CURRICULUM VITAE

THOMAS WILLIAM FERKOL, JR., M.D.

DATE: November 10, 2010

PLACE OF BIRTH Würzburg, Germany

CITIZENSHIP USA

OFFICE ADDRESS Department of Pediatrics, Campus Box 8208

Washington University School of Medicine

One Children's Place St. Louis, MO 63110

PRESENT POSITION Professor of Pediatrics, Cell Biology and Physiology

Director, Division of Allergy, Immunology, and Pulmonary Medicine

EDUCATION

1977-1981	Bachelor of Arts, Biology and Psychology, Case Western Reserve University, Cleveland, Ohio (<i>magna cum laude</i>).
1979-1980	Biology Honours, University of Nottingham, School of Biological Sciences, Nottingham, Great Britain.
1981-1985	<i>Medicinae doctor</i> , The Ohio State University College of Medicine, Columbus, Ohio.
1985-1986	Intern, Department of Pediatrics, North Carolina Memorial Hospital, University of North Carolina at Chapel Hill, Chapel Hill, North Carolina.
1986-1988	Resident, Department of Pediatrics, North Carolina Memorial Hospital, University of North Carolina at Chapel Hill, Chapel Hill, North Carolina.
1988-1989	Chief Resident, Department of Pediatrics, North Carolina Memorial Hospital, College of Medicine, University of North Carolina at Chapel Hill, Chapel Hill, North Carolina.
1989-1992	Pulmonology Fellow, Pediatric Pulmonology, Rainbow Babies and Children's Hospital, Case Western Reserve University School of Medicine, Cleveland, Ohio.

ACADEMIC POSITIONS AND EMPLOYMENT

1988-1989	Clinical Instructor, Department of Pediatrics, North Carolina Memorial Hospital, College of Medicine, University of North Carolina at Chapel Hill, Chapel Hill, North Carolina.
1992-1994	Instructor, Division of Pediatric Pulmonology, Department of Pediatrics, Rainbow Babies and Children's Hospital, Case Western Reserve University School of Medicine, Cleveland, Ohio.
1994-2000	Assistant Professor, Division of Pediatric Pulmonology, Department of Pediatrics, Rainbow Babies and Children's Hospital, Case Western Reserve University School of Medicine, Cleveland, Ohio.

2000-2008 Associate Professor (tenured), Division of Allergy and Pulmonary Medicine, Department of Pediatrics, Washington University School of Medicine, St. Louis, Missouri. 2000-2008 Associate Professor, Department of Cell Biology and Physiology, Washington University School of Medicine, St. Louis, Missouri. Division of Biology and Biological Sciences (DBBS), Washington University 2007-present School of Medicine, St. Louis, Missouri. 2009-present Professor (tenured), Department of Pediatrics, Division of Allergy, Immunology, and Pulmonary Medicine, Washington University School of Medicine, St. Louis, Missouri. Professor, Department of Cell Biology and Physiology, Washington University 2009-present School of Medicine, St. Louis, Missouri.

UNIVERSITY AND HOSPITAL APPOINTMENTS

1989	Attending physician, North Carolina Memorial Hospital, University of North Carolina at Chapel Hill, Chapel Hill, North Carolina.
1992-2000	Teaching attending physician, Department of Pediatrics, Rainbow Babies and Children's Hospital, Cleveland, Ohio.
1992-2000	Bronchoscopy physician, Division of Pediatric Pulmonology, Department of Pediatrics, Rainbow Babies and Children's Hospital.
1992-2000	Attending physician, Division of Pediatric Pulmonology, Rainbow Babies and Children's Hospital.
2000-present	Attending physician, Division of Allergy and Pulmonary Medicine, Washington University School of Medicine, St. Louis, Missouri.
2000-present	Attending physician, Barnes-Jewish Hospital, Washington University School of Medicine, St. Louis, Missouri.
2000-present	Director, Cystic Fibrosis Center, Washington University School of Medicine.
2000-present	Director, Pediatric Pulmonary Fellowship Program, Department of Pediatrics, St. Louis Children's Hospital, Washington University School of Medicine.
2003-2007	Co-director, CFF Therapeutics Development Center, Washington University School of Medicine.
2006-present	Director, Division of Pediatric Allergy, Immunology, and Pulmonary Medicine, Washington University School of Medicine, St. Louis, Missouri
2007-present	Director, Pediatric Pulmonary Function Laboratory, Washington University School of Medicine, St. Louis, Missouri.
2008-present	Consulting physician, Rankin-Jordan Subspecialty Hospital for Children, Maryland Heights, Missouri.

LICENSURE AND BOARD CERTIFICATION

National Board of Medical Examiners, Diplomate

American Board of Pediatrics, General Pediatrics Diplomate, Certified, 1990

American Board of Pediatrics, General Pediatrics Diplomate (Re-certified, 1998, 2005)

American Board of Pediatrics, Pediatric Pulmonology Subboard Diplomate, Certified, 1994

American Board of Pediatrics, Pediatric Pulmonology Subboard (Re-certified, 2002)

US Drug Enforcement Agency, Certified

Medical License, State of Missouri (current)

Medical License, State of Ohio (expired voluntarily)

Medical License, State of North Carolina (expired voluntarily)

INDIVIDUAL HONORS AND AWARDS

1989 Floyd Denny Pediatric Alumni Society Award, Department of Pediatrics, University of North Carolina at Chapel Hill, Chapel Hill, North Carolina.

1989	Cystic Fibrosis Foundation LeRoy Matthews Physician-Scientist Award.
1992	Rainbow Babies and Children's Hospital Trustees New Investigator Award.
1994	American Lung Association Edward Livingston Trudeau Scholar.
1994	Teaching Excellence Award, Department of Pediatrics, Case Western Reserve
	University School of Medicine, Cleveland, Ohio.
1997	Society for Pediatric Research, member.
1998-	Best Doctors of America. W
1999	EDI Innovation Award.
2004	European Cystic Fibrosis Conference Presentation Award.
2006-	America's Top Pediatricians, Consumer's Research Council of America.
2009-	American Pediatric Society, member.

DIVISIONAL HONORS AND AWARDS

2007	Child Magazine Best Children's Hospitals (2 nd in respiratory diseases)
2008	US News and World Report, Best Children's Hospitals (12 th in respiratory diseases)
2009	US News and World Report, Best Children's Hospitals (8 th in respiratory diseases)
2009	Parent Magazine Best Children's Hospitals (4 th in respiratory diseases)
2009	Pediatric Housestaff Teaching Award
2009	Cystic Fibrosis Foundation's Quality Care Award
2010	US News and World Report, Best Children's Hospitals (7 th in respiratory diseases)

REVIEWER AND CONSULTANCIES

Granting Agencies

National Institutes of Health, Gene and Drug Delivery (GDD) study section, 2003-2005, 2008 National Institutes of Health (NCI), temporary reviewer

National Institutes of Health, Medical Biochemistry study section, temporary reviewer

National Institutes of Health, Gene Therapy/Inborn Errors study section, temporary reviewer

American Thoracic Society Career Development Grants Review Committee 2007-2008

Cystic Fibrosis Foundation Research Development Program Review Committee

Cystic Fibrosis Foundation Functional Genomics Review Committee, ad hoc reviewer

Cystic Fibrosis Trust (UK), ad hoc reviewer

German Federal Ministry for Education and Research (BMBF), ad hoc reviewer

L'Agence nationale de la recherche (France), ad hoc reviewer

Mukoviszidose e.V. (Germany) ad hoc reviewer

Italian Cystic Fibrosis Foundation, ad hoc reviewer

Health Research Board (Ireland), ad hoc reviewer

Michael Smith Foundation for Health Research (Canada), ad hoc reviewer

Professional Societies and Conferences

Abstracts and presentations, American Thoracic Society Conference

Abstracts and presentations, North American Cystic Fibrosis Conference.

Abstracts and presentations, Pediatric Academic Societies Annual Meeting

Abstracts and presentations, Society for Gene Therapy Annual Meeting

Institutional programs

University of Iowa, Center for Gene Therapy of Cystic Fibrosis and Other Genetic Diseases, external reviewer

Case Western Reserve University Cystic Fibrosis Research and Development Program, external reviewer

University of Washington Cystic Fibrosis Research and Development Program, external reviewer

University of Florida, General Clinical Research Center, external reviewer

University of Florida, Molecular Therapy Core Center, external advisor

Pharmaceutical Companies

Glycomimetics, Medical Consultant (past)
Kalobios, Medical Advisory Board (past)
Novartis, Cystic Fibrosis Medical Advisory Board (past)
Genentech, Speakers Bureau (past)

Journals

Annals of Internal Medicine American Journal of Physiology Biochimica et Biophysica Acta

Journal of Biological Chemistry

Journal of Clinical Investigation

American Journal of Respiratory Cell and Molecular Biology

American Journal of Respiratory and Critical Care Medicine

American Journal of Physiology

European Respiratory Journal

Experimental Lung Research

Human Gene Therapy

Human Molecular Genetics

Gene Therapy

Infection and Immunity

Journal of Cystic Fibrosis

Journal of Leukocyte Biology

Journal of Pediatrics

Lancet

Molecular Medicine

Molecular Therapeutics

New England Journal of Medicine

Pediatrics

Paediatrics and Child Health

Pediatric Pulmonology

Pharmaceuticals

Proceedings of the American Thoracic Society

Respiration

PROFESSIONAL SOCIETIES, ORGANIZATIONS, AND COMMITTEES

American Thoracic Society 1997-present

Pediatric Assembly Program Committee 2000-2008.

Pediatric Assembly Program Committee Chair-elect, 2006-2007

Pediatric Assembly Program Committee Chair, 2007-2008.

Planning Committee, International Conference 2007-present

Pediatric Assembly, Chair-elect, 2008-2009

Health Policy Committee, 2008-2009

Pediatric Assembly, Chair, 2009-2011

Board of Directors, 2009-2011

American Board of Pediatrics, Member, Subboard of Pediatric Pulmonology 2011-2016.

Pediatric Pulmonary Training Directors Association (PEPTDA) 2000-present

PEPTDA Chair, 2007-2009.

Division of Lung Diseases, NHLBI/NIH Respiratory and Critical Care Training Workshop, 2009

Program Planning Committee, North American Cystic Fibrosis Conference, 2006-2007

Society for Pediatric Research, 1997-present

American Pediatric Society, 2010-present.

Primary Ciliary Dyskinesia Foundation Medical and Scientific Advisory Board 2005-present

Midwest Cystic Fibrosis Center Consortium, co-organizer

State of Missouri Department of Health and Senior Services, Cystic Fibrosis Standing Committee, 2003-present

State of Missouri Department of Health and Senior Service, Neonatal Screening Standing Committee, 2004-present

Heartland Genetics and Newborn Screening Collaborative Advisory Council, 2005-2007

Cystic Fibrosis Foundation Newborn Screening Special Interest Group

Medical Advisor, Gateway Chapter, Cystic Fibrosis Foundation, 2000-present

Association of Pediatric Program Directors, 2006-present

Fellow, American Academy of Pediatrics

UNIVERSITY AND HOSPITAL COMMITTEES

Department of Pediatrics, University of North Carolina at Chapel Hill:

1987-1989	Housestaff Selection Committee, Department of Pediatrics, North Carolina
	Memorial Hospital, University of North Carolina at Chapel Hill.
1988-1989	Education Committee, Department of Pediatrics, North Carolina Memorial
	Hospital, University of North Carolina at Chapel Hill.

Department of Pediatrics, Case Western Reserve University

1994-1995	Pediatric Resuscitation Committee, Department of Pediatrics, Rainbow
	Babies and Children's Hospital.
1994-2000	Intern Selection Committee, Department of Pediatrics, Rainbow Babies and
	Children's Hospital.
1995-2000	Education Committee, Department of Pediatrics, Rainbow Babies and
	Children's Hospital.

Department of Pediatrics, Washington University in St Louis

2004-present	Graduate Medical Education Committee Internal Reviews, Washington
·	University School of Medicine (ad hoc reviewer).
2005 2006	Newborn Corponing Working Croup Department of Redistrice St. Louis

2005-2006 Newborn Screening Working Group, Department of Pediatrics, St. Louis

Children's Hospital, Washington University School of Medicine.

INVITED PROFESSORSHIPS AND LECTURES

Intramural

Pediatric Grand Rounds, Department of Pediatrics, North Carolina Memorial Hospital, University of North Carolina at Chapel Hill, Chapel Hill, North Carolina, May, 1989.

Pediatric Grand Rounds, Department of Pediatrics, Rainbow Babies and Children's Hospital, Case Western Reserve University, Cleveland, Ohio, May, 1990.

Pediatric Grand Rounds, Department of Pediatrics, Rainbow Babies and Children's Hospital, Case Western Reserve University, Cleveland, Ohio, September, 1992.

Seminar, Department of Biochemistry, Case Western Reserve University, Cleveland, Ohio, September, 1993.

Seminars in Hematology/Oncology, Department of Internal Medicine, University Hospitals of Cleveland, Case Western Reserve University, Cleveland, Ohio, December, 1993.

Pediatrics Today, Department of Pediatrics, Rainbow Babies and Children's Hospital, Case Western Reserve University, Cleveland, Ohio, December, 1995.

Pediatric Research Conference, Department of Pediatrics, Rainbow Babies and Children's Hospital, Case Western Reserve University, Cleveland, Ohio, January, 1996.

Grand Rounds, Center for Human Genetics, Case Western Reserve University, Cleveland, Ohio, September, 1996.

Carl F. Doershuk Symposium, Department of Pediatrics, Rainbow Babies and Children's Hospital, Case Western Reserve University, Cleveland, Ohio, April, 1997.

- Pediatric Research Conference, Department of Pediatrics, Rainbow Babies and Children's Hospital, Case Western Reserve University, Cleveland, Ohio, June, 1997.
- Grand Rounds, Department of Pediatrics, Rainbow Babies and Children's Hospital, Case Western Reserve University, Cleveland, Ohio, March, 1999.
- Pediatric Research Conference, Department of Pediatrics, Rainbow Babies and Children's Hospital, Case Western Reserve University, Cleveland, Ohio, April, 1999.
- Pediatric Research Conference, Department of Pediatrics, Rainbow Babies and Children's Hospital, Case Western Reserve University, Cleveland, Ohio, January, 2000.
- Pulmonary and Critical Care Medicine Grand Rounds, Department of Internal Medicine, Barnes-Jewish Hospital, Washington University, St Louis, Missouri, December, 2000.
- Pediatric Research Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, January, 2001.
- Pediatric Research Retreat, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, February, 2001.
- Grand Rounds, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, February, 2001.
- Pediatric Intern Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, March, 2001.
- Early Bird Rounds, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, March, 2001.
- Case Management Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, September, 2001
- Pediatric Research Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, October, 2001.
- Mini-Medical School, Washington University, St Louis, Missouri, November, 2001
- Grand Rounds, Department of Obstetrics and Gynecology, Barnes-Jewish Hospital, Washington University, St Louis, Missouri, November, 2001.
- Pulmonary and Critical Care Medicine Grand Rounds, Department of Internal Medicine, Barnes-Jewish Hospital, Washington University, St Louis, Missouri, November, 2001.
- Pediatric Intern Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, January, 2002.
- Case Management Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, April, 2002.
- Early Bird Rounds, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, April, 2002.
- Case Management Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, August, 2002.
- Pediatric Research Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, October, 2002.
- Mini-Medical School, Washington University, St Louis, Missouri, October, 2002.
- Women and Children's Health Clerkship Lecture Series, Department of Obstetrics and Gynecology, Barnes-Jewish Hospital, Washington University, January, 2003.
- Organizer and speaker, Cystic Fibrosis Parents Night Out, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, March, 2003.
- Organizer and speaker, Cystic Fibrosis Parents Night Out, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, September, 2003.
- Mini-Medical School, Washington University, St Louis, Missouri, October, 2003.
- Pediatric Research Conference, St. Louis Children's Hospital, Washington University, St. Louis, Missouri, October. 2003.
- Early Bird Rounds, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, February, 2004.
- Pediatric Oriented Research Unit (PORU) Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, June, 2004.
- Case Management Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, August, 2004.

- Pediatric Research Conference, St. Louis Children's Hospital, Washington University, St. Louis, Missouri, October. 2004.
- Organizer, Cystic Fibrosis Parents Night Out, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St. Louis, Missouri, October. 2004.
- Lecturer, Infectious Disease Fellowship Lecture Series, Departments of Internal Medicine and Pediatrics, Barnes-Jewish Hospital, Washington University, December, 2004.
- Organizer and speaker, Cystic Fibrosis Parents Night Out, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St. Louis, Missouri, June, 2005.
- Pediatric Research Conference, St. Louis Children's Hospital, Washington University, St. Louis, Missouri, October, 2005.
- Lung Biology Conference, Department of Internal Medicine, Barnes-Jewish Hospital, Washington University, St Louis, Missouri, February, 2006.
- Organizer, Clinical Pediatric Update, *Breathless: A Clinical Review of Pediatric Allergic and Pulmonary Diseases*, St Louis Children's Hospital, St. Louis, Missouri, March, 2006.
- Grand Rounds, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, April, 2006.
- Case Management Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, July, 2006.
- All Resident Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, July, 2007
- Case Management Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, October, 2007.
- Frontiers in Human Pathobiology Lecture Series, Washington University, St. Louis, Missouri, March, 2008.
- Pulmonary and Critical Care Medicine Grand Rounds, Department of Internal Medicine, Barnes-Jewish Hospital, Washington University, St. Louis, Missouri, March, 2008.
- All Resident Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, August, 2008.
- Case Management Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, May, 2009.
- Organizer, Clinical Pediatric Update, *Clinical Review of Pediatric Allergic and Pulmonary Diseases*, St Louis Children's Hospital, St. Louis, Missouri, May, 2009.
- All Resident Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, August, 2009.
- Case Management Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, December, 2009.
- Grand Rounds, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, May, 2010.
- All Resident Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University, St Louis, Missouri, August, 2010.

Extramural

- Invited speaker, Cystic Fibrosis Foundation Conference. Toward New Therapeutic Treatments for Cystic Fibrosis: Gene Therapy, Williamsburg, Virginia. June, 1991.
- Invited speaker, Cystic Fibrosis Foundation Conference. Toward New Therapeutic Treatments for Cystic Fibrosis: Gene Therapy, Williamsburg, Virginia. June, 1992.
- Moderator, Workshop Session, Seventh Annual North American Cystic Fibrosis Foundation Conference. October, 1993.
- Invited speaker, Seventh Annual North American Cystic Fibrosis Foundation Conference. October, 1993.
- Invited speaker, Cystic Fibrosis Foundation Conference. Workshop on Gene Therapy in Cystic Fibrosis, Williamsburg, Virginia. June, 1994.
- Invited seminar, Division of Chest Medicine, Department of Pediatrics, The Hospital for Sick Children, Toronto, Canada. September, 1994.

- Invited seminar, Division of Pediatric Pulmonology, Department of Pediatrics, Cincinnati Children Hospital, Cincinnati, Ohio. September, 1995.
- Moderator, Roundtable, Ninth Annual North American Cystic Fibrosis Foundation Conference, October, 1995.
- Invited speaker, Gene Therapy for Acquired Diseases, Vanderbilt University, Nashville, Tennessee. October, 1995.
- Invited seminar, Howard Hughes Medical Center, University of Iowa, Iowa City, Iowa. December, 1995.
- Invited speaker, Cystic Fibrosis Foundation Conference: Workshop on CFTR Function, Model Systems, Williamsburg, Virginia. June, 1997.
- Invited seminar, Department of Pediatrics, Stanford University, Palo Alto, California. March, 1997.
- Moderator, Workshop Session, Eleventh Annual North American Cystic Fibrosis Foundation Conference. October, 1997.
- Invited speaker, Eleventh Annual North American Cystic Fibrosis Foundation Conference. October, 1997.
- Invited speaker, Keystone Symposia on Cellular and Molecular Biology, "Non-viral Methods of Gene Transfer." January, 1998.
- Invited seminar, Division of Pediatric Pulmonology, Department of Pediatrics, Cincinnati Children's Hospital, Cincinnati, Ohio. March, 1998.
- Invited speaker, American Thoracic Society International Conference, Chicago, Illinois. April, 1998
- Invited speaker, Cystic Fibrosis Foundation Williamsburg Conference, Williamsburg, Virginia. June, 1998.
- Invited speaker, Great Lakes Lung Conference, Niagra-on-the-Lake, Canada. September, 1998.
- Visiting professor, Grand Rounds, Department of Pediatrics, All Children's Hospital, Tampa, Florida. November, 1998.
- Invited speaker, Cystic Fibrosis Family Day, Department of Pediatrics, All Children's Hospital, Tampa, Florida. November, 1998.
- Invited seminar, Howard Hughes Medical Center, University of Iowa, Iowa City, Iowa. February, 1999.
- Invited speaker, Cystic Fibrosis Awareness Day, Department of Pediatrics, Washington University, St. Louis, Missouri. April, 1999.
- Invited speaker, Cystic Fibrosis Foundation Williamsburg Conference, Williamsburg, Virginia. June, 1999.
- Invited speaker, Department of Pediatrics, Washington University, St. Louis, Missouri. July, 1999.
- Moderator, Workshop Session, Thirteenth Annual North American Cystic Fibrosis Foundation Conference. October, 1999.
- Invited speaker, Ohio Regional Cystic Fibrosis Conference. Cincinnati, Ohio. April, 2000. Session chair, Society for Pediatric Research Conference, Baltimore, Maryland. May, 2000.
- Moderator, Workshop Session, Fourteenth Annual North American Cystic Fibrosis Foundation Conference. Baltimore, Maryland. November, 2000.
- Invited speaker and session chair, American Thoracic Society International Conference, San Francisco, California. May, 2001
- Invited speaker and session chair, Cystic Fibrosis Foundation Williamsburg Conference, Williamsburg, Virginia. June, 2001.
- Invited seminar, Department of Pediatrics, University of Michigan. Ann Arbor, Michigan. July, 2001.
- Invited speaker, Infection and Inflammation in the Cystic Fibrosis Lung, Evening Satellite Symposium, Fifteenth Annual North American Cystic Fibrosis Conference, Orlando, Florida. October, 2001.

- Visiting professor, Department of Biochemistry, University of Nebraska Medical Center. Omaha, Nebraska. December, 2001.
- Invited seminar, Department of Pediatrics, Indiana University. Indianapolis, Indiana. May, 2002.
- Session moderator and speaker, Workshop, Sixteenth Annual North American Cystic Fibrosis Foundation Conference. New Orleans, Louisiana. October, 2002.
- Symposium chair and speaker, American Thoracic Society International Conference, Seattle, Washington. May, 2003.
- Session chair and moderator, Cystic Fibrosis Foundation Williamsburg Conference, Williamsburg, Virginia. June, 2003.
- Invited speaker, Symposium, Seventeenth Annual North American Cystic Fibrosis Foundation Conference. Anaheim, California, October, 2003.
- Visiting professor, Grand Rounds, Department of Pediatrics, University of California, Los Angeles. Los Angeles, California, January, 2004.
- Grand Rounds, Department of Pediatrics, Cardinal Glennon Hospital for Children, St Louis University. St Louis, Missouri. February, 2004.
- Visiting scientist, Enzon Inc, Piscataway, New Jersey, February, 2004.
- Session chair, Society for Pediatric Research Conference, San Francisco, California. May, 2004.
- Visiting professor, Grand Rounds, Department of Pediatrics, University of Florida, Gainesville, Florida, May, 2004.
- Co-organizer and speaker, Workshop, Primary Ciliary Dyskinesia, American Thoracic Society International Conference, Orlando, Florida. May, 2004.
- Oral poster presentations, Twenty-seventh Annual European Cystic Fibrosis Conference, Birmingham, England. June, 2004 (awarded Best Poster Presentation, ECFC)
- Moderator, Cystic Fibrosis Foundation Virtual Family Day, June, 2004.
- Session moderator, Workshop, Eighteenth Annual North American Cystic Fibrosis Foundation Conference, St. Louis, Missouri. October, 2004.
- Visiting professor, Grand Rounds, Departments of Internal Medicine and Genetics, Louisiana State University, New Orleans, Louisiana. December, 2004.
- Chair and speaker, CME Lecture Series. *Inflammation in the Cystic Fibrosis Lung*. Chicago, Illinois, St. Louis, Missouri, and San Francisco, California, January-February, 2005.
- Visiting professor, Symposium, European Graduate College, *Pseudomonas: Pathogenicity and Biotechnology*. Medizinische Hochschule Hannover, Hanover, Germany. February, 2005.
- Visiting professor, Symposium, European Graduate College, *Pseudomonas: Pathogenicity and Biotechnology*. Danmarks Tekniske Universitet, Copenhagen, Denmark. February, 2005
- Invited lecturer, Pediatric Year in Review Symposium, American Thoracic Society International Conference, San Diego, California. May, 2005.
- Speaker, Diagnosis and Management of Primary Ciliary Dyskinesia Committee, American Thoracic Society International Conference, San Diego, California. May, 2005
- Invited lecturer, Eastern Ontario Children's Hospital, Ottawa, Ontario. September, 2005.
- Invited lecturer, The Hospital for Sick Children, Toronto, Ontario. September, 2005.
- Moderator, Workshop Session, Nineteenth Annual North American Cystic Fibrosis Foundation Conference. October, 2005.
- Invited lecturer, University of British Columbia, Vancouver, British Columbia, Canada, November, 2005.
- Invited lecturer, University of Manitoba, Winnipeg, Manitoba. November, 2005.
- Visiting professor, McGill University, Montreal, Quebec, Canada, January, 2006.
- Invited lecturer, Dalhousie University, Halifax, Nova Scotia, Canada, January, 2006.
- Invited lecturer, Miami Children's Hospital, Forty-first Annual Pediatric Postgraduate Course, Miami, Florida. February, 2006.

- Invited lecturer, Pulmonary and Allergy Fellows Symposia, American Thoracic Society (ATS) and American Academy of Allergy, Asthma, and Immunology (AAAAI), San Diego, California. May, 2006.
- Invited lecturer, American Thoracic Society International Conference, San Diego, California. May, 2006.
- Invited lecturer, Clinical Advances in Pediatrics, Children's Mercy Hospital, Kansas City, Missouri, November, 2006.
- Invited lecturer, Pulmonary and Allergy Fellows Symposia, American Thoracic Society (ATS) and American Academy of Allergy, Asthma, and Immunology (AAAAI), San Francisco, California. May, 2007.
- Moderator, American Thoracic Society International Conference, San Francisco, California. May, 2007.
- Speaker, CME program: Practicing in a New Era of Expanded Newborn Screening and Preventive Medicine, as Illustrated by the Application of Molecular Biology to the Diagnosis and Treatment of Cystic Fibrosis, St. Louis, Missouri. December, 2007.
- Visiting professor, University of California, San Diego, San Diego, California, March, 2008. Invited lecturer, Pulmonary and Allergy Fellows Symposia, American Thoracic Society (ATS) and American Academy of Allergy, Asthma, and Immunology (AAAAI), Toronto, Canada, May, 2008.
- Moderator, American Thoracic Society International Conference, Toronto, Ontario. May, 2008.
- Co-chair, Outstanding Science Session, American Thoracic Society International Conference. Toronto, Ontario. May, 2008.
- Visiting professor and lecturer, Cotton Club, University of Colorado. Breckenridge, Colorado, April, 2009.
- Lecturer, *Newborn Screening for Cystic Fibrosis*, Heartland Genetics and Newborn Screening Collaborative Conference, Jefferson City, Missouri, April, 2009.
- Moderator, American Thoracic Society International Conference, San Diego, California, May, 2009.
- Invited speaker and moderator, European Respiratory Society Annual Congress, Vienna, Austria, September, 2009.
- Invited symposium speaker, Twenty-third Annual North American Cystic Fibrosis Foundation Conference. Minneapolis, Minnesota, October, 2009.
- Visiting lecturer, National Jewish Health, University of Colorado. Denver, Colorado, October. 2009.
- Moderator, American Thoracic Society International Conference, New Orleans, Louisiana, May, 2010.
- Moderator, European Respiratory Society Annual Congress, Barcelona, Spain, September, 2010.
- Moderator and lecturer, *Primary Ciliary Dyskinesia and Overlapping Syndromes*, St. Louis, Missouri, September 2010.

RESEARCH SUPPORT

A. Governmental

A. Governmenta	
2006-2011	National Institutes of Health (HL082657). Title: Delivering antiproteases to
	the bronchiectatic airway. Position: Principal Investigator. Effort: 25%.
	Annual Direct Costs: \$200,000.
2004-2014	National Institutes of Health (HL096458) Title: Genetic disorders of
	mucociliary clearance. Position: Site Principal Investigator (Principal
	Investigator: Michael Knowles, M.D.). Effort: 10%. Annual Direct Costs:
	\$937,768. Site Budget: \$86,753.
2010-2015	National Institutes of Health (HL101465). Title: Influence of the enteric
	microbiome on the genesis of bronchopulmonary dysplasia. Position: Co-
	principal investigator. Effort: 5%. Annual Direct Costs: \$350,000.

State of Missouri. Title: *Cystic Fibrosis Newborn Screening*. Position: Director. Effort: 2%. Annual Direct Costs: \$64,056. 2006-present

B. Non-Governmental

D. NOII-COVCIIII	III III III III III III III III III II
2008-2010	Children's Discovery Institute. Title: <i>Gene discovery and treatment of primary ciliary dyskinesia</i> . Position: Principal Investigator. Effort: 5%. Annual Direct Costs: \$100,000.
2008-2011	Children's Discovery Institute. Title: Functional, structural, and genetic characterization of ciliopathies. Position: Principal Investigator. Effort: 5%.
2009-2011	Annual Direct Costs: \$200,000. Cystic Fibrosis Foundation. Title: <i>FDG-PET imaging in young cystic fibrosis patients</i> . Position: Principal Investigator. Effort: 10%. Annual Direct Costs: \$100,000.
2008-2011	Cystic Fibrosis Foundation. Title: Characterization of MRSA infection and colonization in cystic fibrosis. Position: Site Principal Investigator. (Principal Investigator: Marianne Muhlebach, M.D.). Effort: 2%. Annual Direct Costs:
0000	\$150,000.

2000-present Cystic Fibrosis Foundation. Title: Cystic Fibrosis Center Grant. Position: Codirector. Effort: 5%. Annual Direct Costs (current): \$215,760.

C. Previous Support		
1989-1995	Cystic Fibrosis Foundation. Title: LeRoy Matthews Physician-Scientist	
	Award. Position: Investigator. Annual Direct Costs: \$75,000.	
1994-1995	American Lung Association Research Grant. Title: Receptor-mediated	
	transfer of the human alpha ₁ -antitrypsin gene. Position: Principal	
	Investigator. Annual Direct Costs: \$25,000.	
1991-1996	National Institutes of Health (DK43999). Title: Targeting genes to cells	
	relevant to cystic fibrosis. Position: Co-Investigator (Principal Investigator,	
	Pamela B. Davis, M.D., Ph.D.). Annual Direct Costs: \$118,350.	
1994-1996	National Institutes of Health (DK27651). Title: Receptor-mediated gene	
	transfer into airway cells. Position: Co-Investigator (Principal Investigator,	
	Pamela B. Davis, M.D., Ph.D.). Annual Direct Costs: \$159,997.	
1995-1997	Cystic Fibosis Foundation. Title: Engineering a protein complex for receptor-	
	mediated gene transfer. Position: Co-Investigator (Principal Investigator,	
	Pamela B. Davis, M.D., Ph.D.). Annual Direct Costs: \$50,000.	
1995-1997	Cystic Fibrosis Foundation. Title: Modulation of host responses to gene	
	delivery systems. Position: Co-Investigator (Principal Investigator, Michael	
	Tosi, M.D.). Annual Direct Costs: \$50,000.	
1996-1998	National Institutes of Health (P30DK27651). Feasibility Project Title:	
	Targeting alpha ₁ -antitrypsin to the airways. Position: Principal Investigator.	
	Annual Direct Costs: \$40,000.	
1994-1998	National Institutes of Health (HL53672). Title: Gene therapy of hemophilias.	
	Position: Co-Investigator (Principal Investigator, Richard W, Hanson, Ph.D.).	
	Annual Direct Costs: \$233,000.	
1994-1999	National Institutes of Health (DK48996). Title: Gene transfer into the	
	respiratory tract in vivo. Position: Principal Investigator. Annual Direct Costs:	
	\$125,669.	
1998-2000	National Institutes of Health (HL60293). Title: Inflammation in the cystic	
	fibrosis lung. Position: Project Leader (Principal Investigator, Pamela B.	
1000 0000	Davis, M.D., Ph.D.). Annual Direct Costs of Project: \$125,901.	
1998-2000	National Institutes of Health (HL60293). Title: <i>Inflammation in the cystic</i>	
	fibrosis lung. Position: Co-investigator. (Principal Investigator, Pamela B.	
	Davis, M.D., Ph.D.). Annual Direct Costs of Project: \$170,140.	

1998-2000	National Institutes of Health (DK52981). Title: Targeting the SEC-receptor for gene therapy. Position: Co-Investigator (Principal Investigator, Pamela B. Davis, M.D., Ph.D.). Annual Direct Costs: \$184,500.
1999-2001	Cystic Fibrosis Foundation. Title: Targeted delivery of interleukin-10. Position: Principal Investigator. Annual Direct Costs: \$100,000.
1998-2003	National Institutes of Health (P01 HL51811). Title: Adeno-associated virus vectors for cystic fibrosis. Position: Consortium (Principal Investigator, William Guggino, M.D.; Project Leader: Terence Flotte, M.D.) Annual Direct Costs: \$34,973.
2002-2004	Cystic Fibrosis Foundation Research Grant. Title: Neutrophil proteases in the pathogenesis of CF lung disease. Position: Principal Investigator. Annual Direct Costs: \$40,000.
2002-2005	Cystic Fibrosis Foundation Clinical Research Grant. Title: <i>Positron emission tomographic imaging in cystic fibrosis</i> . Position: Co-investigator (Principal Investigator: Daniel Schuster, M.D.). Annual Direct Costs: \$79,895.
2004-2006	Cystic Fibrosis Foundation Research Grant. Title: Neutrophil proteases and innate airway defenses in cystic fibrosis. Position: Principal Investigator. Annual Direct Costs: \$60,000.
2003-2006	March of Dimes Research Grant. Title: <i>Genotype-phenotype relationships in the cystic fibrosis airway</i> . Position: Principal Investigator. Annual Direct Costs: \$80,000.
2001-2007	National Institutes of Health (R01 HL64044). Title: <i>Targeted delivery of interleukin-10.</i> Position: Principal Investigator. Annual Direct Costs: \$175,000.
2003-2007	Cystic Fibrosis Foundation. Title: <i>Therapeutic Development Network Center</i> . Position: Co-director. Effort: 5%. Annual Direct Costs: \$100,000.
2004-2007	National Institutes of Health (R21 HL079024) Title: Genetic variants of primary ciliary dyskinesia. Position: Principal Investigator. Effort: 15%. Annual Direct Costs: \$150,000

THERAPEUTIC DEVELOPMENT NETWORK AND CLINICAL RESEARCH PROJECTS

- Epidemiologic study of cystic fibrosis: A multi-center longitudinal follow-up study of patients with cystic fibrosis for monitoring pulmonary function, pulmonary exacerbations, and the safety of long-term treatment with Pulmozyme ZO595n. Position: Site Principal Investigator. Sponsor: Genetech (closed).
- Allergic aspergillosis in cystic fibrosis. Position: Collaborator (Principal Investigator: Raymond Slavin, M.D., St Louis University School of Medicine). Sponsor: National Institutes of Health (closed).
- Genetic modifiers in cystic fibrosis lung disease. Position: Site Investigator. (Principal Investigators: Michael Knowles, M.D. University of North Carolina School of Medicine, Michael Konstan, M.D. and Mitchell Drumm, Ph.D. Case Western Reserve University). Sponsor: National Institutes of Health (enrollment ongoing).
- Genetic modifiers of cystic fibrosis. Position: Site Investigator. (Principal Investigators: Garry Cutting, M.D. Johns Hopkins University). Sponsor: National Institutes of Health (enrollment ongoing).
- Positron emission tomographic imaging in cystic fibrosis. Position: Co-Investigator. Sponsor: Cystic Fibrosis Foundation (closed).
- A randomized, double-blind, placebo-controlled study to investigate the efficacy and safety of 24 weeks of oral treatment with BIIL 284 BS in adult and pediatric cystic fibrosis patients. Position: Site Principal Investigator. Sponsor: Boeringer-Ingelheim (closed).
- Growth hormone use in cystic fibrosis: a multicenter study. Position: Site Principal Investigator. Sponsor: Genentech (closed).

- A multicenter, double-blind, placebo controlled, phase II study of aerosolized TGAAVCF for the treatment of cystic fibrosis (protocol 25E01). Position: Site Principal Investigator. Sponsor: Targeted Genetics (closed).
- Phase 2, multicenter, randomized, controlled, open-label study of the safety and efficacy of Nutropin AQ [somatropin (DNA origin) injection] for the treatment and growth restriction in children with cystic fibrosis. Position: Site Principal Investigator. Sponsor: Genentech (closed).
- Phase 2, randomized, double-blind, parallel dose ranging study of oral theraCLEC-total in cystic fibrosis subjects with exocrine pancreatic insufficiency. Position: Site Principal Investigator. Sponsor: Altus (closed).
- Assessment of induced sputum as a tool to evaluate anti-inflammatory agents in patients with cystic fibrosis. Position: Site Principal Investigator. Sponsor: TDN (closed).
- The EPIC Observational Study: Longitudinal assessment of risk factors for and impact of Pseudomonas aeruginosa acquisition and early anti-Pseudomonal treatment in children with CF. Position: Site Principal Investigator. Sponsor: TDN (enrollment ongoing).
- A randomized, open-label, multicenter, phase 3 trial to assess the safety of tobramycin inhalation powder (TIP) compared to TOBI in cystic fibrosis (CF) subjects. Position: Site Principal Investigator. Sponsor: Novartis (closed).
- A randomized, double-blind within dose, placebo-controlled study to investigate the safety, tolerability and pharmacokinetics of increasing single- and multiple-doses (28-day dosing) of tiotropium bromide administered once daily via the Respimat device in cystic fibrosis patients Position: Site Principal Investigator. Sponsor: Boehringer-Ingelheim (closed).
- Phase 3b study in subjects with cystic fibrosis and chronic Burkholderia spp. infection. Position: Site Principal Investigator. Sponsor: Gilead (enrollment ongoing).

NATIONAL QUALITY IMPROVEMENT PROGRAMS

Improving Care for Children with Cystic Fibrosis Breakthrough Series Collaborative. Position: Site team leader. Sponsor: National Initiative for Children's Healthcare Quality (NICHQ) and Cystic Fibrosis Foundation. 2003-2004.

Cystic Fibrosis Foundation Learning Leadership Collaborative. Position: Team leader. Sponsor: Cystic Fibrosis Foundation. 2004-2005.

CLINICAL TITLE AND RESPONSIBILITIES

1989	Attending physician, North Carolina Memorial Hospital, University of North
	Carolina at Chapel Hill, Chapel Hill, North Carolina.
1992-2000	Bronchoscopy physician, Division of Pediatric Pulmonology, Department of
	Pediatrics, Rainbow Babies and Children's Hospital.
1992-2000	Attending physician, Division of Pediatric Pulmonology, Rainbow Babies and
	Children's Hospital, Cleveland, Ohio.
2000-present	Attending physician, Division of Pediatric Allergy, Immunology, and
	Pulmonary Medicine, St, Louis Children's Hospital, Washington University
	School of Medicine, St. Louis, Missouri.
2000-present	Attending physician, Barnes-Jewish Hospital, Washington University School
	of Medicine, St. Louis, Missouri.

TEACHING TITLE AND RESPONSIBILITIES

1992-2000	Attending physician, Division of Pediatric Pulmonology, Department of Pediatrics, Rainbow Babies and Children's Hospital.
1992-2000	Bronchoscopy physician, Division of Pediatric Pulmonology, Department of
	Pediatrics, Rainbow Babies and Children's Hospital.
1992-2000	Teaching attending physician, Department of Pediatrics, Rainbow Babies and Children's Hospital, Case Western Reserve University.

1992-2000	Lecturer, Pulmonary Physiology Course, Rainbow Babies and Children's Hospital, Case Western Reserve University.
1992-2000	Supervising attending, Pediatric Pulmonology Clinic, Rainbow Babies and Children's Hospital, Case Western Reserve University.
1992-2000	Lecturer, Housestaff Patient Management Conference, Rainbow Babies and Children's Hospital, Case Western Reserve University.
1994-2000	Faculty Preceptor in Senior Housestaff Rounds, Rainbow Babies and Children's Hospital, Case Western Reserve University.
1992-2000	Clinical Instructor, Physical Diagnosis course, Case Western Reserve University.
1994-2000	Lecturer, Medical Student Core Lecture Series, Rainbow Babies and Children's Hospital, Case Western Reserve University.
2000-present	Attending physician, Division of Allergy and Pulmonary Medicine, Department of Pediatrics, St. Louis Children's Hospital, Washington University.
2002-present	Ward attending, Department of Pediatrics, St. Louis Children's Hospital, Washington University.
2000-present	Coordinator and lecturer, Pediatric Pulmonary Physiology Course, Department of Pediatrics, St. Louis Children's Hospital, Washington University.
2000-present	Coordinator and lecturer, Pediatric Pulmonary Pathology Course, Department of Pediatrics, St. Louis Children's Hospital, Washington University.
2000-present	Coordinator and lecturer, Pediatric Research Conference, Department of Pediatrics, St. Louis Children's Hospital, Washington University.
2000-present	Lecturer, Case Management Conference, St. Louis Children's Hospital, Washington University.
2000-present	Director, Pediatric Pulmonary Fellowship Program, Department of Pediatrics, St. Louis Children's Hospital, Washington University.
2000-present	Supervising attending, Pediatric Pulmonology Fellows Clinic, Department of Pediatrics, St. Louis Children's Hospital, Washington University.
2003	Lecturer, Women and Children's Health Clerkship Lecture Series, Department of Obstetrics and Gynecology, Barnes-Jewish Hospital, Washington University.
2004	Lecturer, Infectious Disease Fellowship Lecture Series, Departments of Internal Medicine and Pediatrics, Barnes-Jewish Hospital, Washington University.
2004-2006	Clinical Mentor, Markey Special Emphasis Pathway in Human Pathobiology

FACULTY MENTOR

Trainees: Housestaff

1995-1997 Faculty Mentor, Department of Pediatrics: John Strong, M.D. Faculty Mentor, Department of Pediatrics: Nathan Kraynak, M.D. 1998-2000 1999-2000 Faculty Mentor, Department of Pediatrics: Bradley Arnold, M.D.

RESEARCH MENTOR

Trainees: High School Students

2009-2010 Megan Weber. Senior project, Rockwood Summit High School. Assessing

of inflammation in the cystic fibrosis lung. Missouri Consortium of Science

Research.

Trainees: Undergraduate Students

1993-1994 Jennifer Weber. Senior independent study project, Case Western Reserve

University. Targeting the polymeric immunoglobulin receptor.

2003-2004 Molly Arvin. Senior research project, Washington University in St Louis.

Genetic determinants of inflammation in cystic fibrosis airway epithelia

(recipient of the Cystic Fibrosis Foundation Student Traineeship Award).

2003-2004 **Blaine Fennel.** Senior Thesis, McKendree College. *Anti-inflammatory*

therapy in cystic fibrosis.

2005-2006 Brad Schoss. Research project, Washington University in St Louis. Bacterial colonization of the cystic fibrosis airway before lung transplantation. **Trainees: Medical Students** 1994-1995 Alexandra Pellicena-Palle. Medical student (MSII). Case Western Reserve University School of Medicine. Immunological consequences of gene transfer via the polymeric immunoglobulin receptor (recipient of Cystic Fibrosis Foundation Student Traineeship Award). 1995-1996 Stephanie Lodish. Medical student (MSIV). Case Western Reserve University School of Medicine. Transfer of the human alpha 1-antitrypsin gene to tissue macrophages. 2001-2002 Jessica Pittman. Medical Student (MSII) Washington University School of Medicine. Use of positron emission tomography to measure airway inflammation in murine model of cystic fibrosis lung disease (recipient of the Cystic Fibrosis Foundation Student Traineeship Award). Amber Watters. Medical Student (MSIII) Case Western Reserve University 2008-2009 University School of Medicine. *Modeling primary ciliary dyskinesia*. **Trainees: Graduate Students** 2004-2007 Regina Rowe (doctoral candidate), Thesis committee. Project: Interaction of Andes virus with the respiratory epithelium (A. Pekosz, advisor) 2007-2008 Jessica Marie Esparza (doctoral candidate), Thesis committee. Project: Genetic control of basal body and flagellar assembly (S. Dutcher, advisor) **Trainees: Housestaff** Starla Glick, M.D., Evelyn Jones M.D., Rima Jubran M.D., and William 1994-1995 **Oehlenschlager M.D.** Supervising attending. Senior housestaff projects. Evaluation of a clinical scoring system for hypoxemia in infants with lower respiratory tract infections. John Strong, M.D. Supervising attending. Senior housestaff project. The 1996-1997 impact of housestaff education on asthma morbidity in children. Nathan Kravnack M.D., Lori Racha M.D., and Lisa Cherullo M.D. 1999-2000 Supervising attending. Senior housestaff project. Use of nebulized bronchodilators in infants with acute bronchiolitis. **Trainees: Postdoctoral Fellows** Anna van Heeckeren, D.V.M. Postdoctoral fellow. Project: Effect of 1995-1998 Pseudomonas infection on gene transfer into the lungs. 1996-2000 Starla Glick, M.D. Clinical fellow. Project: Function of anti-epithelial growth factor Fv-based fusion proteins (recipient of the Cystic Fibrosis Foundation Lerov Matthews Physician-Scientist Award). Nada Aldallal, M.D. Clinical fellow. Project: Airway inflammation in cystic 2000-2001 fibrosis. 2000-2002 Julie Sedor, M.D. Clinical fellow. Project: The role of neutrophil-derived proteases in cystic fibrosis lung disease. Michael French, M.D. Clinical fellow. Project: Asymmetric inflammatory 2003-2005 responses of airway epithelia. 2005-2007 Hauw Lie, M.D. Clinical fellow. Project: Genotype-phenotype relationships in primary ciliary dyskinesia.

vectoral chemokine release.

Michelle Farberman, Ph.D. Postdoctoral fellow. Project: Mechanisms of

2004-present

Postgraduate scholars

2009

Amina Selimovic, M.D. Fulbright Visiting Scholar, Medical Sciences. Pediatrician, University Hospital, University of Sarajevo, Sarajevo, Bosnia-Herzegovia. Project: *Bronchoalveolar lavage in children*.

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Original Articles (peer reviewed)

- **1 Ferkol T**, Kaetzel CS, Davis PB: Gene transfer into respiratory epithelial cells by targeting the polymeric immunoglobulin receptor. *Journal of Clinical Investigation* 1993:93:2394-2400.
- **2 Ferkol T**, Lindberg GL, Perales JC, Chen J, Ratnoff OD Hanson RW: Regulation of the phosphoenolpyruvate carboxykinase/human factor IX gene introduced into the livers of adult rats by receptor-mediated gene transfer. *FASEB Journal* 1993;7:1081-91.
- 3 Perales JC, **Ferkol T**, Molas M, Hanson RW: Receptor-mediated gene transfer. *European Journal of Biochemistry* 1994;226:255-266.
- 4 Perales JC, **Ferkol T**, Beegen H, Ratnoff OD, Hanson RW: Persistent expression and transcriptional regulation of genes introduced into the livers of adult animals by receptor targeted uptake. *Proceedings of the National Academy of Science, USA* 1994;91:4086-90.
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- **6 Ferkol TW**, McDowell LM, Gauderer MWL, Alpert SE: Sinopulmonary complications of congenital bronchobiliary fistula. *Clinical Pediatrics* 1994;33:181-4.
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- **Ferkol T**, Pellicena-Palle A, Eckman E, Perales JC, Redman R, Tosi M, Davis PB: Immunologic responses of gene transfer into mice via the polymeric immunoglobulin receptor. *Gene Therapy* 1996;3:669-78.
- **9 Ferkol T**, Perales JC, Mularo F, Hanson RW: Receptor-mediated gene transfer into macrophages. *Proceedings of the National Academy of Science USA* 1996;93:101-5.
- 10 Perales JC, Grossman GA, Molas M, Liu G, **Ferkol T**, Harpst J, Oda H, Hanson RW: Biochemical and functional characterization of DNA complexes capable of targeting genes to hepatocytes *via* the asialoglycoprotein receptor. *Journal of Biological Chemistry* 1997;272:7398-407.
- 11 Ziady A-G, Perales JC, **Ferkol T**, Gerken T, Beegen H, Perlmutter DH, Davis PB: Gene transfer into hepatoma cell lines via the serpin enzyme complex (SEC) Receptor. *American Journal of Physiology: Liver* 1997;273:G545-G52.
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- **13 Ferkol T**, Mularo F, Hilliard J, Lodish S, Perales JC, Ziady A-G, Konstan MW: Transfer of the gene encoding human alpha₁-antitrypsin into macrophages *via* the mannose receptor. *American Journal of Respiratory Cell and Molecular Biology* 1998;18:591-601.
- 14 van Heeckeren A, **Ferkol T**, Zakem-Cloud H, Hamedani A, Mularo F, Konstan M, Tosi M: Effects of chronic *Pseudomonas*-induced lung inflammation on adenovirus-mediated gene transfer. *Gene Therapy* 1998;5:345-51.
- 15 Ziady A-G, **Ferkol T**, Gerkin T, Perlmutter DJ, Davis PB: Ligand substitution of receptor targeted DNA complexes affects gene transfer into hepatoma cells. *Gene Therapy* 1998;5:1685-97.
- 16 Ziady A-G, **Ferkol T**, Dawson D, Perlmutter DH, Davis PB: Chain length of the polylysine in receptor-targeted gene transfer complexes affects duration of reporter gene expression both in vitro and in vivo. *Journal of Biological Chemistry* 1999;274:4908-16.

- 17 Eckman E, Mallender W, Szegletes T, Silski C, Schreiber J, Davis P, **Ferkol T**: *In vitro* transport of active alpha₁-antitrypsin to the apical surface of epithelia by targeting the polymeric immunoglobulin receptor. *American Journal of Respiratory Cell and Molecular Biology* 1999;21:246-52.
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- 19 van Heeckeren A, Tscheikuna J, Davis PB, Erokwu B, Haxhiu M, **Ferkol T**: Effect of *Pseudomonas* infection on weight loss, lung mechanics, and cytokines in mice. *American Journal of Respiratory and Critical Care Medicine* 2000;161:271-9.
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- 22 Mhanna MJ, **Ferkol T**, Dreshaj IA, van Heeckeren A, Kelley T, Martin RJ, Haxhiu MA: Nitric oxide deficiency contributes to the impairment of airway relaxation in cystic fibrosis mice. *American Journal of Respiratory Cell and Molecular Biology* 2001;24:621-6.
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- 25 Ziady AG, Kelley T, Milliken E, **Ferkol T**, Davis PB: Partial correction of the nasal chloride transport defect in CF mice after apical application of serpin enzyme complex receptor targeted DNA complexes. *Molecular Therapy*. 2002;5:413-9.
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- modifiers of liver disease in cystic fibrosis. *Journal of the American Medical Association* 2009;302:1076-83.
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Australia Patent number: 696455 Original Issue date: December 1, 1998

Inventors: Richard W. Hanson, Jose C. Perales, and Thomas W. Ferkol.

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Original Issue date: October 29, 1999

Inventors: Thomas W. Ferkol and Pamela B. Davis.

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