

Maria Sofia Falzarano (PhD)
Curriculum Vitae

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| Name | Maria Sofia Falzarano |
| Place of Birth | Mottola (TA), Italy |
| Date of Birth | 09 October 1975 |
| Telephone number | 3394386373 |
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| Tax code | FLZMSF75R49F784O |
| Nationality | Italian |
| Address | Via Parmeggiani 12, Ferrara |
| Studies | Classical Studies (1994) |
| Graduate | Chemical and pharmaceutical technology (30/10/2001, University of Ferrara) |
| Postgraduate | PhD in Molecular and Cellular Pharmacology (23/02/2005, University of Ferrara) |
| 2020-present: | PhD student in Biomedical sciences and biotechnology (University of Ferrara) |

Work experience

2002: fellowship from MURST for "Immunogenic peptides with partial agonistic activity for the activation of cytotoxic T lymphocytes and with potential use in immunotherapy" research (University of Ferrara).

2003: fellowship from MIUR "Immune recognition of virus-infected cells" research (University of Ferrara).

2004-2005: post-doc contract for "Identification of activated signal transduction pathways in human neutrophils by chemotactic peptides such as for-Met-Leu-Phe and its analogues" research (University of Ferrara).

2006: post-doc contract for "Molecular Genetics of PKC inactivation pathway by PKI55 protein in signal transduction in normal and pathological cells" research (University of Ferrara).

2007-2014: post-doc fellowship for the study of the exon skipping in DMD (human and mdx mouse models) by Antisense Oligo Nucleotides and nanoformulations (Section of Medical Genetics, Department of Medical Sciences, University of Ferrara).

2015-2017: post-doc fellowship for screening of new molecules for DMD therapies (Section of Medical Genetics, Department of Medical Sciences, University of Ferrara).

2017-2020: post-doc fellowship for screening of antisense modified molecules in myogenic cells from DMD patients for therapies (Section of Medical Genetics, Department of Medical Sciences, University of Ferrara).

2020: contract of collaboration for “In situ hybridization di RNA in adult human brain“from EU Horizon 2020 grant Brain Involvement in Dystrophinopathies (BIND), grant n. 847826, website <https://bindproject.eu/>. Europeo “Brain Involvement iN Dystrophinopathies – B I N D” - Nr Grant Agreement : 847826.

Laboratory Skills

- Fibroblasts isolation from skin
- Myoblasts isolation from muscle
- Urine derived stem cells isolation
- Cell cultures: fibrobalsts, myogenic cells, stem cells
- Cell transfection with oligonucleotides antisense
- Isolation of blood cells: neutrophils and monocytes
- RNA and DNA extraction from different tissues and cells
- RNA and DNA analysis:

PCR, Sanger Sequencing, RT-PCR, Real-Time PCR, Agilent 2100 Bioanalyzer, Fluidic card, RNAscope

- Protein extraction and analysis in tissues from human and mice:
Western blot, Odyssey (Licor) analysis, IHC, immunoprecipitation
- Elisa assay

Linguistic knowledge

English: good knowledge

Computer skills

- Windows 7, windows 10, Vista, XP
- Office: Word, Excel, PowerPoint
- Internet: PubMed, UCSC Genome Brower and other

- Software for images analysis: Photoshop, NIS-Elements (Nikon), InageJ

Publications

1. Selvatici R, Falzarano S, Traniello S, Zecchini P, Spisani S.
Formylpeptides triggers selective molecular pathways that are required in the physiological functions of human neutrophils.
Cell Signal. 2003;15:377-83
2. Spisani S, Turchetti M, Varani K, Falzarano S, Cavicchioni G.
Hydrophilic residues at position 3 highlight unforeseen features of the fMLP receptor pocket
Eur J Pharmacol. 2003;469:13-9
3. Cavicchioni G, Turchetti M, Varani K, Falzarano S, Spisani S.
Properties of a novel chemotactic esapeptide, an analogue of the prototypical N-formylmethionyl peptide.
Bioorg Chem. 2003;31:322-30
4. Witkowska R, Zabrocki J, Spisani S, Falzarano MS, Toniolo C, Formaggio F.
Synthetic formyl tripeptide chemoattractant: a C^{α,α}-dialkylated, amphiphilic glycyl residue at position 1.
J Peptide Sci. 2003;9:354-360
5. Spisani S., Falzarano S., Traniello S., Nalli M. and Selvatici R.
A “pure” chemoattractant formylpeptide analogue triggers a specific signalling pathway in human neutrophil chemotaxis.
FEBS J. 2005;272:883-91
6. Cavicchioni G., Fraulini A., Turchetti M., Varani K., Falzarano S. and Spisani S.
Biological activity of for-Met-Leu-Phe-OMe analogs: relevant substitutions specifically trigger killing mechanisms in human neutrophils.
Eur. J. Pharmacol. 2005;512:1-8
7. Manzati E., Aguiari G., Banzi M, Selvatici R., Manzati M., Falzarano S., Maestri I., Pinton P., Rizzuto R. and Del Senno L.
The cytoplasmic C-terminus of polycystin-1 increases cell proliferation in kidney epithelial cells through serum-activated and Ca²⁺-dependent pathway(s).
Exp. Cell. Res. 2005;304:391-406
8. Trombella S., Vergura R., Falzarano S., Guerrini R., Calo' G. and Spisani S.
Nociceptin/Orphanin FQ stimulates human monocyte chemotaxis via NOP receptor activation.
Peptides 2005;26:1497-502
9. Selvatici R., Falzarano S., Mollica A. and Spisani S.
Signal transduction pathways triggered by selective formylpeptide analogues in human neutrophils.
Eur. J. Pharmacol. 2006;534:1-11

10. Selvatici R., Falzarano S., Franceschetti L., Cavallini S., Marino S. and Siniscalchi A. Differential activation of protein kinase C isoforms following chemical ischemia in rat cerebral cortex slices. Neurochem. Int. 2006; 49:729-36
11. Siniscalchi A., Cavallini S., Marino S., Falzarano S., Franceschetti L. and Selvatici R. Effects of chemical ischemia on cerebral cortex slices. Focus on mitogen-activated protein kinase cascade. Ann. N.Y. Acad. Sci. 2006; 1090: 445-454
12. Cavicchioni G., Fraulin A., Falzarano S. and Spisani S. Structure-activity relationship of for-L-Met-L-Leu-L-Phe-OMe analogues in human neutrophils Bioorg. Chem. 2006; 34:298-318
13. Spisani S, Fraulini A, Varani K, Falzarano S, Cavicchioni G. New chemotactic dimeric peptides show high affinity and potency at the human formylpeptide receptor. Eur J Pharmacol. 2007; 567:171-6.
14. Selvatici R, Falzarano S, Franceschetti L, Spisani S, Siniscalchi A. Effects of PKI55 protein, an endogenous protein kinase C modulator, on specific PKC isoforms activity and on human T cells proliferation. Arch Biochem Biophys. 2007; 462:74-82
15. Bruno O, Brullo C, Bondavalli F, Ranise A, Schenone S, Falzarano MS, Varani K, Spisani S. 2-Phenyl-2,3-dihydro-1H-imidazo[1,2-b]pyrazole derivatives: new potent inhibitors of fMLP-induced neutrophil chemotaxis. Bioorg Med Chem Lett. 2007; 17:3696-701
16. Selvatici R, Falzarano S, Franceschetti L, Mollica A, Guerrini R, Siniscalchi A, Spisani S. Study of synthetic peptides derived from the PKI55 protein, a protein kinase C modulator, in human neutrophils stimulated by the methyl ester derivative of the hydrophobic N-formyl tripeptide for-Met-Leu-Phe-OH. FEBS J. 2008 Feb; 275(3):449-57
17. Bovolenta M, Neri M, Fini S, Fabris M, Trabanelli C, Venturoli A, Martoni E, Bassi E, Spitali P, Brioschi S, Falzarano MS, Rimessi P, Ciccone R, Ashton E, McCauley J, Yau S, Abbs S, Muntoni F, Merlini L, Gualandi F, Ferlini A. A novel custom high density-comparative genomic hybridization array detects common rearrangements as well as deep intronic mutations in dystrophinopathies. BMC Genomics. 2008 Nov 28;9:572.
18. Selvatici R, Previati M, Marino S, Marani L, Falzarano S, Lanzoni I, Siniscalchi A. Sodium azide induced neuronal damage in vitro: evidence for non-apoptotic cell death. Neurochem Res. 2009 May;34(5):909-16.
19. Bruno O, Brullo C, Bondavalli F, Schenone S, Spisani S, Falzarano MS, Varani K, Barocelli E, Ballabeni V, Giorgio C, Tognolini M.

1-Methyl and 1-(2-hydroxyalkyl)-5-(3-alkyl/cycloalkyl/phenyl/naphthylureido)-1H-pyrazole-4-carboxylic acid ethyl esters as potent human neutrophil chemotaxis inhibitors.
Bioorg Med Chem. 2009 May 1;17(9):3379-87.

20. Cavicchioni G, Fraulini A, Falzarano S, Spisani S.
Oligomeric formylpeptide activity on human neutrophils.
Eur J Med Chem. 2009 Dec;44(12):4926-30.

21. Borgatti M, Finotti A, Falzarano S, Selvatici R.
Structural characterization of promoter sequences of the gene coding human PKI55 protein, a protein kinase C inhibitor.
Biochimie. 2009 Apr;91(4):466-74

22. Spitali P, Rimessi P, Fabris M, Perrone D, Falzarano S, Bovolenta M, Trabanelli C, Mari L, Bassi E, Tuffery S, Gualandi F, Maraldi NM, Sabatelli-Giraud P, Medici A, Merlini L, Ferlini A.
Exon skipping-mediated dystrophin reading frame restoration for small mutations.
Hum Mutat. 2009 Nov;30(11):1527-34.

23. Rimessi P, Fabris M, Bovolenta M, Bassi E, Falzarano S, Gualandi F, Rapezzi C, Coccolo F, Perrone D, Medici A, Ferlini A.
Antisense modulation of both exonic and intronic splicing motifs induces skipping of a DMD pseudo-exon responsible for x-linked dilated cardiomyopathy.
Hum Gene Ther. 2010 Sep;21(9):1137-46.

24. Ferlini A, Sabatelli P, Fabris M, Bassi E, Falzarano S, Vattemi G, Perrone D, Gualandi F, Maraldi NM, Merlini L, Sparmacci K, Laus M, Caputo A, Bonaldo P, Braghetta P, Rimessi P.
Dystrophin restoration in skeletal, heart and skin arrector pili smooth muscle of mdx mice by ZM2 NP-AON complexes.
Gene Ther. 2010 Mar;17(3):432-8.

25. Bovolenta M, Scotton C, Falzarano MS, Gualandi F, Ferlini A.
Rapid, comprehensive analysis of the dystrophin transcript by a custom micro-fluidic exome array. Hum Mutat. 2012 Mar;33(3):572-81

26. Bassi E, Falzarano S, Fabris M, Gualandi F, Merlini L, Vattemi G, Perrone D, Marchesi E, Sabatelli P, Sparmacci K, Laus M, Bonaldo P, Rimessi P, Braghetta P, Ferlini A.
Persistent Dystrophin Protein Restoration 90 Days after a Course of Intraperitoneally Administered Naked 2'OMePS AON and ZM2 NP-AON Complexes in mdx Mice.
J Biomed Biotechnol. 2012

27. Bovolenta M, Erriquez D, Valli E, Brioschi S, Scotton C, Neri M, Falzarano MS, Gherardi S, Fabris M, Rimessi P, Gualandi F, Perini G, Ferlini A.
The DMD locus harbours multiple long non-coding RNAs which orchestrate and control transcription of muscle dystrophin mRNA isoforms.
PLoS One. 2012;7(9)

28. Brioschi S, Gualandi F, Scotton C, Armaroli A, Bovolenta M, Falzarano MS, Sabatelli P, Selvatici R, D'Amico A, Pane M, Ricci G, Siciliano G, Tedeschi S, Pini A, Vercelli L, De Grandis D, Mercuri E, Bertini E, Merlini L, Mongini T, Ferlini A.
Genetic characterization in symptomatic female DMD carriers: lack of relationship between X-inactivation, transcriptional DMD allele balancing and phenotype.

29. Martoni E, Petrini S, Trabanelli C, Sabatelli P, Urciuolo A, Selvatici R, D'Amico A, Falzarano S, Bertini E, Bonaldo P, Ferlini A, Gualandi F. Characterization of a rare case of Ullrich congenital muscular dystrophy due to truncating mutations within the COL6A1 gene C-terminal domain: a case report. *BMC Med Genet.* 2013, 14:59.
30. Falzarano MS, Passarelli C, Bassi E, Fabris M, Perrone D, Sabatelli P, Maraldi NM, Donà S, Selvatici R, Bonaldo P, Sparnacci K, Laus M, Braghetta P, Rimessi P, Ferlini A. Biodistribution and molecular studies on orally administered nanoparticle-AON complexes encapsulated with alginate aiming at inducing dystrophin rescue in mdx mice. *Biomed Res Int.* 2013.
31. Ferlini A. & Falzarano MS. Nanoparticles to Deliver Antisense Oligonucleotides Aimed at Exon Skipping Therapies. V.A. Erdmann and J. Barciszewski (eds.), *DNA and RNA Nanobiotechnologies in Medicine: Diagnosis and Treatment of Diseases, RNA Technologies*, Springer-Verlag Berlin Heidelberg 2013.
32. Falzarano MS, Passarelli C, Ferlini A. Nanoparticle delivery of antisense oligonucleotides and their application in the exon skipping strategy for duchenne muscular dystrophy. *Nucleic Acid Ther.* 2014 Feb 24:87-100.
33. Falzarano MS, Bassi E, Passarelli C, Braghetta P, Ferlini A. Biodistribution Studies of Polymeric Nanoparticles for Drug Delivery in Mice. *Hum Gene Ther.* 2014.
34. Wein N, Vulin A, Falzarano MS, Szigyarto CA, Maiti B, Findlay A, Heller KN, Uhlén M, Bakthavachalu B, Messina S, Vita G, Passarelli C, Gualandi F, Wilton SD, Rodino-Klapac LR, Yang L, Dunn DM, Schoenberg DR, Weiss RB, Howard MT, Ferlini A, Flanigan KM. Translation from a DMD exon 5 IRES results in a functional dystrophin isoform that attenuates dystrophinopathy in humans and mice. *Nat Med.* 2014;20:992-1000.
35. Falzarano MS, Scotton C, Passarelli C, Ferlini A.. Duchenne Muscular Dystrophy: from Diagnosis to Therapy. *Molecules.* 2015 20:18168-84.
36. Falzarano MS, D'Amario D, Siracusano A, Massetti M, Amodeo A, La Neve F, Maroni CR, Mercuri E, Osman H, Scotton C, Armaroli A, Rossi R, Selvatici R, Crea F, Ferlini A. DMD myogenic cells from urinederived stem cells recapitulate the dystrophin genotype and phenotype. *Hum Gene Ther.* 2016 Aug 16.
37. Scotton C, Bovolenta M, Schwartz E, Falzarano MS, Martoni E, Passarelli C, Armaroli A, Osman H, Rodolico C, Messina S, Pegoraro E, D'Amico A, Bertini E, Gualandi F, Neri M, Selvatici R, Boffi P, Maioli MA, Lochmüller H, Straub V, Bushby K, Castrignanò T, Pesole G, Sabatelli P, Merlini L, Braghetta P, Bonaldo P, Bernardi P, Foley R, Cirak S, Zaharieva I, Muntoni F, Capitanio D, Gelfi C, Kotelnikova E, Yuryev A, Lebowitz M, Zhang X, Hodge BA, Esser KA, Ferlini A. Deep RNA profiling identified CLOCK and molecular clock genes as pathophysiological signatures in collagen VI myopathy. *J Cell Sci.* 2016, 129:1671-84.

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39. Gherardi S, Bovolenta M, Passarelli C, Falzarano MS, Pigini P, Scotton C, Neri M, Armaroli A, Osman H, Selvatici R, Gualandi F, Recchia A, Mora M, Bernasconi P, Maggi L, Morandi L, Ferlini A, Perini G. Transcriptional and epigenetic analyses of the DMD locus reveal novel cis- acting DNA elements that govern muscle dystrophin expression. *Biochim Biophys Acta*. 2017, 1860:1138-1147.
40. Falzarano MS, Flesia C, Cavalli R, Guiot C, Ferlini A. Nanodiagnosis and nanodelivery applications in genetic alterations. *Curr Pharm Des*. 2018 Jan 10.
41. Hiller M, Falzarano MS, Garcia-Jimenez I, Sardone V, Verheul RC, Popplewell L, Anthony K, Ruiz-Del-Yerro E, Osman H, Goeman JJ, Mamchaoui K, Dickson G, Ferlini A, Muntoni F, Aartsma-Rus A, Arechavala-Gomeza V, Datson NA, Spitali P. A multicenter comparison of quantification methods for antisense oligonucleotide-induced DMD exon 51 skipping in Duchenne muscular dystrophy cell cultures. *PLoS One*. 2018 Oct 2;13(10):e0204485.
42. Falzarano MS, Ferlini A. Urinary Stem Cells as Tools to Study Genetic Disease: Overview of the Literature. *J Clin Med*. 2019 May 8;8(5):627.
43. Bigoni S, Neri M, Scotton C, Farina R, Sabatelli P, Jiang C, Zhang J, Falzarano MS, Rossi R, Ognibene D, Selvatici R, Gualandi F, Bosshardt D, Perri P, Campa C, Brancati F, Salvatore M, De Stefano MC, Taruscio D, Trombelli L, Fang M, Ferlini A. Homozygous Recessive Versican Missense Variation Is Associated With Early Teeth Loss in a Pakistani Family. *Front Genet*. 2019 Jan 21;9:723.
44. Neri M, Rossi R, Trabanielli C, Mauro A, Selvatici R, Falzarano MS, Spedicato N, Margutti A, Rimessi P, Fortunato F, Fabris M, Gualandi F, Comi G, Tedeschi S, Seia M, Fiorillo C, Traverso M, Bruno C, Giardina E, Piemontese MR, Merla G, Cau M, Marica M, Scuderi C, Borgione E, Tessa A, Astrea G, Santorelli FM, Merlini L, Mora M, Bernasconi P, Gibertini S, Sansone V, Mongini T, Berardinelli A, Pini A, Liguori R, Filosto M, Messina S, Vita G, Toscano A, Vita G, Pane M, Servidei S, Pegoraro E, Bello L, Travaglini L, Bertini E, D'Amico A, Ergoli M, Politano L, Torella A, Nigro V, Mercuri E, Ferlini A. The Genetic Landscape of Dystrophin Mutations in Italy: A Nationwide Study. *Front Genet*. 2020 Mar 3;11:131.
45. Passarelli C, Selvatici R, Carrieri A, Di Raimo FR, Falzarano MS, Fortunato F, Rossi R, Straub V, Bushby K, Reza M, Zharayeva I, D'Amico A, Bertini E, Merlini L, Sabatelli P, Borgiani P, Novelli G, Messina S, Pane M, Mercuri E, Claustres M, Tuffery-Giraud S, Aartsma-Rus A, Spitali P, T'Hoen PA, Lochmüller H, Strandberg K, Al-Khalili C, Kotelnikova E, Lebowitz M, Schwartz E, Muntoni F, Scapoli C and Ferlini A. Tumor Necrosis Factor Receptor SF10A (TNFRSF10A) SNPs Correlate With Corticosteroid Response in Duchenne Muscular Dystrophy. *Front. Genet.*, 03 July 2020.
46. Falzarano MS, Argenziano M, Marsollier AC, Mariot V, Rossi D, Selvatici R, Dumonceaux J, Cavalli R, Ferlini A. Chitosan- shelled nanobubbles irreversibly encapsulate morpholino conjugate antisense oligonucleotides and are ineffective for PMO-mediated gene silencing of DUX4. *Nucleic Acid Ther*. 2020 Jul 13.