



MAURIZIO SCARPA, MD PhD

Department of Pediatrics
University of Padova and Center for Rare Disorders
Via Giustiniani 3, 35121 Padova, Italy
Tel. +39-049-8213505 FAX +39-049-8213502
Email: maurizio.scarpa@unipd.it

DATE AND PLACE OF BIRTH September 15, 1959, Venice, Italy.
CITIZENSHIP Italian.
LANGUAGES English.
POSITION HELD 3-1991-present Assistant Professor, Head of the Lysosomal Unit, Head Molecular Biology Laboratory and Center for Rare Disorders Dept. of Pediatrics, University of Padova, Italy

EDUCATION

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

November 1985 M.D. Degree, University of Padova, Medical School , Padova, Italy.

December 1985- August 1987 Postdoctoral fellowship
Prof. Gianni Cesareni's laboratory
Dept. Gene Structure and Regulation
European Molecular Biology Laboratory
(E.M.B.L.) Heidelberg, Germany

September 1987- December 1987 Dept. of Pediatrics, University of Padova, Italy.

January 1988-June 1990 Postdoctoral Fellowship.
Prof. Charles Thomas Caskey's laboratory.
Institute for Molecular Genetics, Baylor College of Medicine, Houston,Texas, U.S.A.

July 1990-February 1993 Consultant in Molecular Biology
Prof. GianFranco Bottazzo's Laboratory
Department of Immunology
The London Hospital Medical College, London, UK.

February 1991-Present Head of the Gene Transfer Laboratory and Lysosomal Clinical Unit,
Dept. of Pediatrics and Centre for Rare Disorders
University of Padova, Italy.

CERTIFICATIONS

November 1985 Medical Doctor Degree, University of Padova, Italy.
December 1985 License for Medical Practice, Italian Ministry of Health
July 1989 Diploma of Pediatrics, Department of Pediatrics,
University of Padova, Italy.
July 2004 PhD Degree, University of Padova, Medical School

AWARDS

January 1986- August 1987	Junior travel postdoctoral fellowship, Department of Pediatrics University of Padova, Italy.
January 1988-June 1988	Research Associate, Howard Hughes Medical Institute, Institute for Molecular Genetics, Baylor College of Medicine, Houston, Texas, U.S.A.
July 1988-June 1990	Postdoctoral Fellowship, Cystic Fibrosis Foundation, Bethesda, Maryland, U.S.A.
July 1990-February 1992	Charles Fremes Award, Juvenil Diabetes Foundation, New York, New York. U.S.A.
March 1991	First International Prize "Carlo Baschiroto" on Rare Genetic Diseaseas.

MEMBERSHIPS

1992-present	Founding Member of the European Society for Gene Therapy
1994-present	Member of the Scientific Committe, Italian Association for Mucopolisaccharidosis.
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-present	Founder of BRAINS FOR BRAIN European Task Force for the Research on Pediatric Neurodegenerative Disorders
2009-present	VicePresident BRAINS For BRAIN FOUNDATION
2010-present	Member of the European Science Foundation Pool of reviewers

TEACHING ACTIVITIES

2002-	Present: Director PhD Programme on Molecular Genetics and Biochemistry, University of Padova, Italy
2004-present:	Professor of Applied Biology, School of Neurosurgery, University of Padova

2004-present: Professor of Biochemistry, School of Neuropsychiatry University of Padova

2004-Present: Professor of Molecular Biology School of Neuropsychiatry University of Padova.

2008-Present Vice-Dean for the International Affairs Office University of Padova Medical School

N.	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;TSEKNOGLOU, 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)	<p>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</p>
9)	<p>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</p>
10)	<p>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</p>
11)	<p>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</p>
12)	<p>ANTIRETROVIRAL ACTIVITY OF FUROCOUMARINS PLUS UVA LIGHT DETECTED BY A REPLICATION-DEFECTIVE RETROVIRUS. MIOLO, G; TOMANIN, R; DEROSI, A; DALLACQUA, F; ZACCHELLO, F; SCARPA, M. J PHOTOCH PHOTOBIO B. 1994. ; 26(3):241-247 IF: 1.529 Riferimento WoK</p>
13)	<p>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</p>
14)	<p>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</p>
15)	<p>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</p>
16)	<p>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</p>
17)	<p>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</p>

	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;

	<p>Tomanin, R; Scarpa, M; Gianni, AM. BRIT J HAEMATOL. 2000. ; 111(1):344-350 IF: 3.068 Riferimento WoK</p>
27)	<p>Genetic testing - Part I - Introduction. Scarpa, M. Minerva Biotechnologica. 2000. ; 12(1):3-3 IF: 0.233 Riferimento WoK</p>
28)	<p>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, EFWF. GENE THER. 2001. ; 8(18):1380-1386 IF: 5.893 Riferimento WoK</p>
29)	<p>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</p>
30)	<p>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</p>
31)	<p>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</p>
32)	<p>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; Zacchello, F; Scarpa, M. MOL THER. 2003. ; 7(5):1024 - IF: 6.125 Riferimento WoK</p>
33)	<p>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</p>
34)	<p>Chimeric vectors. Scarpa, M. Current gene therapy. 2004. ; 4(4):- IF: 3.681 Riferimento WoK</p>
35)	<p>Why do we need new gene therapy viral vectors? Characteristics, limitations and future perspectives of viral vector transduction.</p>

	<p>Tomanin, R; Scarpa, M. Current gene therapy. 2004. ; 4(4):357-372 IF: 3.681 Riferimento WoK</p>
36)	<p>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</p>
37)	<p>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</p>
38)	<p>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</p>
39)	<p>Cystic fibrosis carriers have higher neonatal immunoreactive trypsinogen values than non-carriers. Castellani, C; Picci, L; Scarpa, M; Dehecchi, MC; Zanolla, L; Assael, BM; Zacchello, F. AM J MED GENET A. 2005. ; 135(2):142-144 IF: 1.913 Riferimento WoK DOI</p>
40)	<p>A PHASE 3, RANDOMIZED, DOUBLE-BLIND, PLACEBO-CONTROLLED, MULTICENTER, MULTINATIONAL CLINICAL STUDY OF RECOMBINANT HUMAN N-ACETYL GALACTOSAMINE 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: 3.598 Riferimento WoK</p>
41)	<p>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</p>
42)	<p>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; Schwarziz, 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</p>

43)	<p>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..</p> <p>J INHERIT METAB DIS. 2006. ; 29(0):29-29 IF: 3.598</p> <p>Riferimento WoK</p>
44)	<p>Neurological examinations and clinical manifestations in MPS I as reported in the MPS I registry. Scarpa, M..</p> <p>J INHERIT METAB DIS. 2007. ; 30(0):97-97 IF:3.598</p> <p>Riferimento WoK</p>
45)	<p>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..</p> <p>J INHERIT METAB DIS. 2007. ; 30(0):116-116 IF: 3.598</p> <p>Riferimento WoK</p>
46)	<p>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.</p> <p>AM J MED GENET A. 2007. ; 143(16):1936-1937 IF: 2.44</p> <p>Riferimento WoK DOI</p>
47)	<p>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..</p> <p>HAEMATOL-HEMATOL J. 2007. ; 92(6):45-45 IF: 5.516</p> <p>Riferimento WoK</p>
48)	<p>Neurologic examinations and clinical manifestations in mucopolysaccharidosis I: MPS I registry data. Scarpa, M..</p> <p>Clinical Therapeutics. 2007. ; 29(0):S123-S123 IF: 3.261</p> <p>Riferimento WoK</p>
49)	<p>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.</p> <p>Stem Cells and Development. 2008. ; 17(5):953-962 IF: 3.273</p> <p>Riferimento WoK DOI</p>
50)	<p>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..</p> <p>Biochimica et Biophysica Acta - Molecular Basis of Disease. 2008. ; 1782(10):574-580 IF: 4.579</p> <p>Riferimento WoK DOI</p>

51)	<p>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: 3.598 Riferimento WoK</p>
52)	<p>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:3.598 Riferimento WoK</p>
53)	<p>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: 3.598 Riferimento WoK</p>
54)	<p>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</p>
55)	<p>Lysosomal storage diseases and the blood-brain barrier. Begley, David J.; Pontikis, Charles C.; Scarpa, Maurizio. Current Pharmaceutical Design. 2008. ; 14(16):1566-1580 IF: 4.399 Riferimento WoK</p>
56)	<p>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</p>
57)	<p>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 BIO. 2008. ; 49(0):219-223 IF: 1.288 Riferimento WoK DOI</p>
58)	<p>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</p>

	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 Riferimento WoK
61)	Efficient transduction of human amniotic stem cells with an adenovirus-expressing iduronate-2-sulphatase: a potential approach for therapy of mucopolysaccharidosis type II?. Grisafi, D.; Friso, A.; Piccoli, M.; Pozzobon, M.; De Coppi, P.; Chiandetti, L.; Zaramellal, P.; Scarpa, M.; Tomanin, R.. ACTA PAEDIATR. 2008. ; 97(0):102-102 IF: 1.517 Riferimento WoK
62)	Efficacy of adenovirus-mediated gene therapy in the mouse model of mucopolysaccharidosis type II. Tomanin, R.; Zaccariotto, E.; Marigo, I.; Friso, A.; Grisafi, D.; Bordin, M.; Salvalaio, M.; Scarpa, M.. ACTA PAEDIATR. 2008. ; 97(0):102-102 IF: 1.517 Riferimento WoK
63)	Neurological manifestations of Hunter syndrome: new insights from HOS - the Hunter outcome survey. Scarpa, M.; Beck, M.; Wraith, J. E.. ACTA PAEDIATR. 2008. ; 97(0):103-104 IF: 1.517 Riferimento WoK
64)	A rapid testing procedure for Fabry disease: alpha-galactosidase A assay in dried blood spots. Gasparotto, N.; Tomanin, R.; Frigo, A. C.; Pasquini, E.; Donati, A.; Niizawa, G.; Blanco, M.; Scarpa, M.. ACTA PAEDIATR. 2008. ; 97(0):112-112 IF: 1.517 Riferimento WoK
65)	Neurological examinations and clinical manifestations in MPS I Registry patients. Scarpa, Maurizio. MOL GENET METAB. 2008. ; 93(2):81 - IF: 2.629 Riferimento WoK DOI
66)	Brains for brain: A new Consortium for the research and the therapy of LSDs. Scarpa, Maurizio; Begley, David. MOL GENET METAB. 2008. ; 93(2):82 - IF: 2.629 Riferimento WoK DOI
67)	In vivo evaluation of genistein for GAG storage reduction. Scarpa, Maurizio; Friso, Adelaide; Tomanin, Rosella; Salvalaio, Marika; Bordin, Mauro; Zacchello, Franco. MOL GENET METAB. 2008. ; 93(2):83 - IF: 2.629

	Riferimento WoK DOI
68)	Neurological manifestations of hunter syndrome: Insights from HOS, the hunter outcome survey. Scarpa, Maurizio; Beck, Michael; Wraith, Edward J.. MOL GENET METAB. 2008. ; 93(2):84 - IF: 2.629 Riferimento WoK
69)	A zebrafish iduronate-2-sulfatase candidate orthologue is strongly expressed during early embryonic development. Scarpa, Maurizio; Moro, Enrico; Tomanin, Rosella; Friso, Adelaide; Modena, Nicola; Bortolussi, Marino; Argenton, Francesco. MOL GENET METAB. 2008. ; 93(2):85 - IF: 2.629 Riferimento WoK
70)	Mucopolysaccharidosis type II (Hunter syndrome): a clinical review and recommendations for treatment in the era of enzyme replacement therapy. Wraith, J. Edmond; Scarpa, Maurizio; Beck, Michael; Bodamer, Olaf A.; De Meirleir, Linda; Guffon, Nathalie; Lund, Allan Meldgaard; Malm, Gunilla; Van der Ploeg, Ans T.; Zeman, Jiri. EUR J PEDIATR. 2008. ; 167(3):267-277 IF: 1.416 Riferimento WoK DOI
71)	Multidisciplinary Management of Hunter Syndrome. Muenzer, Joseph; Beck, M.; Eng, C. M.; Escolar, M. L.; Giugliani, R.; Guffon, N. H.; Harmatz, P.; Kamin, W.; Kampmann, C.; Koseoglu, S. T.; Link, B.; Martin, R. A.; Molter, D. W.; Munoz Rojas, M. V.; Ogilvie, J. W.; Parini, R.; Ramaswami, U.; Scarpa, M.; Schwartz, I. V.; Wood, R. E.; Wraith, E.. Pediatrics. 2009. ; 124(6):E1228-E1239 IF: 4.789 Riferimento WoK DOI
72)	Segregation analysis in a family at risk for the Maroteaux-Lamy syndrome conclusively reveals c.1151G > A (p.S384N) as to be a polymorphism. Zanetti, Alessandra; Ferraresi, Elena; Picci, Luigi; Filocamo, Mirella; Parini, Rossella; Rosano, Camillo; Tomanin, Rosella; Scarpa, Maurizio. European Journal of Human Genetics. 2009. ; 17(9):1160-1164 IF: 3.925 Riferimento WoK DOI
73)	Mucopolysaccharidosis VI: the Italian experience. Scarpa, Maurizio; Barone, Rita; Fiumara, Agata; Astarita, Luca; Parenti, Giancarlo; Rampazzo, Angelica; Sala, Stefania; Sorge, Giovanni; Parini, Rossella. EUR J PEDIATR. 2009. ; 168(10):1203-1206 IF: 1.416 Riferimento WoK DOI
74)	THE BURDEN OF SURGERY IN HUNTER SYNDROME: DATA FROM THE HUNTER OUTCOME SURVEY. Parini, R.; Furfan, F.; Wraith, J. E.; Scarpa, M.; Beck, M.; Muenzer, J.; Giugliani, R.. MOL GENET METAB. 2009. ; 98(1):440 - IF: 2.629 Riferimento WoK
75)	Rapid diagnostic testing procedures for lysosomal storage disorders: alpha-glucosidase and beta-galactosidase assays on dried blood spots. Gasparotto, Nicoletta; Tomanin, Rosella; Frigo, Anna Chiara; Niizawa, Gabriela; Pasquini, Elisabetta; Blanco, Mariana; Donati, Maria Alice; Keutzer, Joan; Zacchello, Franco; Scarpa,

	<p>Maurizio. Clinica Chimica Acta. 2009. ; 402(1):38-41 IF: 2.96 Riferimento WoK DOI</p>
77)	<p>Novel therapies and future perspectives. Scarpa, M.; Frustaci, A.. International Journal of Clinical Pharmacology and Therapeutics. 2009. ; 47(0):S109-S110 IF: 1.381 Riferimento WoK</p>
78)	<p>CNS imaging evaluation: toward the creation of a severity score. Manara, R.; Mardari, R.; Rampazzo, A.; Scarpa, M.. International Journal of Clinical Pharmacology and Therapeutics. 2010. ; 48(0):S38-S39 IF: 1.381 Riferimento WoK</p>
79)	<p>Genistein reduces glycosaminoglycan levels in a mouse model of mucopolysaccharidosis type II. Friso, A.; Tomanin, R.; Salvalaio, M.; Scarpa, M.. British Journal of Pharmacology. 2010. ; 159(5):1082-1091 IF: 5.204 Riferimento WoK DOI</p>
80)	<p>Enzyme replacement therapy for mucopolysaccharidosis VI: evaluation of long-term pulmonary function in patients treated with recombinant human N-acetylgalactosamine 4-sulfatase. Harmatz, Paul; Yu, Zi-Fan; Giugliani, Roberto; Schwartz, Ida Vanessa D.; Guffon, Nathalie; Teles, Elisa Leao; Sa Miranda, M. Clara; Wraith, J. Edmond; Beck, Michael; Arash, Laila; Scarpa, Maurizio; Ketteridge, David; Hopwood, John J.; Plecko, Barbara; Steiner, Robert; Whitley, Chester B.; Kaplan, Paige; Swiedler, Stuart J.; Hardy, Karen; Berger, Kenneth I.; Decker, Celeste. J INHERIT METAB DIS. 2010. ; 33(1):51-60 IF: 3.598 Riferimento WoK DOI</p>
81)	<p>A novel functional role of iduronate-2-sulfatase in zebrafish early development. Moro, Enrico; Tomanin, Rosella; Friso, Adelaide; Modena, Nicola; Tiso, Natascia; Scarpa, Maurizio; Argenton, Francesco. Matrix Biology. 2010. ; 29(1):43-50 IF: 3.558 Riferimento WoK DOI</p>
82)	<p>A 10-year large-scale cystic fibrosis carrier screening in the Italian population. Picci, Luigi; Cameran, Marilena; Marangon, Oriana; Marzenta, Diana; Ferrari, Stefano; Frigo, Anna Chiara; Scarpa, Maurizio. Journal of Cystic Fibrosis. 2010. ; 9(1):29-35 IF: 2.239 Riferimento WoK DOI</p>
83)	<p>Pathophysiology of neuropathic lysosomal storage disorders. Bellettato CM, Scarpa M. (2010) J Inherit Metab Dis. 2010 Apr 29. [Epub ahead of print] IF: 3.598 PUBMED</p>
84)	<p>Diagnosis and Management of ophthalmologic features in patients with mucopolysaccharidosis</p>

	<p>Ferrari S., Ponzin D, Ashworth JL, Teär Fahnehjelm K., Summers CG. Harmatz P. and Scarpa M. (2010) Brit. J. Ophtalmol. In press IF: 2.91</p>
85)	<p>Enzyme replacement therapy for Mucopolysaccharidosis VI: Groeth abd pubertal development In patients treated with recombinant N-Acetylgalactosamine 4-sulfatase Decker C., Yu Z-F., Giugliani R., Schwartz IVD., Guffin N., Teles ES., Sa Miranda MC., Wraith JE., Beck M., Arash L., Scarpa M., Ketteridge D., Hopwood JJ., Plecko B., Steiner R., Whitley CB., Kaplan P., Swiedler SJ., Conrad S., and Harmatz P.. (2010). J. PEd. Rehabil. Med. 3, 2, 89-100.</p>
86)	<p>Evaluation of disease severity in mucopolysaccharidosis Beck M., Muenzer J., and Scarpa M., (2010) J. Pediat. Rehabil. Med. 3, 39-46</p>
87)	<p>Systemic therapies for mucopolysaccharidosis: ocular changes following haematopoietic stem cell transplantation or enzyme replacement therapy, Gail Summers C., Teär Fahnehjelm K., Pitz S., Guffon N., Koseoglu ST., Harmatz P., Scarpa M. (2010) Clin. Exp. Ophthalmol. (in press)</p>