproject (different P01 from #1), and a subsequent "spin-off" R01 (HL69877, which has been funded in my lab since 2003). As Director of the Powell Gene Therapy Center at UF, I hired Dr. Richard Snyder to bring the UF cGMP facility (5th floor McKnight Brain Institute) into operation and produce the material for both the formal GLP toxicology and the phase I clinical trial. I also applied for (as PI) a grant to become one of two NCRR-funded toxicology centers in the National Gene Vector Laboratory (NGVL) program. This entailed creating a full GLP-compliant molecular toxicology core in the basement of the Academic Research Building at UF. In that lab, we completed the GLP toxicology and biodistribution studies supporting the rAAV1-AAT IND submission. I completed the Preclinical Section, the Clinical Trial section, and worked with Dr. Snyder on the CMC. The University was listed as the Sponsor of Record, but I was the designated contact.

rAAV2-CFTR Phase I sinus: co-PI (first subject enrolled 1996). This study was performed at Stanford by John Wagner. I supplied much of the preclinical data in support of the trial (which was in fact the same studies supporting trial 1).

rAAV2-CFTR Phase I nasal/bronchial: *first-in-humans: Primary PI* (first subject enrolled 1995) This project was initiated on the NHLBI P01 funded at Johns Hopkins in 1993 for Cystic Fibrosis Gene Therapy. I was the PI of the subproject addressing the completion of the preclinical toxicology and phase I clinical trial. The IND was subsequently filed with Targeted Genetics Corporation as the Sponsor during an early phase of start-up of the company. I performed nearly all of the preclinical safety and toxicology studies that went into the preclinical portion of the IND, and I contributed heavily to the CMC and Clinical Trial part of the package and

was the primary PI of the phase I trial as it began at Johns Hopkins and then transitioned to University of Florida (UF). I was senior author on several resulting publications.

GRANTS

Current

1. 2UL1TR001453-05A1 (Luzuriaga, PI) 6/30/2020-5/31/2025
   NIH/NCATS
   University of Massachusetts Center for Clinical and Translational Science (Linked award)
   The major goal of this project is to facilitate greater efficiency and productivity of University of Massachusetts investigators by enhancing engagement in clinical research, training a new generation of researchers, and building an academic home for moving laboratory discoveries into treatment for patients.
   Role: CO-PI/CO-Director

2. National Institutes of Health/ NHLBISP01HL158506-02 8/9/2020-7/31/2026
   (PI: Flotte TR)
   Title: Models and Gene Therapies for AAT Deficiency
   Overall – Project Narrative in this project, we will develop new gene therapy drugs to treat genetic emphysema due to alpha-1 antitrypsin deficiency (AATD), a common genetic disease and form of chronic obstructive pulmonary disease (COPD). In the Proposal, we will use advanced gene editing tools to create genetically defined animal models of AATD (known as transgenic animals), both in mice and in ferrets, which are a good model to study lung diseases. In the course of the study we will use the most cutting-edge tools in gene therapy, gene editing, and immune modulation to treat AATD in both animal models and determine the best ways to measure the success of gene therapy over time.
   $1,669,445 Direct Costs/year ($2,676,698 Total Costs/Year)

Completed

1. NIH/NIDDK 5R01DK098252-05 01/01/2019-12/31/2022 01/01/2019-12/31/2022
   (PI: Flotte TR)
   Title: Nuclease free gene editing approaches to treat alpha-1 antitrypsin disease
   The major goa s to exp ore non-nuc ease dependent gene ed t ng strateg es for ver d rected correct on of A1AT.

2. Applied Genetic Technologies Corp.
   (PI: Flotte TR)
   Title: A bridging study of rAAV1-AAT delivery using regional limb perfusion in AAT-deficient adults 01/01/2018-12/31/2020
   The goa s to manufacture c n ca tr a mater a for treat ng a pha-1 def c ency.
   $750,000 Direct Costs

3. National Institutes of Health /NHLBI1P01HL131471-03 08/01/2016-04/30/2021
   (PI: Flotte TR)
   Title: New Approaches to Gene Therapy for Alpha-1 Antitrypsin Deficiency
   The overa goa of th s trans at ona program s to deve op a def n t ve mo ecu ar therapy for ung d sease due to a pha-1 ant tryps n (AAT) def c ency, a re at ve y common s ng e gene d sease due to mutat ons n the AAT gene.
   $1,412,772 Direct Costs/year ($2,346,142 Total Costs/Year)

4. Applied Genetic Technologies Corp. 01/01/2016-01/01/2018
   (Flotte, PI)
   Title: AAV1 & AAV8 Rhesus AAT c-myc study
   $44,345 Direct Cost

5. Applied Genetic Technologies Corp. 01/01/2016-
(Flotte, PI) 01/01/2018
Title: AAV1 & AAV8 Rhesus AAT c-myc study
$44,345 Direct Cost

6. Voyager Therapeutics
(PI: Flotte TR) 08/01/2015-
Title: Induction of AAV capsid-specific regulatory T cells to enhance rAAV persistence 07/31/2017
Th s project w use ch mer c ant gen receptor-T ce (CAR-T) techno ogy to exper menta y man pu ate the T ce responses to AAV caps d ep topes n the context of rAAV gene therapy. We w eng neer T ce receptors w th spec f c ty to a common AAV caps d ep tope to conf rm that effector T ce s (Teff) w th AAV caps d spec f c ty w m t the durat on of rAAV transgene express on and that regu atory T ce s (Treg) w th AAV caps d spec f c ty w pro ong t.
$62,500 Direct Costs/year

7. MA Lions Eye Research Fund
(PI: Flotte TR) 08/28/2014-
Title: Ocular Gene Therapy for Cockayne Syndrome 08/31/2016
The major goa s of th s project are to f nd an opt ma serotype and de very route to target rAAV to the ret na, and to deve op a gene therapy approach to correct the ret na degenerat on seen n the Cockayne Syndrome mouse mode s n order to preserve v s on.
$15,333 Direct Costs/year

8. National Institutes of Health (NIDDK) R01- DK098252-03 05/27/2013-
(Multi-PI: Flotte) 04/30/2018
Title: Dual-Function vectors for in vivo gene therapy of AAT Liver disease
The major goa s are to deve op better mode s of AAT-re ated ver d sease us ng env ronmenta cha enges and human stem ce der ved ver ch meras, and further to test a comb ned gene therapy/RNA nh b t on strategy as a potent a future therapy
$217,500 /year Direct Cost ($364,313/year Total Cost)

9. National Institutes of Health /NCATS 5 UL1TR000161-05 07/01/2010-
(PI: Luzuriaga) (Co-PI: Flotte TR) 09/30/2015
Title: University of Massachusetts Center for Clinical and Translational Science
(Linked award)
The major goa of th s project s to fac tate greater eff c ency and product v ty of Un vers ty of Massachusetts nvest gators by enhanc ng engagement n c n ca research, tra n ng a new generat on of researchers, and bu d ng an academ c home for mov ng aboratory d scover es nto treatment for pat ents.
$2,419,808 Direct Costs/year ($3,387,443 Total Costs/Year)

10. Genzyme 12/01/2009-
(PI: Flotte TR) 12/31/2017
Title: A Phase 1, Open-Label, Multi-Center, Dose Escalating, Safety and Tolerability Study of a Single Intravitreal Injection of AAV2-sFLT01 in Patients With Neovascular Age-Related Macular Degeneration
$25,000

11.Luke O'Brian Foundation 09/2009-
(Project PI: Dr. Flotte) 09/2011
Title: Gene Therapy for Cockayne Syndrome
$52,000 / year ($104,000 total – no IDC)

12. Alpha 1 Foundation 07/2008-
(PI: Flotte) 06/2011
Title: Gene therapy for AAT liver disease

$65,000/year DC ($130,000 total - no IDC)

13. Applied Genetic Technologies Corp (AGTC) 07/28/2008-06/30/2017
(PI: Flotte TR)
Title: Preclinical & Phase I Trials of AAV-AAT Vectors: Phase I Trial of Intramuscular Injection of a Recombinant Adeno-Associated Virus Alpha1-Antitrypsin (rAAV1-CB-hAAT) Gene Vector to AAT-Deficient Adults
$0 /year ($38,359 Total Cost to date)

14. Cystic Fibrosis Foundation Special Research Grant 09/2006-08/2007
(Project PI: Flotte)
Title: Non-human primate model for comparison of rAAV serotypes
$138,000 onetime award

15. Cystic Fibrosis Foundation 04/2004-03/2006
(Project PI: Flotte)
Title: Anti-Inflammatory Gene Therapy in CF, Special RFA for Cystic Fibrosis Airway Infection and Inflammation.
$125,000/year

16. National Institutes of Health (NHLBI) 1-R01-HL69877 04/2003-06/2015
(PI: Flotte)
Title: Preclinical & Phase I/II Trials of AAV-AAT Vectors
$250,000/year Direct Cost ($2,039,235 total Direct Cost (DC)/Indirect cost (IDC)).

17. National Institutes of Health (NEI) U10 EY13729 (Director: Hauswirth; Module 6A) 01/2003
(Project PI: Flotte)
Title: Gene Therapy for Congenital Leber's Amaurosis
Prec n ca Tox co ogy Stud es, *Transfer PI Role to Barry J. Byrne M.D. Ph.D. - March 2005*
5-year $200,000 Direct Cost per year to Module 6A

18. National Institutes of Health (NCRR) U42 RR16586 01/2003
(Project PI: Flotte when awarded, then transferred to Byrne in mid-cycle)
Title: National Gene Vector Laboratory Toxicology Core Center for Gene Therapy
Permission requested and granted to transfer PI Role to Barry J. Byrne M.D. Ph.D. - March 2005
$750,000 Direct Cost per year. 5-year

19. Alpha One Foundation/Fernandez Liver Disease Award, 11/01/2000-11/01/2002
(Project PI: Flotte)
Title: Molecular Therapies for alpha 1-antitrypsin liver disease
$100,000/year

20. Juvenile Diabetes Research Foundation (JDRF) Program Project Award 10/2000-09/2005
(Project PI: Flotte)
Title: The JDFI Gene Therapy Center for the Prevention of Diabetes and Diabetic Complications at the University of Florida, Subproject 4 "Enhanced AAV Vectors
$166,000/year Direct Costs

21. National Institutes of Health (NIDDK) P01-DK58327 08/2000-07/2011
(PI: Flotte; PPG Director: Flotte/Byrne)
Title: Transduction of Hepatocytes with Recombinant AAV for Correction of Genetic and Metabolic Abnormalities
$98,146/year Direct Cost ($402,312 total DC/IDC)

22. Cystic Fibrosis Foundation 07/01/2001-06/30/2005
(Project PI: Flotte)
Title: Immuno-Modulatory Adeno-Associated Gene Therapy for Hyper IgE-Mediated Lung Inflammation in Cystic Fibrosis

23. Alpha One Foundation Young Investigator Award 07/01/1999-06/30/2001
(Project PI: Flotte, Fellow: Sihong Song)
Title: Adeno-associated virus (AAV) vectors for skeletal muscle mediated gene therapy for alpha 1-antitrypsin (AAT) deficiency: Preclinical study in non-human primates
$25,000/year

24. Juvenile Diabetes Research Foundation (JDRF) 04/01/1999-03/30/2001
(P.I. = Mark Atkinson; Co-PI = Flotte)
Title: Cytokine-mediated gene therapy for type I diabetes mellitus
$98,500/year

25. National Institutes of Health/National Gene Vector Laboratory, 10/1999
(Project PI: Flotte)
Title: Production and Distribution of a recombinant AAV reference standard stock
$75,000

26. National Institutes of Health (NHLBI) P01-HL59412 09/1998-09/2013
(Project PI: Flotte; PPG Director: Muzyczka)
Title: Gene Therapy for Lung and Cardiovascular Disease"/PPG "AAV vector delivery to skeletal muscle as a platform for therapeutic protein delivery/Project 2
$246,800/year Direct Costs, ($2,005,250 total DC/IDC)

27. Cystic Fibrosis Foundation Research Grant 09/1998-09/1999
(Project PI: Flotte)
Title: Adeno-associated virus vectors for gene therapy of cystic fibrosis
$100,000/year (Bridge Funding)

28. National Institutes of Health (NIDDK) R01-DK51809 09/1996-09/2000
(Project PI: Flotte)
Title: Strategies to improve adeno-associated virus vector persistence and expression ** The competitive renewal of this grant scored at the 3rd percentile, but it was incorporated into the PPG DK58327 listed below
$166,000/year Direct Costs

29. Howard Hughes Medical Institutions (HHMI) Pilot and Feasibility Project Grant 07/01/1996-06/30/1998
(Project PI: Flotte)
Title: Strategies to improve the persistence of adeno-associated virus (AAV) vectors
$25,000/year

30. Cystic Fibrosis Foundation Gene Therapy Research Grant, 12/01/1994-11/30/1997
(Project PI: Flotte)
Title: Episomal Persistence of DNA virus vectors
$50,000/year

31. National Institutes of Health (NHLBI) P01-HL51811 10/1993-
(Project PI: Flotte; PPG Director: William Guggino) 03/2009
Title Adeno-associated virus vectors for CF gene therapy
$194,147/year Direct Costs

32. Co-investigator on CF Research Development Project (CF Foundation) 02/01/1992-
(Project PI: Flotte; PPG Director: William Guggino, Ph.D.) 06/30/1996
$50,000/year

33. Cystic Fibrosis Foundation Leroy Matthews Physician Scientist Award 07/01/1991-
(Project PI: Flotte) 06/30/1995
$85,000/year

34. Cystic Fibrosis Foundation/NIH Fellowship Award 07/01/1989-
(Project PI: Flotte) 06/30/1991
$30,000/year

CERTIFICATION and LICENSURE

Massachusetts Medical Society House of Delegates	2007-present
Massachusetts Medical License #23395	2007-present
Massachusetts Medical Society	2007-present
American Board of Pediatrics Pulmonary Sub-board Certification	04/1992-present
American Board of Pediatrics Certification	1989-present
Florida State Medical License	inactive
Maryland State Medical License	inactive

CLINICAL DISCIPLINE

Pediatrics, Pulmonary	1992-present
Pediatrics, General	1989-present

CLINICAL ACTIVITIES / Global Health Service

1. Port-au-Prince, Haiti, 1 week, HUEH & St. Damiens/ Health Frontiers (AAP)	7/2011
2. Monrovia, Liberia, 1 week, Health, Education and Relief through Teaching (HEARTT)/JFK Hospital, University of Liberia	3/2011
3. Port-au-Prince, Haiti, 1 week, HUEH/Health Frontiers (AAP)	1/2011
4. Port-au-Prince, Haiti, 1 week, Hopital de L'Universite d'Etat d'Haiti (HUEH) /Health Frontiers [American Academy of Pediatrics (AAP)]	7/2010
5. Port-au-Prince, Haiti, 1 week, International Ministries, Good Samaritan Hospital	1/2010
6. Port-au-Prince, Haiti, 1 week, International Ministries, Good Samaritan Hospital	1/2010
7. Phang Nga, Thailand (Alachua Medical Society) and Mai Suay, Thailand (Children of the Golden Triangle), 2 weeks	1/2005
8. Guayllabamba, Ecuador, 4 weeks, Catholic Medical Mission Board	5/1989

Scholarship

PEER-REVIEWED PUBLICATIONS

1. Lek A, Wong B, Keeler A, Blackwood M, Ma K, Huang S, Sylvia K, Batista AR, Artinian B, Kokoski D, Parajuli S, Putra J, Carreon CK, Lidov H, Woodman K, Pajusalu S, Spinazzola JM, Gallagher T,

LaRovere J, Baulderson D, Black L, Sutton K, Horgan R, Lek M, **Flotte TR**
Death after High-Dose rAAV9 Gene Therapy in a Patient with Duchenne's Muscular Dystrophy
New England Journal of Medicine 2023 Sep; 389(13):1203-1210. doi: 10.1056/NEJMoa2307798.
PMID: 37754285

2. Arjomandnejad M, Dasgupta I, **Flotte TR**, Keeler AM.
Immunogenicity of Recombinant Adeno-Associated Virus (AAV) Vectors for Gene Transfer
BioDrugs. 2023 Mar 2:1-19. doi: 10.1007/s40259-023-00585-7.
PMID: 36862289

3. Pires Ferreira D, Gruntman AM, **Flotte TR**
Gene therapy for alpha-1 antitrypsin deficiency: an update.
Expert Opin Biol Ther. 2023 Mar 2:1-9. doi: 10.1080/14712598.2023.2183771.
PMID: 36825473

4. McDonald CL, Qasba P, Anderson DG, Bao G, Colvin RA, Kohn DB, Malik P, Mitchell MJ, Pu WT, Rawlings DJ, Williams DA, **Flotte TR**.
Future Directions and Resource Needs for National Heart, Lung, and Blood Institute (NHLBI) Gene Therapy Research: A Report of an NHLBI Workshop.
Hum Gene Ther. 2023 Feb;34(3-4):83-89. doi: 10.1089/hum.2022.29233.tga.
PMID: 36794978

5. **Flotte TR**, Cataltepe O, Puri A, Batista AR, Moser R, McKenna-Yasek D, Douthwright C, Gernoux G, Blackwood M, Mueller C, Tai PWL, Jiang X, Bateman S, Spanakis SG, Parzych J, Keeler AM, Abayazeed A, Rohatgi S, Gibson L, Finberg R, Barton BA, Vardar Z, Shazeeb MS, Gounis M, Tifft CJ, Eichler FS, Brown RH Jr, Martin DR, Gray-Edwards HL, Sena-Esteves M. *AAV Gene Therapy for Tay-Sachs Disease.* Nat Med. 2022 Feb;28(2):251-259. doi: 10.1038/s41591-021-01664-4. Epub 2022 Feb 10

6. Milner RJ, **Flotte TR**, Thorndyke LE.
Defining Scholarship for Today and Tomorrow
J Contin Educ Health Prof. 2022 Dec 21. doi: 10.1097/CEH.0000000000000473.
PMID: 36728995

7. Ke H, Guay KP, **Flotte TR**, Gierasch LM, Gershenson A, Hebert DN.
Secretion of functional α1-antitrypsin is cell type dependent: Implications for intramuscular delivery for gene therapy.
Proc Natl Acad Sci U S A. 2022 Aug 2;119(31):e2206103119. doi: 10.1073/pnas.2206103119. Epub 2022 Jul 28.
PMID: 35901208

8. Zieger M, Borel F, Greer C, Gernoux G, Blackwood M, **Flotte TR**, Mueller C.
Liver-directed SERPINA1 gene therapy attenuates progression of spontaneous and tobacco smoke-induced emphysema in α1-antitrypsin null mice.
Mol Ther Methods Clin Dev. 2022 Apr 13;25:425-438. doi: 10.1016/j.omtm.2022.04.003. eCollection 2022 Jun 9.
PMID: 35592360

9. Davidson BL, Gao G, Berry-Kravis E, Bradbury AM, Bönnemann C, Buxbaum JD, Corcoran GR, Gray SJ, Gray-Edwards H, Kleiman RJ, Shaywitz AJ, Wang D, Zoghbi HY, **Flotte TR,** Tauscher-Wisniewski S, Tifft CJ, Sahin M; Gene Therapy Workshop Faculty.
Gene-based therapeutics for rare genetic neurodevelopmental psychiatric disorders.
Mol Ther. 2022 Jul 6;30(7):2416-2428. doi: 10.1016/j.ymthe.2022.05.014. Epub 2022 May 17.
PMID: 35585789 Review.

10. Arjomandnejad M, Sylvia K, Blackwood M, Nixon T, Tang Q, Muhuri M, Gruntman AM, Gao G, **Flotte TR**, Keeler AM. *Modulating Immune Responses to AAV by expanded polyclonal T-regs and capsid specific chimeric antigen receptor T-regulatory cells.* Mol Ther Methods Clin Dev. 2021 Oct 28;23:490-506. doi: 10.1016/j.omtm.2021.10.010. eCollection 2021 Dec 10.

11. Mueller C, Berry JD, McKenna-Yasek DM, Gernoux G, Owegi MA, Pothier LM, Douthwright CL, Gelevski D, Luppino SD, Blackwood M, Wightman NS, Oakley DH, Frosch MP, **Flotte TR**, Cudkowicz ME, Brown RH Jr. *SOD1 Suppression with adeno-associated virus and microRNA in Familial ALS.* N Engl J Med. 2020 Jul 9;383(2):151-158. Doi 10.1056/NEJMoa2005056. PMID: 32640133.

12. **Flotte TR**, Larkin AC, Fischer MA, Chimienti SN, DeMarco DM, Fan PY, Collins MF. *Accelerated graduation and the deployment of new physicians during the COVID-19 Pandemic*. Acad Med. 2020 Jun 9 doi: 10.1097/ACM.0000000000003540.

13. Jagsi R, Means O, Lautenberger D, Jones RD, Giffith KA, **Flotte TR**, Gordon LK, Rexrode KM, Wagner LW, Chatterjee A. *Women's representation among members and leaders of national medical specialty societies.* Acad Med. 2020 Jul;95(7):1043-1049. doi: 10.1097/ACM.0000000000003038. PMID: 31625994

14. Zieger M, Keeler AM, **Flotte TR**, ElMallah MK. *AAV9 Gene Replacement Therapy for Respiratory Insufficiency in Very-long Chain Acyl-CoA Dehydrogenase Deficiency.* J Inherit Metab Dis. 2019 Apr 17. doi: 10.1002/jimd.12101. [Epub ahead of print] PMID: 30993714

15. Gruntman AM, Gernoux G, Tang Q, Ye GJ, Knop DR, Wang G, Benson J, Coleman KE, Keeler AM, Mueller C, Chicoine LG, Chulay JD, **Flotte TR**. *Bridging from Intramuscular to Limb Perfusion Delivery of rAAV: Optimization in a Non-human Primate Study*. Mol Ther Methods Clin Dev. 2019 Feb 2;13:233-242. doi: 10.1016/j.omtm.2019.01.013. eCollection 2019 Jun 14. PMID: 30828586

16. Chimienti SN, DeMarco DM, **Flotte TR**, Collins MF. *Assuring Integrity in the Residency Match Process.* Acad Med. 2018 Nov 13. doi: 10.1097/ACM.0000000000002531. [Epub ahead of print] PMID: 30431456

17. Wang D, Zhong L, Li M, Li J, Tran K, Ren L, He R, Xie J, Moser RP, Fraser C, Kuchel T, Sena-Esteves M, **Flotte TR**, Aronin N, Gao G. *Adeno-Associated Virus Neutralizing Antibodies in Large Animals and Their Impact on Brain Intraparenchymal Gene Transfer.* Mol Ther Methods Clin Dev. 2018 Oct 4;11:65-72. doi: 10.1016/j.omtm.2018.09.003. eCollection 2018 Dec 14. PMID: 30397628

18. Pennesi ME, Weleber RG, Yang P, Whitebirch C, Thean B, **Flotte TR,** Humphries M, Chegarnov E, Beasley KN, Stout JT, Chulay JD. *Results at 5 Years after Gene Therapy for RPE65-deficient Retinal Dystrophy.*Hum Gene Ther. 2018 Jun 5. doi: 10.1089/hum.2018.014. [Epub ahead of print] PMID: 29869534

19. Wang D, Li S, Gessler DJ, Xie J, Zhong L, Li J, Tran K, Van Vliet K, Ren L, Su Q, He R, Goetzmann JE, **Flotte TR**, Agbandje-McKenna M, Gao G. *A Rationally Engineered Capsid Variant of AAV9 for Systemic CNS-Directed and Peripheral Tissue-Detargeted Gene Delivery in Neonates.* Mol Ther Methods Clin Dev. 2018 Mar 16;9:234-246. doi: 10.1016/j.omtm.2018.03.004. eCollection 2018 Jun 15. PMID: 29766031

20. Gruntman AM, **Flotte TR**, *The rapidly evolving state of gene therapy*. FASEB J. 2018 Apr 32(4):1733-1740, doi: 10.1096/fj.201700982R

21. Song CQ, Wang D, Jiang T, O'Connor K, Tang Q, Cai L, Li X, Weng Z, Yin H, Gao G, Mueller C, **Flotte TR**, Xue W. *In vivo genome editing partially restores alpha1-antitrypsin in a murine model of AAT deficiency.* Hum Gene Ther. 2018 Mar 29. doi: 10.1089/hum.2017.225. [Epub ahead of print] PMID: 29597895

22. Borel F, Sun H, Zieger M, Cox A, Cardozo B, Li W, Oliveira G, Davis A, Gruntman A, **Flotte TR**, Brodsky MH, Hoffman AM, Elmallah MK, Mueller C. *Editing out five Serpina1 paralogs to create a mouse model of genetic emphysema.* Proc Natl Acad Sci U S A. 2018 Mar 13;115(11):2788-2793. doi: 10.1073/pnas.1713689115. Epub 2018 Feb 16.PMID: 29453277

23. **Flotte TR**, Daniels E, Benson J, Bevett-Rose JM, Cornetta K, Diggins M, Johnston J, Sepelak S, van der Loo JCM, Wilson JM, McDonald CL. *The Gene Therapy Resource Program: A Decade of Dedication to Translational Research by the National Heart, Lung, and Blood Institute.*Hum Gene Ther Clin Dev. 2017 Nov 27. doi: 10.1089/humc.2017.170. [Epub ahead of print] PMID: 29130351

24. Bates CK, Jagsi R, Gordon LK, Travis E, Chatterjee A, Gillis M, Means O, Chaudron L, Ganetzky R, Gulati M, Fivush B, Sharma P, Grover A, Lautenberger D, **Flotte TR**. *It is Time for Zero Tolerance for Sexual Harassment in Academic Medicine.* Acad Med. 2017 Nov 7. doi:10.1097/ACM.0000000000002050. [Epub ahead of print]

25. Borel F, Tang Q, Gernoux G, Greer C, Wang Z, Barzel A, Kay MA, Shultz LD, Greiner DL, **Flotte TR**, Brehm MA, Mueller C. *Survival Advantage of Both Human Hepatocyte Xenografts and Genome-Edited Hepatocytes for Treatment of α-1 Antitrypsin Deficiency.* Mol Ther. 2017 Sep 25. pii: S1525-0016(17)30436-7. doi: 10.1016/j.ymthe.2017.09.020. [Epub ahead of print] PMID: 29032169

26. Mueller C, Gernoux G, Gruntman AM, Borel F, Reeves EP, Calcedo R, Rouhani FN, Yachnis A, Humphries M, Campbell-Thompson M, Messina L, Chulay JD, Trapnell B, Wilson JM, McElvaney NG, **Flotte TR**. *5 Year Expression and Neutrophil Defect Repair after Gene Therapy in Alpha-1 Antitrypsin Deficiency.* Mol Ther. 2017 Jun 7;25(6):1387-1394. doi: 10.1016/j.ymthe.2017.03.029. Epub 2017 Apr 10. PMID: 28408179

27. Keeler AM, ElMallah MK, **Flotte TR**. 1. *Gene Therapy 2017: Progress and Future Directions.* Clin Transl Sci. 2017 Apr 6. doi: 10.1111/cts.12466. [Epub ahead of print] Review. No abstract available. PMID: 28383804

28. Wilkins O, Keeler AM, **Flotte TR**. *CAR T-Cell Therapy: Progress and Prospects.* Hum Gene Ther Methods. 2017 Apr;28(2):61-66. doi: 10.1089/hgtb.2016.153. PMID: 28330372

29. Gruntman AM, Su L, **Flotte TR.** *Retro-Orbital Venous Sinus Delivery of rAAV9 Mediates High-Level Transduction of Brain and Retina Compared with Temporal Vein Delivery in Neonatal Mouse Pups.* Hum Gene Ther. 2017 Mar;28(3):228-230. doi: 10.1089/hum.2017.037. PMID: 28319444

30. Stoica L, Keeler AM, Xiong L, Kalfopoulos M, Desrochers K, Brown RH Jr, Sena-Esteves M, **Flotte TR**, ElMallah MK. *Restrictive Lung Disease in the Cu/Zn Superoxide-Dismutase 1 G93A Amyotrophic Lateral Sclerosis Mouse Model.* Am J Respir Cell Mol Biol. 2017 Mar;56(3):405-408. doi: 10.1165/rcmb.2016-0258LE. No abstract available. PMID: 28248134

31. Calcedo R, Somanathan S, Qin Q, Betts MR, Rech AJ, Vonderheide RH, Mueller C, **Flotte TR**, Wilson JM. *Class I-restricted T-cell responses to a polymorphic peptide in a gene therapy clinical trial for α-1-antitrypsin deficiency.* Proc Natl Acad Sci U S A. 2017 Jan 30. pii: 201617726. doi: 10.1073/pnas.1617726114. [Epub ahead of print] PMID: 28137880

32. Antman KH, Berman HA, **Flotte TR**, Flier J, Dimitri DM, Bharel M. *Developing Core Competencies for the Prevention and Management of Prescription Drug Misuse: A Medical Education Collaboration in Massachusetts.* Acad Med. 2016 Oct;91(10):1348-1351. PMID: 27532868

33. Weleber RG, Pennesi ME, Wilson DJ, Kaushal S, Erker LR, Jensen L, McBride MT, **Flotte TR**, Humphries M, Calcedo R, Hauswirth WW, Chulay JD, Stout JT. *Results at 2 Years after Gene Therapy for RPE65-Deficient Leber Congenital Amaurosis and Severe Early-Childhood-Onset Retinal Dystrophy.* Ophthalmology. 2016 Jul;123(7):1606-20. doi: 10.1016/j.ophtha.2016.03.003. PMID: 27102010

34. Loring HS, ElMallah MK, **Flotte TR**. *Development of rAAV2-CFTR: History of the First rAAV Vector Product to be Used in Humans.* Hum Gene Ther Methods. 2016 Apr;27(2):49-58. doi: 10.1089/hgtb.2015.150. Review. PMID: 26895204

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36. Schnepp B, Chulay JD, Ye GJ, **Flotte TR**, Trapnell BC, Johnson PR. *Recombinant adeno-associated virus vector genomes take the form of long-lived, transcriptionally competent episomes in human muscle.* Hum Gene Ther. 2016 Jan:27(1):32-42. Doi: 10.1089/hum.2015.136. PMID: 26650966

37. Li S, Ling C, Zhong L, Li M, Su Q, He R, Tang Q, Greiner DL, Shultz LD, Brehm MA, **Flotte TR**, Mueller C, Srivastava A, Gao G. *Efficient and targeted transduction of nonhuman primate liver with systemically delivered optimized AAV3B vectors*. Mol Ther. 2015 Dec;23(12):1867-76. doi: 10.1038/mt.2015.174. PMID: 26403887

38. Gruntman AM, **Flotte TR**. *Delivery of Adeno-Associated Virus Gene Therapy by Intravascular Limb Infusion Methods.* Hum Gene Ther Clin Dev. 2015 Sep;26(3):159-64. doi: 10.1089/humc.2015.116. Epub 2015 Sep 10. PMID: 26357010

39. Gruntman AM, **Flotte TR**. *Progress with Recombinant Adeno-Associated Virus Vectors for Gene Therapy of Alpha-1 Antitrypsin Deficiency.* Hum Gene Ther Methods. 2015 Jun;26(3):77-81. doi: 10.1089/hgtb.2015.086. PMID: 26067712

40. Gruntman AM, Su, L, Su, Q, Gao G, Mueller C, **Flotte TR**. *Stability and compatibility of recombinant adeno-associated virus (rAAV) under conditions commonly encountered in human gene therapy trials.* Human Gene Ther Methods. 2015 Apr;26(2):71-6. PMID: 25819833

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49. Keeler AM, Conlon T, Walter G, Zeng H, Shaffer SA, Dungtao F, Erger K, Cossette T, Tang Q, Mueller C, **Flotte TR**. *Long-term correction of very long-chain acyl-coA dehydrogenase deficiency in mice using AAV9 gene therapy.* Mol Ther. 2012 Jun;20(6):1131-8. PMID: 22395529

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23. **Flotte TR**, Gao G. *AAV Is Now a Medicine: We Had Better Get This Right.* Hum Gene Ther. 2017 Apr;28(4):307. doi: 10.1089/hum.2017.29041.trf. No abstract available. PMID: 28414552

24. **Flotte TR**. *The Negative Effects of Immigration Restrictions on the Gene Therapy Community.*Hum Gene Ther. 2017 Mar;28(3):227. doi: 10.1089/hum.2017.29040.trf. No abstract available. PMID: 28319443

25. **Flotte TR**. *Empty Adeno-Associated Virus Capsids: Contaminant or Natural Decoy?* Hum Gene Ther. 2017 Feb;28(2):147-148. doi: 10.1089/hum.2017.29039.trf. No abstract available. PMID: 28186848

26. **Flotte TR.** *The Science Policy Implications of a Trump Presidency.* Hum Gene Ther. 2017 Jan;28(1):1-2. doi: 10.1089/hum.2016.29037.trf. No abstract available. PMID: 27922749

27. Naldini L, Parker GC, **Flotte TR**. *The Renaissance of Gene and Cell Therapy: Florence 2016.* Human Gene Therapy. October 2016, 27(10): 727-728. No abstract available. PMID: 29112477

28. **Flotte TR**. *Gene Drives: Biological Shield or Ecological Menace?* Hum Gene Ther. 2016 Aug;27(8):561-2. doi*: 10.1089/hum.2016.29032.trf*. No abstract available. PMID: 27513596

29. **Flotte TR**. *The Target's the Thing.* Hum Gene Ther. 2016 Jul;27(7):477. doi: 10.1089/hum.2016.29030.trf. No abstract available. PMID: 27428657

30. **Flotte TR**. *Why Human Gene Therapy Scientists Should Care About Model Organisms.* Hum Gene Ther. 2016 May;27(5):339-40. doi: 10.1089/hum.2016.29026.trf. No abstract available. PMID: 27082033

31. **Flotte TR**. *A Gene Therapy Scientist's Life Well-Lived.* Hum Gene Ther. 2016 Apr;27(4):279. doi: 10.1089/hum.2016.29023.trf. No abstract available. PMID: 27074150

32. **Flotte TR**. *Special Note from the Editor-in-Chief.* Hum Gene Ther. 2016 Feb;27(2):95. doi: 10.1089/hum.2016.29019 No abstract available. PMID: 26886829

33. **Flotte TR**. *The Role of Patient Advocacy Organizations in Advancing Human Gene Therapy.* Hum Gene Ther. 2015 Dec;26(12):782. doi: 10.1089/hum.2015.29011. No abstract available. PMID: 26690811

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35. **Flotte TR**. *Transcription Activator-Like Nucleases Enable Allogeneic Chimeric Antigen Receptor-T Cell Therapy in Humans.* Hum Gene Ther. 2015 Nov;26(11):iv. doi: 10.1089/hum.2015.29013. No abstract available. PMID: 26565678

36. **Flotte TR**. *Ethical Implications of the Cost of Molecularly Targeted Therapies*. Hum Gene Ther. 2015 Sep;26(9):573-4. PMID: 26355414

37. **Flotte TR**. *The End of the Beginning of Gene Therapy*. Hum Gene Ther. 2015 Jul;26(7):407-8. PMID: 26176429

38. **Flotte TR.** *Therapeutic germ line alteration: Has CRISPR/Cas9 technology forced the question?* Hum Gene Ther. 2015 May;26(5):245-6. PMID: 25927157

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40. **Flotte TR**, Mueller C. *What is suppression of anti-adeno-associated virus capsid T-cells achieving?* Hum Gene Ther. 2014 Mar;25(3):178-9. doi: 10.1089/hum.2014.2506. No abstract available. PMID: 24628440

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43. **Flotte TR**. *Sendai virus vectors. Pushing the envelope in the lung.* Gene Ther. 2011 Feb;18(2):107-8. PMID: 20927135

44. **Flotte TR**, Laube BL. Gene therapy in cystic fibrosis. Chest. 2001 Sep; 120(3 Suppl):124S-131S. PMID: 11555567.

45. **Flotte TR.** Size does matter: overcoming the adeno-associated virus packaging limit. Respir Res. 2000; 1(1):16-8. PMID: 11667959.

46. **Flotte TR**. *Gene therapy for cystic fibrosis*. Curr Opin Mol Ther. 1999 Aug;1(4):510-6. Review. PMID:11713767

PATENTS ISSUED

1.	rAAV-based compositions and methods (US Patent Number 10,077452)	09/18/2018
2.	AAV's and uses thereof (US Patent Number10,035,825) Issued July 31, 2018	07/31/2018
3.	rAAV-based compositions and methods for treating alpha-1 anti-trypsin deficiencies (US Patent Number 9,885,057)	02/06/2018
4.	AAV's and uses thereof (US Patent Number 9,284,357)	03/15/2016
5.	rAAV-based compositions and methods for treating alpha-1 anti-trypsin deficiencies (US Patent Number 9,226,976)	01/05/2016
6.	AAV's and Uses Thereof (US Patent Number 8,734,809)	05/27/2014
7.	Compositions for treating cystic fibrosis (US Patent Number 8,137,962).	03/20/2012
8.	Production of pseudotyped recombinant AAV virions (US Patent Number 7,094,604)	08/22/2006
9.	Materials and methods for gene therapy (US Patent Number 6,461,606)	10/08/2002
10.	Modified adeno-associated virus vector capable of expression from a novel promoter (US Patent Number 6,165,781)	12/26/2000
11.	Amino-terminally truncated cystic fibrosis transmembrane conductance regulator (US Patent Number 5,990,279)	11/23/1999
12.	Modified adeno-associated virus vector capable of expression from a novel promoter (US Patent Number 5,989,540)	11/23/1999
13.	Modified adeno-associated virus vector capable of expression from a novel promoter (US Patent Number 5,866,696)	02/02/1999
14.	Generation of high titers of recombinant AAV vectors (US Patent Number 5,658,776)	08/19/1997
15.	Modified adeno-associated virus vector capable of expression from a novel promoter (US Patent Number 5,587,308)	12/24/1996

Patents Pending/Disclosed/Filed

1.	"Methods and Compositions for Expressing a Nucleic Acid in a Dendritic Cell", Ref: UF# 10851 (Womer, et.al.)	2003
2.	"Adeno-associated viral vectors for the treatment and prevention of Diabetes", U.S. Patent Application Serial No. 10/340/112, Ref: UF#11223, 2003. Inventor(s):	2003

Terence Flotte, Sihong Song, Barry Byrne & Michael Morgan

3. "Treatment for Phenylketonuria", U.S. Patent App. Serial No. 10/427/181. Inventor(s): Phil Laipis, Terence Flotte, Leticia Reyes, Bin Yan. UF# 10834.

4. "Improved rAAV Vectors for Enhancing Transduction of Cells Expressing Low-Density Lipoprotein Receptors". Inventors: Scott A Loiler, Terence R. Flotte, Nicholas Muzyczka and Mark A. Atkinson. Ref.#: UF#10708, Submitted 30 October 2003. Serial No. 10/511,914. 2003

5. "Delivery of Genes to the Pancreas as a Method for Delivery of Therapeutic Molecules for Diabetes and Other Diseases", UF#-11668, 2005.

INVITED PRESENTATIONS

International

1. The European Society of Gene & Cell Therapy 30th Annual Congress: Updates and Immune Profiling in AAV gene therapy trials for patients with Tay-Sachs and Sandhoff diseases. Brussels, Belgium. 10/26/2023
2. The European Society of Gene & Cell Therapy 29th Annual Congress: Overview of Clinical Gene Therapy with AAV from the Safety Perspective 10/12/2022
3. The European Society of Gene & Cell Therapy 27th Annual Congress: First-in-human Gene Therapy for Tay-Sachs Disease: Report of Two Infants Treated on an Expanded Access Clinical Trial of rAAVrh8-HexA/HexB (AX)-AVV-GM2). Barcelona, Spain. 10/23/2019
4. The 2019 National Veterinary Scholars Sumposium: *Impact of Research Collaborations between DVMs and MDs.* Worcester, Massachusetts. 07/26/2019
5. The 2018 International Rare Lung Diseases Research Conference & Patient and Family LAMposium, Rare Lung Disease Intensive: *Obstructive Lung Disease, Technology Focus: Gene Editing*, Covington, Kentucky. 09/07/2018
6. The 2018 International Rare Lung Diseases Research Conference & Patient and Family LAMposium, Gene Therapy 2018: *Lessons for Alpha-1 Antitrypsin Deficiency (Alpha-1) Gene Therapy* Workshop, Covington, Kentucky 09/08/2018
7. The 2018 International Rare Lung Diseases Research Conference & Patient and Family Symposium, LA. 09/09/2018
8. The Advanced Biomanufacturing Meeting of the Biomedical Engineering Society, Plenary Speaker *Gene Therapy 2018,* Worcester Polytechnic Institute, Worcester, MA. 08/23/2018
9. The 2018 International Symposium on Gene and Cell Therapy for Rare Diseases, Chair Plenary Session and Speaker, *Human Gene Therapy 2018: The Rapidly Evolving State of rAAV and Gene Editing*, Shenzhen, China 08/17/2018
10. The 2018 International Symposium on Gene and Cell Therapy for Rare Diseases, Co-Chair, *A Shared Mission: Rare Disease Research,* Shenzhen, China, 08/17/2018
11. The Drug Information Agency Global Annual Meeting (DIA-2018), *The Rapidly Evolving State of Recombinant Adeno-Associated Virus Gene Therapy*, Boston, MA 06/26/2018
12. NIH/NIAID/Division of AIDS Genetic Delivery of Monoclonal Antibodies for Prevention and Cure of HIV Workshop, *Optimization of Venous Limb Perfusion with rAAV for Expression of Secreted Proteins from Muscle and induction of peripheral Treg responses*, Rockville, MD 06/14/2018

13. The 12th Annual ICORD Conference 2017 & 6th China Rare Disease Summit, *Gene Therapy for Genetic Emphysema,* Beijing, China — 09/09/2017
14. ESGCT - ISSCR and ABCD Collaborative Congress 2016 "5 Year Expression and Neutrophil Defect Repair after Gene Therapy in Alpha-1 Antitrypsin Deficiency" Florence, Italy — 10/20/2016
15. ESGCT ISSCR and ABCD Collaborative Congress 2016 Chair Symposium "RNA Based Gene Transfer and Integration Studies", Florence, Italy. — 10/21/2016
16. British Society of Gene Therapy, Gene and Cell Therapy in Inherited Metabolic Disorders Symposium, "Gene Therapy for Fatty Acid Oxidation Disorders". London, England — 04/17/2013
17. 14th Biennial International Parvovirus Workshop "Persistence of Transgene Expression 12 months after Intramuscular Administration of a Recombinant Adeno-Associated Virus Alpha-1 Antitrypsin Vector in a Phase 2 Clinical Trial". Cornell University, Ithaca, NY — 06/20/2012
18. 2011 International Academy of Life Sciences, Advancing In The Life Science And Uniting Universities, Corporate And Government Partners. "The Value Chain – University As Center For Competence & Innovation". University of Massachusetts Boston, Boston, MA — 10/28/2011
19. Bio 2008 International Convention, Invited Speaker/Panelist - "What the Vector is Happening Here?" San Diego, CA. — 06/17/2008
20. 2007 Alpha-1 Foundation International Scientific Conference, New Insights into the Biology of AAT. Invited Speaker, "Gene Therapy", Coral Gables, FL. — 02/08/2007
21. 1995 Organization for Economic Cooperation and Development (OECD) International Workshop on Gene Therapy (Ottawa, Canada): Symposium on Biosafety Issues with AAV Vectors — 06/07/1995

18. 1995 BioEast International Symposium on Gene Therapy (Washington, DC) — 1995

National

1. ASGCT 2020 COVID Symposium, Program Committee Member — 09/15/2020, 09/16/2020
2. ASGCT 2020 COVID Symposium, Chair, COVID-19 Scientific Abstract Session, Virtual — 09/16/2020
3. ASGCT 23ed Annual Meeting, Speaker, Clinical Trials Spotlight Symposium, Virtual — 05/15/2020
4. ASGCT 23ed Annual Meeting, Panelist, Panel for ASGCT *Navigating Career Paths* Lunch Session, Virtual — 05/14/2020
5. ASGCT 23ed Annual Meeting, Chair, Outstanding Achievement Award Symposium, Virtual — 05/14/2020
6. ASGCT 23ed Annual Meeting, Chair, *IND-Enabling NIH Resources to Advance Clinical and Translational Gene Therapy,* Symposium, Virtual — 05/14/2020
7. ASGCT 23ed Annual Meeting, Speaker, Pre-Meeting Workshop: *Clinical Trails Training 1*, Virtual — 05/11/2020
8. ASGCT 23ed Annual Meeting, Program Committee Member — 05/12-05/2020
9. AAMC (Annual Meeting), Council of Deans (COD) Administrative Board, *Report by GWIMS Steering Committee Liaison*, Presentation, Austin, TX. — 11/02/2018
10. 2019 Seminar, MedImmune, *Optimization of Venous Limb Perfusion with rAAV for Expression of Secreted Proteins from Muscle and Induction of Peripheral Treg Responses*, Gaithersburg, MD. — 01/09/2019

11. 2018 Duke University Pulmonary Research Conference, *Gene Therapy and Gene Editing for Alpha -1 Antitrypsin Deficiency*, Duke University, Durham, NC. 11/12/2018
12. 2018 Duke University Pediatric Grand Rounds, *Top Ten Reasons to be Optimistic about The Future of Pediatrics*, Duke University, Durham, NC. 11/12/2018
13. 2018 COD/COTH Joint Spring Meeting *Development of Regional Campuses and Teaching Sites to Better Address Physician Workforce Needs: The Massachusetts Experience*, Phoenix, AZ. 04/13/2018
14. The Rare Disease Clinical Research Network Coalition of Patient Advocacy Groups (RDC RN-CPAG) Webinar, *Gene Therapy 101* 12/13/2017
15. The AAMC Annual Meeting 2017, *UMass Medical School Opioid Training Immersion*, Boston, MA 11/03/2017
16. 2017 National Academic Affiliations Council Meeting, *Experiences as a VA Affiliate*, Washington, DC. 07/12/2017
17. The 26th Annual National Education Conference, Chair of The Alpha-1 Project (TAP) Panel, Chicago, IL. 06/24/2017
18. The 3rd Annual CRISPR Precision Gene Editing Congress, Chairperson and Moderator, *Turning Precision Genome Editing Possibilities of Tomorrow into a Clinical Reality Today,* Boston, MA. 02/21/2017
19. The 3rd Annual CRISPR Precision Gene Editing Congress, Speaker, *Clinical Translation of Genome Editing Solutions to the Problem of Alpha-1 Antitrypsin Deficiency*, Boston, MA. 02/22/2017
20. Learn Serve Lead 2016: The AAMC Annual Meeting. Invited Panelist, "Women in Academic Medicine and Science Careers: How Far Have We Come and Where Are We Going?" Seattle WA. 11/12/2016
21. 2016 Biogen Inc. Biogen's Technical Development, Technical Development Science Day. Keynote Address: "Lessons Learned From 21 years of Clinical Trials With Recombinant AAV", Cambridge, MA. 11/09/2016
22. ASGCT 19th Annual Meeting, Group leader, "Immunological Aspects of Gene Therapy-AAV Vectors" poster session, Washington, DC. 05/06/2016
23. ASGCT 19th Annual Meeting, Chair of the Outstanding Achievement Award Lecture with Sonia Skarlatos Public Service Award Presentation, Washington, DC. 05/06/2016
24. ASGCT 19th Annual Meeting, Chair the Outstanding New Investigator Symposium Washington, DC, May 5, 2016 05/05/2016
25. ASGCT 19th Annual Meeting, Chair Symposium, "Foundations: Their Mission, Use of Donor Funding to Incentivize both Academic Scientists and Corporate Entities to Work on Their Disease, Participate in New Ventures and Challenges Faced in Technology Transfer between Universities whose Faculty Generate New IP and Foundations Engaged in Building New Ventures; Successes and challenges in Accomplishing their Mission" Washington, DC. 05/04/2016
26. ASGCT 19th Annual Meeting, "Sustained Expression with Partial Correction of Neutrophil Defects 5 Years after Intramuscular rAAV1 Gene Therapy for Alpha-1 Antitrypsin Deficiency", Washington DC. 05/04/2016
27. 2016 Congressional Briefing, "How Medical Schools and Teaching Hospitals are Addressing the Opioid Epidemic" - Panelist, Capitol Hill, Washington DC 01/28/2016
28. 2016 APRN Task Force Meeting, "Expanding the Role of Advanced Practice Nurses in Health Care", Burlington, MA 01/26/2016

29. ASGCT 18th Annual Meeting , Chair the Outstanding Achievement Award Lecture, New Orleans, LA. 05/15/2015
30. ASGCT 18th Annual Meeting, Chair the Outstanding New Investigator Symposium New Orleans, LA. 05/14/2015
31. CRISPR Genome Editing Summit 2015, “Alpha-1 Antitrypsin Deficiency Liver Disease: Genome Editing for Animal Models and Gene Therapy”, Cambridge, MA. 02/26/2015
32. 2014 AAMC Group on Faculty Affairs Conference, “Navigating the Changes in LCME Standards for Faculty”, Boston, MA. 07/20/2014
33. 2014 Moderator ASGCT Award Introductions Outstanding New Investigator Symposium, Washington, DC. 05/22/2014
34. ASGCT Standardized Pathways Conference, “Standardized Pathways: A Step Toward A ‘Plug and Play’ Gene Therapy Approach?”. Silver Spring, MD. 02/20/2012
35. American Thoracic Society 2013 International Conference, Symposium Next Generation Treatments for Lung Disease: Gene Therapy and Transplantable Scaffolds, "Human Gene Therapy Trials for Alpha-1 Antitrypsin Deficiency", Philadelphia, PA. 05/20/2013
36. ASGCT 16th Annual Meeting, “Phase 2 rAAV1-AAT Trial: 12-month follow-up”, Salt Lake City, UT. 05/16/2013
37. 2012 Institute of Medicine (IOM) Committee to Review the Clinical and Translational Science Awards Program (CTSA) at National Center for Advancing Translational Sciences (NCATS), “Children’s Health Research: Role of the CTSA Program” Washington, DC. 12/12/2012
38. Oregon Health & Science University, Dept. of Molecular and Medical Genetics Seminar, “Recombinant Adeno-Associated Virus-Based Gene Therapy for Disorders of Fatty Acid Oxidation.” 08/29/2012
39. American Society of Gene & Cell Therapy (ASGCT) 15th Annual Meeting, “Phase 2 rAAV1-AAT Trial: 12-month follow-up”. Philadelphia, PA. 05/18/2012
40. American Society of Gene & Cell Therapy (ASGCT) 15th Annual Meeting, Scientific Symposium Co-Chair Gene and Cell Therapy Impact on Disorders in Pulmonary Medicine: A Broader View. Philadelphia, PA. 05/17/2012
41. 2011 NHLBI Gene and Cell Therapy (GCT) DSMB. “Gene Therapy for Single Gene Disorders Affecting the Lung - review of the field in pulmonary gene therapy, gene/cell therapy trials ongoing and the diseases that should be targeted for gene/cell therapy in the future”. Rockville, MD. 09/22/2011
42. American Society of Gene Therapy, 11th Annual Meeting, Invited Speaker – “Meet the Investigator”, Boston, MA. 06/01/2008
43. 2008 American Thoracic Society, International Conference, Invited Speaker - “Living to Adulthood with CF: Current Concepts in Disease Management”, Toronto, Canada 05/16/2008
44. 2006 NIH/NHLBI 5th Annual Gene Therapy Symposium for Heart, Lung, and Blood Diseases, Invited Speaker – Vector Session, Focus Topic: “Tissue Engineering & Regenerative Medicine”. Sonoma, CA. 11/02/2006
45. 18th Cystic Fibrosis Foundation Williamsburg Conference (CFF) invited lecture “Clinical Trials of AAV Vectors for CF”, 06/05/2006
46. American Society for Gene Therapy (ASGT) invited lecture “Innate Immune Responses to AAV”, Baltimore, MD. 06/01/2006

47. American Association for the Study of Liver Diseases (AASLD) and Alpha-1 Foundation, Single Topic Conference, "Alpha-1 Antitrypsin Deficiency and Other Liver Diseases Caused by Aggregated Proteins", Emory Conference Center, Atlanta, GA. 01/27/2006
48. American Society of Gene Therapy, Challenges in Advancing the Field of Gene Therapy: A Critical Review of the Science, Medicine and Regulation - Stakeholders' Meeting, Arlington, VA. 2005
49. 2004 American Society of Gene Therapy: Clinical Trials Platform Session and Regulatory Issues Education Session Chairman 2004
50. 2004 American Society of Gene Therapy: Long-Term Follow-up Workshop Chairman 2004
51. 2003 American Association for The Study of Liver Diseases (AASLD) Annual Meeting (*invited guest speaker*) Boston, Massachusetts, Symposium: "Hepatotoxicity in Alpha-1 Antitrypsin Deficiency: Molecular Pathogenesis and Therapeutic Approaches. Lecture Title: Gene Therapy Approaches for the Treatment of Liver Diseases in Alpha-1 Antitrypsin Deficiency 2003
52. 2003 32nd Annual Meeting, American College of Clinical Pharmacology, Tampa, Florida (*invited symposium speaker*) "Clinical Applications of Gene Therapy and Pharmacogenetics" 2003
53. 2003 American Thoracic Society Conference (*discussion facilitator*), Seattle, WA. 2003
54. 2003 17th Annual North American CF Conference, Co-Chair of Workshop on CF Gene Therapy with AAV 2003
55. 2002 Second Annual National CF Meeting, Ireland: Invited Speaker 2002
56. 1995 Organization for Economic Cooperation and Development (OECD) International Workshop on Gene Therapy (Ottawa, Canada): Symposium on Biosafety Issues with AAV Vectors 1995
57. 1995 BioEast International Symposium on Gene Therapy (Washington, DC) 1995
58. 2002 Cystic Fibrosis Foundation Williamsburg Conference, Invited Speaker 2002
59. 2001 Gordon Research Conference (GRC) on Viruses and Cells: Poster Presenter 2001
60. 2001 Alpha One Foundation Research Conference (invited speaker) Gene Therapy for AAT Deficiency 2001
61. 2001 Cystic Fibrosis Foundation Williamsburg Conference Chair of Gene Therapy Session 2001
62. 2001 Aspen Lung Conference (invited speaker) Gene Therapy for Inherited Lung Diseases 2001
63. 2001 American Society of Gene Therapy (platform talk) Viral Vectors Workshop Session 2001
64. 2001 American Society of Gene Therapy/Avigen Evening Symposium (invited speaker) Gene Therapy for AAT Deficiency 2001
65. 2001 American Society of Gene Therapy: Chair of Workshop on Adeno-associated virus Vectors 2001
66. 2001 American Society of Gene Therapy: Invited Speaker in Cystic Fibrosis Gene Therapy Symposium 2001
67. 2001 Medical Librarians'Association: Invited Speaker in Symposium on Gene Therapy 2001
68. 2001 FASEB Meeting:Chair of American Society of Investigative Pathology Workshop on Gene Therapy for Liver Disease 2001

69. 2001 FASEB Meeting: Invited Speaker for APS Symposium on Gene Therapy 2001
70. 2000 Alpha One Foundation Research Conference: Speaker on Gene Therapy 2000
71. 2000 North American CF Conference: Chair of Host/Vector Interaction workshop 2000
72. 2000 American Society of Gene Therapy: Chair of DNA viral vector workshop 2000
73. 1999 Alpha One Foundation First Annual Research Conference: Invited Speaker on Gene Therapy 1999
74. 1999 North American CF Conference: Chair of Workshop on Viral Gene Therapy Approaches 1999
75. 1999 CF Foundation Williamsburg Conference: Invited Speaker on Immune Responses to Latent AAV Infection 1999
76. 1999 American Society of Gene Therapy: Chair of Workshop on Cystic Fibrosis and Lung Diseases 1999
77. 1998 American College of Toxicology: Invited Symposium Speaker on AAV Vectors for Cystic Fibrosis 1998
78. 1998 North American CF Conference: Chair of Workshop on Advances in Gene Therapy 1998
79. 1998 CF Foundation Williamsburg Conference: Invited Speaker in AAV Gene Therapy Session 1998
80. 1998 American Thoracic Society Meeting: Chair of Symposium on Airway Inflammation & Host Defense 1998
81. 1997 Invited Presenter 2nd Annual FDA/NIH Conference on Gene Therapy 1997
82. 1997 Invited Speaker National Institutes of Health Gene Therapy Workshop Series, Bethesda, MD 1997
83. 1997 American Thoracic Society: Chairman of Workshop on Respiratory Syncytial Virus 1997
84. 1996 North American CF Conference: Symposium Presentation on New Integrating Viral Vectors 1996
85. 1996 North American CF Conference: Human Gene Therapy Trials Symposium Presentation 1996
86. 1996 American Thoracic Society (ATS) International Conference: Chairman of Symposium on Gene Therapy for Pediatric Lung Disease 1996
87. 1996 American Thoracic Society (ATS) International Conference: Chairman of Workshop on Pathophysiology of Viral Respiratory Tract Infections 1996
88. 1996 North American CF Conference: Plenary Session Presentation on Phase I Trial of AAV-CFTR in Adult CF Patients with Mild Lung Disease 1996
89. 1995 American Thoracic Society (ATS) International Conference: Postgraduate Course Presentation on Gene Therapy and the Lung 1995
90. 1995 American Thoracic Society (ATS) International Conference: Educational Presentation on Molecular Biology for Pulmonary Physicians 1995
91. 1995 Society for Pediatric Research (SPR) Annual Meeting: Symposium on New Therapies for CF 1995
92. 1995 NIDDK Workshop on AAV Vectors: Gene Transfer into Quiescent Cells (Bethesda, MD) 1995
93. 1995 North American CF Conference: Chairman of Roundtable on Surrogate Endpoints for Efficacy in Gene Therapy Clinical Trials 1995

94. 1994 North American CF Foundation (CFF) Conference: 2 Symposium presentations (Viral Vectors and Host-Vector Interactions) 1994
95. 1994 American Thoracic Society (ATS) International Conference: Postgraduate Course Presentation on Mouse Models of Pulmonary Diseases 1994
96. 1993 North American CF Foundation (CFF) Conference: Chairman of Workshop on Viral Vectors for Gene Therapy, 2 Symposium presentations (Viral Vectors and Host-Vector Interactions) 1993

Regional

1. Biogen Presentation. *Clinical Translation of rAAV Gene Therapy,* Boston, MA. 01/29/2020
2. AAMC Annual Meeting *Creating Optimal Environments for Promoting Professionalism,* Phoenix, AZ 11/08/2019
3. The Connecticut Alpha-1 Support Group, *Gene Therapy for Alpha-1 Muscle Delivery and Dual-Function Gene Therapy,* New Britain, CT. 11/11/2017
4. The 2nd Session of the Massachusetts-Quebec Collaborative Research Council (MQCRC), *Genome Editing: Regulation and Ethics,* Massachusetts State House, Boston MA. 08/09/2017
5. The Boston, MA Alpha-1 Education Day, MA, Alpha-1 Liver Disease & Liver Research
6. The Worcester District Medical Society 221st Annual Oration, Society's 2017 Orator, *"A Glass (More than) Half Full: The Top Ten Reasons to be Optimistic about the Next 220 Years of Medicine in Worcester",* Worcester MA. 02/08/2017
7. The Boston Biotech, Genetic Rx Boston 2016 Annual Meeting. Invited Panelist, "Key Collaborations with Academia panel", Harvard Medical School - Joseph B. Martin Conference Center, Boston, MA. 12/08/2016
8. The University of Kansas Medical Center (KUMC) Women in Medicine & Science (WIMS), Keynote Address: "The Mirror: Reflections from Leadership", Kansas City, KS. 08/18/2016
9. 2016 inKNOWvation, Moderator: "The Return of Gene Therapy: Trends and Issues in Gene Therapy", Cambridge, MA. 05/11/2016
10. Louisiana State University, Health Science Center, Medicine Grand Rounds - "Gene Therapy for Genetic Disease Including Genetic Emphysema". New Orleans, LA. 04/15/2016
11. 2016 Alpha Omega Alpha Visiting Professor at Louisiana State University School of Medicine, LSU, AOA Banquet, Guest Speaker for the AOA graduates of LSU medical school's class of 2016, "LSU Reflections", New Orleans, LA. 04/14/2016
12. MHA's 49th Annual Mid-Winter Leadership Forum: Healthcare at the Intersection of Cost and Innovation – Panelist. Framingham, MA. 01/29/2016
13. Boston Biotech Conferences, Genetic Rx Boston, "The Evolving Regulatory Landscape for Genetic Therapies" – Panelist. Boston, MA. 12/03/2015
14. 2013 Educational Symposium "50 Years of Research Towards a Cure for Alpha-1 Antitrypsin Deficiency" University of Massachusetts Medical School in partnership with the Alpha-1 Foundation 2013
15. 2003 Florida Pediatric Society, Florida Chapter of the American Academy of Pediatrics, General Pediatric Update IX Annual Meeting, Lake Buena Vista, FL (*invited guest speaker*) "Top Ten Pediatric Stories of the Year 2003
16. 2002 John W. & Marvelle M. Ridgeway Visiting Scholar, Children's Hospital, Missouri University Health Care 2002

17. 2002 Alpha-1 Foundation Critical Issues Workshop Series, Stem Cell Therapies in Reparative Medicine, Workshop Participant Lecturer — 2002
18. 1999 Southern Society of Pediatric Research: Chair of Workshop on Genetics and Gene Therapy — 1999

Local

1. University of Florida, Florida Genetics 2012, "A Brief History of (AAV) Time AND Gene Therapy for Genetic Emphysema". — 11/28/2012
2. Boston Children's Hospital "Development of Gene Therapy for Single Gene Disorders" Boston, MA. — 07/06/2011
3. Novartis "Gene Therapy Clinical Trials / Cystic Fibrosis and Alpha-1 antitrypsin Deficiency using AAV vectors. — 02/02/2010
4. Boston Children's Hospital "A new outlook on Lung Inflammation in CF" Boston, MA. — 01/11/2010
5. Richard Talamo, M.D. Memorial Lecture, Invited Panelist, Boston, MA. — 11/19/2008
6. Wenzhou Medical College, Wenzhou, China, "Gene Therapy on Eye Diseases. — 11/11/2008
7. Peking Union Medical College, Beijing, China, "Gene Therapy", — 11/07/2008
8. Invited Grand Rounds Speaker – "Adeno-Associated Virus (AAV)-Mediated Gene Therapy for Single Gene Defects: Alpha-1 Antitrypsin Deficiency, CF, and Disorders of Fatty Acid Oxidation", Johns Hopkins University School of Medicine, Baltimore, MD. — 10/25/2006
9. Invited Grand Rounds Speaker – Mt. Sinai School of Medicine, New York, NY. — 10/26/2006
10. Nemours Conference for Primary Care Pediatrics, Amelia Island – 3 invited talks "Metapneumovirus, Bird Flu & Other Emerging Respiratory Pathogens", "Children and Families Living with Cystic Fibrosis", and "Relationship Between Sleep Disordered Breathing & School Problems", — 09/03/2004
11. The Children's Hospital of Philadelphia *(invited guest speaker)* "Preclinical and Phase I clinical trials of AAV-alpha-1 antitrypsin vectors", — 11/21/2005
12. Temple University Lung Center COPD/Alpha-1 Education Day, "Gene Therapy for A1AT Deficiency", Philadelphia, PA. — 10/01/2005
13. Ramathibodi Hospital, Mahidol University, Department of Pediatrics, Division of Pediatric Pulmonary, Bangkok, Thailand: Case Discussion, — 01/19/2005
14. Office of Biotechnology Activities Special Safety Symposium: Invited Speaker — 2001
15. Southern Society for Clinical Investigation: Chairman of Workshop on Gene Therapy — 1997
16. Southern Society for Clinical Investigation: Presidential Mini-Symposium Cystic Fibrosis Gene Therapy — 1997
17. Williamsburg CF Foundation (CFF) Conference: Chairman of Workshop on Preclinical Testing of AAV vectors — 1994

REVIEWER / EDITORIAL RESPONSIBILITIES

1. Editor-in-Chief of *Human Gene Therapy* — 07/01/2015-present
2. Associate Editor of *Human Gene Therapy* — 06/01/2011-07/01/2015
3. Mass. Med. Soc., Publications Committee — 07/1/2011-12/31/2012

4. National Institute of Health College of CSR Reviewers 1/2010-2012
5. Editorial Board, *Genetic Testing* 11/2008-present
6. Ad hoc Reviewer, *CHEST* 2007-present
7. Ad hoc Reviewer, *The Lancet* 2007-present
8. University of Florida Shands Cancer Center, Senior Investigator Awards, Reviewer 2005
9. Permanent Charter member NIH Study Section: Gene Drug Delivery Systems 07/1/2004-06/30/2008
10. Ad hoc Reviewer NIH Study Section: Gene Drug Delivery Systems 07/1/2004-06/30/2008
11. Editorial Board, Gene Vaccines & Therapy 12/2002-present
12. Editorial Board, Current Gene Therapy 2002
13. Ad hoc Reviewer NIH Lung Biology Study Section Sub-committee for review of grants excluded due to conflicts of interest- 3/2001
14. Ad hoc Reviewer, *Nature Biotechnology* 2001
15. Editorial Board, *Gene Therapy* 11/2000-2004
16. Ad hoc Reviewer NINDS Program Project Review 10/29/1998
17. Editorial Board, *Human Gene Therapy* 07/1996-07/1998 & 01/2005-present
18. Editorial Board, *Biodrugs* 11/1996-2000
19. Ad hoc Reviewer, *Journal of Respiratory Diseases* 1996-present
20. Ad hoc Reviewer, *Gene Therapy* 1995-11/2000
21. Ad hoc Reviewer NHLBI Special Emphasis Panel: Cardiovascular Gene Therapy 03/1998 & 12/2/1998
22. Ad hoc Reviewer NINDS Program Project Review 10/29/1998
23. Ad hoc Reviewer NIA Program Project Grant Site Visit 04/30/1996-05/1/1996
24. Cystic Fibrosis Foundation Research and Training Grant Review Committee 6/1995-present
25. Ad hoc Reviewer NIH/NHLBI Program Project Grant Site Visit 05/11/1994-05/12/1994
26. Ad hoc Reviewer NHLBI Study Section: Hemophilia Gene Therapy RFA 07/12/1994-07/13/1994
27. Ad hoc Reviewer NIH General Clinical Research Center Site Visits 04/1/1993-04/2/1993 & 8/21/1995
28. Ad hoc Reviewer, *Archives of Pediatric and Adolescent Medicine* 1992-present
29. Ad hoc Reviewer, *New England Journal of Medicine* 1992

EXTERNAL PROFESSIONAL SERVICE

Committees (Current)

1. UMass Memorial Health Care Corporation Board of Trustees member 2007-present

2. UMass Memorial Health Care Corporation Board of Trustees Chair of the Academic Integration Committee, This is the governing board of a $2.6 billion health care corporation that is UMMS' primary teaching affiliate. 2013-present

National Committees (Past)

1. FDA Advisory Committee Meeting of CBER, Cellular, Tissue and Gene Therapies Advisory Committee (CTGTAC), October 12, 2017 10/12/2017
2. Respiratory and GI Tract Gene & Cell Therapy Committee of the American Society of Gene & Cell Therapy (ASGCT) member (2011-2017) 2011-2017
3. GWIMS Steering Committee, Association of American Medical Colleges (AAMC) Council of Deans Liaison 2016-2021
4. Advisory Council of the American Society of Gene & Cell Therapy (ASGCT) Member. This is one of two major oversight boards for the professional society of approximately 2,000 gene therapy scientists in the US and Europe 2011-2016
5. American Board of Medical Specialties (ABMS), Special Committee on Physician Scientists 09/01/2014-09/01/2010
6. Liaison Committee on Medical Education (LCME) member; The LCME is the primary accrediting body for US allopathic medical schools 07/01/2012-07/01/2016

   LCME Subcommittee on Accreditation Standards
7. MDA Gene Therapy Trial Oversight Steering Committee 2007-2010
8. American Board of Pediatrics, Member 2006-present
9. Society for Pediatric Research Young Investigator's Award Selection Committee 2006-2009
10. The American Board of Pediatrics, Credentials Committee Sub-board 2005-2008
11. Association of Medical School Pediatric Department Chairs Executive Committee 2005-2007
12. American Board of Pediatrics, Associate Member, Pulmonary Committee Sub-Board 2003-2008
13. Association of Medical School Pediatric Department Chairs, Inc. 06/2002-04/2007
14. ASGT Clinical and Regulatory Affairs Committee, Chair 06/2002-06/2006
15. National Gene Vector Laboratory Steering Committee 11/2001-03/2006
16. American Society of Gene Therapy Clinical and Regulatory Affairs Committee 05/1999-06/2006
17. Cystic Fibrosis Foundation Research and Training Grant Review Committee 06/1995-present
18. Advisory Council for Partner's in Care-Together for Kids Program 2006
19. American Society of Gene Therapy Program Committee 03/1997-03/2001
20. American Society of Gene Therapy Scientific Committee on Cystic Fibrosis & Lung Diseases 03/1997-06/2000
21. Chairman of AAV Working Group of FDA Rare Diseases Initiative 07/1998-05/1999
22. American Thoracic Society National Pediatric Assembly Program Committee 05/1995-05/1998
23. Abstract Review Committee for North American CF Conference 1993 & 1995

Committees (Past)

1. Massachusetts Society for Medical Research, Board of Directors 2013-2015
2. Massachusetts Medical Society, Medical Examiners and Health Professionals Task Force 2012

3.	Massachusetts Medical Society, Publications Committee	2011- 2012
4.	Commonwealth of Massachusetts Medico-Legal Commission: an appointment by the governor's office	2007-2012
5.	Greater Worcester Community Foundation	2007-2009
6.	Chancellor's Search Committee	2007-2008
7.	Medical Claims Committee, UMass Memorial Health Care	2007-2008
8.	Task Force Committee for UF, Office of the Provost & Senior Vice President, Chairman	2006
9.	UF Department of Urology Chair Search Committee, Chairman	2005-2006
10.	McKnight Brain Institute Director Search Committee	2004
11.	University of Florida Institutional Biosafety Committee (IBC)	1996-2002
12.	University of Florida Faculty Research Advisory Board	1996-1998
13.	Maryland Thoracic Society Research Planning Committee	1993-1996
14.	Johns Hopkins Pediatric Residency Curriculum Committee	1994-1996