Europass Curriculum Vitae

Personal information

First name / Surname	**MAURIZIO SCARPA, MD PhD**
Address	Center For Rare Diseases, Horst Schmidt Klinik, Ludwig Erhrad Straße 100, Wiesbaden, DE
Telephones	Mobile +39348033390
Fax	
E-mail	maurizio.scarpa@brains4brain.eu
Nationality	Italian
Date of birth	September 15 1959
Gender	Male
Occupational field	Director Center for Rare Diseases, Horst Schmidt Klinik, Wiesbaden DE

WORK EXPERIENCE

CLINICAL Experience

1991- present: Head Lysosomal Unit, Dept. of Pediatrics University of Padova,
1995-1997: Staff Pediatric Intensive Care Unit, Dept. of Pediatrics University of Padova,
1997- present: Consultant in General Pediatrics .Laguna Medical Centre Venice, Italy
1997-2005; Staff, Inherited Metabolic Diseases Unit, Dept.of Pediatrics, Padova
2005-present: Staff, Pediatric Neurology Unit Dept. Of Pediatrics Padova,
2011- present: Director, Centre for Rare Disease IRCCS Casa Sollievo della Sofferenza, San Giovanni Rotondo, Foggia, Research Hospital
2014-present: Director Center fo Rare Diseases, Horst Schmidt Klinik, Wiesbaden. DE

Clinical Trial Activity

2004 Principal Investigator For Phase III Extension Clinical Studies For Idursulfase (Elaprase, SHRIE HGT) And Galsulfase (Naglazyme, Biomarin), Italian Branch;

Principal Instigators of National Clinical Trial Programmes on Mucopolysaccharidosis Type II, Fabry, GM1 gangliosidosis.

TEACHING EXPERIENCES

2004-2011: Director PhD Programme on Molecular Genetics and Biochemistry, University of Padova, I

2004-2010: Professor of Applied Biology, School of Neurosurgery, University of Padova

2004-2010: Professor of Biochemistry, School of Neuropsichiatry University of Padova

2004-2010: Professor of Molecular Biology School of Neuropsichiatry University of Padova.

ACADEMICAL DUTIES

2008-2011: Vice-Dean and Director Of the International Affaires OfficeUniversity of Padova Medical School

2008-2011:Member of the University Commission for International Affaires,University of Padova Medical School

2009-2011: Member of the Development and Planning Commission, University of Padova Medical School

MEMBERSHIPS

1992: Founding Member of the European Society for Gene Therapy

1994: Member of the Scientific Committee, Italian Association for Mucopolysaccharidosis.

1994 Member of the Gene Therapy Committee Italian Minister for the EEC, Commission on Safety of Genetically Modified Microorganisms.

1999-present: Member of the European Study Group for Lysosomal Diseases (ESLGD)

2000-present: Member Society for the Study of the Inborn Errors of Metabolism (SSIEM)

2002-2007: Founding Member: Global Organization for Lysosomal Disease (G.O.L.D.)

2007: Founder of BRAINS FOR BRAIN European Task Force for the Research on Pediatric Neurodegenerative Disorders

2008- present: Board Member of the European Brain Council

2009-2011: VicePresident, BRAINS For BRAIN FOUNDATION (www.brains4brain.eu)

2012- present: President, BRAINS For BRAIN FOUNDATION (www.brains4brain.eu)

2010-present: Member of the European Science Foundation Pool of reviewers

2010-present: Board Member of the Euopean Personalised Medicine Association

EDUCATION AND TRAININGS

1980-1985: Working group on Cystic Fibrosis, Dept. of Pediatrics, University of Padova, Italy.

1985: M.D. DEGREE, UNIVERSITY OF PADOVA, Medical School , Padova, Italy.

1985-1987: MOLECULAR BIOLOGY TRAINING: Postdoctoral fellowship for Prof. Gianni Cesareni's laboratory, Dept. Gene Structure and Regulation European Molecular Biology Laboratory, (E.M.B.L.) Heidelberg, Germany;

1988-1990: MOLECULAR GENETICS AND CLINICAL GENETICS TRAINING: Postdoctoral Fellowship. Prof. Charles Thomas Caskey's laboratory. Institute for Molecular Genetics, Baylor College of Medicine, Houston,Texas, U.S.A.

1989: SPECIALIST ON PEDIATRICS, UNIVERSITY OF PADOVA

1990-1993: CONSULTANT IN MOLECULAR BIOLOGY Prof. GianFranco Bottazzo's Laboratory, Department of Immunology, The London Hospital Medical College, London, UK.

2004: PHD DEGREE: PEDIATRIC SCIENCES

CERTIFICATIONS

1985: Medical Doctor Degree, University of Padova, Italy.

1985: License for Medical Practice, Italian Ministry of Health

1989: Diploma of Pediatrics, Department of Pediatrics, University of Padova, Italy.

2004: PhD Degree, Pediatric Sciences, University of Padova, Medical School

LANGUAGES

Mother tongue(s) **Italian**

Other language **English**

	Understanding		Speaking		Writing
	Listening	Reading	Spoken interaction	Spoken production	
Language	Optimal	Optimal	Optimal	Optimal	Optimal

Other language **German,** started course of basic language

Organisational skills

2006 June: IX International Symposium on Mucopolysaccharide and Related Disorders, Venice. President and Organiser
2007: First Brains For Brain Meeting Madrid, Spain, Organiser
2008-2012: Second to Sixth Brains For Brain Meeting, Frankfurt, Germany, Organiser
2007: First International Meeting on Lysosomal diseases SHIRE HGT, Rome; Organiser
2008: X International Symposium on Mucopolysaccharide and Related Disorders, Vancouver Canada, Scientific Committee
2008: World Biomarin meeting on Bone and MPS, Novato California, USA, Organiser
2009: World Biomarin Meeting on Eye and MPS, Venice Italy, Oganiser
2009: CNS WORKING GROUP SHIRE HGT, Chair
2010: World Biomarin Meeting on Heart and MPS, Rio de Janeiro, Brasil, Organiser
2010: XI International Symposium on Mucopolysaccharide and Related Disorders, Adelaide Australia, Scientific Committee
2010: First International Meeting on Pediatric Neurodegenerative Disorders; EU Parliament, Bruxelles, Organiser
2010: European Expert Group on Hunter Disease, SHIRE HGT Chair
2011: First Gordon Conference on Lysosomal Diseases, Galveston Texas, USA (scientific board)
2012: XII International Symposium on Mucopolysaccharide and Related Disorders, Amsterdam The Netherlands, Scientific Committee
2013: Second Gordon Conference on Lysosomal Diseases, Firenze, Italy (scientific board)
2014: XIII International Symposium on Mucopolysaccharide and Related Disorders, Baja Brasil, Scientific Committee

INTERNATIONAL REVIEWER ACTIVITY

Institutional:
Reviewer for the EU Community FP7 Programme
Member of the Expert panel of the European Science Foundation
Reviewer for the Medical Research Council, UK
Reviewer for the French Research Council
Reviewer for the Cochraine Clinical Trial Organization
Gene Review expert

International Journals:
Gene Therapy, Clinical Chemistry, Pediatrics, J. Pediatrics, Nanomedicine. Orphanet Journal of Rare Diseases
Communicating Editor: Journal Inherited Metabolic Diseases

FINANCIAL MANAGEMENT AND GRANT COMPETENCES

Experiences in drafting and managing EU projects (DGRESEARCH, DGSANCO, European Science Foundation), national and International Grant Proposals.

SCIENTIFIC COUNSELLING ACTIVITIES

Scientific Consultant for Actelion, Biomarin, Genzyme and SHIRE HGT

Additional information

Professional Diploma of Sommelier at the Italian Sommelier Association,

Annexes

List of Scientific Publications, Copy of Certificates, Letters of Support from International Academical and Pharma Colleagues

PUBLICATIONS

1) Antagonistic controls regulate copy number of the yeast 2 mu plasmid..
MURRAY, JA;SCARPA, M;ROSSI, N;CESARENI, G.
EMBO J. 1988. ; 6:4205-12 IF: 13.999
IDPUBMED

2) Genetic and structural analysis of the ColE1 Rop (Rom) protein..
CASTAGNOLI, L;SCARPA, M;KOKKINIDIS, M;BANNER, DW;TSERNOGLOU, D;CESARENI, G.
EMBO J. 1989. ; 8:621-9 IF: 13.999
IDPUBMED

3) Determination of base specificity in 6,4,4'-trimethylangelicin photobinding to single-stranded DNA..
MIOLO, G;SCARPA, M;DALL'ACQUA, F;ZACCHELLO, F.
J PHOTOCH PHOTOBIO B. 1989. ; 3:123-7 IF: 1.529
IDPUBMED

4) Gene therapy: a new approach for the treatment of genetic disorders..
COURNOYER, D;SCARPA, M;JONES, SN;MOORE, KA;BELMONT, JW;CASKEY, CT.
Clinical Pharmacology and Therapeutics. 1990. ; 47:1-11 IF: 5.275
IDPUBMED

5) SCREENING FOR CYSTIC-FIBROSIS GENE-MUTATIONS BY MULTIPLEX DNA AMPLIFICATION.
PICCI, L; ANGLANI, F; SCARPA, M; ZACCHELLO, F.
HUM GENET. 1992. ; 88(5):552-556 IF: 3.422
Riferimento WoK

6) Screening for cystic fibrosis gene mutations by multiplex DNA amplification..
PICCI, L;ANGLANI, F;SCARPA, M;ZACCHELLO, F .
HUM GENET. 1992. ; 88:552-6 IF: 3.422
IDPUBMED

7) Gene therapy..
COURNOYER, D;SCARPA, M;CASKEY, CT.
Current Opinion in Biotechnology. 1992. ; 1:196-208 IF: 4.711
IDPUBMED

8) Evaluation of lymphoid-specific enhancer addition or substitution in a basic retrovirus vector..
MOORE, KA;SCARPA, M;KOOYER, S;UTTER, A;CASKEY, CT;BELMONT, JW.
Human gene therapy. 1992. ; 2:307-15 IF: 6.796
IDPUBMED

9) Gene transfer of adenosine deaminase into primitive human hematopoietic progenitor cells..
COURNOYER, D;SCARPA, M;MITANI, K;MOORE, KA;MARKOWITZ, D;BANK, A;BELMONT, JW;CASKEY, CT.
Human gene therapy. 1992. ; 2:203-13 IF: 6.796
IDPUBMED

10) GENE-TRANSFER IN REGENERATING MUSCLE.
VITADELLO, M; NOVELLI, A; PICARD, A; SCHIAFFINO, MV; SCARPA, M; SCHIAFFINO, S.
J CELL BIOCHEM. 1993. ; 0(0):252-252 IF: 2.775
Riferimento WoK

11) GENE-TRANSFER IN REGENERATING MUSCLE.
VITADELLO, M; SCHIAFFINO, MV; PICARD, A; SCARPA, M; SCHIAFFINO, S.
Human gene therapy. 1994. ; 5(1):11-18 IF: 6.796
Riferimento WoK

12) ANTIRETROVIRAL ACTIVITY OF FUROCOUMARINS PLUS UVA LIGHT DETECTED BY A REPLICATION-DEFECTIVE RETROVIRUS.
MIOLO, G; TOMANIN, R; DEROSSI, A; DALLACQUA, F; ZACCHELLO, F; SCARPA, M.
J PHOTOCH PHOTOBIO B. 1994. ; 26(3):241-247 IF: 1.529
Riferimento WoK

13) Gene transfer in regenerating muscle..
VITADELLO, M;SCHIAFFINO, MV;PICARD, A;SCARPA, M;SCHIAFFINO, S.
Human gene therapy. 1994. ; 5:11-8 IF: 6.796
IDPUBMED

14) Antiretroviral activity of furocoumarins plus UVA light detected by a replication-defective retrovirus..
MIOLO, G;TOMANIN, R;DE, ROSSI, A;DALL'ACQUA, F;ZACCHELLO, F;SCARPA, M.
J PHOTOCH PHOTOBIO B. 1995. ; 26:241-7 IF: 1.529
IDPUBMED

15) In vitro and in vivo gene transfer to pulmonary cells mediated by cationic liposomes.
Fortunati, E; Bout, A; Zanta, MA; Valerio, D; Scarpa, M.
BBA-GENE STRUCT EXPR. 1996. ; 1306(1):55-62 IF: 2.243
Riferimento WoK

16) ExGen 500 is an efficient vector for gene delivery to lung epithelial cells in vitro and in vivo.
Ferrari, S; Moro, E; Pettenazzo, A; Behr, JP; Zacchello, F; Scarpa, M.
GENE THER. 1997. ; 4(10):1100-1106 IF: 5.964
Riferimento WoK

17) The bacteriophage T7 binary system activates transient transgene expression in zebrafish (Danio rerio) embryos.
Verri, T; Argenton, F; Tomanin, R; Scarpa, M; Storelli, C; Costa, R; Colombo, L; Bortolussi, M.
BIOCHEM BIOPH RES CO. 1997. ; 237(3):492-495 IF: 3.055
Riferimento WoK

18) Development and characterization of a binary gene expression system based on bacteriophage T7 components in adenovirus vectors.
Tomanin, R; Bett, AJ; Picci, L; Scarpa, M; Graham, FL.
GENE. 1997. ; 193(2):129-140 IF: 2.461
Riferimento WoK

19) In vitro correction of iduronate-2-sulfatase deficiency by adenovirus-mediated gene transfer.
DiFrancesco, C; Cracco, C; Tomanin, R; Picci, L; Ventura, L; Zacchello, F; DiNatale, P; Anson, DS; Hopwood, JJ; Graham, FL; Scarpa, M.
GENE THER. 1997. ; 4(5):442-448 IF: 5.964
Riferimento WoK

20) In vitro and in vivo antitumor effects of retrovirus-mediated herpes simplex thymidine kinase gene-transfer in human medulloblastoma.
Rosolen, A; Frascella, E; di Francesco, C; Todesco, A; Petrone, M; Mehtali, M; Zacchello, F; Zanesco, L; Scarpa, M.
GENE THER. 1998. ; 5(1):113-120 IF: 5.964
Riferimento WoK

21) ExGen 500 is an efficient vector for gene delivery to lung epithelial cells in vitro and in vivo..
FERRARI, S;MORO, E;PETTENAZZO, A;BEHR, JP;ZACCHELLO, F;SCARPA, M.
GENE THER. 1998. ; 4:1100-6 IF: 5.964
IDPUBMED

22) Polyethylenimine shows properties of interest for cystic fibrosis gene therapy.
Ferrari, S; Pettenazzo, A; Garbati, N; Zacchello, F; Behr, JP; Scarpa, M.
BBA-GENE STRUCT EXPR. 1999. ; 1447(2):219-225 IF: 2.243
Riferimento WoK

23) Recombinant adenoviral vector-LipofectAMINE complex for gene transduction into human T lymphocytes.
Di Nicola, M; Milanesi, M; Magni, M; Bregni, M; Carlo-Stella, C; Longoni, P; Tomanin, R; Ravagnani, F; Scarpa, M; Jordan, C; Gianni, AM.
Human gene therapy. 1999. ; 10(11):1875-1884 IF: 6.796
Riferimento WoK

24) Detection of five rare cystic fibrosis mutations peculiar to southern Italy: Implications in screening for the disease and phenotype characterization for patients with homozygote mutations.
Castaldo, G; Fuccio, A; Cazeneuve, C; Picci, L; Salvatore, D; Raia, V; Scarpa, M; Goossens, M; Salvatore, F.
CLIN CHEM. 1999. ; 45(7):957-962 IF: 4.261
Riferimento WoK

25) A noval nonsense mutation (Y849X) in the CFTR gene of a CF patient from southern Italy..
Castaldo, G;Fuccio, A;Cazeneuve, C;Picci, L;Salvatore, D;Scarpa, M;Goossens, M;Salvatore, F. .
HUM MUTAT. 1999. ; 14:- IF: 3.666
IDPUBMED

26) Large-scale feasibility of gene transduction into human CD34(+) cell-derived dendritic cells by adenoviral/polycation complex.
Di Nicola, M; Carlo-Stella, C; Milanesi, M; Magni, M; Longoni, P; Mortarini, R; Anichini, A; Tomanin, R; Scarpa, M; Gianni, AM.
BRIT J HAEMATOL. 2000. ; 111(1):344-350 IF: 3.068
Riferimento WoK

27) Genetic testing - Part I - Introduction.
Scarpa, M.
Minerva Biotecnologica. 2000. ; 12(1):3-3 IF: 0.233
Riferimento WoK

28) Mucus altering agents as adjuncts for nonviral gene transfer to airway epithelium.
Ferrari, S; Kitson, C; Farley, R; Steel, R; Marriott, C; Parkins, DA; Scarpa, M; Wainwright, B; Evans, MJ; Colledge, WH; Geddes, DM; Alton, EWFW.
GENE THER. 2001. ; 8(18):1380-1386 IF: 5.893
Riferimento WoK

29) Update on enzyme replacement therapy in mucopolysaccharidosis type II.
Muenzer, J; Scarpa, M.
ACTA PAEDIATR. 2002. ; 91(0):81-81 IF: 1.26
Riferimento WoK

30) Non-viral transfer approaches for the gene therapy of mucopolysaccharidosis type II (Hunter syndrome).
Tomanin, R; Friso, A; Alba, S; Puicher, EP; Mennuni, C; La Monica, N; Hortelano, G; Zacchello, F; Scarpa, M.
ACTA PAEDIATR. 2002. ; 91(0):100-104 IF: 1.26
Riferimento WoK

31) Uptake of recombinant iduronate-2-sulfatase into neuronal and glial cells in vitro.
Daniele, A; Tomanin, R; Villani, GRD; Zacchello, F; Scarpa, M; Di Natale, P.
Biochimica et Biophysica Acta - Molecular Basis of Disease. 2002. ; 1588(3):203-209 IF: 3.3
Riferimento WoK

32) Electrogene transfer in Mucopolysaccharidosis type II mouse model induces high-level production of iduronate-2-sulfatase in the skeletal muscle.
Tomanin, R; Friso, A; Alba, S; Puicher, EP; Gasparotto, N; Mennuni, C; La Monica, N; Muenzer, J; Zacchelio, F; Scarpa, M.
MOL THER. 2003. ; 7(5):1024 - IF: 6.125
Riferimento WoK

33) Non-viral transfer approaches for the gene therapy of mucopolysaccharidosis type II (Hunter syndrome)..
TOMANIN, R;FRISO, A;ALBA, S;PILLER, PUICHER, E;MENNUNI, C;LA, MONICA, N;HORTELANO, G;ZACCHELLO, F;SCARPA, M .
ACTA PAEDIATR. 2003. ; 91:100-4 IF: 1.128
IDPUBMED

34) Chimeric vectors.
Scarpa, M.
Current gene therapy. 2004. ; 4(4):- IF: 3.681
Riferimento WoK

35) Why do we need new gene therapy viral vectors? Characteristics, limitations and future perspectives of viral vector transduction.
Tomanin, R; Scarpa, M.
Current gene therapy. 2004. ; 4(4):357-372 IF: 3.681
Riferimento WoK

36) Reduction of GAG storage in MPS II mouse model following implantation of encapsulated recombinant myoblasts.
Friso, A; Tomanin, R; Alba, S; Gasparotto, N; Puicher, EP; Fusco, M; Hortelano, G; Muenzer, J; Marin, O; Zacchello, F; Scarpa, M.
J GENE MED. 2005. ; 7(11):1482-1491 IF: 3.699
Riferimento WoK DOI

37) Gender-sensitive association of CFTR gene mutations and 5T allele emerging from a large survey on infertility.
Morea, A; Cameran, M; Rebuffi, AG; Marzenta, D; Marangon, O; Picci, L; Zacchello, F; Scarpa, M.
MOL HUM REPROD. 2005. ; 11(8):607-614 IF: 3.191
Riferimento WoK DOI

38) Update on enzyme replacement therapy (ERT) with recombinant human arylsulfatase B (rhASB) for MPS VI (Maroteaux-Lamy).
Harmatz, P; Giugliani, R; Schwartz, I; Guffon, N; Miranda, CS; Teles, E; Wraith, JE; Beck, M; Scarpa, M; Yu, ZF; Wittes, J; Berger, K; Newman, M.
PEDIATR RES. 2005. ; 58(2):169 - IF: 2.875
Riferimento WoK

39) Cystic fibrosis carriers have higher neonatal immunoreactive trypsinogen values than non-carriers.
Castellani, C; Picci, L; Scarpa, M; Dechecchi, MC; Zanolla, L; Assael, BM; Zacchello, F.
AM J MED GENET A. 2005. ; 135(2):142-144 IF: 1.913
Riferimento WoK DOI

40) A PHASE 3, RANDOMIZED, DOUBLE-BLIND, PLACEBO-CONTROLLED, MULTICENTER, MULTINATIONAL CLINICAL STUDY OF RECOMBINANT HUMAN N-ACETYLGALACTOSAMINE 4 SULFATASE (RHASB) IN PATIENTS WITH MUCOPOLYSACCHARIDOSIS VI.
Guffon, N.; Harmatz, P.; Giugliani, R.; Schwartz, I.; Miranda, C.; Teles, E.; Wraith, Ed; Beck, M.; Arash, L.; Scarpa, M.; Swiedler, S..
J INHERIT METAB DIS. 2005. ; 28(0):190-190 IF: 1.722
Riferimento WoK

41) A 3-year-old child with abdominal pain and fever..
BONETTO, G;SCARPA, M;CARRARO, S;BARALDI, E.
EUR RESPIR J. 2005. ; 26:974-7 IF: 3.947
IDPUBMED

42) Enzyme replacement therapy for mucopolysaccharidosis VI: A Phase 3, randomized, double-blind, placebo-controlled, multinational study of recombinant human N-acetylgalactosamine 4-sulfatase (recombinant human arylsulfatase B or rhASB) and follow-on, open-label extension study.
Harmatz, P; Giugliani, R; Schwariz, I; Guffon, N; Teles, EL; Miranda, MCS; Wraith, JE; Beck, M; Arash, L; Scarpa, M; Yu, ZF; Wittes, J; Berger, KI; Newman, MS; Lowe, AM; Kakkis, E; Swiedler, SJ.
J PEDIATR. 2006. ; 148(4):533-539 IF: 3.991
Riferimento WoK DOI

43) Long term benefit and safety with recombinant human arylsulfatase, B (rhASB) ERT for MPS VI.
Harmatz, P.; Giugliani, R.; Schwartz, I.; Guffon, N.; Sa Miranda, C.; Teles, E.; Wraith, J. E.; Beck, M.; Scarpa, M.; Yu, Z-F; Rhorer, J.; Swiedler, S. J.; Decker, C..
J INHERIT METAB DIS. 2006. ; 29(0):29-29 IF: 1.574
Riferimento WoK

44) Neurological examinations and clinical manifestations in MPS I as reported in the MPS I registry.
Scarpa, M..
J INHERIT METAB DIS. 2007. ; 30(0):97-97 IF: 1.668
Riferimento WoK

45) Phase 3 extension 96-week study data for naglazyme (galsulfase) enzyme replacement therapy (ERT) in MPS VI (Maroteaux-Lamy syndrome) patients.
Harmatz, P.; Gingliani, R.; Schwartz, I; Guffon, N.; Sa, Miranda C.; Teles, E.; Wraith, J.; Beck, M.; Scarpa, M.; Yu, Z. F.; Rhorer, J.; Swiedler, S.; Turbeville, S.; Nicely, H.; White, J.; Decker, C..
J INHERIT METAB DIS. 2007. ; 30(0):116-116 IF: 1.668
Riferimento WoK

46) TG15 T5 allele in clinically discordant monozygotic twins with cystic fibrosis.
Picci, Luigi; Cameran, Marilena; Scarpa, Maurizio; Pradal, Ugo; Melotti, Paola; Assael, Baroukh M.; Castellani, Carlo.
AM J MED GENET A. 2007. ; 143(16):1936-1937 IF: 2.44
Riferimento WoK DOI

47) Efficiency of stem cell infections of amniotic fluid by means of adenoviral carriers.
Piccoli, M.; Grisafi, D.; Pozzobon, M.; Bollini, S.; Marangoni, P.; Carli, M.; Zanesco, L.; Scarpa, M.; De, Coppi P.; Tomanin, R..
HAEMATOL-HEMATOL J. 2007. ; 92(6):45-45 IF: 5.516
Riferimento WoK

48) Neurologic examinations and clinical manifestations in mucopolysaccharidosis I: MPS I registry data.
Scarpa, M..
Clinical Therapeutics. 2007. ; 29(0):S123-S123 IF: 3.261
Riferimento WoK

49) High Transduction Efficiency of Human Amniotic Fluid Stem Cells Mediated by Adenovirus Vectors.
Grisafi, Davide; Piccoli, Martina; Pozzobon, Michela; Ditadi, Andrea; Zaramella, Patrizia; Chiandetti, Lino; Zanon, Giovanni Franco; Atala, Anthony; Zacchello, Franco; Scarpa, Maurizio; De Coppi, Paolo; Tomanin, Rosella.
Stem Cells and Development. 2008. ; 17(5):953-962 IF: 3.273
Riferimento WoK DOI

50) Gene therapy of Hunter syndrome: Evaluation of the efficiency of muscle electro gene transfer for the production and release of recombinant iduronate-2-sulfatase (IDS).
Friso, A.; Tomanin, R.; Zanetti, A.; Mennuni, C.; Calvaruso, F.; La Monica, N.; Marin, O.; Zacchello, F.; Scarpa, M..
Biochimica et Biophysica Acta - Molecular Basis of Disease. 2008. ; 1782(10):574-580 IF: 4.579
Riferimento WoK DOI

51) Molecular analysis of 82 mucopolysaccharidosis type I patients: Mutational spectrum in the European population and identification of 28 novel mutations.
Bertola, F.; Parini, R.; Casati, G.; Tylki-Szymanska, A.; Okur, I; Tuysuz, B.; Dalmau, J.; Gonzales, Meneses A.; Antuzzi, D.; Barone, R.; Dionisi, Vici C.; Donati, A.; Filocamo, M.; Gabrielli, O.; Parenti, G.; Scarpa, M.; Uziel, G.; Biondi, A..
J INHERIT METAB DIS. 2008. ; 31(0):426P - IF: 2.691
Riferimento WoK

52) Morquio syndrome: Gene expression profiling and elastic fiber assembly in patients' fibroblasts.
Caciotti, A.; Carraresi, L.; Filoni, C.; Parini, R.; Antuzzi, D.; Ricci, R.; Scarpa, M.; Procopio, E.; Dazzo, A.; Zammarchi, E.; Guerrini, R.; Donati, M. A.; Morrone, A..
J INHERIT METAB DIS. 2008. ; 31(0):447P - IF: 2.691
Riferimento WoK

53) Clinical phenotype of Italian patients with Hunter syndrome: Data from HOS - The Hunter Outcome Survey.
Parini, R.; Melzi, M. L.; Rigoldi, M.; Sala, S.; Rampazzo, A.; Gabrielli, O.; DiRocco, M.; Feliciani, C.; Castorina, M.; Cicognani, A.; Scarpa, M..
J INHERIT METAB DIS. 2008. ; 31(0):456P - IF: 2.691
Riferimento WoK

54) Long-term follow-up of endurance and safety outcomes during enzyme replacement therapy for mucopolysaccharidosis VI: Final results of three clinical studies of recombinant human N-acetylgalactosamine 4-sulfatase.
Harmatz, Paul; Giugliani, Roberto; Schwartz, Ida Vanessa D.; Guffon, Nathalie; Teles, Elisa Leao; Miranda, M. Clara Sa; Wraith, J. Edmond; Beck, Michael; Arash, Laila; Scarpa, Maurizio; Ketteridge, David; Hopwood, John J.; Plecko, Barbara; Steiner, Robert; Whitley, Chester B.; Kaplan, Paige; Yu, Zi-Fan; Swiedler, Stuart J.; Decker, Celeste.
MOL GENET METAB. 2008. ; 94(4):469-475 IF: 2.629
Riferimento WoK DOI

55) Lysosomal storage diseases and the blood-brain barrier.
Begley, DJ.; Pontikis, Charles C.; Scarpa, Maurizio.
Current Pharmaceutical Design. 2008. ; 14(16):1566-1580 IF: 4.399
Riferimento WoK

56) Eighteen-year follow-up of the first Italian MPSI patient treated with bone marrow transplantation.
Messina, C.; Rampazzo, A.; Cesaro, S.; Monciotti, C.; Gasparotto, N.; Tomanin, R.; Scarpa, M..
Bone Marrow Transplantation. 2008. ; 41(10):905-906 IF: 3.4
Riferimento WoK DOI

57) Molecular markers for the follow-up of enzyme-replacement therapy in mucopolysaccharidosis type VI disease.
Di Natale, Paola; Villani, Guglielmo R. D.; Parini, Rossella; Scarpa, Maurizio; Parenti, Giancarlo; Pontarelli, Gianfranco; Grosso, Michela; Sersale, Giovanna; Tomanin, Rosella; Sibilio, Michelina; Barone, Rita; Fiumara, Agata.
BIOTECHNOL APPL BIOC. 2008. ; 49(0):219-223 IF: 1.288
Riferimento WoK DOI

58) Seventh international symposium on lysosomal storage diseases.
Aerts, Johannes; Beck, Michael; Martin, Rick; Scarpa, Maurizio.
ACTA PAEDIATR. 2008. ; 97(0):1-2 IF: 1.517
Riferimento WoK DOI

59) Lysosomal storage diseases: new challenges.
Scarpa, Maurizio; Eto, Yoshikatsu.
ACTA PAEDIATR. 2008. ; 97(0):5-6 IF: 1.517
Riferimento WoK DOI

60) Mucopolysaccharidosis type II in a female patient: clinical presentation and follow-up examination after 6 months of enzyme replacement therapy.
Rampazzo, A.; Cananzi, M.; Salviati, L.; Tomanin, R.; Gasparotto, N.; Drigo, P.; Scarpa, M..
ACTA PAEDIATR. 2008. ; 97(0):99-99 IF: 1.517
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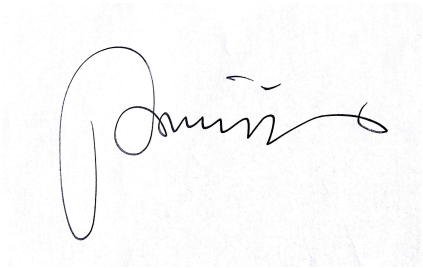

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