BIOGRAPHICAL SKETCH

NAME	POSITION TITLE
AIUTI Alessandro	*Full professor* of Pediatrics, "Vita-Salute San Raffaele" University School of Medicine, Milan, Italy *Head of Unit and Coordinator of Clinical Research*, San Raffaele Telethon Institute for Gene Therapy, Milan, Italy *Chief of Clinic,* Pediatric Immunohematology Unit, San Raffaele Hospital, Milan, Italy

EDUCATION/TRAINING

INSTITUTION	DEGREE	YEAR(s)	FIELD OF STUDY
School of Medicine, University of Rome "La Sapienza"	M.D.	1990	Immunology
School of Medicine, University of Rome "La Sapienza"	Ph.D.	1996	Molecular and Cell Biology
School of Medicine, University of Milan	National Board	1998	Hematology

EMPLOYMENT AND EXPERIENCE

1987	Summer student, Molecular Biology Lab. (Head, Dr. R.A. Gatti), Department of Pathology, UCLA School of Medicine, CA, USA
1987-1990	Pre-doctoral student, Laboratory of Medical Genetics, Department of Human Biopathology, School of Medicine, Rome, Italy
1991-1992	Medical Officer, DASRS (Air Force Research and Study Division) Immunology Laboratory, (Head, Prof. R. D'Amelio), Pratica di Mare, Italy
1991-1995	PhD Student, Department of Human Biopathology (Head, Prof. A. Fantoni), School of Medicine, Rome, Italy
1994-1996	Post-doctoral fellow, Lab of Dr. J.C. Gutierrez-Ramos, The Center for Blood Research, Department of Genetics, Harvard Medical School, Boston, USA
1996-1997	Post-doctoral fellow, SR-TIGET (Head, Prof. C. Bordignon), Scientific Institute H.S. Raffaele, Milan, Italy
1997-2000	Research Scientist, Telethon Foundation, Rome, Italy
1997-2003	Group Leader, SR-TIGET, Scientific Institute H.S. Raffaele, Milan, Italy
2000-2007	MD Research Scientist, Scientific Institute H.S. Raffaele, Milan, Italy
2000-2007	Haematologist, Pediatric Clinical Research Unit, SR-TIGET, Scientific Institute H.S. Raffaele, Milan, Italy
2001-2004	Temporary assignment of Professorship, Course of "Gene transfer into human hematopoietic cells", School of Medicine, "Vita-Salute" San Raffaele University, Milan, Italy
2003-2007	Head of Research Unit, SR-TIGET, Scientific Institute H.S. Raffaele, Milan, Italy

2004-2007	Member of the Committee for the Appointment and Promotions, Scientific Institute H.S. Raffaele, Milan, Italy
2004-2010	Temporary assignment of Professorship, Course of "Molecular Pediatrics", School of Medicine, "Vita-Salute" San Raffaele University, Milan, Italy
2007-present	Head of Unit. Pathogenesis and therapy of primary immunodeficiencies, SR-TIGET, Scientific Institute H.S. Raffaele, Milan, Italy
2007-2014	Haematologist, Pediatric Immunohematology Unit, San Raffaele Hospital, Milan, Italy
2007-10/2014	Associate Professor of Pediatrics, University of Roma Tor Vergata, Rome, Italy
2009-present	Coordinator of Clinical Research, SR-TIGET, Scientific Institute H.S. Raffaele, Milan, Italy
2010-2013	Head, Gene Therapy Unit, Department of Pediatrics, University of Rome "Tor Vergata", Bambino Gesù Pediatric Hospital, Rome, Italy
2011-2014	Head, Primary Immunodeficiencies (PID) outpatients' clinic, Department of Pediatrics, University of Rome "Tor Vergata", Bambino Gesù Pediatric Hospital, Rome, Italy
05/2011-present	Head, Clinical Research Unit, SR-TIGET, Scientific Institute H.S. Raffaele, Milan, Italy
11/2014-12/2015	Associate Professor of Pediatrics, "Vita-Salute" San Raffaele University, Milan, Italy
11/2014-present	Director, Pediatric Immunohematology Unit, San Raffaele Hospital, Milan, Italy
01/2016-present	Full Professor of Pediatrics, "Vita-Salute" San Raffaele University, Milan, Italy
04/2018-present	Deputy Director, San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), Milan, Italy

PROFESSIONAL ACTIVITIES

Since 2017	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 2016	Member of the ASGCT Hematologic and Immunologic Gene and Cell Therapy Committee.
Since 2015	Member of the Inborn Errors Working Party (IEWP) Studies Committee of the European Society for Blood and Marrow Transplantation (EBMT).
2013 and 2017	Member of the Scientific Evaluation Committee (SEC) for E-Rare Joint Transnational Call European Commission.
2013-2017	Chair of the ASGCT Hematologic and Immunologic Gene and Cell Therapy Committee
2013	Member of the Evaluation Committee of Genethon Institute (Evry, France) on behalf of AFM.
Since 2012	Board Member of ESGCT.
2012-2014	Ad hoc drafting group member of Committte for Advanced Therapies (CAT) EMA.
2011-2013	Member of the AIEOP Governing Council.
Since 2010	Member of the Board of the Italian Working Group on Immunodeficiencies (IPINET)
2010-2012	Italian Member of the Gene Therapy Working Party of European Medicines Agency (EMA).
2008-2013	Member of the ASGCT Hemopoietic Cell Gene Therapy Committee.

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 1990.

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.

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.

2015 Award from the Centro Studi Marche (CE.S.MA) "Picus del Ver Sacrum" Marchigiani of the year 2014, XXX edition.

PARTICIPATION IN SCIENTIFIC SOCIETIES

- Italian Society of Paediatrics (SIP)
- Italian Society of Paediatric Research (SIRP)
- Italian Society of Paediatric Oncology and Hematology (AIEOP)
- Italian Strategic Committee on Primary Immunodeficiencies (AIEOP-IPINET)
- European Society of Immunodeficiencies (ESID)
- European Society for Bone and Marrow Transplantation (EBMT)
- Inborn Error Working Party (IEWP) of EBMT
- American Society of Hematology (ASH)
- American Society of Gene and Cell Therapy (ASGCT)
- European Society of Gene and Cell Therapy (ESGCT)

CLINICAL RESEARCH ACTIVITY

Training in clinical trials

- HSR Course of Biosafety (Milan, 2002)
- HSR Clinical Experimentation Course (Milan, December 14 and 15, 2004)
- "Corso AIFA in collaborazione con ISS e CNT per l'utilizzo dei medicinali per terapia cellulare" (Rome, November 13 and 14, 2007)
- "Terapie innovative- Dalla ricerca preclinical ai trials 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,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) (pending AIFA approval)
- 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)

SCIENTIFIC ACTIVITIES

Alessandro Aiuti has published more than 160 papers in international scientific peer review journals and national journals, and contributed 7 chapters to books. Quantitative parameters of his scientific production in international journals can be detailed as follows:
h index (Scopus): 44
Total citations (Scopus): 9534
Average citations: 59,59
Total impact factor (IF) of publications: 1316,82
Average impact factor (IF): 8,23
Invited speaker or lecturer to more than 100 National and International Meetings, Workshops in the last 5 years.
Dr. Aiuti started his scientific career as intern student in the University of Rome La Sapienza Medical School, studying recombinant human clotting Factor XII and characterizing monoclonal antibodies against FXII protein. During his PhD at University La Sapienza his studies focused on models to study HIV infection and immune responses to HIV antigens.
Dr. Aiuti spent two years at the Center for Blood Research, Harvard Medical School, Boston, focusing on the role of stromal cells in supporting proliferation, differentiation and migration of human hematopoietic stem/progenitor cell. His work focused on identifying specialized stromal cell with defined characteristics in the bone marrow microenvironment that were able to controlled differentially the fate of human progenitors. His main research achievement was

the identification of a novel chemotactic factor, SDF-1, produced by stromal cells, which attracted human lymphocytes and more potently human CD34+ cells. SDF-1 was the first chemokine identified able to induce migration of human HSPC and the observation that mobilized HSPC cells migrated less efficiently indicates that SDF-1 played a crucial role in HSC homing and mobilization. This discovery led to the subsequent identification by other groups of the development of an inhibitor of CXCR4, which is currently used in the clinics as mobilizing agent.

Dr. Aiuti was recruited in 1996 by Claudio Bordignon at TIGET as post-doctoral fellow. He initially continued the work on SDF-1 and its receptor CXCR4 in the trafficking of human hematopoietic progenitors and its role in HIV infection. He then joined clinical trials of ADA-SCID gene therapy led by Dr. Bordignon showing the key role of PEG-ADA discontinuation in facilitating the expansion of ADA-transduced cells. He then built up his own research team and gained full independence as a group leader at SR-TIGET and clinician in the Pediatric Clinical Research Unit headed by Dr. Roncarolo.

He has contributed with Dr Bordignon and Dr Roncarolo to the successful treatment of ADA-SCID children by HSC gene therapy thanks to the introduction of a reduced intensity and withdrawal of PEG-ADA. The pilot HSC gene therapy study resulted in multilineage and engraftment of gene corrected HSC, immune reconstitution and metabolic correction Aiuti, Science 2002) whereas efficacy and safety of ADA-SCID gene therapy was confirmed long-term and published on the NEJM in 2009.

His other main research area involves lentiviral–mediated gene therapy for Wiskott-Aldrich Syndrome. He participated to the preclinical safety and efficacy studies and set up the gene transfer protocol for human CD34+ cells supervising the work on biodistribution studies in immunodeficient mice. He led all the preparatory activity for regulatory authority approval of the TIGET-WAS trial and acted as PI with MG Roncarolo) of the TIGET-WAS study, which has shown biological activity, safety, efficacy of gene therapy for WAS Aiuti et al., 2013). He participated to the MLD gene therapy clinical trial becoming the PI of the TIGET-MLD study and more recently becoming involved on the MPSI clinical development. He is working to develop a gene therapy strategy for X-CGD using regulated lentiviral vectors in murine models of the disease and humanized immunodeficient mice.

In the past 10 years, Dr. Aiuti and his group have studied the safety of gene therapy by vector integration analyses in vitro and in vivo in patients, using insertion sites to follow the fate and dynamics of transplanted HSC and lymphocytes. Studies at low resolution and at high throughput level allowed to characterize the profile of vector integration and the effect of host cells on vector and vice versa, identifying cell specific features which control retroviral vector insertions in lymphocytes and HSC. More recent studies proved the existence of HSC sharing common integrations sites with a multilineage progeny and provided key information on HSC biology.

Dr. Aiuti has a strong background in immunology and hematology and has been involved since many years in studying the pathogenesis of primary and secondary immunodeficiencies and the correction of disease phenotype after different treatment. Specifically, he has performed studies aimed at assessing the cellular and molecular bases of immunological and/or hematological defects in affected by ADA-SCID, Combined Immunodeficiencies due to different gene defects, CGD, Wiskott-Aldrich Syndrome, Ataxia Teleangectasia, CVID, DiGeorge Syndrome, and has studied immune reconstitution after allogeneic transplantation.

The solid experience in the field of primary immunodeficiencies of Dr. Aiuti is also witnessed by the coordinator role in the European Community funded Grant CELL PID 2010-2015) involving all major European centers in the field of basic studies and innovative therapeutic approaches for PID and an Italian grant Network on PID funded by the Ministry of Health.

PUBLICATIONS

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
30. Guazzi V., Aiuti F., Mezzaroma I., Mazzetta F., Andolfi G., Mortellaro A., Pierdominici M., Fantini R., Marziali M., and Aiuti A. (2002). Assessment of thymic output in common variable immunodeficiency patients by evaluation of T cell receptor excision circles. Clin Exp Immunol 129, 346-353. IF 2.716

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
41. Deola S., Scaramuzza S., Birolo R. S., Carballido-Perrig N., Ficara F., Mocchetti C., Dando J., Carballido J. M., Bordignon C., Roncarolo M. G., Bregni M., and Aiuti A. (2004). Mobilized blood CD34+ cells transduced and selected with a clinically applicable protocol reconstitute lymphopoiesis in SCID-Hu mice. Hum Gene Ther 15, 305-311. IF 4.857
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
43. 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. IF 5.204

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
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