Curriculum Vitae

Personal information

Alessandro Aiuti

Work experience

1. Employer: Dr. R.A. Gatti
   - Start date: 1987
   - End date: 1987
   - Position: Summer Student
   - Activities:
   - Country: United States
2. Employer: Laboratory of Molecular Genetics
   - Start date: 1987
   - End date: 1990
   - Position: Pre_doctoral student
   - Activities:
   - Country: Italy
3. Employer: Head Prof. R. D'Amelio
   - Start date: 1991
   - End date: 1992
   - Position: Medical Officer
   - Activities:
   - Country: Italy
4. Employer: Head, Prof. A. Fantoni
   - Start date: 1991
   - End date: 1995
   - Position: PhD Program Student
   - Activities:
   - Country: Italy
5. Employer: J.C. Gutierrez_Ramos
   - Start date: 1994
   - End date: 1996
   - Position: Post_doctoral fellow
   - Activities:
   - Country: United States
6. Employer: Head, Dr. C. Bordignon
   - Start date: 1996
   - End date: 1997
   - Position: Post_doctoral fellow
   - Activities:
   - Country: Italy
7. Employer: Telethon Foundation, Italy
   - Start date: 1997
   - End date: 2000
   - Position: Research Scientist
   - Activities:
   - Country: Italy
8. Employer: SR_Tiget, Scientific Institute H.S. Raffaele
   - Start date: 1997
   - End date: 2003
   - Position: Group leader
   - Activities:
   - Country: Italy
9. Employer: Scientific Institute HS Raffaele
   - Start date: 2000
   - End date: 2007
   - Position: MD Research Scientist
   - Activities:
   - Country: Italy
10. Employer: Pediatric Clinical Research Unit, SR_Tiget
    - Start date: 2000
    - End date: 2007
    - Position: Haematologist
    - Activities:
    - Country: Italy
11. Employer: Course of “Gene transfer into human hematopoietic cells”, School of Medicine, Univ. “Vita_Salute”, San Raffaele, Milano
    - Start date: 2001
    - End date: 2004
    - Position: Temporary assignment of Professorship
    - Activities:
    - Country: Italy
12. Employer: SR_Tiget, Scientific Institute H.S. Raffaele
    - Start date: 2003
    - End date: 2007
    - Position: Head of a Research Unit
    - Activities:
    - Country: Italy

13. Employer: Scientific Institute H.S. Raffaele
    - Start date: 2004
    - End date: 2007
    - Position: Member of the Committee for the Appointment and Promotions
    - Activities:
    - Country: Italy
14. Employer: Course of “Molecular Pediatrics”, School of Medicine, University “Vita_Salute”, San Raffaele
    - Start date: 2004
    - End date: 2010
    - Position: Temporary assignment of Professorship
    - Activities:
    - Country: Italy
15. Employer: SR_Tiget
    - Start date: 2007
    - End date:
    - Position: Head of Unit.Pathogenesis and therapy of primary immunodeficiencies.
    - Activities:
    - Country: Italy
16. Employer: University of Rome Tor Vergata
    - Start date: 2007
    - End date: 102014
    - Position: Associate Professor of Pediatrics
    - Activities:
    - Country: Italy
17. Employer: SR_Tiget, San Raffaele Hospital
    - Start date: 2009
    - End date:
    - Position: Coordinator of Clinical Research
    - Activities:
    - Country: Italy
18. Employer: Dpt. of Pediatrics, University of Rome Tor Vergata _ Bambino Gesù Pediatric Hospital
    - Start date: 2010
    - End date: 2013
    - Position: Head of Gene Therapy Unit
    - Activities: Pediatric Immunology
    - Country: Italy
19. Employer: Dpt. of Pediatrics, University of Rit, San Raffaele Hospital
    - Start date: 2011
    - End date: 2014
    - Position: Head, Primary Immunodeficiencies (PID) outpatients' clinic
    - Activities:
    - Country: Italy
20. Employer: SR_Tiget, San Raffaele Hospital
    - Start date: 052011
    - End date:
    - Position: Head, Clinical Research Unit
    - Activities:
    - Country: Italy
21. Employer: "Vita_Salute" San Raffaele University, Milan
    - Start date: 112014
    - End date: 122015
    - Position: Associate Professor of Pediatrics
    - Activities:
    - Country: Italy
22. Employer: San Raffaele Hospital
    - Start date: 112014
    - End date:
    - Position: Chief of Clinic, Pediatric Immunohematology Unit
    - Activities:
    - Country: Italy
23. Employer: "Vita_Salute" San Raffaele University
    - Start date: 012016
    - End date:
    - Position: Full Professor of Pediatrics
    - Activities:
    - Country: Italy
24. Employer: SR_Tiget, San Raffaele Scientific Institute
    - Start date: 042017
    - End date:
    - Position: Deputy Director, clinical research
    - Activities:
    - Country: Italy

Education and training

1. Subject: School of Medicine, University of Rome “La Sapienza”, Italy
    - Start date: 1990
    - End date: 1990
    - Qualification: M.D
    - Organisation:
    - Country: Italy
2. Subject: School of Medicine, University of Rome
    - Start date: 1996
    - End date: 1996
    - Qualification: Ph.D.
    - Organisation:
    - Country:
3. Subject: School of Medicine, University of Milan
    - Start date: 1998
    - End date: 1998
    - Qualification: Natl. Board
    - Organisation:
    - Country: Italy

Additional information

Publications

Alessandro Aiuti has published more than 300 papers in international scientific peer review journals and national journals, and contributed 8 chapters to books.

Quantitative parameters of his scientific production in internationaI journals can be detailed as follows: h index (Scopus): 73 , Total citations (Scopus): 24.642 , Average citations: 86.16 Total impact factor (IF) of publications: 3707.08 , Average impact factor (IF): 12.44

List of Selected publications

1. **Aiuti A.**, Webb I. J., Bleul C., Springer T., and Gutierrez-Ramos J. C. (1997). The chemokine SDF-1 is a chemoattractant for human CD34+ hematopoietic progenitor cells and provides a new mechanism to explain the mobilization of CD34+ progenitors to peripheral blood. J Exp Med 185, 111-120. Times cited:1233 IF 15.572
2. **Aiuti A.,** Slavin S., Aker M., Ficara F., Deola S., Mortellaro A., Morecki S., Andolfi G., Tabucchi A., Carlucci F., Marinello E., Cattaneo F., Vai S., Servida P., Miniero R., Roncarolo M. G., and Bordignon C. (2002). Correction of ADA-SCID by stem cell gene therapy combined with nonmyeloablative conditioning. Science (New York, NY) 296, 2410-2413. Times cited:1022 IF 23.329
3. **Aiuti A.**, Vai S., Mortellaro A., Casorati G., Ficara F., Andolfi G., Ferrari G., Tabucchi A., Carlucci F., Ochs H. D., Notarangelo L. D., Roncarolo M. G., and Bordignon C. (2002). Immune reconstitution in ADA-SCID after PBL gene therapy and discontinuation of enzyme replacement. Nat Med 8, 423-425. Times cited:189 IF 27.906
4. Ficara F., Superchi D. B., Hernandez R. J., Mocchetti C., Carballido-Perrig N., Andolfi G., Deola S., Colombo A., Bordignon C., Carballido J. M., Roncarolo M. G., and **Aiuti A.** (2004). IL-3 or IL-7 increases ex vivo gene transfer efficiency in ADA-SCID BM CD34+ cells while maintaining in vivo lymphoid potential. Mol Ther 10, 1096-1108. Times cited:16 IF 5.204
5. Mortellaro A., Hernandez R. J., Guerrini M. M., Carlucci F., Tabucchi A., Ponzoni M., Sanvito F., Doglioni C., Di Serio C., Biasco L., Follenzi A., Naldini L., Bordignon C., Roncarolo M. G., and **Aiuti A.** (2006). Ex vivo gene therapy with lentiviral vectors rescues adenosine deaminase (ADA)-deficient mice and corrects their immune and metabolic defects. Blood 108, 2979-2988. Times cited:66 IF 10.370
6. **Aiuti A.**, Cassani B., Andolfi G., Mirolo M., Biasco L., Recchia A., Urbinati F., Valacca C., Scaramuzza S., Aker M., Slavin S., Cazzola M., Sartori D., Ambrosi A., Di Serio C., Roncarolo M. G., Mavilio F., and Bordignon C. (2007). Multilineage hematopoietic reconstitution without clonal selection in ADA-SCID patients treated with stem cell gene therapy. J Clin Invest 117, 2233-2240. Times cited:230 IF 15.754
7. **Aiuti A.**, Cattaneo F., Galimberti S., Benninghoff U., Cassani B., Callegaro L., Scaramuzza S., Andolfi G., Mirolo M., Brigida I., Tabucchi A., Carlucci F., Eibl M., Aker M., Slavin S., Al-Mousa H., Al Ghonaium A., Ferster A., Duppenthaler A., Notarangelo L., Wintergerst U., Buckley R.H., Bregni M., Marktel S., Valsecchi M.G., Rossi P., Ciceri, F. Miniero R., Bordignon C., and Roncarolo M.G. (2009). Gene therapy for immunodeficiency due to adenosine deaminase deficiency. N Engl J Med 360, 447-458. Times cited:882 IF 52.589
8. Biasco L., Ambrosi A., Pellin D., Bartholomae C., Brigida I., Roncarolo M.G., Di Serio C., von Kalle C., Schmidt M., and **Aiuti A.** (2010) Integration profile of retroviral vector in gene therapy treated patients is cell-specific according to gene expression and chromatin conformation of target cell. EMBO Mol Med. 3, 89-101. Times cited:94 IF 10.333
9. Sauer A.V., Brigida I., Carriglio N., Jofra Hernandez R., Scaramuzza S., Clavenna D., Sanvito F., Poliani P.L., Gagliani N., Carlucci F., Tabucchi A., Roncarolo M.G., Traggiai E., Villa A., and **Aiuti A.** (2012). Alterations in the adenosine metabolism and CD39/CD73 adenosinergic machinery cause loss of Treg cell function and autoimmunity in ADA-deficient SCID. Blood. 119, 1428-1439. Times cited:90 IF 9.898
10. Scaramuzza S., Biasco L., Ripamonti A., Castiello M.C., Loperfido M., Draghici E., Jofra Hernandez R., Benedicenti F., Radrizzani M., Salomoni M., Ranzani M., Bartholomae CC., Vicenzi E., Finocchi A., Bredius R., Bosticardo M., Schmidt M., von Kalle C., Montini E., Biffi A., Roncarolo M.G., Naldini L., Villa A., and **Aiuti A.** (2013). Preclinical safety and efficacy on human CD34+ cells transduced with lentiviral vector for the treatment of Wiskott-Aldrich Syndrome. Mol Ther. 21, 175-184. Epub 2012 Feb 28. Times cited:70 IF 7.149
11. **Aiuti A.@**, Biasco L., Scaramuzza S., Ferrua F., Cicalese M.P., Baricordi C., Dionisio F., Calabria A., Giannelli S.,.Castiello M.C, Bosticardo M., Evangelio C., Assanelli A., Casiraghi M., Di Nunzio S., Callegaro L., Benati C., Rizzardi P., Pellin D., Di Serio C., Schmidt M., Van Kalle C., Gardner J.,. Mehta N, Neduva V., Dow D.J., Galy A., Miniero R., Finocchi A., Metin A., Banerjee P., Orange J., Galimberti S., Valsecchi M.G., Biffi A., Montini E., Villa A., Ciceri F., Roncarolo M.G., and Naldini L. (2013). Lentiviral hematopoietic stem cell gene therapy in patients with Wiskott-Aldrich syndrome. Science 341(6148):1233151. Epub 2013 Jul 11. Times cited:920 IF 31.207 @corresponding author
12. Biffi A., Montini E., Lorioli L., Cesani M., Fumagalli F., Plati T., Baldoli C., Martino S., Calabria A., Canale S., Benedicenti F., Vallanti G., Biasco L., Leo S., Kabbara N., Zanetti G., Rizzo W.B., Mehta N., Cicalese M.P., Casiraghi M., Boelens J.J., Del Carro U., Dow D.J., Schmidt M., Assanelli A., Neduva V., Di Serio C., Stupka E., Gardner J., Van Kalle C., Bordignon C., Ciceri F., Rovelli A., Roncarolo M.G., **Aiuti A.**, Sessa M. and Naldini L. (2013). Lentiviral hematopoietic stem cell gene therapy benefits metachromatic leukodystrophy. Science 341(6148):1233158. Epub 2013 Jul 11. Times cited:1031 IF 31.207
13. Chiriaco M., Farinelli G., Capo V., Di Matteo G., Zonari E., Scaramuzza S., Sergi Sergi L., Migliavacca M.,Hernandez R.J., Bombelli F., Giorda E., Kajaste-Rudnitski A., Trono D., Grez M., Rossi P., Finocchi A., Naldini L., Gentner B., and **Aiuti A.** (2014). Dual-regulated lentiviral vector for gene therapy of X-linked chronic granulomatosis. Mol Therapy. 22, 1472-1483. Epub 2014 May 29. Times cited: 57 IF. 6.82
14. Biasco L., Scala S., Basso Ricci L., Dionisio F., Baricordi C., Calabria A., Giannelli S., Cieri N., Barzaghi F., Pajno R., Al-Mousa H., Scarselli A., Cancrini C., Bordignon C., Roncarolo M.G., Monti E., Bonini C., and **Aiuti A.** (2015). In vivo tracking of T cells in humans unveils decade-long survival and activity of genetically modified T memory stem cells. Sci Transl Med. 7: 273ra13. Times cited:146 IF 14.414
15. Cicalese M.P., Ferrua F., Castagnaro L., Pajno R., Barzaghi F., Giannelli S., Dionisio F., Brigida I., Bonopane M., Casiraghi M., Tabucchi A., Carlucci F., Grunebaum E., Adeli M., Bredius R.G., Puck J.M., Stepensky P., Tezcan I., Rolfe K., De Boever E., Reinhardt R.R., Appleby J., Ciceri F., Roncarolo M.G., and **Aiuti A.** (2016). Update on the safety and efficacy of retroviral gene therapy for immunodeficiency due to adenosine deaminase deficiency. Blood. 128: 45-54. Epub April 29, 2016. Times cited:184 IF 9.898
16. Biasco L., Pellin D., Scala S., Dionisio F., Basso-Ricci L.,Leonardelli L., Scaramuzza S., Baricordi C., Ferrua F., Cicalese M.P., Giannelli S., Neduva V., Dow D.J., Schmidt M., Von Kalle C.,Roncarolo M.G., Ciceri F., Vicard P., Wit E., Di Serio C., Naldini L., and **Aiuti A.** (2016). In vivo tracking of human hematopoiesis reveals patterns of clonal dynamics during early and steady-state seconstitution phases. Cell Stem Cell. 19: 107-119. Epub May 25, 2016. Times cited:196 IF 23.394
17. Farinelli G., Hernandez RJ, Rossi A, Ranucci S, Sanvito F, Migliavacca M,Brombin C, Pramov A, Serio CD, Bovolenta C, Gentner B, Bragonzi A, and **Aiuti A.** (2016). Lentiviral vector gene therapy protects XCGD mice from Acute Staphylococcus aureus pneumonia and inflammatory response. Mol Ther. 24: 1873-1880. Epub July 26, 2016. Times cited:11 IF 6.688
18. **Aiuti A.**, Roncarolo M.G. and Naldini L. (2017). Gene therapy for ADA-SCID, the first marketing approval of an ex vivo gene therapy in Europe: paving the road for the next generation of advanced therapy medicinal products. EMBO Mol Med. 9: 737-740. Epub April 10, 2017. Times cited:213 IF 10.293
19. Scala S., Basso-Ricci L., Dionisio F., Pellin D., Giannelli S., Salerio F.A., Leonardelli L., Cicalese M.P., Ferrua F., **Aiuti A.***, and Biasco L.*(2018). Dynamics of hematopoietic stem/progenitor cells after autologous transplantation in humans. Nat Med. 24:1683-1690. Epub 2018 Oct 1. *Equal contribution. Times cited:97 IF 30.641
20. Marktel S., Scaramuzza S., Cicalese M.P., Giglio F., Galimberti S., Lidonnici M.R., Calbi V., Assanelli A., Bernardo M.E., Rossi C., Calabria A., Milani R., Gattillo S., Benedicenti F., Spinozzi G., Aprile A., Bergami A., Casiraghi M., Consiglieri G., Masera N., D'Angelo E., Mirra N., Origa R., Tartaglione I., Perrotta S., Winter R., Coppola M., Viarengo G., Santoleri L., Graziadei G., Gabaldo M., Valsecchi M.G., Montini E.,

Naldini L., Cappellini M.D., Ciceri .F*, **Aiuti A.***, and Ferrari G.* (2019). Intrabone hematopoietic stem cell gene therapy for adult and pediatric patients affected by transfusion-dependent ß-thalassemia. Nat Med.25:234-241. Times cited:194 IF 36.130 *Equal contribution

21. Ferrua F. *, Cicalese M.P.*, Galimberti S., Giannelli S., Dionisio F., Barzaghi F., Migliavacca M., Bernardo M.E., Calbi V., Assanelli A.A., Facchini M., Fossati C., Albertazzi E., Scaramuzza S., Brigida I., Scala S., Basso-Ricci L., Pajno R., Casiraghi M., Canarutto D., Salerio F. A., Albert M. H., Bartoli A., Wolf H.M., Fiori R., Silvani P., Gattillo S., Villa A., Biasco L., Dott C., Culme-Seymour E.J., van Rossem K., Atkinson G., Valsecchi M.G., Roncarolo M.G., Ciceri F., Naldini L., and **Aiuti A.** (2019) . Lentiviral haematopoietic stem/progenitor cell gene therapy for the treatment of Wiskott-Aldrich syndrome: interim results of a non-randomized, open-label, phase 1/2 clinical study. Lancet Hematology. 6: e239-e253. Epub 2019 April 10. Times cited:165 IF 10.406
22. Ferrari G., Thrasher A.J., and **Aiuti A.** (2021). Gene therapy using haematopoietic stem and progenitor cells. Nat Rev Genet. 22: 216-234. Epub 2020 Dec 10. Times cited:174 IF 59.924
23. Gentner B., Tucci F., Galimberti S., Fumagalli F., De Pellegrin M., Silvani P., Camesasca C., Pontesilli S., Darin S., Ciotti F., Sarzana M., Consiglieri G., Filisetti C., Forni G., Passerini L., Tomasoni D., Cesana D., Calabria A., Spinozzi G., Cicalese M.P., Calbi V., Migliavacca M., Barzaghi F., Ferrua F., Gallo V., Miglietta S., Zonari E., Cheruku P.S., Forni C., Facchini M., Corti A., Gabaldo M., Zancan S., Gasperini S., Rovelli A., Boelens J.J., Jones S.A., Wynn R., Baldoli C., Montini E., Gregori S., Ciceri F., Valsecchi M.G., la Marca G., Parini R., Naldini L., **Aiuti A.*@**, Bernardo M.E*. and MPSI Study Group. (2021). Hematopoietic Stem- and Progenitor-Cell Gene Therapy for Hurler Syndrome. N Engl J Med. 385:1929-1940. Times cited:99 IF 176.082 @corresponding author, *equal contribution
24. Fumagalli F., Calbi V., Natali Sora M.G., Sessa M., Baldoli C., Rancoita P.M.V., Ciotti F., Sarzana M., Fraschini M., Zambon A.A., Acquati S., Redaelli D., Attanasio V., Miglietta S., De Mattia .F, Barzaghi F., Ferrua F., Migliavacca M., Tucci F., Gallo V., Del Carro U., Canale S., Spiga I., Lorioli L., Recupero S., Fratini E.S., Morena F., Silvani P., Calvi M.R., Facchini M., Locatelli S., Corti A., Zancan S., Antonioli G., Farinelli G., Gabaldo M., Garcia-Segovia J., Schwab L.C., Downey G.F., Filippi M., Cicalese M.P., Martino S., Di Serio C., Ciceri F., Bernardo M..E, Naldini L., Biffi A., and **Aiuti A.** (2022). Lentiviral haematopoietic stem-cell gene therapy for early-onset metachromatic leukodystrophy: long-term results from a non-randomised, open-label, phase 1/2 trial and expanded access. Lancet.399:372-383. Times cited:152 IF 168.9
25. Tucci F., Galimberti S., Naldini L., Valsecchi M.G., and **Aiuti A.** (2022). A systematic review and meta-analysis of gene therapy with hematopoietic stem and progenitor cells for monogenic disorders. Nat Commun. 13:1315. Times cited:82 IF 16.6
26. Scala S, Ferrua F, Basso-Ricci L, Dionisio F, Omrani M, Quaranta P, Jofra Hernandez R, Del Core L, Benedicenti F, Monti I, Giannelli S, Fraschetta F, Darin S, Albertazzi E, Galimberti S, Montini E, Calabria A, Cicalese MP, **Aiuti A.** (2023). Hematopoietic reconstitution dynamics of mobilized- and bone marrow-derived human hematopoietic stem cells after gene therapy. Nat Commun. 14:3068. Times cited:9 IF 14.7
27. Migliavacca M, Barzaghi F, Fossati C, Rancoita PMV, Gabaldo M, Dionisio F, Giannelli S, Salerio FA, Ferrua F, Tucci F, Calbi V, Gallo V, Recupero S, Consiglieri G, Pajno R, Sambuco M, Priolo A, Ferri C, Garella V, Monti I, Silvani P, Darin S, Casiraghi M, Corti A, Zancan S, Levi M, Cesana D, Carlucci F, Pituch-Noworolska A, AbdElaziz D, Baumann U, Finocchi A, Cancrini C, Ladogana S, Meinhardt A, Meyts I, Montin D, Notarangelo LD, Porta F, Pasquet M, Speckmann C, Stepensky P, Tommasini A, Rabusin M, Karakas Z, Galicchio M, Leonardi L, Duse M, Guner SN, Di Serio C, Ciceri F, Bernardo ME, **Aiuti A.@**, Cicalese MP.(2024). Long-term and real-world safety and efficacy of retroviral gene therapy for adenosine deaminase deficiency Nat Med. 30:488-497. Epub 2024 Feb 14. Times cited:8 IF 58.7 @corresponding author
28. Quaranta P, Basso-Ricci L, Jofra Hernández RJ, Pacini G, Naldini MM, Barcella M, Seffin L, Pais G, Spinozzi G, Benedicenti F, Pietrasanta C, Cheong JG, Ronchi A, Pugni L, Dionisio F, Monti I, Giannelli S, Darin S, Fraschetta F, Barera G, Ferrua F, Calbi V, Ometti M, Di Micco R, Mosca F, Josefowicz SZ, Montini E, Calabria A, Bernardo ME, Cicalese MP, Gentner B, Merelli I, **Aiuti A***., Scala S.* (2024). Circulating Hematopoietic Stem/Progenitor Cells subsets contribute to human hematopoietic homeostasis. Blood. 2024 Mar 6 blood.2023022666. Online ahead of print. Times cited:5 IF 21.1 *Equal contribution
29. Calabria A, Spinozzi G, Cesana D, Buscaroli E, Benedicenti F, Pais G, Gazzo F, Scala S, Lidonnici MR, Scaramuzza S, Albertini A, Esposito S, Tucci F, Canarutto D, Omrani M, De Mattia F, Dionisio F, Giannelli S, Marktel S, Fumagalli F, Calbi V, Cenciarelli S, Ferrua F, Gentner B, Caravagna G, Ciceri F, Naldini L, Ferrari G, **Aiuti A**., Montini E. Long-term lineage commitment in haematopoietic stem cell gene therapy. Nature. 2024 Dec;636(8041):162-171. doi: 10.1038/s41586-024-08250-x. Epub 2024 Oct 23. PMID: 39442556; PMCID: PMC11618100. Times cited:3 IF 50.5
30. Fumagalli F., Calbi V., Gallo V., Zambon A. A., Recupero S., Ciotti F., Sarzana M., Fraschini M., Scarparo S., De Mattia F., Miglietta S., Pierini C., Soncini M., Morena F., Montini E., Barzaghi F., Consiglieri G., Ferrua F., Migliavacca M., Tucci F., Fratini E. S., Ippolito A., Silvani P., Calvi M. R., Clerici A., Corti A., Facchini M., Locatelli S., Sangalli M., Zancan S., Miotto F., Natali Sora M. G., Baldoli C., Martino S., Córdoba Claros A., Moro S. L., Gollop N. D., Abate J., Yarzi M. N., Nutkins P., Shenker A., Calissano M., Brooks J., Richardson A., Campbell L., Filippi M., Naldini L., Cicalese M. P., Ciceri F., Bernardo M. E., **Aiuti A.** Long-term Effects of Atidarsagene Autotemcel for Metachromatic Leukodystrophy. N Engl J Med 2025

The complete list of his publications can be https://pubmed.ncbi.nlm.nih.gov/?term=aiuti+a&sort=date&size=50

Projects

Recent grants/funding

-Hematopoietic stem/progenitor cell gene therapy for inborn errors of metabolism and immunity by innovative gene addition and gene editing approaches", funded by the Else-Kroener-Fresenius-Stiftung Prize for Medical Research. (2020-2025).

-Expanding the spectrum of adenosine deaminase 2 (ADA2) deficiency: towards a gene therapy approach. Italian Ministry of Health, Bando della Ricerca Finalizzata 2019. 2020-2022 (PI of research Unit)

-SR-Tiget Core Grant, Human Hematopoietic Stem/Progenitor cell trafficking and clonal tracking, Fondazione Telethon (2022-2026).

-Map, join and drive European activities for advanced therapy medicinal product development and implementation for the benefit of patients and society" (Join4ATMP). Funded by EU Commission (Horizon Europe) (2024-2026)

-"Implementation of an Italian Network for advanced diagnosis and targeted treatment of Inborn errors of Immunity". Funded by European Union/Next Generation EU. PNRRMR1-2022-12376594 (2023-2025)

-National Research Centre - Development of Gene Therapy and Drugs with RNA Technology, from the resources of the National Recovery and Resilience Plan (PNRR), Mission 4 'Education and Research', Component 2 'From research to enterprise', Investment Line 1. 4 'Strengthening research structures and creation of national R&S champions on certain Key Enabling Technologies", finanziato dall'Unione Europea – Next Generation EU, Progetto CN00000041, CUP B93D21010860004, Spoke n. 10 " Preclinical. Target diseases: MPSIVA, MPSIVB, alfa-mannosidase, beta thalassemia, osteopetrosis.

-PNC PROGETTO HUB LIFE SCIENCE - ADVANCED THERAPY (LSH-TA) PNC-E3-2022-23683269 financed by the Ministry of Health within the framework of the National Complementary Plan Innovative Health Ecosystem' Unique investment code: PNC-E.3 (local Project Leader)

-"Pathogenetic mechanisms and development of innovative gene therapies for Mucopolysaccharidosis type IVA.

Funded by PRIN, Ministry of University and Research. (2023-2025)

-PI of academic observational studies on biological samples for research purposes conducted at Sr-TIGET on disease pathogenesis and hematopoietic stem cell gene therapy.

Memberships

MEMBERSHIPS: • Italian Society of Pediatrics (SIP) • Italian Society of Pediatric Research (SIRP) • Italian Society of Pediatric Oncology and Hematology (AIEOP) • Italian Strategic Committee on Primary Immunodeficiencies (AIEOP_IPINET) • European Society of Immunodeficiencies (ESID) • European Working Party on Inborn Errors (EBMT) • American Society of Hematology (ASH) • American Society of Gene and Cell Therapy (ASGCT) • European Society of Gene and Cell Therapy (ESGCT), President of the Italian Immunodeficiency Association (ImmunITA) (03/2024 ongoing), Board Member of Agora not for profit Foundation (Access to Gene Therapy for Rare Diseases)(2022 – present), Member of the Executive Board of the Italian Society of Gene and Cell Therapy (SITGEC) (2024 – present).

Other Relevant Information

1993: Fellowship from the Italian Ministry of Health for scientists working on AIDS research in a foreign country.
1996: Travel Award from the American Society of Hematology for presenting an oral communication at the ASH annual meeting in San Diego.
2002: Top abstract presented at the Plenary Session of the American Society of Gene Therapy
2003: Young Investigator Award, American Society of Gene Therapy
2004: "JOUAN BIOTHERAPY AWARD" for the best clinical research project
2009: HSR Scientific Retreat: Best paper award in translational medicine
2010: XVIIIth ESGCT Meeting: Award for an outstanding career and pioneering contributions to the field
2010: Award from AACS of Rome – Special Mention "Heart of Rome"
2014:National Award "Cultura della Solidarietà" for an outstanding activity in science, which has granted so many benefits to human being. This award has obtained the acknowledgment of the President of Italy.
2015: Award from the Centro Studi Marche (CE.S.MA) "Picus del Ver Sacrum" Marchigiani of the year 2014, XXX edition.
2020: The Else Kroener Fresenius Prize for Medical Research 2020 for groundbreaking successes in the development of gene therapies.
2022: Callahan Award from the Wiskott – Aldrich Foundation for outstanding service to the WAS Community
2024: European Paediatric Association (EPA-UNESPA) Medal of Science.
2025: International Society for Stem Cell Research (ISSCR) Public Service Award