EUROPEAN CURRICULUM VITAE FORMAT

PERSONAL INFORMATION

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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

Director Regional Coordinating Center for Rare Diseases, Udine

Associate Professor of Pediatrics; University of Padova, Italy, National Abilitation to Full Professor of Pediatrics 03-2017.

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- 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

EDUCATION AND TRAINING	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
ACADEMICAL TEACHING ACTIVITIES	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. 2014-present: Professor of Pediatrics, School of Medicine , University of 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
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 Cochraine 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 Gentics, Communicating Editor: Journal Inherited Metabolic Diseases
CONFLICT OF INTERESTS	GRANTS FOR RESEARCH, UNRESTRICTED EDUCATIONAL ACITIVITES ADVISORY BOARD HONORARIA FROM: ACTELION, ALEXION, BIOMARIN, BIOASIS, 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;TSERNOGLOU, 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.
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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; DEROSSI, 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.
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16. ExGen 500 is an efficient vector for gene delivery to lung epithelial cells in vitro and in vivo.
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17. The bacteriophage T7 binary system activates transient transgene expression in zebrafish (Danio rerio) embryos.
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18. Development and characterization of a binary gene expression system based on bacteriophage T7 components in adenovirus vectors.
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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.
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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.
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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.
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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.
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Castaldo, G;Fuccio, A;Cazeneuve, C;Picci, L;Salvatore, D;Scarpa, M;Goossens, M;Salvatore, F. .
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Di Nicola, M; Carlo-Stella, C; Milanesi, M; Magni, M; Longoni, P; Mortarini, R; Anichini, A; Tomanin, R; Scarpa, M; Gianni, AM.
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29. Non-viral transfer approaches for the gene therapy of mucopolysaccharidosis type II (Hunter syndrome).
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Tomanin, R; Friso, A; Alba, S; Puicher, EP; Gasparotto, N; Mennuni, C; La Monica, N; Muenzer, J; Zacchelio, F; Scarpa, M.
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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.
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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.
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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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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..
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Scarpa, M..
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44. 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..
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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.
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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..
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47. Neurologic examinations and clinical manifestations in mucopolysaccharidosis I: MPS I registry data.
Scarpa, M..
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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.
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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.
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J INHERIT METAB DIS. 2008. ; 31(0):426P -

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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..
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52. 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..
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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.
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54. Lysosomal storage diseases and the blood-brain barrier.
Begley, DJ.; Pontikis, Charles C.; Scarpa, Maurizio.
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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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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.
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57. Seventh international symposium on lysosomal storage diseases.
Aerts, Johannes; Beck, Michael; Martin, Rick; Scarpa, Maurizio.
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Scarpa, Maurizio; Eto, Yoshikatsu.
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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?.
Grisafi, D.; Friso, A.; Piccoli, M.; Pozzobon, M.; De Coppi, P.; Chiandetti, L.; Zaramellal, P.; Scarpa, M.; Tomanin, R..
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61. 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..
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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..
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63. A rapid testing procedure for Fabry disease: alpha-galadosidase A assay in dried blood spots.
Gasparotto, N.; Tomanin, R.; Frigo, A. C.; Pasquini, E.; Donati, A.; Niizawa, G.; Blanco, M.; Scarpa, M..
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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.
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66. In vivo evaluation of genistein for GAG storage reduction.
Scarpa, Maurizio; Friso, Adlelaide; Tomanin, Rosella; Salvalaio, Marika; Bordin, Mauro; Zacchello, Franco.
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69. Mucopolysaccharidosis type II (Hunter syndrome): a clinical review and recommendations for treatment in the era of enzyme replacement therapy.
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Scarpa, Maurizio; Barone, Rita; Fiumara, Agata; Astarita, Luca; Parenti, Giancarlo; Rampazzo, Angelica; Sala, Stefania; Sorge, Giovanni; Parini, Rossella.
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73. The burden of surgery in Hunter Syndrome: data from the Hunter OUTCOME SURVEY.
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74. Rapid diagnostic testing procedures for lysosomal storage disorders: alpha-glucosidase and beta-galactosidase assays on dried blood spots.
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