CURRICULUM VITAE ET STUDIORUM

Prof. Alberto Auricchio

(May, 2009)

Date and place of birth	:	August 14, 1969, Napoli
Citizenship	:	Italian
Language	:	Italian, English, French
Address	:	Telethon Institute of Genetics and Medicine (TIGEM), Via P. Castellino 111, 80131 Napoli, Italy. Tel +11-39-081-6132229, e-mail: auricchio@tigem.it

Current Position: **"Full Investigator"**, Telethon Institute of Genetics and Medicine (TIGEM), Napoli, Italy.
Supervisor, AAV Vector Core, Telethon Institute of Genetics and Medicine (TIGEM), Naples, ITALY.
"Lecturer", European School of Molecular Medicine (SEMM), Napoli, Italy.
Associate Professor, Medical Genetics, Department of Pediatrics, University of Napoli "Federico II" Medical School, Napoli, Italy.

CURRICULUM STUDIORUM

Year	Institute	Title
1987	Liceo Umberto I, Napoli.	BA
1987-1993	University of Napoli "Federico II", Medical School.	Medical Degree (cum laude)
1993	University of Napoli "Federico II", Medical School.	Medical License
1998	III Pediatric Clinic, University of Milan, Medical School.	Pediatric Board Certification (cum laude)
1998	Educational Commission for Foreign Medical Graduates, Philadelphia, PA, USA.	USMLE (#0-550-664-7)
1998	Institute for Human Gene Therapy, University of Pennsylvania, Philadelphia, PA, USA.	Post-doctoral Researcher
2001	Institute for Human Gene Therapy, University of Pennsylvania, Philadelphia, PA, USA.	Research Associate
2002	Telethon Institute of Genetics and Medicine (TIGEM), Napoli, Italy.	Full Investigator and AAV Vector Core Supervisor
2002	European School of Molecular Medicine (SEMM), Napoli-Milano, Italy.	Lecturer
2004	"Federico II" University, Medical School, Napoli, Italy.	Lecturer in Medical Genetics
2005	Department of Pediatrics "Federico II" University, Medical School, Napoli, Italy.	Associate Professor of Medical Genetics

Italian and foreign pre-doctoral activity

1990-1993 Intern fellow in the the Dept. of Molecular and Cellular Biology, University of Napoli "Federico II" Medical School
Mentor: Prof. B. Bruni.

Summers 1990 and 1991 Summer Student, Laboratory of Molecular and Cellular Biology, National Cancer Institute, N.I.H., Bethesda, Md, USA.
Mentor: Prof. P.P. Di Fiore.

1993 Intern fellow in the Dept. of Pediatrics, University of Napoli "Federico II" Medical School
Mentor: Prof. R. Troncone.

Post-doctoral activity

1994-1995 and 1996-1998 Fellow scientist at Telethon Institute of Genetics and Medicine (TIGEM), Milan
Mentor: Prof. A. Ballabio.

1998-2001 Post-doctoral Researcher, Institute for Human Gene Therapy, University of Pennsylvania, Philadelphia, PA, USA.
Director: Dr. J. M. Wilson.

1999-2002 "Joint appointment", Kirby Center for Molecular Ophthalmology, University of Pennsylvania, Philadelphia, PA, USA.
Mentor: Dr. J. Bennett.

2000-2002 "Principal Investigator", Dept. of Radiology, University of Pennsylvania, Philadelphia, PA, USA.
Mentor: Dr. P. Acton.

2001-2002 Research Associate, Institute for Human Gene Therapy, University of Pennsylvania, Philadelphia, PA, USA.
Director: Dr. J. M. Wilson.

Scientific Awards

- Telethon Fellowship 371/B (1999-2001) for Italian Scientists Abroad.
- Winner of the "Image of the Year" Award, 49th Annual Meeting of the Society of Nuclear Medicine, Los Angeles (June 15-19, 2002).
- Ruth and Milton Steinbach Fund Award, NYC (July 2003-July 2006).
- Startcup Award for the "Angiotech" business plan, "Federico II" University, Napoli, Italy (2005).

- Winner of the "Outstanding New Investigator Award", American Society of Gene Therapy (2006).
- "Cavaliere" of the Italian Republic (2007)

TEACHING ACTIVITY

Tutorial Activity

Marilisa Carpentieri	MD- University of Naples "Federico II", Naples, Italy	training completed in 1996
Erin O' Connor	pre-Med Student- University of Pennsylvania	training completed in 2000
Deena Bernett	pre-Med Student- University of Pennsylvania	training completed in 2001
Fabiana Parisi	BA in Biotechnology, University of Naples "Federico II", Naples, Italy	training completed in 2006
Maria Garcia Hoyos	Post Doc Student	training completed in 2007
Anita Capalbo	Student in Biological Sciences ,University of Naples "Federico II", Naples, Italy	training completed in 2008
Maria Carmela Allocca	PhD Student, in Molecular Medicine, European School of Molecular Medicine (SEMM), Italy	training completed in 2008
Marco Petrillo	PhD Student in Medical Genetics, Second University of Naples, Naples, Italy	training completed in 2008
Gabriella Cotugno	PhD Student in Molecular Medicine, European School of Molecular Medicine (SEMM), Italy	training completed in 2009
Alessandra Tessitore	Post Doc Student	training completed in 2009
Pasqualina Colella	PhD Student in Human Genetics, Open University,	training to be completed in 2010

	London, UK	
Anna Manfredi	PhD student University of Naples "Federico II", Naples, Italy	training to be completed in 2011
Caterina Strisciuglio	Residency in Pediatrics University of Naples "Federico II", Naples, Italy	training to be completed in 2012

Courses

"Gene and Cell Therapy Course" for the PhD Programs in Human Genetics, Open University London, UK, in Human Genetics, European School of Molecular Medicine (S.E.M.M.), Naples, Italy and in Medical Genetics, Second University of Naples, Naples, Italy ,October 2003 to present.

Medical Genetics Course, for the Audiometry Degree, in the three year undergraduate course, May-June 2004 (University of Naples, Federico II School of Medicine)

Gene Therapy Lectures in the Master Program in Advanced Biotechnology for Diagnosis and Molecular Therapy, CEINGE, Naples, Italy, May and September 2004.

Medical Genetics Lectures for the Degree in Pediatrics Nursering Science, May- June 2005 (University of Naples, Federico II School of Medicine)

Medical Genetics Course, in the three year undergraduate course the three year undergraduate course for the Audiometry, Audioprotesi, Orthopedic Techniques, Dental Hygiene 2006-present University of Naples (University of Naples, Federico II School of Medicine).

Genetics, Pediatrics and Clinal Genetics Lectures for the Specialistic Medicine and Surgery Degree, 2006- present (University of Naples, Federico II School of Medicine)

Medical Genetics for the Graduate School Medicine and Surgery Degree, 2005 at present (University of Naples, Federico II School of Medicine)

Seminars

<u>Wilson's Laboratory Semi-Annual Seminars:</u>

Dicember 10, 1998 "AAV-based gene therapy for the treatment of OTC deficiency"

June 12, 1999 "Constitutive insulin-based gene therapy for the treatment of type 1 diabetes"

February 12, 2000	“Pharmacologically-regulated expression of the insulin gene via viral vectors from muscle and liver”
Septemebr 6, 2000	“31-NMR spectroscopy detection of creatine-kinase gene transfer to liver”
February 27, 2001	“Pharmacologically-regulated gene expression in the retina transduced with viral vectors”
July 24, 2001	“In vivo non-invasive imaging of gene transfer using SPECT’
November 27, 2001	“Gene transfer to lung for delivery of systemic therapeutic proteins”
February 20, 2001	“Gene transfer to muscle, liver and retina using viral vectors”, TIGEM Napoli, Italy
September 24, 2002	“Gene transfer to lung and retina using AAV vectors”, TIGEM, Napoli, Italy
December 6, 2002	"AAV vectors for in vivo gene transfer to lung and retina", Dept. of Biology and Molecular and Cellular Pathology, "Federico II" University, Napoli, Italy
December 13, 2002	"AAV Vectors for in vivo gene transfer", Research Institute in Molecular Biology (IRBM), Pomezia, Italy
March 26, 2003	"AAV vectors for in vivo gene transfer", Dept. of Genetics and Molecular Biology, University of Rome "La Sapienza", Italy
October 31, 2003	"AAV vectors for retinal gene transfer", Dept. of Ophthalmology, Insitute of Child Health, University College, London, UK
March 30, 2005	“Retinal degeneration, neovascularization and ocular albinism: AAV-mediated gene transfer in animal models“ TIGEM – Telethon Institute of Genetics and Medicine, Napoli, Italy
September 20, 2005	“AAV vectors serotypes as tools for in vivo gene transfer”, Center for Cell and Gene Therapy, Baylor College of Medicine, Houston, USA
June 17, 2008	“Advancements in AAV-mediated gene transfer for inherited retinal diseases”, Targeted Genetics, Seattle, WA, USA

June 19 , 2008	"Advancements in AAV-mediated gene transfer for inherited retinal diseases", Amsterdam Molecular Therapeutics (AMT), Amsterdam, Netherlands
September 29, 2008	"AAV mediated gene transfer for human inherited diseases", Dept. of General Pathology, " Federico II" University, Napoli, Italy
May 13, 2009	"Expanding the utility of AAV vectors for gene therapy of human Inherited diseases", Dept. of Biological and Technological Research (DiBIT), "San Raffaele" hospital, Milan, Italy
October 6, 2009	"AAV-mediated gene transfer to the retina and liver for human inherited diseases" International Centre for Genetic Engineering and Biotecnology (ICGEB), Trieste, Italia
November 27, 2009	"Gene therapy for inherited retinal dystrophies: from viral vector to patient", UCL Institute of Ophthalmology, Genetics and Molecular Therapy, Londra, Uk

RESEARCH ACTIVITY

Memberships

Member of:
European Society of Gene Therapy (ESGT)
American Society of Gene Therapy (ASGT)
Association for Research in Vision and Ophthalmology (ARVO)
2004-2005: Member of the New York Academy of Sciences

Member of Editorial Boards:

Human Gene Therapy (2005-2009)
Molecular Therapy (2008-2011)

Reviewer Activity (Scientific Journals):

Human Gene Therapy
Molecular Therapy
Gene Therapy
Investigative Ophthalmology and Visual Science
Vision Research
Diabetologia
Cell Death and Differentiation

EMBO Journal
Journal of Gene Medicine
Journal of Pediatric Gastroenterology and Nutrition
Journal of Clinical Investigation

Reviewer Activity (Scientific Agencies):

Italian Ministry of University and Scientific Research
Department of Health, UK Government, UK.
Association Francaise pour le Myopathies (AFM), France.
Swiss Ministry of Scientific Research

Recent oral presentations at international congresses by members of the group supervised by Prof.Auricchio

G. Cotugno, P. Formisano, F. Beguinot, R. Pollock and A. Auricchio. AP20187-Inducible activation of a chimeric insulin receptor mimics insulin action in hepatocytes transduced with AAV. XII Annual Congress of the European Society of Gene Therapy (ESGT), Tampere, Finland. 4-7 Novembre 2004 (Oral Presentation)
Recipient of the European Society of Gene Therapy Travel Award (ESGT), Tampere, Finland, November 4-7, 2004

M. Allocca, T. S. Rex, L. Domenici, E. M. Surace, A. M. Maguire, A. Cellerino, J. Bennett and A. Auricchio. Systemic but not intraocular Epo gene transfer protects the retina from light-and genetic-induced degeneration. European Society of Gene Therapy (ESGT), Tampere, Finland, November 4 - 7 2004. (Oral presentation)

G. Cotugno. Winner of the Best Poster Award - 19th course in Medical Genetics of the European School of Genetic Medicine, Bertinoro, Italia. April 26 – May 2 2006

G. Cotugno, P. Formisano, F. Giacco, V. Rivera, F. Beguinot, A. Auricchio. AP20187-mediated activation of a chimeric insulin receptor in AAV transduced murine muscles and livers results in insulin-like effects. The American Society of Gene Therapy's 9th Annual Meeting (ASGT), Baltimore, (USA) 31 Maggio - 4 Giugno, 2006. (Oral Presentation)
Recipient of the American Society of Gene Therapy Travel Grant (ASGT), Baltimore, MD,(USA) May 31 - June 4 2006

A. Tessitore, Armida Faella, Fabiana Parisi, Mark Haskins and Alberto Auricchio. AAV-mediated gene transfer to muscle and liver of MPS VI animal models. 9th International Symposium on Mucopolysaccharide and Related Disease Venezia Lido, Italia 29 Giugno - 2 Luglio, 2006 (Oral presentation)

G. Cotugno. Recipient of the Association for Research in Vision and Ophtalmology International (ARVO) Travel Grant, Fort Lauderdale, FL, (USA) May 6 - 10 2007 (Poster presentation)

A. Tessitore, G. Cotugno, A. Capalbo, A. Faella, C. Strisciuglio, Thomas O'Malley, M. Haskins and A. Auricchio. Immune suppression increases the therapeutic efficacy of AAV-mediated gene transfer in animal models of mucopolysaccharidosis VI with null but not with missense mutations.CLINIGENE 2nd Anniversary meeting "Les Pensières, Mérieux Foundation". Annecy, France 3 - 7 Aprile 2008 (Oral presentation)

M. Allocca, M. Garcia-Hoyos, M. Doria, M. Petrillo, S. R. Kim, A. Maguire, T. S. Rex, U. Di Vicino, J. R. Sparrow, J. Bennett and A. Auricchio. Gene therapy of a mouse model of Stargardt's disease using adeno-associated viral vectors packaging large genes. ARVO's 2008 Annual Meeting, Eyes on Innovation, Fort Lauderdale, FL (USA) April 27 – May 1 2008.(Oral presentation)
Travel Grant award ($1000) provided by the ARVO Fondazione per l'oftalmologia GB Bietti.

G. Cotugno, P. Colella, E. M. Surace and Alberto Auricchio. Inhibition of Sonic Hedgehog by RNA-Interference and by Gene Transfer of a Decoy Receptor Reduces Murine Retinal Neovascularization.The ARVO 2008 Annual Meeting, Fort Lauderdale, Fl (USA) April 27 - May 1 2008 (Oral Presentation)

G. Cotugno, P. Colella, M. Savarese, E. M. Surace and Alberto Auricchio Inhibition of the Sonic Hedgehog Pathway by RNA-Interference and by Gene Transfer of a Decoy Receptor Reduces Retinal Neovascularization in ROP mice. The American Society of Gene Therapy's 11th Annual Meeting (ASGT), Boston, (USA) May 28 – June 1, 2008. (Oral Presentation)

M. Allocca, M. Doria, M. Petrillo, P. Colella, M. Garcia-Hoyos, D. Gibbs, S. R. Kim, A. Maguire, U. Di Vicino, J. R. Sparrow, D. S. Wiliams, J. Bennett and A. Auricchio. Serotype-dependent packaging of large genomes in adeno-associated viral vectors results in efficient in vivo gene delivery. The 11th Annual Meeting of the American Society of Gene Therapy (ASGT), Boston, MA (USA) May 28 – June 1 2008 (Oral presentation)

A. Tessitore, G. Cotugno, A. Capalbo, A. Faella, C. Strisciuglio, Thomas O'Malley, M. Haskins and A. Auricchio. Immune suppression increases the therapeutic efficacy of AAV-mediated gene transfer in animal models of mucopolysaccharidosis VI with null but not with missense mutations. American Society of Gene Therapy (ASGT) 2008 Boston, MA (USA) May 28 - June 1, 2008 (Oral presentation)

P. Colella, U. Di Vicino and A. Auricchio. AAV-Mediated Gene Transfer of the Erythropoietin Derivative S100E in Models of Retinal Degenerative Diseases", American Society of Gene Therapy (ASGT) 2008 Boston, MA (USA) May 28 - June 1, 2008 (Oral presentation)

G.Cotugno, A. Tessitore, A. Capalbo, Patrizia Annunziata, C.Strisciuglio, A. Faella, T. O'Malley, E. De Leonibus, L. Aloj, M. Haskinsand A. Auricchio. Optimizing gene

therapy for MPSVI: overcoming immune responses and° highvector doses. ASGT meeting 2009, May 27 - 30, 2009, San Diego, CA (Oral presentation)
Travel Grant award provided by the ASGT

Invited Presentations at National and International Meetings

"Genetic aspects of neurocrystopathies". International Symposium on Intestinal Motility Disorders, Castelvolturno, Naples, Italy 1996.

"Hirschsprung disease and X-linked chronic intestinal pseudoobstruction". Third International Meeting on Hirschsprung disease and related neurochrystopathies, Evian, Francia, February 5-8, 1998.

"Pharmacologically-regulated gene expression in the retina following transduction with viral vectors". 7th Congress of the Interantional Society of Ocular Toxicology, Kiawah Island Resort, SC, September 30-October 5, 2000.

"Pseudotyped viral vectors as gene delivery agents for the retina". New Therapeutic Approaches in Hereditary Eye Disease-From Gene To Cure (European Union), Prague, July, 13-15 2001.

"Gene transfer to the adult retina". 2002 World Symposium on Ocular Albinism (Vision of Children Foundation), S. Diego, CA, March 6-8, 2002.

"The effect of modification of viral vector surface proteins on cellular specificity and transduction characteristics in the retina" Sixth Annual Vision Research Conference on "Retinal Cell Rescue", Ft. Lauderdale, FL, May 3-4, 2002.

"Gene Therapy for Diabetic Retinopathy" 7th EASD/JDRF OXFORD WORKSHOP
Keble College, Oxford, August, 3-5 2002.

"Gene transfer using adeno-associated viral vectors", 15th Meeting of the Insternational Institute of Genetics and Biophysics, Capri, October 12-15, 2002.

"AAV vectors for in vivo gene transfer to retina and lung", Telethon Convention 2002, Riva del Garda, Novembre 24-26, 2002.

"AAV vectors for gene tranfer to the adult retina", European Science Foundation Workshop, Sorrento, March 17-18, 2003.

"AAV vectors for retinal gene transfer", American Society of Gene Therapy, Washington DC, June 4-8, 2003.

"Vettori AAV per il trasferimento di geni alla retina", Retina 2003, Ischia, September 12-13, 2003.

"Terapia Genica della retina", Societa' Italiana di Neuroscienze, Pisa, September 26-28, 2003.

"A strategy for Oa1 gene transfer using AAV vectors", 2003 World Symposium on Ocular Albinism, San Diego, California, USA, Novembre 9-11, 2003.

"Gene Transfer to the retina with AAV vectors for Ocular Neovascularization", Ruth & Milton And Steinbach Fund Annual Meeting, Rockfeller University, NYC, USA, April 4, 2004.

"In vivo gene transfer to the retina with AAV vectors", European Society of Human Genetics, Monaco, Germania, June 12-15, 2004.

"AAV-mediated gene transfer in animal models of retinal diseases", The Biology and Development of the Eye in Health and Diseases, 17th IGB Meeting, Capri, October 9-12, 2004.

"AAV-mediated Oa1 gene transfer in an animal model of ocular albinism" Vision Of Children Fourth World's Symposium on Ocular Albinism, New York, October 18-19, 2004.

"Shh involvement in ocular neovascularization identifies a novel pathway for therapeutic targeting" Ruth & Milton And Steinbach Fund Annual Meeting, Rockfeller University, NYC, USA, April 4, 2005.

"Animal library for diagnostic molecular imaging in neuroscience & cardiovascular" DiMI (Diagnostic Molecular Imaging) Kick-off Meeting, Colonia, Germany. Aprile 7-8 2005.

"Ocular gene transfer for retinal degenerations, dystrophies and neovascularization" FASEB meeting on The Biology and Chemistry of Vision, Tucson, AZ, USA, June 18-23, 2005.

"Adeno-associated vectors for gene therapy of lysosomial diseases" National Congress of SISMME – SISN – GENCLI, Pollenzo – Bra (Cn) October 16-18, 2005.

"Gene transfer systems and theirapplication to diabetes"; "Production and purification of adeno-associated vectors" 1st Eugene 2 Training Course, Napoli, October 17-19, 2005.

"AAV-mediated gene transfer for inherited diseases", Clinigen Kick OFF Meeting, Annecy, France. March 31 - April 3 2006.

"The gene therapy" First National Scientific Meeting Retina Italia ", Milan, Italy, May 12 2006.

"AAV-mediated gene transfer for retinal diseases"; "Outstanding New Investigator

Symposium" American Society of Gene Therapy 9th Annual Meeting, Baltimore, USA, May 31-June 4, 2006.

"Gene therapy and the gut: where are we in the 21st century?"; Second ESPGHAN (European Society for Pediatric Gastroenterology Hepatology & Nutrition) Capri Meeting, Capri, Italy, October 12-15, 2006.

"Gene therapy for retinal diseases" "Italian Society of Genetics Ophthalmology, Milan, Italy, Dicember 1 2006.

"Gene therapy for severe inherited photoreceptor disease", Vision of Children Foundation, Sixth Annual Symposium on Oculac Albinism, San Diego, California - USA, March 1-3, 2007.

"Intramuscular AAV (MPS VI)", National Institute of Neurological Disorders and Stroke "Towards Clinical Progress in the Mucopolysaccharidoses", Washington March 7-8, 2007.

"Exploiting AAV capsids for photoreceptor gene transfer in animal models of retinal degeneration"; "Retinal Degeneration and Gene Therapy" Eleventh Annual Vision Research Conference, Convention Center - Ft. Lauderdale, FL – USA, May 4-5, 2007.

"AAV vectors for retinal gene therapy" Euretina Congress 2007 Montecarlo, France May 18-20, 2007.

"Principles of gene therapy and pediatrics application" Italian Society of Pediatrics (SIP), Salerno, Italy, November 17 2007.

"New possibilities to treat inherited retinal diseases with AAV vectors" "Day of Science" Foundation Fighting Blindness San Diego, CA, USA, February 29, 2008.

"Advancement in AAV-Mediated Retinal Gene Transfer for Inherited Retinal Diseases" 5th Annual Conference of the British Society for Gene Therapy Heriot-Watt University, Edinburgh in 7-9 April, 2008.

"Kick-off Meeting" AAVEYE – FP7, Naples, Italy, 25-26 June, 2008.

"Gene therapy for retinal diseases" European Society of Gene Terapy Annual meeting, Brugge 11-16 November, 2008.

"Serotype- dependent packaging of large genomes in adeno-associatedviral vectorsresult in efficient in vivo gene delivery" European Society of Gene Terapy Annual meeting, Brugge 11-16 November, 2008.

"Gene Therapy of retinal diseases" Neuro- Ophthalmology Update Course Madrid, Spain 20 February, 2009.

"Advancement in AAV – mediated retinal gene transfer for inherited retinal diseases" Convention Telethon 2009 Riva del Garda, Italy 9-11 March, 2009

"Trasferimento genico per malattieretiniche" 8th master in clinical genetics, International School of Pediatrics Science, Genova, Italy 22 April, 2009.

"Advancements in AAV-mediated gene transfer for inherited retinal diseases", Vision of Children Foundation International Symposium, San Diego, California - USA, 1-3 June, 2009.

"AAV-mediated gene transfer for Mucopolysaccharridosis VI" In vivo application of AAv meeting, Nantes, France, 3-4 September 2009.

"Therapy I: gene therapy for inharited retinal diseases" Mucolpolysaccharidosis (MPS) and the Eye, Venice, Italy, 7-9 October 2009.

"AAV-mediated gene transfer to the retina: from bench to bedside" ESGT combined meeting, Hannover, Germany, 21-25 November 2009.

"Treatrush" Kick-off meeting, Paris, France, 17-18 February 2010.

"AAV-mediated liver gene transfer in mucopolysaccharidosis VI cats" Clinigene 4th Anniversary meeting, Annecy, France, 25-28 March 2010.

"Gene therapy of inherited retinal diseases" 5th Canadian Gene Therapy and Vaccines Symposium, Montreal, Canada, 2-4 May 2010.

"Terapia genica delle distrofie retiniche ereditarie" 27th Congresso Nazionale S.I.O.P., Napoli, 10-12 June 2010-04-27

"Genes and Gene Therapy" Keynote lecture, Retinal International, Stresa- lago Maggiore, Italy, 27 June 2010.

INTERNATIONAL PATENTS

1) Recombinant AAV vectors with AAV5 capsids and AAV5 vectors pseudotyped in heterologous capsids. International patent number 01928750.7-2405-US0113000. Date of filing: 23 April 2001. This patent covers the use for gene therapy applications of a novel hybrid adeno-associated viral vector (AAV) 2/5 between serotypes 2 and 5. Capsid exchange between 2 and 5 allows for transduction of cells resistant to AAV2 infection (i.e. airway epithelia).

2) Method for purification of viral vectors having proteins which bind sialic acid. International patent PCT/WO03/014367-A1. Date of filing: 06 August 2002 This patents covers the use of sialic acid for purification of viruses and more specifically those with an AAV 5 capisd. The sialic acid is a component of the receptor complex

which binds to the AAV5 capsid. The sialic acid has been therefore used to build a chromatographic column for AAV5 affinity purification. Vector preps obtained with this method are as infectious as and purer than those obtained with traditional physical methods of purification.

3) Methods and composition for treatment of obesity. UPN-N2603. This patent covers the use of genes of the uncoupling protein (UCP) family, and more specifically UCP3, for the treatment of obesity and type 2 diabetes. UCP proteins uncouple the mithocondrial oxidative chain from the ATP production resulting in both ATP loss and inability to build up ATP stores. UCP over-expression in muscle trough transgenic technologies results in mice that eat more than wild type animals but weight less and are more resistant to a glucose load. Here this observation is brought to a gene therapy level and UCP3 somatic gene transfer to muscle of genetically obese mice aims at reducing their weight and blood glucose levels. Theoretically, following gene transfer of pharmacologically regulated UCP3 one could take a pill before a rich meal and therefore burn calories without storing them.

4) Composition and methods useful for non-invasive delivery therapeutic molecules to the bloodstream. Date of filing: 11 September 2002. UPN-N2605. This patent covers the use of gene transfer to lung using viral vectors to release systemic proteins, as clotting factors, erythropoietin, growth hormone, insulin, etc…The lung is easily accessible trough the airways and offers a large exchange surface with the systemic circulation, comparable to an additional easily accessible tissue, the gut. The lung transduced by viral vectors can therefore work as a factory for production of systemic proteins, in that being even more desirable than classic gene therapy targets as muscle and liver.

5) Laser controlled temporal and spatial regulation of gene delivery. Date of filing: 16 August 2002. UPN-O2798. This patent covers the use of a heat sensititve promoter (heat shock protein 70, HSP70) included in a viral vector for regulation of gene expression in the retina using laser. Therefore, using this promoter following intraocular gene transfer, i.e. of antiangiogenic or neuronal growth factors, their expression could be regulated in a specific retinal region simply heating the area with laser. Laser photocoagulation is commonly used to treat age-related macular degeneration or diabetic retinopathy.

6) Use of DAT for in vivo, non-invasive imaging of gene transfer. Year of filing: 2002. UPN-O2722. The dopamine transporter (DAT) is a membrane protein specifically expressend in the central nervous system. DAT binds with high affinity to 99Tc-TRODAT, a radioligand that can be imaged trough Single Photon Emission Computerized Tomography (DAT). This system can be used to monitor gene transfer. The DAT coding sequence can be introduced in a vector expressing a therapeutic gene. Following the transduction of a specific tissue with the vector (any tissue but the central nervous system can be targeted for this purpose), and the 99Tc-TRODAT injection, SPECT imaging of the transduced tissue can be obtained in vivo, repeatedly and non-invasively.

7) Use of compounds that interfere with the hedghehog signaling pathway for the manufacture of a medicament for preventing, inhibiting, and/or reversing ocular diseases related to ocular neovascularization. Date of filing: 2 December 2005. SG/VC.AE25783. This patent application covers the use of Sonic Hedhehog (Shh) inhibitors, as cyclopamine, RNA interference or Shh binding proteins, for treatment of common causes of blindness, as age-related amcular degeneration and diabetic retinopathy.

8) Methods and compositions for recovering or improving visual function. Date of filing: 5 March 2006. US patent. Application Serial No. 11/416,453. This patent application covers the use of AAV-mediated gene transfer to rescue visual function in individuals affected with type I Ocular Albinism.

9) Method for recovering tyrosinase albino retinal anomalies. Date of filing: 15 February 2008 US patent. Application Serial No.12/032,508. This patent application covers the use of AAV-mediated gene transfer to rescue visual function in individuals affected with Ocular-Cutaneous Albinism.

10) Method of treating ocular diseases by gene therapy. Date of filing: 21 February 2008 US patent. Application Serial No.12/071,508. This patent application covers the use of AAV-mediated gene transfer to rescue visual function in individuals affected with Stardgart disease.

11) Method of treating genetic disorders. Date of filing: 2 April 2008 US provisional n. 61/041,746. This patent application covers the use of AAV-mediated gene transfer to rescue visual function in individuals affected with tupe IB Usher Syndrome and Congenital Leber Amaurosi due to CEP290 mutation.

Publications

1- F. Fazioli, D. P. Bottaro, L. Minichiello, A. Auricchio, W. T. Wong, O. Segatto, P. P. Di Fiore (1992) Identification and biochemical characterization of novel putative substrates for the Epidermal Growth Factor Receptor kinase. *J. Biol. Chem*.267(8): 5155-61. PMID: 1347529.

2- A. Auricchio, M. di Domenico, G. Castoria, A. Bilancio, A. Migliaccio (1994) Epidermal Growth Factor induces protein tyrosine phosphorylation and association of p190 with ras-GTPase activating protein in Caco 2 cells. *FEBS Letters*. 353(1):16-20. PMID: 7926013.

3- A. Auricchio, G. Casari, A. Staiano, A. Ballabio (1996) Endothelin-B receptor mutations in patients with isolated Hirschsprung disease from a non inbred population. *Hum. Mol. Genet*. 5(3):351-4. PMID: 8852659.

4- A. Auricchio, V. Brancolini, G. Casari, P. J. Milla, V. V. Smith, M. Devoto, A. Ballabio (1996) The locus for a novel syndromic form of neuronal intestinal pseudoobstruction maps to Xq28. *Am. J. Hum. Genet*. 58(4):743-8. PMID: 8644737.

5- A. Staiano, L. Santoro, R. De Marco, E. Miele, F. Fiorillo, A. Auricchio, M. L. Carpentieri, J. Celli, S. Auricchio (1999) Autonomic dysfunction in children with Hirschsprung's disease. *Dig. Dis. Sci*. 44(5):960-5. PMID: 10235604.

6- A. Auricchio , P. Griseri, M. L. Carpentieri, N. Betsos, A. Staiano, A. Tozzi, M. Priolo, H. Thompson, R. Bocciardi, G. Romeo, A. Ballabio, I. Ceccherini (1999) Double heterozigosity for a RET substitution interfering with splicing and an EDNRB missense mutation in Hirschsprung disease. *Am. J. Hum. Genet*. 64(4):1216-21. PMID: 10090908.

7- A. Auricchio*, M. Hildinger*, E. O'Connor, G. Gao, J. M. Wilson (2001) Isolation of highly infectious and pure AAV2 vectors with a single-step gravity-flow column. *Hum. Gene Ther*.12(1):71-6. *these two authors contributed equally to the work. PMID: 11177544.

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