

EUROPEAN
CURRICULUM VITAE
FORMAT



PERSONAL INFORMATION

Name

FERRUA FRANCESCA

Nationality

Italian

WORK EXPERIENCE

- Dates (from – to)
- Name and address of employer
 - Type of business or sector
 - Occupation or position held
- Main activities and responsibilities

August 2014 – August 2016; October 2017 - *present*

Prof. A. Aiuti – San Raffaele Scientific Institute, Milan, Italy

Paediatric Immunohaematology and Bone Marrow Transplantation Unit

Paediatric consultant

Diagnostic work-up, care management and treatment of paediatric patients affected by immunological and/or haematological diseases.

Gene therapy in patients affected by primary immunodeficiencies (ADA-SCID, Wiskott-Aldrich Syndrome - WAS) and lysosomal storage disorders (Metachromatic Leukodystrophy – MLD; Mucopolysaccharidosis type I, MPSI).

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 - Type of business or sector
 - Occupation or position held

October 2018 - *present*

Prof. A. Aiuti – San Raffaele Scientific Institute, Milan, Italy

San Raffaele Telethon Institute for Gene Therapy (SR-Tiget)

Co-investigator in the clinical trial: “A Single Arm, Open Label Clinical Study of Haematopoietic Stem Cell Gene Therapy with Cryopreserved Autologous CD34+ Cells Transduced with Lentiviral Vector encoding WAS cDNA in Subjects with Wiskott-Aldrich Syndrome (WAS)”.

Clinical trial management.

- Main activities and responsibilities

- Dates (from – to)
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 - Type of business or sector
 - Occupation or position held

November 2014 – May 2019

Director of Studies: Dr. A. Villa – “Vita Salute” San Raffaele University, Milan, Italy

San Raffaele Telethon Institute for Gene Therapy (SR-Tiget)

PhD student – Molecular Medicine International PhD Course (Basic and Applied Immunology Curriculum)

PhD student lab research training.

Research Project Title: “Cellular and Molecular Characterization of Thymic Defects in Primary Immunodeficiencies”.

- Main activities and responsibilities

- Dates (from – to)
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 - Type of business or sector
 - Occupation or position held

July 2014 – November 2014

Prof. A. Aiuti – San Raffaele Scientific Institute, Milan

San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET)

Research Fellow

Clinical management and care of patients enrolled in clinical trials. Data collection and analysis.

Preparation of clinical trial reports and supervision of regulatory documentation management.

- Main activities and responsibilities

- Dates (from – to)
- Name and address of employer

March 2014 – May 2014

Supervisor: Dr. A.R. Gennery, Head of Department: Prof. A.J. Cant – Paediatric Immunology, Allergy and Infectious Diseases/Bone Marrow Transplant Unit, Great North Children’s Hospital, Newcastle upon Tyne, UK

Bone Marrow Transplant Unit and Outpatient clinics (*paediatric immunology, paediatric bone marrow transplant follow up, paediatric periodic fevers and paediatric heart transplant follow-up*)

Clinical attachment, ESID Fellow

- Type of business or sector
- Occupation or position held

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 - Occupation or position held
- Diagnostic work-up, care management and treatment of patients with different and complex primary immunodeficiencies.
 Training in Paediatric Bone Marrow Transplantation for primary immunodeficiencies and haematological malignancies.
 Active participation in a European project about HSCT outcome in CD40 Ligand deficiency patients (data collection and analysis, *ongoing*) – Project Leader: A.R. Gennery.
 Oral presentations at Ground Round, journal club and Annual Joint Meeting with Great Ormond Street Hospital transplant team.
- May 2012 - *present*
 Prof. A. Aiuti, Prof. MG Roncarolo – San Raffaele Scientific Institute, Milan, Italy
 San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET)
Co-Investigator in the Clinical Trial: “*A Phase I/II Clinical Trial of Hematopoietic Stem Cell Gene Therapy for the Wiskott-Aldrich Syndrome*”
 Clinical trial management.
- April 2010 – May 2012
 Prof. A. Aiuti, San Raffaele Scientific Institute, Milan, Italy
 San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET)
Data Manager of the Clinical Trial: “*A Phase I/II Clinical Trial of Hematopoietic Stem Cell Gene Therapy for the Wiskott-Aldrich Syndrome*”
 Data record and CRF filing.
- July 2009 – June 2014
 Residency Program in Paediatrics, “Vita-Salute” San Raffaele Medical School and San Raffaele Scientific Institute, Milan, Italy – Director: Prof. M.G. Roncarolo – Prof. G. Weber
 Specialized Paediatric Training – Clinical rotations:
 - Paediatric Immunohematology and Bone Marrow Transplant Unit (*Jul 2009 – Jun 2014*)
 - Paediatric Emergency Department (*Dec 2009 – Jun 2014*)
 - Neonatology and Neonatal Intensive Care Unit (*Mar/Apr 2011; Sept 2012 – Jan 2013*)
 - Paediatric Outpatient clinics (haematology, immunology, allergology, gastroenterology) (*Jul 2009 – Jun 2014*)
 - General Paediatric Department (*Oct 2013 – Jan 2014*).
- Resident Fellow training in Paediatrics**
 Clinical Practice.
 Diagnosis and treatment of childhood and neonatal diseases.
 Diagnosis and treatment of childhood emergencies (day and night shifts under the supervision of a Senior Staff Member in the ED).
 Haematopoietic stem cell transplantation in children affected by haemoglobinopathies and primary immunodeficiencies.
 Gene therapy in patients affected by primary immunodeficiencies (ADA-SCID, Wiskott-Aldrich Syndrome - WAS) and lysosomal storage disorders (Metachromatic Leukodystrophy - MLD).
- July 2009 - May 2012
 Prof. A. Aiuti, San Raffaele Scientific Institute, Milan, Italy
 San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET)
Co-investigator and data manager in the Clinical Trial: “*ADA gene transfer into hematopoietic stem/progenitor cells for the treatment of ADA-SCID*”
 Clinical Trial Management. Data record and CRF filing.
- September 2008 – June 2009
 Prof. M.G. Roncarolo, San Raffaele Scientific Institute, Milan, Italy
 Paediatric Immunohematology outpatient clinic and Bone Marrow Transplant Unit
Medical doctor training
 Haematopoietic stem cell transplantation in children affected by haemoglobinopathies and primary immunodeficiencies.
 Gene therapy in patients affected by primary immunodeficiencies (ADA-SCID).

- Dates (from – to)
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- Main activities and responsibilities

October 2007 – September 2008

Prof. A. Aiuti, San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET), Milan, Italy
Gene Therapy Research Laboratory

Final thesis internship

Lab research practice.

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 - Occupation or position held
- Main activities and responsibilities

August 2007 – September 2007

Supervisor: Dr. P. Fiorina, Lab Chief: Prof. MH Sayegh - Transplantation Research Center – Children’s Hospital – Harvard Medical School, Boston, MA, USA

Transplantation Immunology Laboratory

Observership

Lab research activities (mainly immunological methods) and advanced seminars.

- Dates (from – to)
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 - Occupation or position held
- Main activities and responsibilities

July 2005

Dr. L. Ronzoni, São Rafael Hospital, Salvador de Bahia, Brasil

Emergency Department

Medical student training

Clinical Practice.

- Dates (from – to)
- Name and address of employer
 - Type of business or sector

November 2004 – September 2008

“Vita-Salute” San Raffaele Medical School and San Raffaele Scientific Institute, Milan, Italy

Clinical rotations:

- Paediatric Immunohematology Strategic Research Program (Prof. MG Roncarolo)
- Haematology Division – Bone Marrow Transplantation Unit (Prof. F. Ciceri)
- Internal Medicine Division – Transplantation Unit (Prof. A. Secchi)
- Heart Surgery Division (Prof. O. Alfieri)
- General Surgery Division (Prof. V. Di Carlo)

Medical student training

Clinical Practice.

- Occupation or position held
- Main activities and responsibilities

EDUCATION AND TRAINING

- Dates (from – to) November 2014 – May 2019
- Name and type of organisation providing education and training “Vita-Salute” San Raffaele University and San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), Milan, Italy
- Principal subjects/occupational skills covered Molecular Medicine International PhD Course (Basic and Applied Immunology Curriculum)
- Title of qualification awarded PhD in Molecular Medicine (22nd May 2019)
PhD thesis title: “*Study of thymic epithelial cell dysfunction in primary immunodeficiencies*”
Cum laude
- Level in national classification

- Dates (from – to) July 2009 – June 2014
- Name and type of organisation providing education and training “Vita-Salute” San Raffaele Medical School and San Raffaele Scientific Institute, Milan, Italy – Director: Prof. M.G. Roncarolo – Prof. G. Weber
- Principal subjects/occupational skills covered Paediatric Residency Program
- Title of qualification awarded Paediatrician (14th July 2014)
Final thesis title: “*Lentiviral haematopoietic stem cell gene therapy for children affected by Wiskott-Aldrich Syndrome*”
70/70 cum laude
- Level in national classification

- Dates (from – to) November 2008 - February 2009
- Name and type of organisation providing education and training “Vita-Salute” San Raffaele Medical School and San Raffaele Scientific Institute, Milan, Italy
- Principal subjects/occupational skills covered Internship in General Surgery, Internal Medicine and General Medicine
- Title of qualification awarded Italian Government Licence for Medical Profession and admission to Italian Professional Medical Register (OMCEOCN number 4170)

- Dates (from – to) September 2002 – September 2008
- Name and type of organisation providing education and training “Vita-Salute” San Raffaele Medical School, Milan, Italy
- Principal subjects/occupational skills covered Specialistic Degree Course in Medicine and Surgery
- Title of qualification awarded Medical doctor (17th September 2008)
Final thesis title: “*Study of thymic function reconstitution in ADA-SCID paediatric patients treated with bone marrow transplantation or gene therapy*”.
110/110 cum laude and mention of honour
- Level in national classification

- Dates (from – to) September 1997 – July 2002
- Name and type of organisation providing education and training “G. Ancina” Scientific Secondary School, Fossano (CN), Italy
- Principal subjects/occupational skills covered Physics and Computer Science
- Title of qualification awarded Scientific High School Certificate (2nd July 2002)
- Level in national classification 100/100

**PERSONAL SKILLS
AND COMPETENCES**

*Acquired in the course of life and career
but not necessarily covered by formal
certificates and diplomas.*

MOTHER TONGUE

OTHER LANGUAGES

- Reading skills
- Writing skills
- Verbal skills

SOCIAL SKILLS
AND COMPETENCES

ORGANISATIONAL SKILLS
AND COMPETENCES

TECHNICAL SKILLS
AND COMPETENCES
*With computers, specific kinds of
equipment, machinery, etc.*

OTHER SKILLS
AND COMPETENCES
Competences not mentioned above.

DRIVING LICENCE(S)

ADDITIONAL INFORMATION

ANNEXES

ITALIAN

ENGLISH

Excellent
Excellent
Excellent

Good in teamwork and cooperation, strong communication skills.

Good skills in coordinating groups of work.

Good knowledge of both MAC and Windows operating systems. Very good knowledge of Excel, Word, Power Point, GraphPad Prism and Flow Jo.

- **BLS-D** (Basic Life Support and Defibrillation) Certification (10th June 2010).
- **PBLS-D** (Paediatric Basic Life Support and Defibrillation) Certification (last on 13th April 2022).
- **EPALS** (European Paediatric Advanced Life Support) Certification (last on 22th-23th March 2022).
- **Knowledge of ICH GCP guidelines** and expertise to review and evaluate clinical and experimental data. Management, organization and maintenance of clinical trials' study files and documentation ensuring high levels of accuracy. Expert in CRF fling and clinical trial data analysis. Last GCP course on May 24, 2022.
- **Teaching and tutoring activities** in Pathology, Immunology and Paediatric courses for students of "Vita Salute" San Raffaele Medical School, Milan.
- **Teaching activities** in Paediatric Residency Program of "Vita Salute" San Raffaele Medical School, Milan.

B type

- Member of the European Society for Immunodeficiencies since May 2009
- Member of the Italian Society of Paediatrics (SIP) since July 2014
- **ESID Medium Term Fellowship Award 2013**
- **ASH "G. Bigi" Abstract Achievement Award supported, December 2015**

- I) PUBLICATIONS
- II) MEETING PRESENTATIONS

Milan, 24.01.2024



I authorize the treatment of my personal data according to GDPR 2016/679 and DL 196/2003.

ANNEX I - PUBLICATIONS

1. Asperti C, Canarutto D, Porcellini S, Sanvito F, Cecere F, Vavassori V, Ferrari S, Rovelli E, Albano L, Jacob A, Sergi L, Montaldo E, **Ferrua F**, González-Granado LI, Lougaris V, Badolato R, Finocchi A, Villa A, Radrizzani M, Naldini L. *Scalable GMP-compliant gene correction of CD4+ T cells with IDLV template functionally validated in vitro and in vivo*. Mol Ther Methods Clin Dev. 2023 Aug 23;30:546-557. eCollection 2023 Sep 14. PMID: 37693944. **IF 4.7** (2022)
doi: 10.1016/j.omtm.2023.08.020.
2. Canarutto D, Asperti C, Vavassori V, Porcellini S, Rovelli E, Paulis M, Ferrari S, Varesi A, Fiumara M, Jacob A, Sergi L, Visigalli I, **Ferrua F**, González-Granado LI, Lougaris V, Finocchi A, Villa A, Radrizzani M, Naldini L. *Unbiased assessment of genome integrity and purging of adverse outcomes at the target locus upon editing of CD4+ T-cells for the treatment of Hyper IgM1*. EMBO J. 2023 Dec 1;42(23):e114188. Epub 2023 Nov 2. PMID: 37916874. **IF 11.4** (2022)
doi: 10.15252/embj.2023114188.
3. Canarutto D, Oltolini C, Barzaghi F, Calbi V, Migliavacca M, Tucci F, Gallo V, Consiglieri G, **Ferrua F**, Recupero S, Cervi MC, Al-Mousa H, Pituch-Noworolska A, Tassan Din C, Scarpellini P, Silvani P, Fossati C, Casiraghi M, Cirillo DM, Castagna A, Bernardo ME, Aiuti A, Maria Pia Cicalese MP. *Outcome of BCG Vaccination in ADA-SCID Patients: A 12-Patient Series*. Biomedicines. 2023 Jun 24;11(7):1809. PMID: 37509449. **IF 4.7** (2022)
doi: 10.3390/biomedicines11071809.
4. Fratini ES, Migliavacca M, Barzaghi F, Fossati C, Giannelli S, Monti I, Casiraghi M, **Ferrua F**, Recupero S, Consiglieri G, Calbi V, Tucci F, Gallo V, Bernardo ME, Cenciarelli S, Palmoni M, Moni M, Galimberti L, Duse M, Leonardi L, Sieni E, Soncini E, Porta F, Notarangelo LD, De Santis R, Ladogana S, Aiuti A, Cicalese MP. *Hemophagocytic inflammatory syndrome in ADA-SCID: report of two cases and literature review*. Front Immunol. 2023 Jun 26;14:1187959. **IF 7.3** (2022)
doi: 10.3389/fimmu.2023.1187959. eCollection 2023.
5. 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, Frascetta F, Darin S, Albertazzi E, Galimberti S, Montini E, Calabria A, Cicalese MP, Aiuti A. *Hematopoietic reconstitution dynamics of mobilized- and bone marrow-derived human hematopoietic stem cells after gene therapy*. Nat Commun. 2023 May 27;14(1):3068. **IF 16.6** (2022)
doi: 10.1038/s41467-023-38448-y.
6. Marcello AP, Visconti C, Vercellati C, Zaninoni A, Bianchi P, Barcellini W, Aiuti A, Fermo E, **Ferrua F**. *Overhydrated hereditary stomatocytosis: A rare cause of familial persistent macrocytosis due to SLC4A1 variants*. Pediatr Blood Cancer. 2023 Apr 14:e30344. Online ahead of print. PMID: 37057369. **IF 3.2** (2022)
doi: 10.1002/pbc.30344. Online ahead of print.
7. Barzaghi F, Cicalese MP, Zoccolillo M, Brigida I, Barcella M, Merelli I, Sartirana C, Zanussi M, Calbi V, Bernardo ME, Tucci F, Migliavacca M, Giglio F, Doglio M, Canarutto D, **Ferrua F**, Consiglieri G, Prunotto G, Saettini F, Bonanomi S, Rovere-Querini P, Di Colo G, Jofra T, Foustieri G, Penco F, Gattorno M, Hershfield MS, Bongiovanni L, Ponzoni M, Markt S, Milani R, Peccatori J, Ciceri F, Mortellaro A, Aiuti A. *Case Report: Consistent disease manifestations with a staggered time course in two identical twins affected by adenosine deaminase 2 deficiency*. Front Immunol. 2022 Sep 29;13:910021. eCollection 2022. **IF 7.3** (2022)
doi: 10.3389/fimmu.2022.910021.
8. Cattaneo A, Liguori M, Trombetta E, Ceriotti F, Pagni L, Ronchi A, Carracchia G, Notarangelo LD, **Ferrua F**, Barzaghi F, Giovanettoni C, Zuccotti G, Cirillo E, Pignata C, Meroni F, Maietta A, Farruggia P, Porretti L. *Neonatal alloimmune neutropenia: diagnosis and management of 31 Italian patients*. Blood Transfus. 2023 May;21(3):227-234. Epub 2022 Aug 5. PMID: 35969137. **IF 3.7** (2022)
doi: 10.2450/2022.0088-22
9. Migliavacca M, Basso Ricci L, Farinelli G, Calbi V, Tucci F, Barzaghi F, **Ferrua F**, Cicalese MP, Darin S, Barzaghi LR, Giglio F, Peccatori J, Fumagalli F, Nicoletti R, Giannelli S, Sartirana C, Bandiera A, Esposito M, Milani R, Mazzi B, Finocchi A, Markt S, Assanelli A, Locatelli F, Ciceri F, Aiuti A, Bernardo ME. *A Novel Assay in Whole Blood Demonstrates Restoration of Mitochondrial Activity in Phagocytes After Successful HSCT in Hyperinflamed X-Linked Chronic Granulomatous Disease*. J Clin Immunol. 2022 Nov;42(8):1742-1747. Epub 2022 Aug 10. **IF 9.1** (2022)
doi: 10.1007/s10875-022-01338-x.
10. Milardi G, Di Lorenzo B, Gerosa J, Barzaghi F, Di Matteo G, Omrani M, Jofra T, Merelli I, Barcella M, Filippini M, Conti A, **Ferrua F**, Pozzo Giuffrida F, Dionisio F, Rovere-Querini P, Markt S, Assanelli A, Piemontese S, Brigida I, Zoccolillo M, Cirillo E, Giardino G, Danieli MG, Specchia F, Pacillo L, Di Cesare S, Giancotta C, Romano F, Matarese A, Chetta AA, Trimarchi M, Laurenzi A, De Pellegrin M, Darin S, Montin D, Marinoni M, Dellepiane RM, Sordi V, Lougaris V, Vacca A, Melzi R, Nano R, Azzari C, Bongiovanni L, Pignata C, Cancrini C, Plebani A, Piemonti L, Petrovas C, Di Micco R, Ponzoni M, Aiuti A, Cicalese MP, Foustieri G. *Follicular helper T cell signature of replicative exhaustion, apoptosis, and senescence in common variable immunodeficiency*. Eur J Immunol. 2022 May 13. Online ahead of print. **IF 5.4** (2022)

doi: 10.1002/eji.202149480.

11. Lucchese A, Cenciarelli S, Manuelli M, Marcolina M, Barzaghi F, Calbi V, Migliavacca M, Bernardo ME, Tucci F, Gallo V, Frascchetta F, Darin S, Casiraghi M, Aiuti A, **Ferrua F**, Cicalese MP. *Wiskott-Aldrich syndrome: Oral findings and microbiota in children and review of the literature*. Clin Exp Dent Res. 2022 Feb;8(1):28-36. **IF 1.8** (2022)
doi: 10.1002/cre2.503.
12. Cicalese MP, **Ferrua F**, Barzaghi F, Cerri F, Moro M, Aiuti A, Silvani P. *Third cranial nerve palsy in an 88-year-old man after SARS-CoV-2 mRNA vaccination: change of injection site and type of vaccine resulted in an uneventful second dose with humoral immune response*. BMJ Case Rep. 2022 Feb 8;15(2):e246485. **IF 0.9** (2022)
doi: 10.1136/bcr-2021-246485.
13. Fumagalli F, Calbi V, Natali Sora MG, Sessa M, Baldoli C, Rancoita PMV, Ciotti F, Sarzana M, Fraschini M, Zambon AA, 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 ES, Morena F, Silvani P, Calvi MR, Facchini M, Locatelli S, Corti A, Zancan S, Antonioli G, Farinelli G, Gabaldo M, Garcia-Segovia J, Schwab LC, Downey GF, Filippi M, Cicalese MP, Martino S, Di Serio C, Ciceri F, Bernardo ME, Naldini L, Biffi A, Aiuti A. *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. 2022 Jan 22;399(10322):372-383. **IF 168.9** (2022)
doi: 10.1016/S0140-6736(21)02017-1.
14. 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 MP, Calbi V, Migliavacca M, Barzaghi F, **Ferrua F**, Gallo V, Miglietta S, Zonari E, Cheruku PS, Forni C, Facchini M, Corti A, Gabaldo M, Zancan S, Gasperini S, Rovelli A, Boelens JJ, Jones SA, Wynn R, Baldoli C, Montini E, Gregori S, Ciceri F, Valsecchi MG, la Marca G, Parini R, Naldini L, Aiuti A, Bernardo ME; MPSI Study Group. *Hematopoietic Stem- and Progenitor-Cell Gene Therapy for Hurler Syndrome*. N Engl J Med. 2021 Nov 18;385(21):1929-1940. **IF 176.079** (2021)
doi: 10.1056/NEJMoa2106596.
15. Consiglieri G, **Ferrua F**; San Raffaele Hospital Consortium, Aiuti A, Cicalese MP. *A Case of Two Adult Brothers with Wiskott-Aldrich Syndrome, One Treated with Gene Therapy and One with HLA-Identical Hematopoietic Stem Cell Transplantation*. J Clin Immunol. 2022 Feb;42(2):421-425. **IF 9.1** (2022)
doi: 10.1007/s10875-021-01157-6. Epub 2021 Nov 4.
16. Lankester AC, Neven B, Mahlaoui N, von Asmuth EGJ, Courteille V, Alligon M, Albert MH, Serra IB, Bader P, Balashov D, Beier R, Bertrand Y, Blanche S, Bordon V, Bredius RG, Cant A, Cavazzana M, Diaz-de-Heredia C, Dogu F, Ehlert K, Entz-Werle N, Fasth A, **Ferrua F**, Ferster A, Formankova R, Friedrich W, Gonzalez-Vicent M, Gozdzik J, Gungör T, Hoening M, Ikinogullari A, Kalwak K, Kansoy S, Kupesiz A, Lanfranchi A, Lindemans CA, Meisel R, Michel G, Miranda NAA, Moraleda J, Moshous D, Pichler H, Rao K, Sedlacek P, Slatter M, Soncini E, Speckmann C, Sundin M, Toren A, Vetterranta K, Worth A, Yeşilipek MA, Zecca M, Porta F, Schulz A, Veys P, Fischer A, Gennery AR. *Hematopoietic cell transplantation in severe combined immunodeficiency: The SCETIDE 2006-2014 European cohort*. J Allergy Clin Immunol. 2021 Oct 27:S0091-6749(21)01629-8. **IF 14.19** (2021)
doi: 10.1016/j.jaci.2021.10.017. Online ahead of print.
17. Canarutto D, Tucci F, Gattillo S, Zambelli M, Calbi V, Gentner B, **Ferrua F**, Markt S, Migliavacca M, Barzaghi F, Consiglieri G, Gallo V, Fumagalli F, Massariello P, Parisi C, Viarengo G, Albertazzi E, Silvani P, Milani R, Santoleri L, Ciceri F, Cicalese MP, Bernardo ME, Aiuti A. *Peripheral blood stem and progenitor cell collection in pediatric candidates for ex vivo gene therapy: a 10-year series*. Mol Ther Methods Clin Dev. 2021 Jun 1;22:76-83. **IF 5.849** (2021)
doi: 10.1016/j.omtm.2021.05.013. eCollection 2021 Sep 10.
18. **Ferrua F**, Bortolomai I, Fontana E, Di Silvestre D, Rigoni R, Marcovecchio GE, Draghici E, Brambilla F, Castiello MC, Delfanti G, Moshous D, Picard C, Tagnon T, Bordon V, Schulz AS, Schuetz C, Giliani S, Soresina A, Gennery AR, Signa S, Dávila Saldaña BJ, Delmonte OM, Notarangelo LD, Roifman CM, Poliani PL, Uva P, Mauri PL, Villa A, Bosticardo M. *Thymic Epithelial Cell Alterations and Defective Thymopoiesis Lead to Central and Peripheral Tolerance Perturbation in MHCII Deficiency*. Front Immunol. 2021 Jun 15;12:669943. **IF 8.787** (2021)
doi: 10.3389/fimmu.2021.669943. eCollection 2021.
19. Marcovecchio GE*, **Ferrua F***, Fontana E, Beretta S, Genua M, Bortolomai I, Conti A, Montin D, Cascarano MT, Bergante S, D'Oria V, Giamberti A, Amodio D, Cancrini C, Carotti A, Di Micco R, Merelli I, Bosticardo M, Villa A. *Premature Senescence and Increased Oxidative Stress in the Thymus of Down Syndrome Patients*. Front Immunol. 2021 Jun 1;12:669893. **IF 8.787** (2021)
doi: 10.3389/fimmu.2021.669893. eCollection 2021.
20. Vavassori V, Mercuri E, Marcovecchio GE, Castiello MC, Schirotti G, Albano L, Margulies C, Buquicchio F, Fontana E, Beretta S, Merelli I, Cappelleri A, Rancoita PM, Lougaris V, Plebani A, Kanariou M, Lankester A, **Ferrua F**, Scanziani E, Cotta-Ramusino C, Villa A, Naldini L, Genovese P. *Modeling, optimization, and comparable efficacy of T cell and hematopoietic stem cell gene editing for treating hyper-IgM syndrome*. EMBO Mol Med. 2021 Mar 5;13(3):e13545. **IF 14.26** (2021)
doi: 10.15252/emmm.202013545. Epub 2021 Jan 21.

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* *Equal contribution*

Quantitative parameters of the scientific production in international journals:

- ***h* index (Scopus): 23**
- **Citations number: 2517 (*Scopus*)**
- **Average citations number: 45.0**
- **Total Impact Factor of publications: 833.89**
- **Average Impact Factor: 14.891**

Source of IF data: <https://jcr.clarivate-com.sanraffaele.idm.oclc.org/jcr/home>

ANNEX II – MEETING PRESENTATIONS

- Oral presentation at the 2022 Meeting of the European Society for Immunodeficiencies (ESID) – 12th – 15th October 2022, Gothenburg, Sweden. Title: *Lentiviral Hematopoietic Stem and Progenitor cell gene therapy for Wiskott-Aldrich Syndrome: safety and clinical benefit in 23 patients up to 10.5 years of follow-up.*
- Oral presentation at the 29th Annual Meeting of the European Society of Gene and Cell Therapy (ESGCT) – 11th October 2022, Edinburgh, UK. Title: *Safety and clinical benefit of lentiviral haematopoietic stem and progenitor cell gene therapy in 23 patients with Wiskott-Aldrich Syndrome with up to 10.5 years of follow-up.*
- Oral presentation at the International Consortium of Personalized Medicine Conference (ICPerMED) – 5th – 6th October 2022, Paris, France. Title: *Hematopoietic stem cell gene therapy for the treatment of rare genetic diseases.*
- Oral presentation at the 3rd International Symposium for researchers and clinicians on Wiskott-Aldrich Syndrome (WAS2022) – 17th June 2022, Munich, Germany. Title: *Hematopoietic Stem and Progenitor Cell Lentiviral Gene Therapy for Wiskott-Aldrich Syndrome: Up to 10.5 Years of Follow-Up in 17 Subjects.*
- Oral presentation at the 25th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) – 16th – 19th May, 2022, Washington D.C., USA. Title: *Up to 10.5 Years of Follow-Up in 17 Subjects Treated with Hematopoietic Stem and Progenitor Cell Lentiviral Gene Therapy for Wiskott-Aldrich Syndrome.*
- Oral presentation at the 2017 Meeting of the European Society for Immunodeficiencies (ESID) – 11th – 14th September 2017, Edinburgh, UK. Title: *Cellular and molecular characterization of defective thymopoiesis in MHC class II deficiency*
- Oral presentation at the 34th API Meeting – 25th – 27th May 2017, Kloster Banz, Germany. Title: *From basic research to gene therapy of immunologic disorders.*
- Oral presentation at the 10th Meeting of the EBMT Paediatric Diseases Working Party – 26th – 29th May 2016, Island of Rhodes, Greece. Title: *Gene Therapy for ADA-SCID: Long Term Results.*
- Oral presentation at the 57th ASH Annual Meeting – 5th – 8th December 2015, Orlando (FL), USA. Title: *Safety and Clinical Benefit of Lentiviral Hematopoietic Stem Cell Gene Therapy for Wiskott-Aldrich Syndrome.*
- Oral presentation at the EBMT Inborn Errors Working Party (IEWP) Meeting – 16th – 18th October 2015, Seville, Spain. Title: *Update on TIGET-WAS Clinical Trial.*
- Oral presentation at the 41st Annual EBMT Meeting – 22nd – 25th March 2015, Istanbul, Turkey. Title: *Haematopoietic stem cell transplantation for CD40 ligand deficiency: results from an EBMT Inborn Errors Working Party (IEWP) study.*
- Oral presentation at the EBMT Inborn Errors Working Party (IEWP) Meeting – 17th – 19th October 2014, Munich, Germany. Title: *Haematopoietic Stem Cell Transplantation for CD40 Ligand Deficiency: Preliminary Results From An EBMT/ESID Inborn Error Working Party Study.*
- Oral presentation at the National Commissioning Group Audit Meeting – Bone Marrow Transplant Units, Great North Children's Hospital, Newcastle upon Tyne, and Great Ormond Street Hospital, London – 4th – 5th June 2014, Newcastle upon Tyne, UK. Title: *Haematopoietic stem cell transplantation for CD40 ligand deficiency: preliminary results from an EBMT/ESID Inborn Error Working Party study.*
- Oral presentation at the CELL-PID & PERSIST Annual Meeting - 21st February 2013, Stresa, Italy. Title: *Update on the TIGET-WAS Clinical Trial of Haematopoietic Stem Cell Gene Therapy for the Wiskott-Aldrich Syndrome.*
- Oral presentation at the EBMT Inborn Errors Working Party (IEWP) Meeting - 2nd – 4th November 2012, Barcelona, Spain. Title: *Gene Therapy for Wiskott-Aldrich Syndrome.*
- Oral presentation of a clinical case at ESID Junior Summer School (Tutor: Prof. AJ Cant) – 28th September – 2nd October 2011, Il Ciocco, Barga, Italy.
- Oral presentation at the 6th International Cystinosis Conference – 24th- 26th September 2010, Lignano Sabbiadoro (UD), Italy. Title: *Gene Therapy for Inherited Metabolic Disorders: ADA SCID and beyond.*
- Oral presentation of a clinical case at ESID Junior Workshop (T cell deficiencies section – AJ Cant) – 11th – 12th January 2010, Florence, Italy.
- Oral presentation at the Educational Day of the XIIIth Meeting of the European Society for Immunodeficiencies – 16th October 2008, 's-Hertogenbosch, the Netherlands. Title: *Reconstitution of thymic function after gene therapy for ADA-SCID: preliminary observations by imaging and immunological studies.*