EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Curriculum Vitae

PERSONAL INFORMATION

Alessandro Aiuti

WORK EXPERIENCE

1987- 1987 **Summer Student**
Dr. R.A. Gatti (United States)

1987- 1990 **Pre-doctoral student**
Laboratory of Molecular Genetics (Italy)

1991- 1992 **Medical Officer**
Head Prof. R. D'Amelio (Italy)

1991- 1995 **PhD Program Student**
Head, Prof. A. Fantoni (Italy)

1994- 1996 **Post-doctoral fellow**
J.C. Gutierrez-Ramos (United States)

1996- 1997 **Post-doctoral fellow**
Head, Dr. C. Bordignon (Italy)

1997- 2000 **Research Scientist**
Telethon Foundation, Italy (Italy)

1997- 2003 **Group leader**
SR-Tiget, Scientific Institute H.S. Raffaele (Italy)

2000- 2007 **MD Research Scientist**
Scientific Institute HS Raffaele (Italy)

2000- 2007 **Haematologist**
Pediatric Clinical Research Unit, SR-Tiget (Italy)

2001- 2004 **Temporary assignment of Professorship**
Course of Gene transfer into human hematopoietic cells, School of Medicine, Univ. Vita-Salute, San Raffaele, Milano (Italy)

2003- 2007 **Head of a Research Unit**
SR-Tiget, Scientific Institute H.S. Raffaele (Italy)

2004- 2007 **Member of the Committee for the Appointment and Promotions**
Scientific Institute H.S. Raffaele (Italy)

2004- 2010 **Temporary assignment of Professorship**
Course of Molecular Pediatrics, School of Medicine, University Vita-Salute, San Raffaele (Italy)

2007- Present **Head of Unit.Pathogenesis and therapy of primary immunodeficiencies.**
SR-Tiget (Italy)

2007-October 2014 **Associate Professor of Pediatrics**
University of Rome Tor Vergata (Italy)

2009- Present **Coordinator of Clinical Research**
SR-Tiget, San Raffaele Hospital (Italy)

2010- 2013 **Head of Gene Therapy Unit**
Dpt. of Pediatrics, University of Rome Tor Vergata - Bambino Gesù Pediatric Hospital (Italy)
Pediatric Immunology

2011- 2014 **Head, Primary Immunodeficiencies (PID) outpatients' clinic**
Dpt. of Pediatrics, University of Rit, San Raffaele Hospital (Italy)

May 2011- Present **Head, Clinical Research Unit**
SR-Tiget, San Raffaele Hospital (Italy)

November 2014-December 2015 **Associate Professor of Pediatrics**
"Vita-Salute" San Raffaele University, Milan (Italy)

November 2014- Present **Chief of Clinic, Pediatric Immunohematology Unit**
San Raffaele Hospital (Italy)

January 2016- Present **Full Professor of Pediatrics**
"Vita-Salute" San Raffaele Univer (Italy)

April 2017- Present **Deputy Director, clinical research**
SR-Tiget, San Raffaele Scientific Institute (Italy)

EDUCATION AND TRAINING

1990- 1990 **M.D**
School of Medicine, University of Rome La Sapienza, Italy (Italy)

1996- 1996 **Ph.D.**
School of Medicine, University of Rome ()

1998- 1998 **Natl. Board**
School of Medicine, University of Milan (Italy)

ADDITIONAL INFORMATION

Expertise

Pediatric Immunology and Hematology
Gene therapy
Advanced therapies
Primary immunodeficiencies
Hematopoietic stem cells
Non clinical and clinical studies

Publications

Alessandro Aiuti has published more than 200 papers in international scientific peer review journals and national journals, and contributed 8 chapters to books. Quantitative parameters of his scientific production in international journals can be detailed as follows:
h index (Scopus): 52
Total citations (Scopus): 12345
Average citations: 60,51
Total impact factor (IF) of publications: 1.753,63
Average impact factor (IF): 8,60
Publications on international journals:
1. Fattorossi A, Le Moli S, Pontesilli O, Aiuti A Jr, Nisini R, Galli E, Carbonari M, D'Amelio R. (1988). Complement activation is variably affected by fibronectin preparations obtained through different procedures. Boll Ist Sieroter Milan 67, 128-134. IF NA
2. Citarella F., Aiuti A., La Porta C., Russo G., Pietropaolo C., Rinaldi M., and Fantoni A. (1992). Control of human coagulation by recombinant serine proteases. Blood clotting is activated by recombinant factor XII deleted of five regulatory domains. Eur J Biochem / FEBS 208, 23-30. IF 3.499
3. D'Amelio R., Biselli R., Nisini R., Matricardi P. M., Aiuti A., Mezzaroma I., Pinter E., Pontesilli O., and Aiuti F. (1992). Spectrotype of anti-gp120 antibodies remains stable during the course of HIV disease. J Acquir Immune Defic Syndr 5, 930-935. IF 4.125
4. Citarella F., Misiti S., Felici A., Aiuti A., La Porta C., and Fantoni A. (1993). The 5' sequence of human factor XII gene contains transcription regulatory elements typical of liver specific, estrogen-modulated genes. Biochim Biophys Acta 1172, 197-199. IF 2.467
5. Forte P., Aiuti A., Pozzi L., Citarella F., Fattorossi A., Rossi G. B., and Fantoni A. (1993). Human CD4 produced in lymphoid cells of transgenic mice binds HIV gp120 and modifies the subsets of mouse T-cell populations. Immunogenetics 38, 455-459. IF 3.085
6. Nisini R., Aiuti A., Matricardi P. M., Fattorossi A., Ferlini C., Biselli R., Mezzaroma I., Pinter E., and D'Amelio R. (1994). Lack of evidence for a superantigen in lymphocytes from HIV-discordant monozygotic twins. AIDS (London, England) 8, 443-449. IF 5.294
7. Aiuti A., Forte P., Simeoni L., Lino M., Pozzi L., Fattorossi A., Giacomini P.,Ginelli E., Beretta A., Siccardi A., and et al. (1995). Membrane expression of HLA-Cw4 free chains in activated T cells of

transgenic mice. Immunogenetics 42, 368-375. IF 3.373
8. Giovannetti A., Aiuti A., Pizzoli P. M., Pierdominici M., Agostini E., Oliva A., Dianzani F., Aiuti F., and Pandolfi F. (1995). Tyrosine phosphorylationpathway is involved in interferon-gamma (IFN-gamma) production; effect of sodium ortho vanadate. Clin Exp Immunol 100, 157-163. IF 2.680
9. Bleul C. C., Fuhlbrigge R. C., Casasnovas J. M., Aiuti A., and Springer T. A. (1996). A highly efficacious lymphocyte chemoattractant, stromal cell-derived factor 1 (SDF-1). J Exp Med 184, 1101-1109. IF 15.126
10. 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. IF 15.572
11. Finco O., Nuti S., De Magistris M. T., Mangiavacchi L., Aiuti A., Forte P., Fantoni A., van der Putten H., and Abrignani S. (1997). Induction of CD4+ T cell depletion in mice doubly transgenic for HIV gp120 and human CD4. Eur J Immunol 27, 1319-1324. IF 5.701
12. Ruggieri L., Aiuti A., Salomoni M., Zappone E., Ferrari G., and Bordignon C. (1997). Cell-surface marking of CD(34+)-restricted phenotypes of human hematopoietic progenitor cells by retrovirus-mediated gene transfer. Hum Gene Ther 8, 1611-1623. IF 7.429
13. Aiuti A., Cicchini C., Bernardini S., Fedele G., Amicone L., Fantoni A., and Tripodi M. (1998). Hematopoietic support and cytokine expression of murine-stable hepatocyte cell lines (MMH). Hepatology (Baltimore, Md) 28, 1645-1654. IF 5.849
14. Aiuti A., Friedrich C., Sieff C. A., and Gutierrez-Ramos J. C. (1998). Identification of distinct elements of the stromal microenvironment that control human hematopoietic stem/progenitor cell growth and differentiation. Exp Hematol 26, 143-157. IF 3.591
15. Simeoni L., Forte P., Aiuti A., Candido A., Campese A. F., Fedele G., Di Tommaso F., Navarra M., and Fantoni A. (1998). Transgenic mice expressing human HIV receptors become persistently recipient of HIV DNA after injection with infected human cell lines. Folia Microbiol (Praha) 43, 525-526. IF 0.312
16. Aiuti A., Tavian M., Cipponi A., Ficara F., Zappone E., Hoxie J., Peault B., and Bordignon C. (1999). Expression of CXCR4, the receptor for stromal cell-derived factor-1 on fetal and adult human lympho-hematopoietic progenitors. Eur J Immunol 29, 1823-1831. IF 5.438
17. Aiuti A., Turchetto L., Cota M., Cipponi A., Brambilla A., Arcelloni C., Paroni R., Vicenzi E., Bordignon C., and Poli G. (1999). Human CD34(+) cells express CXCR4 and its ligand stromal cell-derived factor-1. Implications for infection by T-cell tropic human immunodeficiency virus. Blood 94, 62-73. IF 8.372
18. Arcelloni C., Aiuti A., Cipponi A., and Paroni R. (1999). High-performance liquid chromatographic purification and capillary electrophoresis quantification of the chemokine stromal cell-derived factor-1. J Chromatogr B Biomed Sci Appl 729, 369-374. IF NA
19. Grande A., Piovani B., Aiuti A., Ottolenghi S., Mavilio F., and Ferrari G. (1999). Transcriptional targeting of retroviral vectors to the erythroblastic progeny of transduced hematopoietic stem cells. Blood 93, 3276-3285. IF 8.372
20. Isgrò A., De Vita L., Mezzaroma I., Aiuti A., and Aiuti F. (1999). Recovery of haematopoietic abnormalities in HIV-1 infected patients treated with HAART. AIDS (London, England) 13, 2486-2488. IF 8.372
21. Isgrò A., Mezzaroma I., Aiuti A., De Vita L., Franchi F., Pandolfi F., Alario C., Ficara F., Riva E., Antonelli G., and Aiuti F. (2000). Recovery of hematopoietic activity in bone marrow from human immunodeficiency virus type 1-infected patients during highly active antiretroviral therapy. AIDS Res Hum Retroviruses 16, 1471-1479. IF 2.499
22. Dando J. S., Aiuti A., Deola S., Ficara F., and Bordignon C. (2001). Optimisation of retroviral supernatant production conditions for the genetic modification of human CD34+ cells. J Gene Med 3, 219-227. IF 3.103
23. Dando J. S., Roncarolo M. G., Bordignon C., and Aiuti A. (2001). A novel human packaging cell line with hematopoietic supportive capacity increases gene transfer into early hematopoietic progenitors. Hum Gene Ther 12, 1979-1988. IF 6.796

24. Aiuti A. (2002). Advances in gene therapy for ADA-deficient SCID. Curr Opin Mol Ther 4, 515-522. IF 5.640
25. 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. IF 23.329
26. 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. IF 27.906
27. Duprè L., Aiuti A., Trifari S., Martino S., Saracco P., Bordignon C., and Roncarolo M. G. (2002). Wiskott-Aldrich syndrome protein regulates lipid raft dynamics during immunological synapse formation. Immunity 17, 157-166. IF 18.866
28. Faedo A., Ficara F., Ghiani M., Aiuti A., Rubenstein J. L., and Bulfone A. (2002). Developmental expression of the T-box transcription factor T-bet/Tbx21 during mouse embryogenesis. Mech Dev 116, 157-160. IF 3.687
29. Giovannetti A., Mazzetta F., Caprini E., Aiuti A., Marziali M., Pierdominici M., Cossarizza A., Chessa L., Scala E., Quinti I., Russo G., and Fiorilli M. (2002). Skewed T-cell receptor repertoire, decreased thymic output, and predominance of terminally differentiated T cells in ataxia telangiectasia. Blood 100, 4082-4089. IF 9.273
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31. Isgrò A., Aiuti A., Mezzaroma I., Addesso M., Riva E., Giovannetti A., Mazzetta F., Alario C., Mazzone A., Ruco L., and Aiuti F. (2002). Improvement of interleukin 2 production, clonogenic capability and restoration of stromal cell function in human immunodeficiency virus-type-1 patients after highly active antiretroviral therapy. Br J Haematol 118, 864-874. IF 2.815
32. Isgrò A., Aiuti F., Mezzaroma I., Franchi F., Mazzone A. M., Lebba F., and Aiuti A. (2002). Interleukin 7 production by bone marrow-derived stromal cells in HIV-1-infected patients during highly active antiretroviral therapy. AIDS (London, England) 16, 2231-2232. IF 6.881
33. Simeoni L., Rufini A., Moretti T., Forte P., Aiuti A., and Fantoni A. (2002). Human CD26 expression in transgenic mice affects murine T-cell populations and modifies their subset distribution. Hum Immunol 63, 719-730. IF 2.373
34. Aiuti A., Ficara F., Cattaneo F., Bordignon C., and Roncarolo M.G. (2003). Gene therapy for adenosine deaminase deficiency. Curr Opin Allergy Clin Immunol 3, 461-466. IF N.A.
35. Bonini C., Grez M., Traversari C., Ciceri F., Marktel S., Ferrari G., Dinauer M., Sadat M., Aiuti A., Deola S., Radrizzani M., Hagenbeek A., Apperley J., Ebeling S., Martens A., Kolb H. J., Weber M., Lotti F., Grande A., Weissinger E., Bueren J. A., Lamana M., Falkenburg J. H., Heemskerk M. H., Austin T., Kornblau S., Marini F., Benati C., Magnani Z., Cazzaniga S., Toma S., Gallo-Stampino C., Introna M., Slavin S., Greenberg P. D., Bregni M., Mavilio F., and Bordignon C. (2003). Safety of retroviral gene marking with a truncated NGF receptor. Nat Med 9, 367-369. IF 30.550
36. Carlucci F., Tabucchi A., Aiuti A., Rosi F., Floccari F., Pagani R., and Marinello E. (2003). Capillary electrophoresis in diagnosis and monitoring of adenosine deaminase deficiency. Clin Chem 49, 1830-1838. IF 5.538
37. Pierdominici M., Mazzetta F., Caprini E., Marziali M., Digilio M. C., Marino B., Aiuti A., Amati F., Russo G., Novelli G., Pandolfi F., Luzi G., and Giovannetti A. (2003). Biased T-cell receptor repertoires in patients with chromosome 22q11.2 deletion syndrome (DiGeorge syndrome/velocardiofacial syndrome). Clin Exp Immunol 132, 323-331. IF 2.347
38. Aiuti A. (2004). Gene therapy for adenosine-deaminase-deficient severe combined immunodeficiency. Best Pract Res Clin Haematol 17, 505-516.IF 2.549
39. Carlucci F., Tabucchi A., Aiuti A., Rosi F., Floccari F., Pagani R., and Marinello E. (2004). Evaluation of ADA gene expression and transduction efficiency in ADA/SCID patients undergoing

gene therapy. Nucleosides Nucleotides Nucleic Acids 23, 1245-1248. IF 0.429
40. Dando J. S., Ficara F., Deola S., Roncarolo M. G., Bordignon C., and Aiuti A. (2004). Efficient gene transfer into primitive hematopoietic progenitors using a bone marrow microenvironment cell line engineered to produce retroviral vectors. Haematologica 89, 462-470. IF 4.192
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42. Duprè L., Trifari S., Follenzi A., Marangoni F., Lain de Lera T., Bernad A., Martino S., Tsuchiya S., Bordignon C., Naldini L., Aiuti A., and Roncarolo M. G. (2004). Lentiviral vector-mediated gene transfer in T cells from Wiskott-Aldrich syndrome patients leads to functional correction. Mol Ther 10, 903-915. IF 5.204
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44. Isgrò A., Mezzaroma I., Aiuti A., Fantauzzi A., Pinti M., Cossarizza A., and Aiuti F. (2004). Decreased apoptosis of bone marrow progenitor cells in HIV-1-infected patients during highly active antiretroviral therapy. AIDS (London, England) 18, 1335-1337. IF 5.893
45. Duprè L., Andolfi G., Tangye S. G., Clementi R., Locatelli F., Arico M., Aiuti A., and Roncarolo M. G. (2005). SAP controls the cytolytic activity of CD8+ T cells against EBV-infected cells. Blood 105, 4383-4389. IF 10.131
46. Isgrò A., Aiuti A., Leti W., Gramiccioni C., Esposito A., Mezzaroma I., and Aiuti F. (2005). Immunodysregulation of HIV disease at bone marrow level. Autoimmun Rev 4, 486-490. IF 3.091
47. Isgrò A., Aiuti A., Mezzaroma I., Ruco L., Pinti M., Cossarizza A., and Aiuti F. (2005). HIV type 1 protease inhibitors enhance bone marrow progenitor cell activity in normal subjects and in HIV type 1-infected patients. AIDS Res Hum Retroviruses 21, 51-57. IF 2.531
48. Isgrò A., Marziali M., Mezzaroma I., Luzi G., Mazzone A. M., Guazzi V., Andolfi G., Cassani B., Aiuti A., and Aiuti F. (2005). Bone marrow clonogenic capability, cytokine production, and thymic output in patients with common variable immunodeficiency. J Immunol 174, 5074-5081. IF 6.387
49. Duprè L., Marangoni F., Scaramuzza S., Trifari S., Hernandez R. J., Aiuti A., Naldini L., and Roncarolo M. G. (2006). Efficacy of gene therapy for Wiskott-Aldrich syndrome using a WAS promoter/cDNA-containing lentiviral vector and nonlethal irradiation. Hum Gene Ther 17, 303-313. IF 4.514
50. 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. IF 10.370
51. Trifari S., Sitia G., Aiuti A., Scaramuzza S., Marangoni F., Guidotti L. G., Martino S., Saracco P., Notarangelo L. D., Roncarolo M. G., and Dupre L. (2006). Defective Th1 cytokine gene transcription in CD4+ and CD8+ T cells from Wiskott-Aldrich syndrome patients. J Immunol 177, 7451-7461. IF 6.293
52. Aiuti A., Bachoud-Levi A. C., Blesch A., Brenner M. K., Cattaneo F., Chiocca E. A., Gao G., High K. A., Leen A. M., Lemoine N. R., McNeish I. A., Meneguzzi G., Peschanski M., Roncarolo M. G., Strayer D. S., Tuszynski M. H., Waxman D. J., and Wilson J. M. (2007). Progress and prospects: gene therapy clinical trials (part 2). Gene Ther 14, 1555-1563. IF 4.782
53. 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. IF 15.754
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proceedings of the EBMT satellite workshop (Hamburg, March 2006). Clin immunol (Orlando, Fla) 123, 139-147. IF 3.606
55. Trifari S., Marangoni F., Scaramuzza S., Aiuti A., Roncarolo M.G., Duprè L. (2007). Current understanding of the Wiskott-Aldrich syndrome and prospects for gene therapy. Expert Rev Clin Immunol 3, 205-215. IF 3.342
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57. Charrier S., Duprè L., Scaramuzza S., Jeanson-Leh L., Blundell M. P., Danos O., Cattaneo F., Aiuti A., Eckenberg R., Thrasher A. J., Roncarolo M. G., and Galy A. (2007). Lentiviral vectors targeting WASp expression to hematopoietic cells, efficiently transduce and correct cells from WAS patients. Gene Ther 14, 415-428. IF 4.782
58. Deola S., Scaramuzza S., Birolo R. S., Cergnul M., Ficara F., Dando J., Voena C., Vai S., Monari M., Pogliani E., Corneo G., Peccatori J., Selleri S., Bordignon C., Roncarolo M. G., Aiuti A., and Bregni M. (2007). Molecular purging of multiple myeloma cells by ex-vivo culture and retroviral transduction of mobilized-blood CD34+ cells. J Transl Med 5, 35. IF 3.300
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60. Marangoni F., Trifari S., Scaramuzza S., Panaroni C., Martino S., Notarangelo L.D., Baz Z., Metin A., Cattaneo F., Villa A., Aiuti A., Battaglia M., Roncarolo M.G., and Duprè, L. (2007). WASP regulates suppressor activity of human and murine CD4(+)CD25(+)FOXP3(+) natural regulatory T cells. J Exp Med 204, 369-80. IF 14.484
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63. de Lalla C., Festuccia N., Albrecht I., Chang H. D., Andolfi G., Benninghoff U., Bombelli F., Borsellino G., Aiuti A., Radbruch A., Dellabona P., and Casorati G. (2008). Innate-like effector differentiation of human invariant NKT cells driven by IL-7. J Immunol 180, 4415-4424. IF 6.068
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65. 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. IF 52.589
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hematopoietic stem cell gene therapy for inherited monogenic diseases. Mol Ther. 2020 Nov 19Online ahead of print. IF (2018): 8.402

202. Cirillo E., Giardino G., Ricci S., Moschese V., Lougaris V., Conti F., Azzari C.., Barzaghi F, Canessa C., Martire B., Badolato R., Dotta L., Soresina A., Cancrini C., Finocchi A., Montin D., Romano R., Amodio D., Ferrua F., Tommasini A., Baselli L.A., Dellepiane R.M., Polizzi A., Chessa L., Marzollo A., Cicalese M.P., Putti M.C., Pession A., Aiuti A., Locatelli F., Plebani A., and Pignata C.(2020). Consensus of the Italian Primary Immunodeficiency Network on transition management from pediatric to adult care in patients affected with childhood-onset inborn errors of immunity. J Allergy Clin Immunol. 146:967-983. Epub 2020 Aug 19. IF (2018): 14.110

Publications as part of Network:

1. Soresina A., Nacinovich R., Bomba M., Cassani M., Molinaro A., Sciotto A., Martino S., Cardinale F., De Mattia D., Putti C., Dellepiane R. M., Felici L., Parrinello G., Neri F., Plebani A; Italian Network for Primary Immunodeficiencies. (2009). The quality of life of children and adolescents with X-linked agammaglobulinemia. J Clin Immunol 29, 501-507. IF 2.654

2. Lougaris V., Pession A., Baronio M., Soresina A., Rondelli R., Gazzurelli L., Benvenuto A., Martino S., Gattorno M., Biondi A., Zecca M., Marinoni M., Fabio G., Aiuti A., Marseglia G., Putti M.C., Agostini C., Lunardi C., Tommasini A., Bertolini P., Gambineri E., Consolini R., Matucci A., Azzari C., Danieli M.G., Paganelli R., Duse M., Cancrini C., Moschese V., Chessa L., Spadaro G., Civino A., Vacca A., Cardinale F., Martire B., Carpino L., Trizzino A., Russo G., Cossu F., Badolato R., Pietrogrande M.C., Quinti I., Rossi P., Ugazio A., Pignata C., and Plebani A.(2020). The Italian Registry for Primary Immunodeficiencies (Italian Primary Immunodeficiency Network; IPINet): Twenty Years of Experience (1999-2019). J Clin Immunol. 40:1026-1037. Epub 2020 Aug 15. IF (2018): 4.128

Projects

CLINICAL RESEARCH ACTIVITY

Training in clinical trials

HSR Course on Biosafety (Milan, 2002)

HSR Clinical Experimentation Course (Milan, December 14 and 15, 2004)

Corso AIFA in collaborazione con ISS e CNT per lutilizzo dei medicinali per terapia cellulare (Rome, November 13 and 1,4 2007)

Terapie Innovative Dalla ricerca preclinica ai trial clinici - Terapie avanzate e targeted therapies (Milan, December 12 and 13, 2007)

Gianni Benzi Foundation "II Foresight training course" (Pavia, September 2 to 4, 2009)

AIFA Annual Meeting on Independent Research (Rome, October 27, 2009)

AIFA Annual Meeting on Independent Research (Rome,Ocober 27, 2009)

OSR Course on Clinical Trial Management from the P.I. point of view (Milan, April 27, 2017)

PI of clinical trials

ADA gene transfer into hematopoietic stem/progenitor cells for the treatment of ADA SCID (AD1115611)

A phase I/II clinical trial of hematopoietic stem cell gene therapy for the Wiskott Aldrich Syndrome (201228)

A phase I/II clinical trial of hematopoietic stem cell gene therapy for the treatment of Metachromatic Leukodystrophy (201222)

A single arm, open label, clinical study of cryo preserved autologous CD34+ cells transduced with lentiviral vector containing human ARSA cDNA for the treatment of early onset Metachromatic Leukodystrophy (205756)

A phase I/II study evaluating safety and efficacy of autologous hematopoietic stem cells genetically modified with GLOBE lentiviral vector encoding for the human beta globin gene for the treatment of patients affected by transfusion dependent beta thalassemia (TIGET BTHAL)

A phase I/II study evaluating safety and efficacy of autologous hematopoietic stem and progenitor cells genetically modified with IDUA lentiviral vector encoding for the

humana-L-iduronidase gene for the treatment of patients affected by Mucopolysaccharidosis Type I, Hurler variant (TIGET t10-MPS1)

Methodology study to investigate the utility of retroviral insertion site analysis in samples from subjects treated with Strimvelis gene therapy (205813)

A prospective outcome study on patients with Profound Combined Immunodeficiency (P-CID)

Retrospective-prospective observational study on patient enrolled in AIEOP/IPINET clinical centers

In-depth diagnostic and pathogenic analysis on immunodeficiencies and immune disorders, on both known and unknown genetic basis (TIGET 02)

Diagnostic and pathogenic studies on immunodeficiencies and immune disorders, on both known and unknown genetic basis (TIGET 06)

Biological sample collection for study of blood cells and their microenvironment, and for the development of new therapeutic approaches for genetic diseases and tumors (TIGET 09b)

Neonatal screening on dried peripheral blood spot for combined severe primary immunodeficiencies (TIGET 11b).

RESEARCH INTERESTS

He has explored several issues in the field of Immunology and Hematology:

1989-1992

- Production of recombinant human Factor XII proteins in eukaryotic expression system.
- Immunological and biochemical characterization of recombinant FXII proteins.
- Characterization of monoclonal antibodies against human Factor XII and validation of a competitive immunoenzymatic assay for FXII.

1991-1994

- Study of T-cell and B-cell immune responses to HIV in human subjects.
- Role of superantigens in the pathogenesis of HIV.

1992-1995

- Immunological characterization of transgenic mice for human receptors (CD4, HLA-Cw4, CD26).
- Transgenic mice for human CD4 as an animal model for HIV infection.

1994-1996

- Transplantation of human CD34+ cells into the fetal liver or adult bone marrow of immunodeficient mice (RAG-2).
- Role of stromal factors in the control of the survival, proliferation and differentiation of human hematopoioetic progenitor cells.
- Analysis of the expression of adhesion molecules on the surface of human CD34+ progenitor cells from peripheral blood, cord blood and bone marrow.
- Identification of a stromal derived chemoattractant for lymphocytes and human progenitor cells.

1996-2000

Role of chemokines and their receptor in the trafficking of human hematopoietic progenitors.

2000-2009

Immunological characterization of patients and the study of pathogenesis in various forms of primary immunodeficiencies: ADA-SCID, CVID, Ataxia-telangiectasia, Wiskott-Aldrich syndrome (WAS), SAP deficiency (XLP), perforin deficiency, hyper-IgE syndrome, IPEX. Study of hematopoiesis in patients with HIV infection.

-Transfer Gene in lymphocytes and hematopoietic stem cells.

-Gene therapy of primary immunodeficiencies: adenosine deaminase-deficient SCID, Wiskott-Aldrich syndrome, a defect of SAP.

Current research activities

-Gene transfer into human hematopoietic stem/progenitor cells using retroviral and lentiviral vectors.

-Safety and efficacy of hematopoietic stem cell gene therapy for inherited disorders (ADA, WAS, MLD, MPSI, CGD).

-Study of dynamics and fate of human hematopoietic stem cells after gene therapy.

-Genetic and immunological characterization of primary immunodeficiencies due to unknown genetic defect.

Dr. Aiuti is co-inventor in a UK patent application (priority application, subject to secrecy period)

related to a method for identifying the frequency of hematopoietic cell subtypes in blood (Basso Ricci L. et al. Cytometry 2017).

FUNDING

PI of SR-Tiget Core Grant, project B2: HSPC biology: in vivo clonal tracking and lineage modeling. 2016-2021.

Co-PI of SR-Tiget Core Grant, project E6: Use of mesenchymal stromal cells to optimize transplantation outcome of gene edited hematopoietic stem cells. 2016-2021.

Program Leader of Telethon Core Grant Program A: Clinical Trial Pipeline.

Coordinator and Unit PI of Italian Ministry of Health funded grant NET2011: New strategies for gene therapy of primary immunodeficiencies using regulated lentiviral vectors and gene targeting approaches. 2015-2018.

PI of WP2 of European Community funded grant E-RARE EUROCID: Identification of immunological biomarkers for the diagnosis and prognosis of patients with combined immunodeficiencies. 2015-2018.

Previous Funds

PI of SR-Tiget Core Grant, projects TGT11A01 (Loss f central and peripheral tolerance mechanisms leading to autoimmune manifestations in ADA-deficient SCID9 and TGT11C01 (Tracking and modeling of HSC clonal dynamics by vector marking). 2013-2016

PI of Telethon Foundation grant, SR-TIGET Core Grant. Loss of central and peripheral tolerance mechanisms leading to autoimmune in ADA-deficient SCID (A1). 2011-2015.

PI of Telethon Foundation grant, SR-TIGET Core Grant. Principal Investigator of project Tracking and modeling of HSC clonal dynamics by vector marking (C1). 2011-2015.

PI Telethon Foundation grant Gene Therapy of Wiskott-Aldrich Syndrome. 2011-2015.

Coordinator of grant from Italian Ministry of Health RF-2009-1485896 Combined gene and cell therapy approaches for the treatment of primary immunodeficiencies. 2011-2014

Coordinator of European Community grant FP. Advanced cell-based therapies for the treatment of primary immunodeficiencies (CELL-PID). 2010-2015

Coordinator of European Community E-RARE Genetics and pathogenesis of chronic granulomatous disease and development of new gene transfer therapeutic approaches (EURO-CGD). 2010-2013

PI of Fondazione Roma grant Stem Cells based approaches to monogenic diseases. SR-TIGET 2009-2012

PI of Telethon Foundation grant Clinical trial of gene therapy for ADA-SCID, SR-TIGET 2006-2010

PI of grant from Italian Drug Agency (AIFA). Study of safety and efficacy of gene therapy with autologous CD34+ cells transfected with a retroviral vector encoding ADA gene) (EMEA/OD/053/05) for treatment of ADA-SCID. 2006-2008

PI of Italian Research Association for Cancer (AIRC). Activated upar fragments in cancer. Evaluation of their diagnostic, prognostic and therapeutic protein 2005-2007.

PI of Telethon Foundation grant: Gene Therapy for ADA-Deficient SCID. 2003-2005

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)

Other Relevant Information

REVIEWER AND EVALUATOR ACTIVITY:

Peer reviewer for the following international Journals: Nature, Blood, Human Gene Therapy,

Haematologica, Molecular Therapy, European Journal of Immunology, Frontiers in Primary immunodeficiencies, The Journal of Allergy and Clinical Immunology.
Reviewer for meeting abstracts (ESGCT and ASGCT).
Ad hoc scientific evaluator for the following funding agencies: Israeli Science Foundation, Foundation of Polish Science, French National Research Agency.
PROFESSIONAL ACTIVITIES
Assistant European Editor, Human Gene Therapy Journal. 1998-2003
Member of the Editorial Board, Human Gene Therapy Journal. 2005-2009
Member of the ASGCT Hemopoietic Cell Gene Therapy Committee (2008- 2013)
Member of the Board of the Italian Working Group on Immunodeficiencies (IPINET) (2010-present).
Member of the AIEOP Governing Council (2011-2012).
Board Member of ESGCT (2012 present).
Chair of ASGCT Hematologic and Immunologic Gene and Cell Therapy Committee (2013-05/2016).

Member of the Scientific Evaluation Committee (SEC) for E-Rare Joint Transnational Call European Commission (2013 and 2017).
Member of the Inborn Errors Working Party (IEWP) Studies Committee of the European Society for Bone and Marrow Transplantation (EBMT) (since 2015).
Member of ASGCT Hematologic and Immunologic Gene and Cell Therapy Committee (since 2016).

Co-Chair of the Stem Cell and Gene Therapy WP of European Reference Network (ERN) on Rare Immunodeficiency, Autoinflammatory and Autoimmune Diseases Network (RITA) (since 2017).
2019-present. Editorial Board of the Italian journal Prospettive in Pediatria, co-responsible of the section Frontiers.
Member of ASGCT Global Outreach Committee (since 06/2020)
INSTITUTIONAL COMMITMENTS
Italian Member of the Gene Therapy Working Party of European Medicines Agency (EMA) (2010-2012).
Ad hoc drafting group member of Committte for Advanced Therapies (CAT) EMA (2012-2014).
Member of the Evaluation Committee of Genethon Institute (Evry, France) on behalf of AFM.
Member (representing clinicians) of the Committee for Advanced Therapies (CAT) of the European Medicines Agency (EMA) (as from July 1, 2019).
HONORS
1989: Award from the University of Rome and the Fondazione Sigma Tau for the best research work on basic sciences presented at the "National Research Forum of students from the Faculty of Medicine, University of Rome".
1991: Award from the Istituto Pasteur Fondazione Cenci Bolognetti, University of Rome "La Sapienza", for the best experimental thesis on basic sciences in year
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 Price for Medical Research 2020 for groundbreaking successes in the development of gene therapies.

MENTORSHIP
Prof. Aiuti has directly supervised 12 PhD student at University Rome Tor Vergata and Vita Salute San Raffaele University, and 15 students discussing Thesis (Medical School, Biotechnology or Biology, Nurse School, Resident fellows in Pediatrics). Among the young scientists he has mentored of particular mention are Dr. Francesca Ficara (post-doc at Stanford University and Junior PI at CRN/Humanitas), Dr. Alessandra Mortellaro (PI at the Singapore Immunology Network, and presently Project Leader at SR-Tiget), Dr. Barbara Cassani (researcher, Humanitas Hospital), Dr. Luca Biasco (Assistant Professor, Harvard Medical School).
INVITED LECTURES OF THE LAST 3 YEARS
Jeffrey Modell Foundation Global Summit. 8-12 June 2016. Beverly Hills, California, USA.
2016 MLD Family Conference. 15-16 July 2016. Tarrytown, New York, USA.
SSIEM 2016 Annual Symposium. 6-9 September 2016. Rome, Italy.
ESID 2016 17th Biennial Meeting. 21-24 September 2016. Barcelona, Spain.
ESGCT 2016 24th Congress. 18-21 October 2016. Florence, Italy.
IEWP Autumn meeting. 4-6 November 2016. Gand, Belgium.
2nd Functional Genomics: Nature Via Nurture Symposium.12-14 December 2016. Sidra Medical and Research Center, Doha, Qatar.
Pediatric Medicine into the Future, an Italian Israeli Symposium. 14-15 February 2017.Sheba Medical Center, Tel Aviv, Israel.
XIX Telethon Convention. 13-15 March 2017. Riva del Garda, Italy.
37th Annual Meeting of the French Society of Hematology (Société Française d'Hématologie, SFH). 15-17 March 2017. Paris, France.
43° Annual Meeting of the European Society for Blood and Marrow Transplantation. 26-29 March 2017. Marseille, France.
European Hematology Association (EHA) Paediatric Course. 5-8 April 2017. Sorrento, Italy.
International Pediatric Clinical Trial Day Helping children creating lives they deserve. 9 May 2017. Milan, Italy.
XLII National Congress of AIEOP. 21-23 May 2017. San Giovanni Rotondo, Italy.
IPINET Meeting. 25-26 May 2017. Turin, Italy.
2nd Annual Meeting of the Hellenic Society of Gene Therapy and regenerative medicine. 26-27 May 2017. Athens, Greece.
The future of medicine starts now. How science and new technology are reshaping health science. 29-30 June 2017. Genoa, Italy.
E-Rare Scientific Evaluation Committee Meeting. 6-7 September 2017. Cluj-Napoca, Romania.
Shaping the future of Pediatrics. How technology empowers clinicians, researchers and patients. 20-22 September 2017. Rome, Italy.
Allo-BMT and Natural History in MLD Retrospective analysis. 29th September 2017. Amsterdam, the Netherlands.
MeuSIX meeting. 3rd October 2017. Neaples, Italy.
European Society of Gene and Cell Therapy (ESGCT) 25th Anniversary Congress. 17-20 October 2017. Berlin, Germany.
38th Congress of the Italian Society of Pharmacology. 25-28 October 2017. Rimini, Italy.
Joint IEWP/ADWP Annual Conference. 3-5 November 2017. Newcastle, UK.
International Primary Immunodeficiencies Congress (IPIC). 8-10 November 2017. Dubai, U.A.E.
Giornate AIEOP 2017. 13-14 November 2017. Rimini, Italy.
Al Sissizio dinvero si parla di dolore, 5th Edition. 15-16 December 2017. Trani, Italy.
Worksohp at Ospedale Pediatrico Bambino Gesù in occasion of the Rare Disease Day 2018. 27 February 2018. Rome, Italy.
44th Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT). 18-21

March 2018. Lisbon, Portugal.

Spring School 2018 of the European Society of Gene and Cell Therapy. (ESGCT). 21-23 March 2018. Oxford, UK.

SCIDNET/RECOMB Kick-off meeting. 9-10 April, 2018. London, UK.

Clinical Immunology Society (CIS) Annual Meeting. 26-29 April, 2018. Toronto, Canada.

Symposium North American Guidelines for the management of adenosine deaminase deficiency. 29 April 2018. Toronto, Canada.

Primary Immune Deficiency Treatment Consortium (PIDTC) Annual Scientific Workshop and Educational Day. 8-11 May 2018. Philadelphia, USA.

American Society of Gene and Cell Therapy (ASGCT) 21st Annual Meeting. 16-19 May 2018. Chicago, USA.

AFI 58th Symposium. 6-8 June 2018. Rimini, Italy.

11th PDWP, IDWP, IEWP and Pediatric Nurse Group Meeting. 7-9 June 2018. Verona, Italy.

74th Annual Meeting of the Italian Society of Pediatrics. 12-16 June, 2018. Rome, Italy.

Invited lecture at Morgan Stanley Childrens Hospital of NewYork -PresbyterianColumbia University Medical Center- 27 September 2018

European Society of Gene and Cell Therapy (ESGCT) Annual Meeting. 15-19 October 2018. Lousanne, Switzerland.

ERN Conference. 21-22 November 2018. Bruxelles. Belgium.

TED-conference dedicated to the Russian Science Day. 7-8 February 2019. Moscow. Russia.

Mumbai International PID Conference. 9-11 March 2019. Bombay. India.

45° Annual Meeting of the European Society for Bone and Marrow Transplantation (EBMT). 24-27 March 2019. Frankfurt. Germany.

10th Biannual Meeting of the International Society of Systemic Auto Inflammatory Diseases (ISSAID). 31 March-3 April 2019. Genova. Italy.

Spring School 2019 of the European Society of Gene and Cell Therapy. (ESGCT). 3-5 April 2019. Naples, Italy.

AIEOP-IPINET Meeting, 9-10 May 2019. Bologna, Italy.

XXI Meeting of the Italian Society of Pediatric Allergology and Immunology (SIAIP). 16-18 May 2019. Milan, Italy.

75th Congress of the Italian Society of Pediatrics (SIP). 29 May-1 June 2019. Bologna, Italy.

11TH National Congress of the Italian Conference on AIDS and Antiviral Research. 5-7 June 2019. Milan, Italy.

Immune Deficiency Foundation (IDF) Conference. June 20-22, 2019. National Harbor, MD, USA.

7th Annual German Stem Cell Network (GSCN) Conference. September 23-25, 2019. Berlin, Germany.

27th Annual Congress of the European Society of Gene and Cell Therapy (ESGCT). October 22-25, 2019. Barcelona, Spain.

XX Telethon Scientific Convention. October 28-30, 2019. Riva del Garda, Italy.

1st International Rumi Pediatric Congress (IRUPEC), December 4-7, 2019. Konya, Turkey.

23rd virtual Congress of the American Society of Gene and Cell Therapy (ASGCT), May 12-15, 2020.

XXII virtual meeting of the Italian Society of Pediatric Allergology and Immunology (SIAIP). 23-24 June, 2020.

51st virtual Meeting of the Società Italiana di Neurologia (SIN). November 28-30, 2020.