

EUROPEAN  
CURRICULUM VITAE  
FORMAT



**PERSONAL INFORMATION**

Name **MAURIZIO SCARPA**

Address Regional Coordinating Centre for Rare Disease – University Hospital “Santa Maria della Misericordia” – Piazzale Santa Maria della Misericordia, 15 – 33100 Udine (Italy)

Telephone +39 0432 559883                      Mobile +39 3480 33390

Fax

E-mail **maurizio.scarpa@asufc.sanita.fvg.it**

Nationality Italian

Date of birth and place SEPTEMBER 15, 1959, VENICE ITALY

**WORK EXPERIENCE**

**MAIN ACTIVITIES AND RESPONSIBILITIES**

Coordinator, MetabERN European Reference Network for Hereditary Metabolic Diseases, Past-Chair ERN Coordinators Group.

Associate Professor of Pediatrics; University of Padova, Italy,  
National Abilitation to Full Professor of Pediatrics 03-2017.  
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1991- 2014: Head Lysosomal Unit, Dept. of Pediatrics University of Padova,  
1995-1997: Staff Pediatric Intensive Care Unit, Dept. of Pediatrics University of Padova,  
1997- 2016: Consultant in General Pediatrics . Laguna Medical Centre Venice, Italy  
1997-2005; Staff, Inherited Metabolic Diseases Unit, Dept.of Pediatrics, Padova  
2005-2014: Staff, Pediatric Neurology Unit Dept. Of Pediatrics Padova,  
2011- 2015: Director, Centre for Rare Disease IRCCS Casa Sollievo della Sofferenza, San Giovanni Rotondo, Foggia, Research Hospital  
2014-2018: Director Center for Rare Diseases, Horst Schmidt Klinik, Wiesbaden, DE  
2017- present: Coordinator European Reference Network for Hereditary Metabolic Diseases: MetabERN and Past-Chair ERN Coordinators Group  
2019-present: Director, Regional Coordinating Center for Rare Diseases, Udine University Hospital, Udine, Italy  
2019-present: Scientific Director UDINE University Hospital, Udine, Italy

EDUCATION AND TRAINING	<p>1980-1985: Working group on Cystic Fibrosis, Dept. of Pediatrics, University of Padova, Italy.</p> <p>1985: M.D. DEGREE, UNIVERSITY OF PADOVA, Medical School , Padova, Italy.</p> <p>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;</p> <p>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.</p> <p>1989: SPECIALIST ON PEDIATRICS, UNIVERSITY OF PADOVA</p> <p>1990-1993: CONSULTANT IN MOLECULAR BIOLOGY Prof. GianFranco Bottazzo's Laboratory, Department of Immunology, The London Hospital Medical College, London, UK.</p> <p>2004: PHD DEGREE: PEDIATRIC SCIENCES</p> <p>15/Jan/1986: Medical Registry Number :4331</p>
CERTIFICATIONS	<p>1985: Medical Doctor Degree, University of Padova, Italy.</p> <p>1985: License for Medical Practice, Italian Ministry of Health</p> <p>1989: Diploma of Pediatrics, Department of Pediatrics, University of Padova, Italy.</p> <p>2004: PhD Degree, Pediatric Sciences, University of Padova, Medical School</p> <p>16 Jul 2019: ICH GOOD CLINICAL PRACTICE E6(R2)</p>
ACADEMICAL TEACHING ACTIVITIES	<p>2004-2011: Director PhD Programme on Molecular Genetics and Biochemistry, University of Padova, I</p> <p>2004-2010: Professor of Applied Biology, School of Neurosurgery, University of Padova</p> <p>2004-2010: Professor of Biochemistry, School of Neuropsychiatry University of Padova</p> <p>2004-2010: Professor of Molecular Biology School of Neuropsychiatry University of Padova.</p> <p>2014-present: Professor of Pediatrics, School of Medicine , University of</p>

Padova, Italy

## MEMBERSHIP

1990-pres: Member of the Italian Society of Pediatrics

1992: Founding Member of the European Society for Gene Therapy

1994- pres.: Member of the Scientific Committee,

Italian Association for Mucopolysaccharidosis.

1994-1996 Member of the Gene Therapy Committee Italian Minister for the EEC, Commission on Safety of Genetically Modified Microorganisms.

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

2000- pres. Society for the Study of the Inborn Errors of Metabolism (SSIEM)

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

2012-2017: President BRAINS For BRAIN FOUNDATION

2017: Present, Member Scientific Committee for Therapy Development, IRDIRC International Rare Diseases Research Consortium

## REVIEWER ACTIVITY

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 Cochrane Clinical Trial Organization

Gene Review expert

International Journals:

Gene Therapy, Human Gene Therapy, Clinical Chemistry, Pediatrics, J. Pediatrics, Nanomedicine. Orphanet Journal of Rare Diseases, Molecular Genetics and Metabolism, American Journal Human Genetics,

Communicating Editor: Journal Inherited Metabolic Diseases

## CONFLICT OF INTERESTS

GRANTS FOR RESEARCH, UNRESTRICTED EDUCATIONAL ACITIVITES  
ADVISORY BOARD HONORARIA FROM: ACTELION, ALEXION, BIOMARIN,  
BIOASIS, CHIESI, GENZYME SANOFY, SHIRE, PTC THERAPEUTICS,  
VTESSE

## 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
2. Genetic and structural analysis of the ColE1 Rop (Rom) protein..  
CASTAGNOLI, L;SCARPA, M;KOKKINIDIS, M;BANNER, DW;TSEBNOGLOU, D;CESARENI, G.  
EMBO J. 1989. ; 8:621-9
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
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
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
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
7. Gene therapy..  
COURNOYER, D;SCARPA, M;CASKEY, CT.  
Current Opinion in Biotechnology. 1992. ; 1:196-208
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
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

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
  
11. GENE-TRANSFER IN REGENERATING MUSCLE.  
VITADELLO, M; SCHIAFFINO, MV; PICARD, A; SCARPA, M; SCHIAFFINO, S.  
Human gene therapy. 1994. ; 5(1):11-18
  
12. 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
  
13. Gene transfer in regenerating muscle..  
VITADELLO, M;SCHIAFFINO, MV;PICARD, A;SCARPA, M;SCHIAFFINO, S.  
Human gene therapy. 1994. ; 5:11-8
  
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:
  
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
  
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
  
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
  
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
  
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
  
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.  
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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
  
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
  
23. Recombinant adenoviral vector-LipofectAMINE complex for gene transduction into human T lymphocytes.  
Di Nicola, M; Milanese, 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
  
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
  
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. .  
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26. Large-scale feasibility of gene transduction into human CD34(+) cell-derived dendritic cells by adenoviral/polycation  
complex.  
Di Nicola, M; Carlo-Stella, C; Milanese, M; Magni, M; Longoni, P; Mortarini, R; Anichini, A; Tomanin, R; Scarpa, M;  
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27. Mucus altering agents as adjuncts for nonviral gene transfer to airway epithelium.  
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WH; Geddes, DM; Alton, EFWF.  
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28. Update on enzyme replacement therapy in mucopolysaccharidosis type II.  
Muenzer, J; Scarpa, M.  
ACTA PAEDIATR. 2002. ; 91(0):81
  
29. 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
  
30. 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.  
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31. Electrogene transfer in Mucopolysaccharidosis type II mouse model induces high-level production of iduronate-2-sulfatase in the skeletal muscle.  
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32. 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 .  
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33. Chimeric vectors.  
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34. Why do we need new gene therapy viral vectors? Characteristics, limitations and future perspectives of viral vector transduction.  
Tomanin, R; Scarpa, M.  
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35. 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
36. 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
37. 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.  
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38. Cystic fibrosis carriers have higher neonatal immunoreactive trypsinogen values than non-carriers.  
Castellani, C; Picci, L; Scarpa, M; Dechecchi, MC; Zanolta, L; Assael, BM; Zacchello, F.  
AM J MED GENET A. 2005. ; 135(2):142-144
39. 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..  
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40. A 3-year-old child with abdominal pain and fever..  
BONETTO, G;SCARPA, M;CARRARO, S;BARALDI, E.  
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41. 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.; Schwarz, 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
  
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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
  
43. 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
  
44. Phase 3 extension 96-week study data for naglazyme (galsulfase) enzyme replacement therapy (ERT) in MPS VI (Maroteaux-Lamy syndrome) patients.  
Harmatz, P.; Giugliani, 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
  
45. 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:
  
46. 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
  
47. Neurologic examinations and clinical manifestations in mucopolysaccharidosis I: MPS I registry data.  
Scarpa, M..  
Clinical Therapeutics. 2007. ; 29(0):S123-S123
  
48. 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
  
49. 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
  
50. 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.; Tytki-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 -



51. 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 -
52. Clinical phenotype of Italian patients with Hunter syndrome: Data from HOS - The Hunter Outcome Survey.  
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J INHERIT METAB DIS. 2008. ; 31(0):456P -
53. 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.  
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54. Lysosomal storage diseases and the blood-brain barrier.  
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55. 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..  
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56. 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
57. Seventh international symposium on lysosomal storage diseases.  
Aerts, Johannes; Beck, Michael; Martin, Rick; Scarpa, Maurizio.  
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58. Lysosomal storage diseases: new challenges.  
Scarpa, Maurizio; Eto, Yoshikatsu.  
ACTA PAEDIATR. 2008. ; 97(0):5-6
59. 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..  
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60. Efficient transduction of human amniotic stem cells with an adenovirus-expressing iduronate-2-sulphatase: a potential approach for therapy of mucopolysaccharidosis type II?  
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61. Efficacy of adenovirus-mediated gene therapy in the mouse model of mucopolysaccharidosis type II.  
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62. 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
63. A rapid testing procedure for Fabry disease: alpha-galactosidase A assay in dried blood spots.  
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64. Neurological examinations and clinical manifestations in MPS I Registry patients.  
Scarpa, Maurizio.  
MOL GENET METAB. 2008. ; 93(2):81 -
65. 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 -
66. In vivo evaluation of genistein for GAG storage reduction.  
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67. Neurological manifestations of hunter syndrome: Insights from HOS, the hunter outcome survey.  
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68. 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 -
69. 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.  
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Date

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