

CURRICULUM VITAE

Name:

Paola Leone

Professional Address:

Rowan University - School of Osteopathic Medicine

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Education:

University of Padua, Italy

M.S-Ph.D. Neuroscience

1987

Citizenship:

American, Italian

Career:

RESEARCH:

1988-1989

Ph.D. Research Internship

Institute of Pharmacology and Experimental Toxicology,

UNIVERSITY OF CAGLIARI, Cagliari, Italy

1990-1992

Postdoctoral Research Fellow

Center for Studies in Behavioral Neurobiology,

CONCORDIA UNIVERSITY, Montreal, Canada

1993-1995

Postdoctoral Research Fellow

Section of Neurosurgery,

Gene Therapy and Neurogenetics Laboratories

YALE UNIVERSITY, New Haven, USA

1995-1998

Research Scientist

Department of Neurosurgery,

Gene Therapy and Neurogenetics Laboratories

YALE UNIVERSITY, New Haven, USA

1997-1998

Visiting Scientist

Gene Therapy Center

Department of Molecular Medicine

UNIVERSITY OF AUCKLAND, Auckland, New Zealand

1998-2001

Assistant Professor

Department of Neurosurgery

JEFFERSON MEDICAL COLLEGE, Philadelphia, PA, USA

1998-2001 **Associate Director**
CNS Gene Therapy Center
JEFFERSON MEDICAL COLLEGE, Philadelphia, PA, USA

2002-2007 **Associate Professor**
Department of Surgery &
Department of Molecular Genetics, Microbiology, and Immunology
UMDNJ / Robert Wood Johnson Medical School
Camden, NJ, USA

2002-2007 **Adjunct Associate Professor** Department
of Neuroradiology, CHILDREN'S
HOSPITAL OF PHILADELPHIA,
Philadelphia, PA, USA

2007 to June 2013 **Associate Professor**
Department of Cell Biology
UMDNJ-School of Osteopathic Medicine
Stratford, NJ, USA

2007 to June 2013 **Director**
Cell & Gene Therapy Center
UMDNJ-School of Osteopathic Medicine
Stratford, NJ, USA

July 2013 to date **Associate Professor**
Department of Cell Biology
ROWAN-School of Osteopathic Medicine
Stratford, NJ, USA

July 2013 to date **Director**
Cell & Gene Therapy Center
ROWAN-School of Osteopathic Medicine
Stratford, NJ, USA

2006-2007 **HOSPITAL APPOINTMENTS:**

Director of Surgical Research
Department of Surgery
Cooper University Hospital, Camden, NJ, USA

TEACHING:

1989	Instructor , Pharmacology, Concordia University, Montreal, Canada
1990	Instructor , Pharmacology, Concordia University, Montreal, Canada
1991	Instructor , Pharmacology, Concordia University, Montreal, Canada
1992	Instructor , Pharmacology, Concordia University, Montreal, Canada
2003	Instructor , Medical Physiology, RWJMS, NJ
2004	Instructor , Medical Physiology, RWJMS, NJ
2007	Lecturer : Pharmacology, UMDNJ/SOM, NJ
2007	Lecturer : Pharmacology, GSBS/SOM, NJ
2008	Lecturer : PBL: Topics in Cell Biology, GSBS/SOM, NJ
2008	Lecturer : Pharmacology, UMDNJ/SOM, NJ
2008	Lecturer : Pharmacology, GSBS/SOM, NJ
2009	Lecturer : Pharmacology, UMDNJ/SOM, NJ
2009	Lecturer : Pharmacology, GSBS/SOM, NJ
2010	Lecturer : PBL: Topics in Cell Biology, GSBS/SOM, NJ
2010	Lecturer : Pharmacology, UMDNJ/SOM, NJ
2010	Lecturer : Pharmacology, GSBS/SOM, NJ
2011	Lecturer : Pharmacology, UMDNJ/SOM, NJ
2011	Lecturer : Pharmacology, GSBS/SOM, NJ
2011	Course Director : Neuroscience, GSBS/SOM, NJ
2012	Lecturer : Pharmacology, UMDNJ/SOM, NJ
2012	Lecturer : Pharmacology, GSBS/SOM, NJ
2012	Lecturer : Human Genetics, UMDNJ/SOM, NJ
2012	Course Director : Neuroscience, GSBS/SOM, NJ
2013	Lecturer : Pharmacology, UMDNJ/SOM, NJ
2013	Lecturer : Pharmacology, GSBS/SOM, NJ
2013	Lecturer : Ethics of Clinical Trials, GSBS/SOM, NJ
2013	Lecturer : Human Genetics, UMDNJ/SOM, NJ
2013	Course Director : Epigenetics of Learning and Memory, GSBS/SOM, NJ

PROFESSIONAL MEMBERSHIPS:

American Society of Cell and Gene Therapy
European Society of Cell and Gene Therapy
The American Society of Human Genetics
International Brain Research Organization
Society for Neuroscience

HONORS/AWARDS:

1999 UNESCO Award, Career Achievement Award, Italy
2003 Canavan Research Illinois, Best Scientist of the Year Award, USA
2006 Woman of the Year, Lions Club, Italy
2009 Foundation of UMDNJ's Excellence in Research Award, USA
2010 Canavan Research Illinois, Scientific Achievement Award, USA
2011 International Rare Disease Day, Hall Of Fame
2012 International Rare Disease Day, Hall Of Fame
2012 Edward J. III Medical Foundation Award in Biomedical Research, USA
2013 International Rare Disease Day, Hall Of Fame
2013 American Academy of Neurology – NeuroFilm Festival 3rd Prize

COMMITTEE MEMBER

2002 Judging Committee for Resident's Research, Cooper Health System
2003 Research Oversight Committee, Cooper Health System, Camden, NJ
2003 Search Committee for Neurosurgery Associates and Chief, Cooper Health System, Camden, NJ
2003 Judging Committee for Resident's Research, Cooper Health System
2004 Judging Committee for Resident's Research, Cooper Health System
2005 Search Committee for the Director of the Coriell Institute, Camden, NJ
2005 Search Committee for Associate Research Dean, UMDNJ, Camden, NJ
2005 Reviewer, Duchenne Muscular Dystrophy Grants, MVP, Tucson, AZ
2005 Member, Research Oversight Committee, Cooper University Hospital, NJ
2005 Judging Committee for Resident's Research, Cooper Health System, NJ
2006 Chair of Data Safety Monitor Board, Gene Therapy for Duchenne Muscular Dystrophy Phase I Protocol, Columbus, OH
2007 NIH Study Section, Inborn Errors Of Metabolism, Bethesda, MD
2008 Executive Committee, UMDNJ-SOM, Stratford, NJ
2008 Recruitment Committee, UMDNJ-SOM, Stratford, NJ
2008 Research Committee, UMDNJ-SOM, Stratford, NJ
2009 IST Committee, UMDNJ-SOM, Stratford, NJ
2009 Reviewer, Duchenne Muscular Dystrophy Grants, MVP, Tucson, AZ
2009 Scientific Committee / Reviewer, Gene Therapy for MPSIIIA, France
2009 Scientific Committee / Reviewer, Partnership For Cures, Chicago, IL
2009 Executive Committee, UMDNJ-SOM, Stratford, NJ
2010 Scientific Committee / Reviewer, Gene Therapy for MPSIIIA, France
2010 Scientific Committee / Reviewer, Gene Therapy for MPSIIIA & MPSIIIB, France
2010-2014 NIH-Data Safety Monitoring Board, Gene Therapy for Batten Disease, New York, NY

SCIENTIFIC ADVISORY BOARD MEMBER

National Tay Sachs and Allied Disorders, USA
Late Onset Tay-Sachs Foundation, PA, USA Canavan Research, IL, USA
Jacob's Cure, NY USA
Canavan Research Foundation, CT
Canavan Foundation, NY, NY
Partnership for Cures, IL, USA
Giant Axonal Neuropathy Foundation, NY
National Organization of Rare Disease

SERVICE ON MAJOR COMMITTEES:

International

Therapy for MPSIIIA, France, 2009
Gene Therapy for MPSIIIA, France, 2010
Gene Therapy for MPSIIIA & MPSIIIB, France, 2010

National

Duchenne Muscular Dystrophy Grants, MVP, 2005
Search Committee for the Director of the Coriell Institute, 2005
Data Safety Monitor Board- Gene Therapy for Duchenne Muscular, 2006
Dystrophy Phase I Protocol
NIH Study Section- Inborn Errors Of Metabolism, 2007
Duchenne Muscular Dystrophy Grants, 2009
Partnership For Cures, 2009

Medical School/University

Search Committee for Associate Dean of Research - UMDNJ-RWJMS 2005
Research Committee, UMDNJ-SOM, 2008
Research Committee, UMDNJ-SOM, 2009
Research Committee, UMDNJ-SOM, 2010
Faculty Affairs Committee. UMDNJ-SOM2011
The American Osteopathic Association Commission on Osteopathic College
Accreditation 2012
Faculty Affairs Committee, UMDNJ-SOM 2012
Faculty Affairs Committee, UMDNJ-SOM 2013

Department (Name, Inclusive Dates)

IST Committee, UMDNJ-SOM Cell Biology, 2008
Executive Committee, UMDNJ-SOM Cell Biology, 2008
IST Committee, UMDNJ-SOM Cell Biology, 2009
Executive Committee, UMDNJ-SOM Cell Biology, 2009
Research Committee, UMDNJ-SOM Cell Biology, 2010
IST Committee, UMDNJ-SOM Cell Biology, 2010
Executive Committee, UMDNJ-SOM Cell Biology, 2010
IST Committee, UMDNJ-SOM Cell Biology, 2011
Executive Committee, UMDNJ-SOM Cell Biology, 2011

IST Committee, UMDNJ-SOM Cell Biology, 2012
Executive Committee, UMDNJ-SOM Cell Biology, 2012
IST Committee, UMDNJ-SOM Cell Biology, 2013
Executive Committee, UMDNJ-SOM Cell Biology, 2013

SERVICE ON HOSPITAL COMMITTEES:

Judging Committee for Resident's Research, Cooper Health System, 2002
Research Oversight Committee, Cooper Health System, 2003
Search Committee for Neurosurgery Associates and Chief, Cooper Health System, 2003
Judging Committee for Resident's Research, Cooper Health System, 2003
Judging Committee for Resident's Research, Cooper Health System, 2004
Member, Research Oversight Committee, Cooper University Hospital, 2005
Judging Committee for Resident's Research, Cooper Health System, 2005

INVITED SPEAKER – SCIENTIFIC MEETINGS

2000 Hunter's Hope Conference, IL, USA
2001 Metabolic Disorder Conference, Netherland
2001 Late Onset Tay Sachs Conference, PA USA
2002 European Pediatric Conference, Italy
2002 Carlo Besta National Neurological Institute, Italy
2002 Milano Pediatria, Milano, Italy
2002 San Raffaele Research Institute, Italy
2002 Myelin Project Conference, FL, USA
2003 United Leukodystrophy Annual Conference, Chicago, IL, USA
2003 Workshop on Genetic Systems in the Rat, Kyoto, Japan
2003 Pelizaeus-Merzbacher Conference, VA, USA
2004 Center for Drug Evaluation, Taipei, Taiwan
2004 Jichi Medical School, Kawachi, Japan
2004 Myelin Project Annual Meeting, Aquiterme, Italy
2004 NAA Symposium, Bethesda, MD
2004 CNS Foundation, Tarrytown, NY
2005 National Tay Sachs & Allied Disorders, Annual Conference, New Orleans
2005 American Society of Gene Therapy. St Louis, MO
2005 Pharamness Symposium, Pula, Italy
2006 Basic Science Seminar, UMDNJ, Newark
2006 Nuclean Medicine Conference, Cagliari, Italy
2006 Cell Biology Seminar, UMDNJ/SOM, Stratford, NJ
2006 Transgenic Rat Symposium, Tokyo, Japan
2007 Metachromatic Leukodystrophy Foundation, Gavoi, Italy
2008 European Leukodystrophy Association, Sevres, France
2008 Canavan Research Summit, White Plains, NY
2008 Annual Canavan Foundation Meeting, New York, New York
2009 Canavan Disease Conference, Tel Aviv, Israel
2010 Pediatric Leukodystrophy Workshop, Italy
2010 Columbia University-Medical Center, Neurology Grand Round, NY
2011 Canavan Research Summit, Harrison, NY
2012 The Translational Science of Rare Diseases, AAAS, Vienna, 2013

2013 Grand Round-Neurology, Hackensack UMC, NJ
2013 European Leukodystrophy Association, Paris
2013 NIH Recombinant DNA Advisory Committee, Bethesda, MD
2013 United Leukodystrophy Foundation, IL

INVITED SPEAKER - FUNDRAISING EVENTS

2002 Jacob's Cure – Fundraising Bowling Event, Strike Out Canavan Disease, NY
2002 Canavan Research Foundation – Tennis Tournament, CT
2003 Jacob's Cure – Fundraising Bowling Event, Strike Out Canavan Disease, NY
2003 Canavan Research Illinois, Annual Event, IL
2003 Canavan Research Foundation – Golf Tournament, CT
2004 As Daylight Breaks – Documentary Release, NY
2005 Canavan Research Foundation - Golf Tournament, CT
2005 Jacob's Cure - Fundraising Bowling Event, Strike Out Canavan Disease, NY
2006 Women For Stem Cells Research - StemCellebration Foundation, NY
2007 Jacob's Cure - Lucky Magazine Event, LA
2009 Saving Lavi Life Fundraising Event – Tel Aviv, Israel
2010 Canavan Research Illinois, Annual Fundraising Event, IL
2010 Jacob's Cure, Fundraising Event, Dream Big Gala, NY
2011 Canavan Research Illinois, Annual Fundraising Event, IL
2012 Canavan Research Illinois, Annual Fundraising Event, IL

CONFERENCE ORGANIZER & CHAIR

2000 Neurodegenerative Diseases Symposium, PA
2005 American Society of Gene Therapy, Symposium, St. Louis, MI
2008 Canavan Research Summit, White Plains, NY
2011 Canavan Research Summit, Harrison, NY

Institutional Review Board Protocols:

Institutions: Cooper Hospital (NJ), UMDNJ (NJ), Children's Hospital of Philadelphia (PA)
Title: Gene Therapy of Canavan Disease using AAV-ASPA
Role: Research P.I. at Cooper Hospital and UMDNJ; Co-PI at CHOP.
Completed

Institution: Cooper University Hospital (NJ),
Title: Use of Lithium for treatment of patients with Canavan Disease
Role: Research P.I.
Completed

Institution: Cooper University Hospital (NJ)
Title: Use of Buspirone for treatment of patients with spinocerebellar ataxias
Role: Co-Investigator
Completed

Institution: Cooper University Hospital (NJ),
Title: Warfarin for Metachromatic Leukodystrophy
Role: Research P.I.
Completed

Institution: Rowan University - SOM
Title: Neuroradiologic Markers of Disease Progression in Canavan Disease
Role: Research P.I.
Active

Institution: Rowan University - SOM
Title: Role of Invariant Natural Killer T Cells in Multiple Sclerosis
Role: Research P.I.
Pending

Sponsor of FDA-Investigational New Drug (IND) Protocols:

IND# 7303 (1998-2002) - Phase I: LPD-ASPA for Canavan Disease
IND# 9119 (2001- 2009) - Phase I/II: AAV-ASPA for Canavan Disease
Pre-IND filed: Triheptanoin Therapy for Canavan Disease

**FUNDING
HISTORY**

GRANT SUPPORT:

Principal Investigator

Funding Agency: UMDNJ Foundation
Title: Stem Cell Therapy for Canavan Disease
Project Dates: 04/01/13 – 03/31/14
Total Direct Costs: \$25,000

Funding Agency: Canavan Research Illinois
Title: Dietary Therapy for Canavan Disease
Project Dates: 02/01/13 – 01/31/14
Total Direct Costs: \$100,000

Funding Agency: Jacob's Cure
Title: human Stem Cell Therapy for Canavan Disease
Project Dates: 08/01/12 – 07/31/13
Total Direct Costs: \$100,000

Funding Agency: NIH-NINDS
Title: Murine Neural Stem Cells in an animal model of Canavan Disease
Project Dates: 07/01/07 - 06/30/12
Direct Costs: \$275,000

Funding Agency: NIH/NINDS
Project number: RO1/NS42120
Title: Gene Therapy for Canavan Disease using AAV2-ASPA

Project Dates: 09/01/02 - 07/31/08

Total Direct Costs: \$3,250,000

Funding Agency: Jacob's Cure, NY

Title: "Characterization of an Animal Model of Canavan Disease"

Project Dates: 07/01/07 - 06/30/09

Total Costs: 400,000

Funding Agency: ProEdoardo Foundation

Title: Warfarin for Metachromatic Leukodystrophy

Project Dates: 06/01/07-05/30/08

Total Costs: \$150,000

Funding Agency: Canavan Research Illinois & Jacob's Cure, NY

Title: Analysis of Stem Cell Therapy in the Tremor Rat Model of Canavan Disease

Project Dates: 06/01/03- 05/31/07

Total Direct Costs: \$500,000

Funding Agency: Canavan Research Foundation, CT

Title: Human Fetal Neural Stem Cell for Canavan Disease

Project Dates: 06/01/06 - 05/31/07

Total Direct Costs: \$50,000

Funding Agency: Canavan Research Illinois

Title: Lithium Therapy for Canavan Disease

Project Dates: 12/01/01- 07/31/04

Total Direct Costs: \$200,000

Funding Agency: Canavan Fund, NY

Title: Preclinical Studies for Gene Transfer of Canavan Disease

Project Dates: 05/31/99 - 06/01/01

Total Direct Costs: \$250,000

Funding Agency: Canavan Foundation, NY

Type: Canavan Disease Fellowship

Project Dates: 04/01/97 - 03/31/98

Total Direct Costs: \$100,000

Funding Agency: Canavan Research Foundation, CT

Title: Liposome-Based Vector Delivery for Canavan Disease

Project Dates: 06/01/95 - 05/31/98

Total Direct Costs: \$300,000

B, Co-Investigator

NIH Center Grant on Epilepsy (1997-1998), Gene Therapy of Epilepsy, \$2,700,000

NIH Program, Project Grant on Transmissible Dementias, (1995-2000), Etiology of

Creutzfeldt-Jakob Disease, \$1,700,000, Yale University, New Haven, CT

C. Pending

- 1) Phenotype and polyfunctionality of peripheral blood invariant Natural Killer T (iNKT) cells as an immunological marker for different forms of Multiple Sclerosis and following different immunomodulatory treatments. NMSS (P.I.) Direct Costs \$579,387, Indirect Costs \$20,502.
- 2) AAV2-ASPA-Based Metabolic Intervention for Alzheimer's Disease. NIH-SBIR R-43 (Co-Investigator), Direct Costs \$150,000, Indirect Costs \$84,000.
- 3) Triheptanoin for Canavan Disease. NIH- UO1, (P.I.) Direct Costs \$350,000, Indirect Costs \$206,500.
- 4) Regulation of Energy Homeostasis in Neurodegenerative Disease by N-acetylaspartate. NIH RFA-RO1 (co-Investigator), Direct Costs \$800,000, Indirect Costs \$ 472,000

BIBLIOGRAPHY:

1. **Leone P.**, and Di Chiara G. (1987) Blockade of D₁ receptors by SCH 23390 antagonizes morphine- and amphetamine-induced place preference conditioning. *Eur. J. Pharm.*, 135: 251-254.
2. Acqvas, E., Carboni, E., **Leone, P.**, and Di Chiara, G. (1988) 5HT₃ receptor antagonists block morphine and nicotine- but not amphetamine-induced reward. *Eur. J. Pharm.*, 97: 175-178.
3. Acqvas, E., Carboni, E., **Leone, P.**, and Di Chiara, G. (1989) SCH 23390 blocks drug-conditioned place preference and place-aversion: anhedonia (lack of reward) or apathy (lack of motivation) after dopamine-receptor blockade? *Psychopharmacology*, 99:151-155.
4. **Leone, P.**, Pocock, D. and Wise, R.A. (1991) Morphine-dopamine interaction: ventral tegmental morphine increases nucleus accumbens dopamine release as measured by *in vivo* microdialysis. *Pharm Biochem. & Behav.*, 39: 469-472.
5. During, M.J., Mirchandani, G., Williamson, A., **Leone, P.**, deLanerolle, N., Bergold, P.J., Federoff, H.J. (1993) Intrahippocampal injection of a HSV-1 vector expressing GluR6 induces spontaneous limbic seizures and CA1 and CA3 hyperexcitability. *Epilepsia*, 34, (Suppl. 6): 75.
6. Devine, D.P., **Leone, P.**, Pocock, D. and Wise, R.A. (1993) Differential Involvement of ventral tegmental Mu, Delta and Kappa opioid receptors in modulation of basal mesolimbic dopamine release: *In vivo* microdialysis studies *J. Pharm. Exp. Ther.*, 266: 1236-1246.
7. Devine, D.P., **Leone, P.**, Carlezon, A.W. Jr., and Wise, R.A. (1993) Ventral mesencephalic & opioid are involved in modulation of basal mesolimbic dopamine neurotransmission: an anatomical localization study. *Brain Research*, 622: 348-352.

8. Devine, D.P., **Leone, P.**, and Wise, R.A. (1993) Striatal tissue preparation facilitates early sampling in microdialysis and reveals an index of neuronal damage. *J. Neurochemistry*, 61: 1246-1254.
9. Devine, D.P., **Leone, P.**, and Wise, R.A.. (1993) Mesolimbic dopamine neurotransmission is increased by administration of μ -opioid receptor antagonists. *Eur. J. Pharmacology*, 243: 55-64.
10. During, M.J., Fried, I., **Leone, P.**, Katz, A. and Spencer, D.D. (1994) Direct measurement of extracellular lactate in the human hippocampus during spontaneous seizures. *J. Neurochem.*, 62, 2356-2361.
11. Kaplitt, M.G., **Leone, P.**, Xiao, X., Pfaff, D.W., O'Malley, K.L., Samulski, R.J., and During, M.J. (1994) Adeno-associated virus vectors yield long-term expression of potentially therapeutic genes in the mammalian brain. *Nature Genetics*, 8: 148-154.
12. During, M.J., **Leone, P.**, Davis, K.E., Kerr, D., and Sherwin, R.S. (1995) Glucose availability influences hippocampal GABA release in the freely-moving rat via an effect on ATP-sensitive potassium channels. *J. Clin. Inv.*, 95: 2403-2408.
13. Badiani, A., **Leone, P.**, Noel, M.B., and Stewart J. (1995) Ventral tegmental area opioid mechanisms and modulation of ingestive behavior. *Brain Research*, 670: 264-276.
14. Maggs, D.G., Jacob, R., Langer, R., **Leone, P.**, During M.J., Tamborlane, W.V., and Sherwin, R.S. (1995) Interstitial fluid concentrations of glycerol, glucose and amino acids in human quadriceps muscle and adipose tissue: evidence for significant lipolysis in skeletal muscle. *J. Clin. Inv.*, 96: 370-377.
15. Wise, P., **Leone, P.**, Rivest, R., and Leeb, K. (1995) Elevations of nucleus accumbens dopamine and DOPAC levels during intravenous heroin self-administration. *Synapse*, 21, 140-148.
16. During, M.J. and **Leone, P.** (1995) Adeno-associated virus vectors for gene therapy for neurodegenerative disorders. *Clinical Neuroscience*, 3, 292-300.
17. Jacob R.J., Dziura J., Medwick, M.B., **Leone, P.**, During, M.J., Shulman, G.I., and Sherwin, R.S. (1997) The effect of Leptin is enhanced by microinjection into the ventromedial hypothalamus. *Diabetes* 46 (1) 150-152.
18. Freese, A., Kaplitt, M.G., O'Connor, W.M., Abbey, M., Langer, D., **Leone, P.**, During, M.J. (1997) Direct gene transfer into human epileptogenic hippocampal tissue with an adeno-associated virus vector - implications for a gene therapy approach to epilepsy. *Epilepsia*, 38: 759-766.

19. During, M.J. and **Leone, P.** (1997) Targets for Gene Therapy of Parkinson's Disease: Growth Factors, signal transduction and promoters. *Exp. Neurol.*, 144, 74-81.
20. O'Connor, W.M., Davidson, B.L., Kaplitt, M.G., Abbey, M.V., During, M.J., **Leone, P.**, Langer, D., O'Connor, M.J., Freese, A. (1997) Adenovirus vector-mediated gene transfer into human epileptogenic brain slices: prospects for gene therapy in epilepsy. *Exp. Neurol.* 148, 167-78.
21. During, M.J., Samulski, R.J., **Leone, P.**, Kaplitt, M.G., Freese, A. Xiao, X., Elsworth, J.D., Roth, R.H., Sladek, J.R.Jr., O'Malley, K.L. and Redmond, D.E. (1998) In vivo expression of therapeutic human genes for dopamine production in the caudates of MPTP-treated monkeys using an AAV vector. *Gene Therapy* 5, 820-827.
22. During, M.J., Xu, R., Young, D., Kaplitt, M.G., Sherwin, R.S., and **Leone, P.** (1998) Peroral gene therapy of lactose intolerance using an AAV vector. *Nature Medicine*, 4, 1131-1135.
23. Young, D.D., Lawlor, P., **Leone, P.**, Dragunow, M., During, M.J. (1999) Environmental enrichment inhibits spontaneous neuronal apoptosis and prevents neuronal injury in the rat. *Nature Medicine*, 5, 448-453.
24. **Leone, P.**, Janson, C.G., McPhee, S.J., During, M.J. (1999) Global CNS Gene Transfer for Childhood Neurogenetic Enzyme Deficiency: Canavan Disease. *Current Opinions in Molecular Therapeutics*, 1:4, 487-492
25. Janson, C.G., McPhee S.W.J., **Leone, P.**, During, M.J. (1999) Gene Therapy in the Central Nervous System. *Science and Medicine*, 6, 4-5.
26. **Leone, P.**, McPhee, S.W., Janson, C.G., Davidson, B.L., Freese, A., During, M.J. (2000) Multisite partitioned delivery of human tyrosine hydroxylase gene with phenotypic recovery in Parkinsonian rats. *Neuroreport*, 2:6, 1145-1151.
27. During, M.J., Symes, C.A., Lawlor, P.A., Lin, J., Dunning, J., Fitzsimons, H.L., Poulsen, D., **Leone, P.**, Xu, R., Dicker, B.L., Lipski, J., Young, D. (2000) Targeted autoimmunity: a genetic NMDAR1 vaccine with efficacy in experimental stroke and epilepsy. *Science*, 287, 1453-1460.
28. **Leone, P.**, Janson, C.G., Bilianiuk, L., Wang, Z., Huang, L., Sorgi, F., Matalon, R., Kaul, R., Zeng, Z., Freese, A., McPhee S.W., Mee, E., During, M.J. (2000) Aspartoacylase gene transfer to the mammalian central nervous system with therapeutic implication for Canavan Disease. *Ann. of Neurol*, 48:1, 27-38.
29. Telfeian, A.E., Federoff, H.J., **Leone, P.**, During, M.J., Williamson, A. (2000) Overexpression of GluR6 in rat hippocampus produces seizures and spontaneous nonsynaptic bursting in vitro. *Neurobiology of Disease*, 4, 362-374.
30. Janson CG, McPhee SW, **Leone P.**, Freese A, During MJ. (2001) Viral-based gene transfer to the mammalian CNS for functional genomic studies. *Trends Neurosci.* 1;24(12):706-12.

31. Xu R, Janson CG, Mastakov M, Lawlor P, Young D, Mouravlev A, Fitzsimons H, Choi KL, Ma H, Dragunow M, **Leone P.**, Chen Q, Dicker B, During MJ. (2001) Quantitative comparison of expression with adeno-associated virus (AAV-2) brain-specific gene cassettes. *Gene Therapy* 8 (17):1323-32.
32. Janson CG, Ramesh T.M., During, M.J., **Leone, P.**, Heywood, J.(2001) Human intrathecal transplantation of peripheral blood stem cells in amyotrophic lateral sclerosis. *Hematotherapy & Stem Cell Research* 10:6, 913-16.
33. Abi-Saab W.M., Maggs D.G., Jones T, Jacob R, Srihari V., Thompson J., Kerr D, **Leone P**, Krystal J.H., Spencer D.D., During MJ, Sherwin R.S. (2002) Striking Differences in Glucose and Lactate Levels Between Brain Extracellular Fluid and Plasma in Conscious Human Subjects: Effects of Hyperglycemia and Hypoglycemia. *J Cereb Blood Flow Metab.* (3): 271-279.
34. Janson, C.G, McPhee, S.W.J., Bilaniuk, L., Haselgrove, J., Testaiuti, M., Freese, A., Wang, D.J., Shera, D., Hurh P., Rupin J., Saslow, E., Goldfarb, O., Goldberg, M., Larijani, G., Sharrar, W., Camp A., Liouterman, L., Kolodny, E., Samulski, J., **Leone, P.** (2002) Gene Therapy of Canavan Disease: AAV-2 Vector for Neurological Delivery of Aspartoacylase Gene (ASPA) to the Human Brain. *Human Gene Therapy*, 13: 1391-1412.
35. Janson C.G., **Leone P.**, Freese, A. (2002) Current medical and surgical approaches to Parkinson's Disease. *Science and Medicine.* 8(6), 328-338.
36. Janson C.G., **Leone P.**, Freese, A. (2003) Current medical and surgical approaches to Parkinson's Disease. *Science & Medicine* 9(1): 24-35.
37. Janson C.G., **Leone P.**, Freese, A. (2003) Current medical and surgical approaches to Parkinson's Disease. *Science & Medicine* 9(2): 82-95.
38. Zeng, B., Wang, Z-H., Ribeiro, L.A., **Leone, P.**, De Gasperi, R., Kim, S-J., Raghavan, S., Ong, E., Naum, M., Pastores, G.M., Kolodny, E.H. (2003) Identification and Characterization of Novel Mutations of the Aspartoacylase Gene in Non-Jewish Patients with Canavan Disease. *Journal of Inherited Metabolic Diseases.* 2002; 25: 557-570.
39. Janson C.G., During M., Shifman Y, **Leone P.** (2003) Transcriptional Activation of Human CREB gene promoter using bis-PNA (peptide nucleic acid). *Letters in Peptide Science.* 10 (3), 373-376.
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76. Assadi-Khansari M., Velazquez-Rodriguez Y., Zerafati G., and **Leone P.** The Phenotypic Differences between Probable Migraine and Definite Migraine. (2013) P03-017, *San Diego, AAN*
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Patents:

Leone, P., Sorgi, F., and During, M.J. (1997) Delivery of Genes in the Central Nervous System.

Tavazzi, B., **Leone, P.**, Lazzarino (2005) Analytical Kits for the Chemical Diagnosis of Leukodystrophies & Non-hereditary Brain Pathologies, Organic Acidosis, Metabolic Abnormalities of Purine & Pyrimidines, Congenital Metabolic Diseases Vitamin C Deficiency & Abnormal Oxidative States.

Leone, P., Francis, JS (2012) PCT & US
Metabolic Therapy of Neurodegenerative Diseases via the Catabolism of NAA.

Extracurricular Activities

1) Photographs displayed at the American Museum of Natural History, New York, NY. **Genomic Revolution Exhibit**, May 26th 2001 -January 2nd, 2002; Museum of Natural History, North Carolina. **Genomic Revolution Exhibit**, April 26th, January 2nd, 2003; Fernbank Museum of Natural History, Atlanta, GA, June 12th 2004 - January 2nd, 2005.

2) Editor of the documentary entitled " **Little Heroes**" , 2013