

Luca Guzzardi

Curriculum Vitae

PERSONAL AND CONTACT INFORMATION

CURRENT POSITION:	Since Jan. 2019	Senior Assistant Professor of Logic and Philosophy of Science at the University of Milan, Department of Philosophy.
Research areas & topics:		Integrated History and Philosophy of Science. <i>Ongoing research:</i> (i) The natural philosophy of Ruggiero G. Boscovich. (ii) Epistemic constraints in scientific discourses. (iii) Collaborative scientific practices (iv) Patterns of intersection between philosophy and history of science.
CAREER:		
	2015-2018	Junior Assistant Professor of Logic and Philosophy of Science at the University of Milan, Department of Philosophy.
	2012-2014	Post-Doc in History of Science (Department of Physics, University of Pavia) & Associate Researcher of INAF (Istituto Nazionale di Astrofisica), Brera Observatory Milan.
	Dec 2013	National Scientific Qualification for the function of an associate professor (11/C2).
	2007-2012	Scientific Collaborator and editor of Edizione Nazionale delle Opere e della Corrispondenza Ruggiero G. Boscovich (R. Boscovich's Opera Omnia). Co-editor of the Web Edition (Biblioteca Digitale). From 2009: Associate Researcher of INAF, Brera Observatory Milan.
	2005-2006	Research contract – INAF-Osservatorio Astronomico di Brera. Project: The correspondence of Ruggiero G. Boscovich (sponsored by Fondazione CARIPLO). Head of the project: Dr. Elio Antonello.
	2003-2004	DAAD Post-Doc, Universität der Bundeswehr München / Forschungsinstitut des Deutschen Museum München. (Advisor: Prof. Dr. Ivo Schneider.)
EDUCATION:		
	Jan 2004	PhD in Philosophy. Dissertation title: <i>Descrittività e fenomenismo</i> . Università degli Studi di Milano. Starting tutor: Prof. Dr. Francesco Moisot. Final tutor: Prof. Dr. Giovanni Micheli.
	2000	Master Degree in Philosophy. Thesis title: <i>La filosofia della scienza di Wilhelm Ostwald</i> . Tutor: Prof. Dr. Francesco Moisot. Università degli Studi di Milano. Thesis Mark: 110/110 cum laude.

Foreign languages: Good spoken and written English and German.

GRANTS & FELLOWSHIPS:	May 2020	PI of the Inter-departmental Project “SEED” of the University of Milan, “Re-assessing Scientific Collaboration” (15.000 €).
	Oct.-Dec. 2018	DAAD Re-invitation Programme for Former Scholarship Holders, 2018 – Berlin-Brandenburg Academy of Sciences and Humanities. Host: Prof. Dr. Eberhard Knobloch.
	2017	Individual funding grant for basic activity in research (FFABR).
	2011	Co-funded grant “Archivi storici astronomici lombardi”, sponsored by Regione Lombardia and Istituto Nazionale di Astrofisica at INAF-Osservatorio Astronomico di Brera (6.000 €) [Ref.: Dr. Giovanni Pareschi, past director OA Brera].
	May-July 2008	Alumni-DAAD Research Scholarship «Wiedereinladung» – Ludwig Maximilian Universität / Forschungsinstitut des Deutschen Museums München. Host: Prof. Dr. Ivo Schneider.
	Oct 2003 – July 2004	DAAD Research Scholarship.

TEACHING:

University of Milan	2019-2020	Philosophy of Science (B.A. in Philosophy), 60h regular course. History and Philosophy of the Sciences (M.A. in Biodiversity and Evolutionary Biology), 48h regular course. Ethical, Legal and Social Issues of Science (Post-graduate School for Medical Genetics), 8h regular course in medical specialty school. Methodology of natural sciences (Post-graduate School for Medical Statistics at Biometry), 8h regular course in medical specialty school
	2018-2019	Philosophy of Science (B.A. in Philosophy), 60h regular course. Ethical, Legal and Social Issues of Science (Post-graduate School for Medical Genetics), 8h regular course in medical specialty school. Methodology of natural sciences (Post-graduate School for Medical Statistics at Biometry), 8h regular course in medical specialty school
	2017-2018	Philosophy of Science (B.A. in Philosophy), 60h regular course. History and Philosophy of the Sciences (M.A. in Biodiversity and Evolutionary Biology), 48h regular course. Ethical, Legal and Social Issues of Science (Post-graduate School for Medical Genetics), 8h regular course in medical specialty school.
	2016-2017	Philosophy of Science (B.A. in Philosophy), 60h regular course.

History and Philosophy of the Sciences (M.A. in Biodiversity and Evolutionary Biology), 48h regular course.

Ethical, Legal and Social Issues of Science (Post-graduate School for Medical Genetics), 8h regular course in medical specialty school.

2015-2016 Philosophy of Science (B.A. in Philosophy), 60h regular course.

Course for PhD students (with Dr Tzuchien Tho): "The Philosophy of Motion", 10h special course in the Doctoral School in Philosophy and Human Sciences.

Ethical, Legal and Social Issues of Science (Post-graduate School for Medical Genetics), 8h regular course in medical specialty school.

ORGANIZING:	Sept 2020	Member of the International Committee for the Centennial of the Birth of Paul K. Feyerabend.
	2018	Advisory Committee of the 38th National Congress of the Italian Society for the History of Physics and Astronomy 2018, Messina.
	Since 2016	University of Milan: Seminar of Historical Epistemology.

SELECT CONFERENCES AND SEMINARS (SINCE 2015)

2019 Sept 20: *A Philosophy of Heap. On Mach's Historical Epistemology*. Open Epistemologies. Mach, Bachelard, Feyerabend, FCUL, Lisbon, 20-21 September 2019.

2018 Sept. 14-17: *Boscovich and the Newtonians: Converging Divergences*. 8th Biennial Conference of the European Society for the History of Science 2018, London: UCL Institute of Education, the Royal Institution and the Science Museum.

2018 May 26: *Does Philosophy of Science Meet History of Science? Epistemic Constraints*. 4èmes Journées d'études sur l'Épistémologie Historique: "L'épistémologie historique et les désunités des sciences / Historical Epistemology and the Disunities of the Sciences", Paris, 24-25-26 mai 2018.

2017 December 6: *Il concetto di vincolo epistemico come strumento teorico per l'epistemologia storica*. University of Urbino, Department of Pure and Applied Sciences.

2017 June 5: *Jesuit Physico-Mathesis after Clavius: The Shaping of a Mathematical Style*. Cohn Institute for the History and Philosophy of Science and Ideas, Tel Aviv University.

2016 September 19: *God only knows. Ruggiero Boscovich, the temptation of forces and their rejection*. Conferências HoST, Centro Interuniversitário de História das Ciências e da Tecnologia, Universidade de Lisboa.

2016 July 28: *Ruggiero Boscovich at the intersection of traditions. Newtonianism, Jesuit Science, and... Max-Planck-Institut für Wissenschaftsgeschichte Berlin*.

2015 November 26-27: *The project of the Ruggiero Boscovich National Edition in digital perspective*. Workshop "Jesuit science from a digital perspective" (Universität Wuppertal).

VISITING POSITIONS

2014 February-May: Max-Planck-Institut für Wissenschaftsgeschichte, Berlin (group “Modern Geometry and the Concept of Space”, chair Vincenzo de Risi).

2016 September: Centro Interuniversitário de História das Ciências e da Tecnologia, Universidade de Lisboa.

2018 January-February: Berlin-Brandenburgische Akademie der Wissenschaften, Berlin (Leibniz-Edition Arbeitsstelle).

Luca Guzzardi

Publications

Update: February 2021

1

PHD THESIS:

Descrittività e fenomenismo. Declinazioni dell'oggettività. Tesi di dottorato di ricerca in filosofia. Università degli Studi di Milano, Milano 2004, pp. 1-218.

BOOKS:

1. **Guzzardi, L.** (2020): *Ruggiero Boscovich's Theory of Natural Philosophy. Points, Distances, Determinations.* Basel et al.: Birkhäuser. ISBN 9783030520922.
2. **Guzzardi, L.** (2010): *Lo sguardo muto delle cose. Oggettività e scienza nell'età della crisi.* Milano: Raffaello Cortina. ISBN: 8860303257.

Critical editions and edited books (with notes, apparatus, etc.):

1. R.G. Boscovich (2017): *Theoria philosophiae naturalis 1758-1763.* Ed. **Guzzardi, L.** Introduzione di L. Guzzardi. Edizione Nazionale delle Opere e della Corrispondenza di R.G. Boscovich, Opere, vol. VII. Roma-Milano. ISBN: 9788896700259.
2. R.G. Boscovich (2016): *Opere precedenti la Theoria.* Eds. Bevilacqua, F., **Guzzardi, L.** Introduzione di L. Guzzardi. Edizione Nazionale delle Opere e della Corrispondenza di R.G. Boscovich, Opere, vol. VI. Roma-Milano. ISBN: 9788896700198.
3. R.G. Boscovich (2013): *Les Éclipses, poème en six chants.* Ed. **Guzzardi, L.** Edizione Nazionale delle Opere e della Corrispondenza di R.G. Boscovich, Opere, vol. XIII/2. Roma-Milano. ISBN: 9788896700105.
4. E. Mach (2005): *Scienza tra storia e critica.* Ed. **Guzzardi L.**, [Scientific-historical introduction, translation and scientific footnotes by L. Guzzardi]. Monza: Polimetrica.

PEER-REVIEWED ARTICLES AND BOOK CHAPTERS:

1. **Guzzardi, L.** (2021). "Imaging a black hole through the Event Horizon Telescope. A case study in the philosophy of scientific collaboration." Submitted (Sept. 2020) to *European Journal for the Philosophy of Science*.
2. **Guzzardi, L.** (2021). "Holding the Hand of History: Ernst Mach on the History of Science, the Analysis of Sensations, and the Economy of Thought". In: *Interpreting Mach: Critical Essays*, ed. by J.M. Preston, Cambridge: Cambridge University Press, *Forthcoming* (May 2021).
3. **Guzzardi, L.** (2018): "The logic that governs each step of scientific research". *Isis*, 109(1): 105-108. <https://doi.org/10.1086/697110>.
4. **Guzzardi, L.** (2017): "Ruggiero Boscovich and 'the Forces Existing in Nature'". *Science in Context*, 30(4): 385-422. doi: 10.1017/S0269889717000266.

5. **Guzzardi, L.**, Tagliagambe, Silvano (2017): "Classical physics as a metaphorical tool for evoking quantum world". In: *Metaphor in Communication, Science and Education* (Applications of Cognitive Linguistics, 36), ed. by F. Ervas, E. Gola, M.G. Rossi, Berlin: De Gruyter, 171-187. ISBN: 9783110549928. ISSN: 1861-4078. doi: 10.1515/ 9783110549928-010.
6. **Guzzardi, L.** (2015): "Sharing Discoveries. Boscovich's Network and the Discovery of Uranus". *Archives Internationales d'Histoire des Sciences*, 64: 429-447. doi: 10.1484/ J.ARIHS.5.110297.
7. **Guzzardi, L.** (2015): "Personaggi. Ruggiero Giuseppe Boscovich". In: *Almum Studium Papiense. Storia dell'Università di Pavia*. Vol. II. Ed. D. Mantovani (Milano: Monduzzi Editoriale Cisalpino), 349-352.
8. **Guzzardi, L.** (2014): "Energy, Metaphysics, and Space. Ernst Mach's Interpretation of Energy Conservation as the Principle of Causality". *Science and Education*, 23(6): 1269-1291 (Special Issue *Energy Conservation: History, Philosophy and Education*). DOI: 10.1007/ s11191-012-9542-9.
9. **Guzzardi, L.** (2013): "Robert Musil, i numeri reali e la matematica". *Intersezioni. Rivista di Storia delle Idee*, XXXIII/ 3, 395-410.
10. Graziani, P., **Guzzardi, L.**, Sangoi, M. (Eds.) (2013): *Open Problems in Philosophy of Sciences*. SILFS 2. London. College Publication. VII + 258 pp. ISBN: 9781848900622.
11. **Guzzardi, L.** (2010): "Some Remarks on a Heuristic Point of View about the Role of Experiment in the Physical Sciences". In *New Essays in Logic and Philosophy of Science*, Eds. D'Agostino, M., Giorello, G., Laudisa, F., Pievani, T., Sinigaglia, C. (London: College Publications), 698-708.
12. **Guzzardi, L.** (2005): "Masse, moti nascosti, etere e spazio nella meccanica hertziana". *Physis. Rivista internazionale di storia della scienza*, XLII/ 2, 379-416.
13. **Guzzardi, L.** (2005): "Il Kantismo anomalo di Heinrich Hertz". *Studi Kantiani*, XVIII, 117-131.
14. **Guzzardi, L.** (2005): "Lettere di Aurelio Pelazza a Piero Martinetti e a Ernst Mach". *Rivista di Storia della Filosofia*, LX, 1, 109-125.
15. **Guzzardi, L.** (2004): "Conoscenza e corporeità". *Epistemologia*, XXVII, 2, 179-210.
16. **Guzzardi, L.** (2002): "Teorie stravaganti di un redattore invadente. Eugenio Rignano, un positivista italiano nel dibattito scientifico europeo". *Intersezioni. Rivista di Storia delle Idee*, XXII/ 3, 419-439.
17. **Guzzardi, L.** (2000): "Trasformazioni e inneschi. Occasionalismo nel XIX secolo". *Rivista di Estetica*, XLI, n.s., 18/ 3, 142-155.

OTHER ARTICLES, BOOK CHAPTERS, AND EDITED BOOKS:

1. Giammarchi, M., **L. Guzzardi** (2021): "A unified vision of Everything". In: *Isonomia — Epistemologica* (Special issue: "MQ90. Dualismo, Entanglement, Olismo —Un dibattito ancora aperto"), XI, 51-58.
2. **Guzzardi, L.** (2018): Prefazioni e schede storico-filosofiche in: James S. Walker, *Dialogo con la Fisica*, 3 voll. Torino: Pearson Italia.
3. **Guzzardi, L., ed.** (2017): *Hertz, La scoperta delle onde elettromagnetiche*. Milano: RCS/ Corriere della Sera.
4. **Guzzardi, L., ed.** (2015): *Il pensiero acentrico*. Milano: elèuthera [ISBN: 8896904811].

5. **Guzzardi, L.** (2014): "The end of a world? Ruggiero Boscovich and the tradition of didactic poetry". In: *Boscovich and his times. Contributions from Pavia 2011 International Conference*. Eds. Bevilacqua, F., Contardini, P., **Guzzardi, L.** (Pavia: Pavia University Press.). (Accepted for publication).
6. **Guzzardi, L.** (2014): "The One or the Many? Boscovich, Kant, and the Metaphysical Puzzle of Space". In: *Filozofija Ruđera Josipa Boškovića*. Eds. Stanković, N., Kutleša, S., Šestak, I. Special Issue of *Filozofski niz* (Zagreb: FTI – Filozofsko-teološki Institut), Knjiga 30, pp. 65-82.
7. **Guzzardi, L.** (2013): "Una vaga idea di spazio. Il concetto di spazio fra Settecento e Ottocento". In: *Pavia 1878. Il mondo della Fisica onora Volta*. Eds. Cantoni, V., Morando, A.P., Zucca, F. (Milano: Cisalpino), 107-125.
8. **Guzzardi, L.** (2013): "Boscovich, the discovery of Uranus and his inclination to theoretical astronomy". In: *Ruggiero Boscovich: Astronomer, Man of Science and Letters, 300 Years after His Birth*. Special Issue of: *Memorie della Società Astronomica Italiana. Supplementi*, 23, 26-33.
9. **Guzzardi, L.** (2012): "Un'applicazione della 'Legge di continuità' di Ruggiero Giuseppe Boscovich alla musica". In: *SISFA 2010 – Società Italiana degli Storici della Fisica e dell'Astronomia. Atti del XXX Congresso Nazionale (2010)*. Urbino: Argalà Editore. 139-145.
10. G. Giorello, **L. Guzzardi** (2012): "La natura della prova: tra scienza, filosofia e diritto". In: *L'uso della prova scientifica nel processo penale*. Eds. Cucci, M., Gennari, G., Gentilomo, A. (Santarcangelo di Romagna: Maggioli Editore), 21-43.
11. G. Giorello, **L. Guzzardi** (2011): "G.V. Schiaparelli: from scientific observations to scientific imagination". In: *Memorie della Società Astronomica Italiana*, 82/ 2, 219-224.
12. G. Giorello, **L. Guzzardi** (2011): "Italiani, ancora uno sforzo...". In: *L'Illuminismo e i suoi critici*. Ed. Bolognesi, D., Mattarelli, S. (Milano: Franco Angeli), 35-42.
13. **Guzzardi, L.** (2011): "Lo specchio della natura. Colombe e la cultura scientifica del suo tempo". In: *Eugenio Colombe e la cultura italiana fra le due guerre*. Eds. Cerchiai, G., Rota, G. (Manduria-Roma-Bari: Piero Lacaita Editore), 177-195.
14. G. Giorello, **L. Guzzardi** (2010): "Ricerca scientifica e libertà politica". In: *L'eredità dell'Occidente. Cristianesimo, Europa, nuovi mondi*. Eds. Olmi, A., (Firenze: Nerbini), 129-142.
15. **Guzzardi, L.** (2009): "Boscovich, Boltzmann und 'die Unentbehrlichkeit der Atomistik in der Naturwissenschaft'". In: *Ruđer Bošković (Boscovich) und sein Modell der Materie*. Eds. Grössing, H., Ullmaier, H. (Wien: Österreichische Akademie der Wissenschaften), 135-144.
16. **Guzzardi, L.** (2009): "Die italienische 'Edizione Nazionale' der Werke und der Korrespondenz von Ruggiero Giuseppe Boscovich". In: *Ruđer Bošković (Boscovich) und sein Modell der Materie*. Eds. Grössing, H., Ullmaier, H. (Wien: Österreichische Akademie der Wissenschaften), 189-193.
17. **Guzzardi, L.** (2009): "La corrispondenza di Ruggiero Giuseppe Boscovich e il Web: problemi e prospettive". In: *Rendiconti della Accademia Nazionale delle Scienze detta dei XL. Memorie di Scienze Fisiche e Naturali*, Serie V, vol. XXXIII, Parte II, Tomo I, 1-9.
18. C. Bartocci, **L. Guzzardi** (2007): "Gödel e l'irrealtà del tempo: Kant, Einstein e gli universi rotanti". In: *Lettera matematica Pristem*, 62-63, 69-78.
19. **Guzzardi, L.** (2005): "Tra filosofia della natura e morfologia dei saperi: un ruolo per l'enciclopedia". In: *Francesco Moiso. Testimonianze di colleghi e allievi* (Torino: Trauben), 69-79.

20. **Guzzardi, L.** (2005): "Scienza come Morfologia. Da Mach a Goethe". In: *La natura osservata e compresa. Saggi in memoria di Francesco Moiso*. Ed. F. Viganò (Milano: Guerini & Associati), 339-362.
21. **Guzzardi, L.** (2003): "La scienza nell'ottica dell'artista. Hermann von Helmholtz". In: *Magazzino di Filosofia*, 10, A4, 164-185.
22. **Guzzardi, L.** (2000): "Die 'Energetik' – Von einer wissenschaftlichen Theorie zur Wissenschaftslehre". In: *Mitteilungen der Wilhelm-Ostwald-Gesellschaft*. Wilhelm-Ostwald-Gesellschaft zu Grossbothen, 2, 66-69.

REVIEWS

1. **Guzzardi, L.** (2013): "P. Giordanetti, *L'avventura della ragione. Kant e il giovane Nietzsche*, Georg Olms Verlag, Hildesheim 2011". In: *Giornale critico della filosofia italiana*, XCII/ 3 (Settima Serie), pp. 686-688.
2. **Guzzardi, L.** (2011): "S. Boscani Leoni (Hg.), *Wissenschaft – Berge – Ideologien. Johann Jakob Scheuchzer (1672-1733) und die frühneuzeitliche Naturforschung*, Schwabe Verlag, Basel 2010". In: *Rivista di Storia della Filosofia*, LXVI, 2, 347-350.
3. **Guzzardi, L.** (2006): "J. Lützen, *Mechanistic Images in Geometric Form. Heinrich Hertz's 'Principles of Mechanics'*, Oxford University Press, Oxford 2005". In: *Rivista di Storia della Filosofia*, LXI, 3, 826-830.
4. **Guzzardi, L.** (2006): "C. Cappelletto, *Il rito delle pulci. Wittgenstein morfologo*, il Castoro, Milano, 2004". In: *Rivista di Estetica*, XLVI, n.s., 31/ 1, 223-225.
5. **Guzzardi, L.** (2005): "E.C. Banks, *Ernst Mach's World Elements. A Study in Natural Philosophy*, Kluwer, Dordrecht 2003". In: *Rivista di Storia della Filosofia*, LX, 3, 596-599.
6. **Guzzardi, L.** (2003): "F.W. Schelling, *Le divinità di Samotracia*, Milano, Mimesis, 2002; M. Detienne, *Apollo con il coltello in mano*, Milano, Adelphi, 2002". In: *Rivista di Estetica*, XLIII, n. s., 23/ 2, 246-248.
7. **Guzzardi, L.** (2003): "V. Mathieu, *Goethe e il suo diavolo custode*, Adelphi, Milano, 2002". In: *Filosofia*, 53/ 3, 101.

TRANSLATED BOOKS:

1. Halpern, P. (2016): I dadi di Einstein e il gatto di Schrödinger. Transl. **L. Guzzardi**. Milano. Raffaello Cortina. [Italian edition of: *Einsteins dice and Schrödingers cat*].
2. Gefter, A. (2015): Due intrusi nel mondo di Einstein. Un padre, sua figlia, il significato del nulla e l'inizio di tutto. Transl. **L. Guzzardi**. Milano. Raffaello Cortina. 494 pp. [Italian edition of: *Trespassing on Einstein's Lawn*].
3. Young, R.F. (2012): Bolle, gocce, schiume: Fisica della vita quotidiana. Transl. **L. Guzzardi**. Milano. Raffaello Cortina. 148 pp. [Italian edition of: *Fizzics. The Science of Bubbles, Droplets, and Foams*].
4. Close, F. (2012): Neutrino. Transl. **L. Guzzardi**. Milano. Raffaello Cortina. 192 pp. [Italian edition of: *Neutrino*].

5. Sloterdijk, P. (2011): Caratteri filosofici. Da Platone a Foucault. Transl. **L. Guzzardi**. Milano. Raffaello Cortina. 126 pp. [Italian edition of: *Philosophische Temperamente. Von Platon bis Foucault*].
6. Frith, C. (2011): Inventare la mente: Come il cervello crea la nostra vita mentale. Transl. M. Berlingeri & **L. Guzzardi**. Milano. Raffaello Cortina. 304 pp. [Italian edition of: *Making up the Mind*].
7. Clark, P. (2011): I paradossi dalla A alla Z. Nuova edizione. Transl. **L. Guzzardi** & A. Pedferri. Milano. Raffaello Cortina. 300 pp. [Italian edition of: *Paradoxes from A to Z*].
8. Gubser, S. (2010): Il piccolo libro delle stringhe. Transl. **L. Guzzardi**. Milano. Raffaello Cortina. 182 pp. [Italian edition of: *The Little Book of String Theory*].
9. Aczel, A. (2010): Le cattedrali della preistoria: Il significato dell'arte rupestre. Transl. **L. Guzzardi**. Milano. Raffaello Cortina. 238 pp. [Italian edition of: *The Cave and the Cathedral*].
10. Goodchild, P. (2009): Il vero dottor Stranamore: Edward Teller e la guerra nucleare. Italian edition, translation and scientific notes by **L. Guzzardi**. Milano. Raffaello Cortina. 592 pp. [Italian edition of: *Edward Teller: The Real Dr. Strangelove*].
11. Montague, R. (2008): Perché l'hai fatto? Come prendiamo le nostre decisioni. Transl. **L. Guzzardi**. Milano. Raffaello Cortina. 362 pp. [Italian edition of: *Why Choose This Book? How We Make Decisions*].
12. Baron, J. (2008): Contro la bioetica. Italian edition, translation and scientific notes by **L. Guzzardi**. Milano. Raffaello Cortina. 322 pp. [Italian edition of: *Against Bioethics*].
13. Klein, E. (2006): Sette volte la rivoluzione: I grandi della fisica contemporanea. Italian edition and scientific notes by **L. Guzzardi**. Milano. Raffaello Cortina. 196 pp. [Italian edition of: *Il Etait Sept Fois la Révolution: Albert Einstein et les Autres*].
14. Vilenkin, A. (2007): Un solo mondo o infiniti? Alla ricerca di altri universi. Transl. **L. Guzzardi**. Milano. Raffaello Cortina. 304 pp. [Italian edition of: *Many Worlds in One: The Search for Other Universes*].
15. Bruce, C. (2006): I conigli di Schrödinger. Fisica quantistica e universi paralleli. Transl. **L. Guzzardi**. Milano: Raffaello Cortina. 350 pp. [Italian edition of: *Schrödinger's Rabbits: The Many Worlds of Quantum*].
16. Dalai Lama (2006): Nuove immagini dell'Universo: Dialoghi con fisici e cosmologi. Italian edition, translation and scientific notes by **L. Guzzardi**. Milano. Raffaello Cortina. 344 pp. [Italian edition of: *The New Physics and Cosmology. Dialogues with the Dalai Lama*, ed. by A. Zajonc].

BIOGRAPHICAL SKETCH

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

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 Committee 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 “Il Foresight training course” (Pavia, September 2 to 4, 2009)
- AIFA Annual Meeting on Independent Research (Rome, October 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 integration 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 Teleangiectasia, 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
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., Tuszyński 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
54. Booth C., Hershfieeld M., Notarangelo L., Buckley R., Hoenig M., Mahlaoui N., Cavazzana-Calvo M., Aiuti A., and Gaspar H. B. (2007). Management options for adenosine deaminase deficiency; 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
56. Cattoglio C., Facchini G., Sartori D., Antonelli A., Miccio A., Cassani B., Schmidt M., von Kalle C., Howe S., Thrasher A. J., Aiuti A., Ferrari G., Recchia A., and Mavilio F. (2007). Hot spots of retroviral integration in human CD34+ hematopoietic cells. *Blood* 110, 1770-1778. IF 10.370

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., Vena 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
59. Husain M., Grunebaum E., Naqvi A., Atkinson A., Ngan B. Y., Aiuti A., and Roifman C. M. (2007). Burkitt's lymphoma in a patient with adenosine deaminase deficiency-severe combined immunodeficiency treated with polyethylene glycol-adenosine deaminase. *J Pediatr* 151, 93-95. IF 3.991
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
61. Benninghoff U., Cattaneo F., Aiuti A., Flores-D'Arcais A., Gelmetti C., Viscardi M., Callegaro L., Mirolo M., Ambrosi A., Roncarolo M. G., and Bacchetta R. (2008). Clinical improvement and normalized Th1 cytokine profile in early and long-term interferon-alpha treatment in a suspected case of hyper-IgE syndrome. *Pediatr Allergy Immunol* 19, 564-568. IF 2.454
62. Cassani B., Mirolo M., Cattaneo F., Benninghoff U., Hershfield M., Carlucci F., Tabucchi A., Bordignon C., Roncarolo M.G., and Aiuti A. (2008). Altered intracellular and extracellular signaling leads to impaired T-cell functions in ADA-SCID patients. *Blood* 111, 4209-19. IF 10.896
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
64. Aiuti A., Brigida I., Ferrua F., Cappelli B., Chiesa R., Marktel S., and Roncarolo M.G. (2009). Hematopoietic stem cell gene therapy for adenosine deaminase deficient-SCID. *Immunol Res* 44, 150-159. IF 2.364
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
66. Aiuti A., and Roncarolo M.G. (2009). Ten years of gene therapy for primary immune deficiencies. *Hematology Am Soc Hematol Educ Program*. 2009, 682-689. IF 1.333
67. Bosticardo M., Marangoni F., Aiuti A., Villa A., and Roncarolo M.G. (2009). Recent advances in understanding the pathophysiology of Wiskott-Aldrich syndrome. *Blood* 113, 6288-6295. IF 10.896
68. Cassani B., Montini E., Maruggi G., Ambrosi A., Mirolo M., Selleri S., Biral E., Frugnoli I., Hernandez-Trujillo V., Di Serio C., Roncarolo M. G., Naldini L., Mavilio F., and Aiuti A. (2009). Integration of retroviral vectors induces minor changes in the transcriptional activity of T cells from ADA-SCID patients treated with gene therapy. *Blood* 114, 3546-3556. IF 10.896
69. Chiesa R., Cappelli B., Crocchiolo R., Frugnoli I., Biral E., Noe A., Evangelio C., Fossati M., Roccia T., Biffi A., Finizio V., Aiuti A., Broglia M., Bartoli A., Ciceri F., Roncarolo M. G., and Marktel S. (2010). Unpredictability of iv Busulfan pharmacokinetics in children

- undergoing hematopoietic stem cell transplant for advanced beta thalassemia: Limited toxicity with a dose adjustment policy. *Biol Blood Marrow Transplant.* 16, 622-628. Epub Dec 4, 2009. IF 3.732
70. Gabriel R., Eckenberg R., Paruzynski A., Bartholomae C. C., Nowrouzi A., Arens A., Howe S. J., Recchia A., Cattoglio C., Wang W., Faber K., Schwarzwaelder K., Kirsten R., Deichmann A., Ball C. R., Balaggan K. S., Yanez-Munoz R. J., Ali R. R., Gaspar H. B., Biasco L., Aiuti A., Cesana D., Montini E., Naldini L., Cohen-Haguenauer O., Mavilio F., Thrasher A. J., Glimm H., von Kalle C., Saurin W., and Schmidt M. (2009). Comprehensive genomic access to vector integration in clinical gene therapy. *Nat Med.* 15, 1431-1436. IF 27.136
71. Gaspar H. B., Aiuti A., Porta F., Candotti F., Hershfield M. S., and Notarangelo L. D. (2009). How I treat ADA deficiency. *Blood* 114, 3524-3532. IF 10.896
72. Locci M., Draghici E., Marangoni F., Bosticardo M., Catucci M., Aiuti A., Cancrini C., Marodi L., Espanol T., Bredius R. G., Thrasher A. J., Schulz A., Litzman J., Roncarolo M. G., Casorati G., Dellabona P., and Villa A. (2009). The Wiskott-Aldrich syndrome protein is required for iNKT cell maturation and function. *J Exp Med* 206, 735-742. IF 15.162
73. Marangoni F., Bosticardo M., Charrier S., Draghici E., Locci M., Scaramuzza S., Panaroni C., Ponzoni M., Sanvito F., Doglioni C., Liabeuf M., Gjata B., Montus M., Siminovitch K., Aiuti A., Naldini L., Duprè L., Roncarolo M. G., Galy A., and Villa A. (2009). Evidence for long-term efficacy and safety of gene therapy for Wiskott-Aldrich syndrome in preclinical models. *Mol Ther* 17, 1073-1082. IF 5.896
74. Sauer A.V., and Aiuti A. (2009) New insights into the pathogenesis of adenosine deaminase-severe combined immunodeficiency and progress in gene therapy. *Curr Opin Allergy Clin Immunol* 9, 496-502. IF 3.497
75. Sauer A. V., Mrak E., Hernandez R. J., Zucchi E., Cavani F., Casiraghi M., Grunebaum E., Roifman C. M., Cervi M. C., Ambrosi A., Carlucci F., Roncarolo M. G., Villa A., Rubinacci A., and Aiuti A. (2009). ADA-deficient SCID is associated with a specific microenvironment and bone phenotype characterized by RANKL/OPG imbalance and osteoblast insufficiency. *Blood* 114, 3216-3226. IF 10.896
76. Trifari S., Scaramuzza S., Catucci M., Ponzoni M., Mollica L., Chiesa R., Cattaneo F., Lafouresse F., Calvez R., Vermi W., Medicina D., Castiello M.C., Marangoni F., Bosticardo M., Doglioni C., Caniglia M., Aiuti A., Villa A., Roncarolo M.G., and Dupré L. (2010). Revertant T lymphocytes in a patient with Wiskott-Aldrich syndrome: Analysis of function and distribution in lymphoid organs. *J Allergy Clin Immunol.* 125, 439-448.e8. IF 9.273
77. Cappelli B., and Aiuti A. (2010). Gene therapy for adenosine deaminase deficiency. *Immunol Allergy Clin. North Amer.* 30, 249-260. IF 2.959
78. Cancrini C., Ferrua F., Scarselli A., Brigida I., Romiti M.L., Barera G., Finocchi A., Roncarolo M.G., Caniglia M., and Aiuti A. (2010). Role of reduced intensity conditioning in T-cell and B-cell immune reconstitution after HLA-identical bone marrow transplantation in ADA-SCID. *Haematologica.* 95, 1778-1782. IF 6.532
79. Ferrua F., Brigida I., and Aiuti A. (2010). Update on gene therapy for adenosine deaminase-deficient severe combined immunodeficiency. *Curr Opin Allergy Clin Immunol* 10, 551-556. IF 3.431
80. Cohen-Haguenauer O., Creff N., Cruz P., Tunc C., Aiuti A., Baum C., Bosch F., Blomberg P., Cichutek K., Collins M., Danos O., Dehaut F., Federspiel M., Galun E., Garritsen H., Hauser H., Hildebrandt M., Klatzmann D., Merten O., Montini E., O'Brien T., Panet A., Rasooly L., Scherman D., Schmidt M., Schweitzer M., Tiberghien P., Vandendriessche T., Ziehr H., Ylä-Herttuala S., von Kalle C., Gahrton G., and Carrondo M. (2010). Relevance of an Academic GMP Pan-European Vector Infra-structure (PEVI). *Curr Gene Ther.* 10, 414-422. IF 4.482
81. Biasco L., Ambrosi A., Pellin D., Bartholomae C., Brigida I., Roncarolo M.G., Di Serio C., von Kalle C., Schmidt M., and Aiuti A. (2010) Integration profile of retroviral vector in

- gene therapy treated patients is cell-specific according to gene expression and chromatin conformation of target cell. *EMBO Mol Med.* 3, 89-101. IF 10.333
82. Selleri S., Brigida I., Casiraghi M., Scaramuzza S., Cappelli B., Cassani B., Ferrua F., Aker M., Slavin S., Scarselli A., Cancrini C., Marktel S., Roncarolo M.G., and Aiuti A. (2011). In vivo T-cell dynamics during immune reconstitution after hematopoietic stem cell gene therapy in adenosine deaminase severe combined immune deficiency. *J Allergy Clin Immunol.* 127, 1368-1375. IF 11.003
 83. Grunebaum E., Chung C.T., Dadi H., Kim P., Brigida I., Ferrua F., Cicalese M.P., Aiuti A., and Roifman C.M. (2011). Purine metabolism, immune reconstitution, and abdominal adipose tumor after gene therapy for adenosine deaminase deficiency. *J Allergy Clin Immunol.* 127, 1417-1419. IF 11.003
 84. Bosticardo M., Draghici E., Schena F., Sauer A.V., Fontana E., Castiello M.C., Catucci M., Locci M., Naldini L., Aiuti A., Roncarolo M.G., Poliani P.L., Traggiai E., and Villa A. (2011) Lentiviral-mediated gene therapy leads to improvement of B-cell functionality in a murine model of Wiskott-Aldrich syndrome. *J Allergy Clin Immunol.* 127, 1376-1384. IF 11.003
 85. Angelino G., Natali G.L., Falappa P., Folgori L., Moretti R., Cantarutti N., Di Matteo G., Chiriaco M., Rossi P., Roos D., Aiuti A., and Finocchi A. (2011) Successful treatment with percutaneous transhepatic alcoholization of a liver abscess in a child with chronic granulomatous disease. *Pediatr Infect Dis J.* 30, 819-820. IF 3.064
 86. Deichmann A., Brugman M.H., Bartholomae C.C., Schwarzwaelter K., Verstegen M.M., Howe S.J., Arens A., Ott M.G., Hoelzer D., Seger R., Grez M., Hacein-Bey-Abina S., Cavazzana-Calvo M., Fischer A., Paruzynski A., Gabriel R., Glimm H., Abel U., Cattoglio C., Mavilio F., Cassani B., Aiuti A., Dunbar C.E., Baum C., Gaspar H.B., Thrasher A.J., von Kalle C., Schmidt M., and Wagemaker G. (2011). Insertion sites in engrafted cells cluster within a limited repertoire of genomic areas after gammaretroviral vector gene therapy. *Mol Ther.* 19, 2031-2039. IF 6.873
 87. Cancrini C., Scarselli A., Scaramuzza S., Chiriaco M., Di Cesare S., Di Matteo G., Romiti M.L., Palma P., De Felice L., Palumbo G., Pinto R.M., De Vito R., Racioppi L., Livadiotti S., Fischer A., Rossi P., Caniglia M., and Aiuti A. (2011). Early-onset monocyte-B-natural killer-dendritic cells' deficiency successfully treated with hematopoietic stem cell transplantation. *J Allergy Clin Immunol.* 128, 897-900. IF 11.003
 88. Catucci M., Prete F., Bosticardo M., Castiello M.C., Draghici E., Locci M., Roncarolo M.G., Aiuti A., Benvenuti F., and Villa A. (2012). Dendritic cell functional improvement in a preclinical model of lentiviral-mediated gene therapy for Wiskott-Aldrich syndrome. *Gene Ther.*, 19, 1150-1159. Epub 2011 Dec 22. IF 4.538
 89. Sauer A.V., Brigida I., Carriglio N., Jofra Hernandez R., Scaramuzza S., Clavenna D., Sanvitto F., Poliani P.L., Gagliani N., Carlucci F., Tabucchi A., Roncarolo M.G., Traggiai E., Villa A., and Aiuti A. (2012). Alterations in the adenosine metabolism and CD39/CD73 adenosinergic machinery cause loss of Treg cell function and autoimmunity in ADA-deficient SCID. *Blood.* 119, 1428-1439. IF 9.898
 90. Mariani S.A., Brigida I., Kajaste-Rudnitski A., Vicenzi E., Aiuti A., and Poli G. (2012). HIV-1 envelope dependent post-entry restriction of CXCR4-using viruses in children but not adult derived CD4+ T lymphocytes. *Blood.* 119, 2013-2023. IF 9.898
 91. Biasco L., Baricordi C., and Aiuti A. (2012). Retroviral integrations in gene therapy trials. *Mol. Ther.* 20, 709-716. IF 6.873
 92. Sauer A.V., Morbach H., Brigida I., Ng Y.S., Aiuti A., and Meffre E. (2012). Defective B cell tolerance due to adenosine deaminase deficiency is corrected by gene therapy. *J Clin Investig.* 122, 2141-2152. IF 13.069
 93. Corrigan-Curay J., Cohen-Haguenuer O., O'Reilly M., Ross S.R., Fan H., Rosenberg N., Somia N., King N., Friedmann T., Dunbar C., Aiuti A., Naldini L., Baum C., von Kalle C., Kiem H.P., Montini E., Bushman F., Sorrentino B.P., Carrondo M., Malech H., Gahrton G.,

- Shapiro R., Wolff L., Rosenthal E., Jambou R., Zaia J., and Kohn D.B. (2012). Challenges in Vector and Trial Design using Retroviral vectors for Long Term Gene Correction in Hematopoietic Stem Cell Gene Therapy: Summary of a Symposium Sponsored by the NIH Office of Biotechnology Activities and the EC DG-research NoE for the Advancement of Clinical Gene Transfer and Therapy. *Mol Ther.* 20, 1084-1094. IF 6.873
94. Vago L., Oliveira G., Bondanza A., Noviello M., Solati C., Ghio D., Brigida I., Greco R., Lupo Stanghellini M.T., Peccatori J., Fracchia S., Del Fiacco M., Traversari C., Aiuti A., Del Maschio A., Bordignon C., Ciceri F., and Bonini C. (2012). T cell suicide gene therapy prompts thymic renewal in adults after hematopoietic stem cell transplantation. *Blood* 120, 1820-30. IF 9.898
95. Hassan A., Booth C., Brightwell A., Allwood Z., Veys P., Rao K., Hoenig M., Friedrich W., Gennery A.R., Slatter M., Bredius R., Finocchi A., Cancrini C., Aiuti A., Lanfranchi A., Porta F., Ridella M., Graham Steward C., Filipovich A., Marsh R., Bordon V., Al-Muhsen S., Al-Mousa H., Alsum Z., Al-Dhekri H., Al Ghonaium A., Speckmann C., Fischer A., Mahlaoui N., Nichols K. E., Grunebaum E., Al Zaharani D., Roifman C., Boelens J.J., Davies E. G., Cavazzana-Calvo M., Notarangelo L., and Gaspar H.B. (2012). Outcome of hematopoietic stem cell transplantation for adenosine deaminase-deficient severe combined immunodeficiency. *Blood* 120, 3615-24. IF 9.898
96. Aiuti A., Bacchetta R., Seger R., Villa A., Cavazzana-Calvo M. (2012) Gene therapy for primary immunodeficiencies: Part 2 *Curr. Opin. Immunol.*, 24, 585-591. IF 9.522
97. Cavazzana-Calvo M., Fischer A., Hacein-Bey-Abina S., Aiuti A. (2012) Gene therapy for primary immunodeficiencies: Part 1. *Curr. Opin. Immunol.* 24, 580-584. IF 9.522
98. Sauer A.V., Brigida I., Carriglio N., Aiuti A. (2012) Autoimmune dysregulation and purine metabolism in adenosine deaminase deficiency. *Front. Immunol.* 3, 265. IF N.A.
99. Scaramuzza S., Biasco L., Ripamonti A., Castiello M.C., Loperfido M., Draghici E., Jofra Hernandez R., Benedicenti F., Radrizzani M., Salomoni M., Ranzani M., Bartholomae CC., Vicenzi E., Finocchi A., Bredius R., Bosticardo M., Schmidt M., von Kalle C., Montini E., Biffi A., Roncarolo M.G., Naldini L., Villa A., and Aiuti A. (2013). Preclinical safety and efficacy on human CD34+ cells transduced with lentiviral vector for the treatment of Wiskott-Aldrich Syndrome. *Mol Ther.* 21, 175-184. Epub 2012 Feb 28. IF 7.149
100. Prete F., Catucci M., Labrada M., Gobessi S., Castiello M.C., Bonomi E., Aiuti A., Vermi W., Cancrini C., Metin A., Hambleton S., Bredius R., Notarangelo L.D., Van der Burg M., Kalinke U., Villa A., and Benvenuti F. (2013). Wiskott-Aldrich syndrome protein-mediated actin dynamics control type-I interferon production in plasmacytoid dendritic cells. *J. Exp. Med.*, 210, 355-374. IF 13.214
101. Goudy K., Aydin D., Barzaghi F., Gambinieri E., Vignoli M., Doglioni C., Ponzoni M., Cicalese M.P., Assanelli A., Tommasini A., Brigida I., Dellepiane R.M., Martino S., Olek S., Aiuti A., Ciceri F., and Roncarolo M.G. (2013). Human IL2RA null mutation mediates immunodeficiency with lymphoproliferation and autoimmunity. *Clin Immunol.*, 146, 248-261. IF 3.771
102. Salfa I., Cantarutti N., Angelino G., Matteo G.D., Capo V., Farinelli G., Cancrini C., Aiuti A., Palma P., and Finocchi A. (2013). Serratia marcescens osteomyelitis in a newborn with chronic granulomatous disease. *Pediatric Infect Dis J.*, 32: 926. IF 3.569
103. Aiuti A., Biasco L., Scaramuzza S., Ferrua F., Cicalese M.P., Baricordi C., Dionisio F., Calabria A., Giannelli S., Castiello M.C., Bosticardo M., Evangelio C., Assanelli A., Casiraghi M., Di Nunzio S., Callegaro L., Benati C., Rizzardi P., Pellin D., Di Serio C., Schmidt M., Van Kalle C., Gardner J., Mehta N., Nedeva V., Dow D.J., Galy A., Miniero R., Finocchi A., Metin A., Banerjee P., Orange J., Galimberti S., Valsecchi M.G., Biffi A., Montini E., Villa A., Ciceri F., Roncarolo M.G., and Naldini L. (2013). Lentivirus based gene therapy of hematopoietic stem cell in Wiskott-Aldrich Syndrome. *Science* 341(6148):1233151. Epub 2013 Jul 11. I.F. 31.207

104. Biffi A., Montini E., Lorioli L., Cesani M., Fumagalli F., Plati T., Baldoli C., Martino S., Calabria A., Canale S., Benedicenti F., Vallanti G., Biasco L., Leo S., Kabbara N., Zanetti G., Rizzo W.B., Mehta N., Cicalese M.P., Casiraghi M., Boelens J.J., Del Carro U., Dow D.J., Schmidt M., Assanelli A., Nedeva V., Di Serio C., Stupka E., Gardner J., Van Kalle C., Bordignon C., Ciceri F., Rovelli A., Roncarolo M.G., Aiuti A., Sessa M. and Naldini L. (2013). Therapeutic benefit in metachromatic leukodystrophy by lentiviral hematopoietic stem cell gene therapy. *Science* 341(6148):1233158. Epub 2013 Jul 11. I.F. 31.207
105. Aiuti A., Cossu G., de Felipe P., Galli M.C., Narayanan G., Renner M., Stahlbom A., Schneider C.K., and Voltz-Girold C. (2013). The Committee for Advanced Therapies' (CAT) reflection paper on management of clinical risks deriving from insertional mutagenesis. *Hum Gene Ther Clin Dev.* Jul 18. 24:47-54. I.F. 4.019
106. Castiello M.C., Bosticardo M., Pala F., Catucci M., Chamberlain N., van Zelm M.C., Driessen G.J., Pac M., Bernatowska E., Scaramuzza S., Aiuti A., Sauer A.V., Traggiai E., Meffre E., Villa A., van der Burg M. (2014). Wiskott-Aldrich Syndrome protein deficiency perturbs the homeostasis of B-cell compartment in humans. *J Autoimmun.* 50, 42-50. I.F. 8.41
107. Angelino G., Caruso R., D'Argenio P., Calò Carducci F.I., Pascone R., Lanciotti M., Cancrini C., Palma P., Aiuti A., Rossi P., and Finocchi A. (2014). Etiology, clinical outcome, and laboratory features in children with neutropenia: analysis of 104 cases. *Pediatr Allergy Immunol.* 25: 283-289. Epub 2013 Dec 10. IF 3.376
108. Brigida I., Sauer A.V., Ferrua F., Giannelli S., Scaramuzza S., Pistoia V., Castiello M.C., Barendregt B.H., Cicalese M.P., Casiraghi M., Brombin C., Puck J., Müller K., Notarangelo L.D., Montin D., van Montfrans J.M., Roncarolo M.G., Traggiai E., van Dongen J.J.M., van der Burg M., and Aiuti A. (2014). B-cell development and functions and therapeutic options in adenosine deaminase deficient patients. *J Allergy Clin Immunol* 133:799-806. Epub 2014 Feb 5. IF 11.248
109. Finocchi A., Claps A., Serafinelli J., Saifa I., Longo D., Di Matteo G., Aiuti A., and Rossi P. (2014). Chronic granulomatous disease presenting with *Salmonella* brain abscesses. *Pediatr Infect Dis J* 33: 525-528. IF 3.135
110. Farinelli G., Capo V., Scaramuzza S., and Aiuti A. (2014). Lentiviral vectors for the treatment of primary immunodeficiencies. *J Inher Metab Dis.* 37: 525-533. Epub 2014 Mar 12. IF 4.07
111. Cancrini C., Puliafito P., Digilio M.C., Soresina A., Martino S., Ruga E.M., Rondelli R., Consolini R., Milanesi O., Cardinale F., Finocchi A., Romiti M.L., Martire B., Corsello G., Bacchetta R., Albano V., Pignata C., Azzari C., Carotti A., Specchia F., Montin D., Cirillo E., Cocchi G., Trizzino A., Bossi G., Aiuti A., Pietrogrande M.C., Marino B., Ugazio A.G., Plebani A. and Rossi P. for the Italian Network for Primary Immunodeficiencies (IPINET). (2014). Clinical features of a cohort of 22q11.2 deletion syndrome patients: an Italian multicenter study. *J of Pediatr* 164, 1475-1480.e2. Epub 2014 Mar 20. IF 4.05
112. Chiriaco M., Farinelli G., Capo V., Di Matteo G., Zonari E., Scaramuzza S., Sergi L., Migliavacca M., Hernandez R.J., Bombelli F., Giorda E., Kajaste-Rudnitski A., Trono D., Grez M., Rossi P., Finocchi A., Naldini L., Gentner B., and Aiuti A. (2014). Dual-regulated lentiviral vector for gene therapy of X-linked chronic granulomatosis. *Mol Therapy.* 22, 1472-1483. Epub 2014 May 29. IF. 6.82
113. Sauer A.V., Di Lorenzo B., Carriglio N., and Aiuti A. (2014). Progress in gene therapy for primary immunodeficiencies using lentiviral vectors. *Curr Opin Allergy Clin Immunol.* 14, 527-534. IF 3.659
114. Bosticardo M., Ferrua F., Cavazzana M., and Aiuti A. (2014). Gene therapy for Wiskott Aldrich Syndrome. *Curr Gene Ther.* 14:413-421. Epub 2014 Sep 17. IF 4.096
115. Biasco L., Scala S., Basso Ricci L., Dionisio F., Baricordi C., Calabria A., Giannelli S., Cieri N., Barzaghi F., Pajno R., Al-Mousa H., Scarselli A., Cancrini C., Bordignon C., Roncarolo

- M.G., Monti E., Bonini C., and Aiuti A. (2015). In vivo tracking of T cells in humans unveils decade-long survival and activity of genetically modified T memory stem cells. *Sci Transl Med.* 7: 273ra13. I.F. 14.414
116. Castiello M.C., Scaramuzza S., Pala F., Ferrua F., Uva P., Brigida I., Sereni L., van den Burg M., Ottaviano G., Albert M.H., Roncarolo M.G., Naldini L., Aiuti A., and Bosticardo M. (2015). B-cell reconstitution after lentiviral vector-mediated gene therapy in patients with Wiskott-Aldrich syndrome. *J Allergy Clin Immunol.* 136, 692-702. Epub 2015 March 16. I.F. 11.248
117. Cotugno N., Finocchi A., Cagigi A., Di Matteo G., Chiriaco M., Di Cesare S., Rossi P., Aiuti A., Palma P., and Douagi I. (2015). Defective B-cell proliferation and maintenance of long-term memory in patients with chronic granulomatous disease. *J Allergy Clin Immunol.* 135, 753-761. Epub 2014 Aug 29. IF 11.248
118. Lorioli L., Cicalese M.P., Silvani P., Assanelli A., Salvo I., Mandelli A., Fumagalli F., Fiori R., Ciceri F., Aiuti A., Sessa M., Roncarolo M.G., Lanzani C., and Biffi A. (2015). Abnormalities of acid-base balance and predisposition to metabolic acidosis in Metachromatic Leukodystrophy patients. *Mol Genet Metab.* 115:48-52. Epub 2015 March 6. I.F. 2.827
119. Cicalese M.P., and Aiuti A. (2015). Clinical applications of gene therapy for primary immunodeficiencies. *Hum Gene Ther* 26: 210-219. I.F. 3.623
120. Scarselli A., Di Cesare S., Capponi C., Romiti M.L., Di Matteo G., Simonetti A., Palma P., Finocchi A., Lucarelli B., Pinto R.M., Rana I., Palumbo G., Caniglia M., Rossi P., Carsetti R., Cancrini C., and Aiuti A. (2015). Longitudinal evaluation of immune reconstitution and B-cell function after hematopoietic cell transplantation for primary immunodeficiency. *J Clin Immunol.* 35:373-383. Epub 2015 April 15. I.F. 2.654
121. Di Cesare S., Puliafito P., Ariganello P., Marcovecchio G.E., Mandolesi M., Capolino R., Digilio M.C., Aiuti A., Rossi P., and Cancrini C. (2015). Autoimmunity and regulatory T cells in 22q11.2 deletion syndrome patients. *Pediat Allergy Immunol* 26:591-594. Epub 2015 June 9.I.F.3.397
122. Pala F., Morbach H., Castiello M.C., Schickel J.N., Scaramuzza S., Chamberlain N., Cassani B., Glauzy S., Romberg N., Candotti F., Aiuti A., Bosticardo M., Villa A., and Meffre E. (2015). Lentiviral-mediated gene therapy restores B cell tolerance in Wiskott-Aldrich syndrome patients. *J Clin Invest.* 125: 3941-3951. I.F. 13.262
123. Oliveira G., Ruggiero E., Stanghellini M.T., Cieri N., D'Agostino M., Fronza R., Lulay C., Dionisio F., Mastaglio S., Greco R., Peccatori J., Aiuti A., Ambrosi A., Biasco L., Bondanza A., Lambiase A., Traversari C., Vago L., von Kalle C., Schmidt M., Bordignon C., Ciceri F., and Bonini C. (2015). Tracking genetically engineered lymphocytes long-term reveals the dynamics of T cell immunological memory. *Sci Transl Med.* 7: 317ra198. I.F. 15.843
124. Scarselli A., Di Cesare S., Di Matteo G., De Matteis A., Ariganello P., Romiti M.L., Cascioli S., De Vito R., Bertaina A., Locatelli F., Gaspar H.B., Aiuti A., Rossi P., Gilmour K., and Cancrini C. (2015). Combined immunodeficiency due to JAK3 mutation in a child presenting with skin granuloma. *J Allergy Clin Immunol.* S0091-6749 Epub 2015 November 3. I.F. 11.476
125. Migliavacca M., Assanelli A., Ferrua F., Cicalese M.P., Biffi A., Frittoli M., Silvani P., Chidini G., Calderini E., Mandelli A., Camporesi A., Milani R., Farinelli G., Nicoletti R., Ciceri F., Bernardo M.E., and Aiuti A.(2016). Pioglitazone as a novel therapeutic approach in Chronic Granulomatous Disease. *J Allergy Clin Immunol.* 137: 1913-1915. Epub 2016 April 4. IF (2015) 12.485
126. Brigida I., Scaramuzza S., Lazarevic D., Cittaro D., Ferrua F., Leonardelli L., Alessio M., Forma O., Lanzani C., Viarengo G., Ciceri F., Jankovic M., Pesce F., Aiuti A., and Cicalese M.P. (2016). A novel genomic inversion in Wiskott-Aldrich-associated autoinflammation. *J Allergy Clin Immunol.* 138: 619-622.e.7. Epub 2016 April 22. IF (2015) 12.485
127. Cicalese M.P., Ferrua F., Castagnaro L., Pajno R., Barzaghi F., Giannelli S., Dionisio F., Brigida I., Bonopane M., Casiraghi M., Tabucchi A., Carlucci F., Grunebaum E., Adeli M.,

- Bredius R.G., Puck J.M., Stepensky P., Tezcan I., Rolfe K., De Boever E., Reinhardt R.R., Appleby J., Ciceri F., Roncarolo M.G., and Aiuti A.(2016). Update on the safety and efficacy of retroviral gene therapy for immunodeficiency due to adenosine deaminase deficiency. *Blood.* 128: 45-54. Epub April 29, 2016. IF (2015) 11.841
128. Biasco L., Pellin D., Scala S., Dionisio F., Basso-Ricci L., Leonardelli L., Scaramuzza S., Baricordi C., Ferrua F., Cicalese M.P., Giannelli S., Neduva V., Dow D.J., Schmidt M., Von Kalle C., Roncarolo M.G., Ciceri F., Vicard P., Wit E., Di Serio C., Naldini L., and Aiuti A. (2016). In vivo tracking of human hematopoiesis reveals patterns of clonal dynamics during early and steady-state reconstitution phases. *Cell Stem Cell.* 19: 107-119. Epub May 25, 2016. IF (2015) 22.387
129. Sessa M*, Lorioli L*, Fumagalli F, Acquati S, Redaelli D, Baldoli C, Canale S, Lopez ID, Morena F, Calabria A, Fiori R, Silvani P, Rancoita PM, Gabaldo M, Benedicenti F, Antonioli G, Assanelli A, Cicalese MP, Del Carro U, Natali Sora MG, Martino S, Quattrini A, Montini E, Di Serio C, Ciceri F, Roncarolo MG, Aiuti A, Naldini L, and Biffi A.(2016). Lentiviral haemopoietic stem-cell gene therapy in early-onset metachromatic leukodystrophy: an ad-hoc analysis of a non-randomised, open-label, phase 1/2 trial. *Lancet.* 388: 476-487. Epub June 8, 2016. IF (2015) 44.002 * equal contribution
130. Magnani CF, Turazzi N, Benedicenti F, Calabria A, Tenderini E, Tettamanti S, Giordano Attianese GM, Cooper LJ, Aiuti A, Montini E, Biondi A, and Biagi E. (2016). Immunotherapy of acute leukemia by chimeric antigen receptor-modified lymphocytes using an improved Sleeping Beauty transposon platform. *Oncotarget.* 7: 51581-51597. Epub June 13, 2016. IF (2015) 5.008
131. Aiuti A., and Naldini L. (2016). Safer conditioning for blood stem cell transplants. *Nat Biotechnol* 34: 721-723. IF (2015) 43.113
132. Farinelli G., Hernandez RJ, Rossi A, Ranucci S, Sanvito F, Migliavacca M, Brombin C, Pramov A, Serio CD, Bovolenta C, Gentner B, Bragonzi A, and Aiuti A. (2016). Lentiviral vector gene therapy protects XCGD mice from Acute Staphylococcus aureus pneumonia and inflammatory response. *Mol Ther.* 24: 1873-1880. Epub July 26, 2016. IF (2015) 6.938
133. Ingo D.M., Redaelli D., Rossella V., Perini O., Santoleri L., Ciceri F., Aiuti A., and Bernardo M.E. (2016). Bone marrow-derived CD34- fraction: A rich source of mesenchymal stromal cells for clinical application. *Cytotherapy.* 18: 1560-1563. Epub October 16, 2016. IF (2015) 3.625
134. Bertolotti M., Farinelli G., Galli M., Aiuti A., and Sitia R. (2016). AQP8 transports NOX2-generated H₂O₂ across the plasma membrane to promote signaling in B cells. *J Leukoc Biol.* 100: 1071-1079. Epub June 2, 2016. IF (2015) 4.165
135. Bernardo M.E., and Aiuti A. (2016). The role of conditioning in hematopoietic stem cell gene therapy. *Hum Gene Ther.* 27:741-748. Epub August 16, 2016. IF (2015) 4.062
136. Stray-Pedersen A. *, Sørmo Sorte H. *, Gambin T. , Samarakoon P. , Chinn I.K., Coban Akdemir Z.H. , Erichsen H.C., Forbes L.R. , Gu S. , Yuan B., Jhangiani S.N., Muzny D.M. , Rødningen O.K., Sheng Y., Nicholas S.K. , Noroski L.M., Seeborg F.O., Davis C. , Canter D., Mace E.M., Vece T., Allen C.E., Abhyankar H.A. , Boone P., Beck C.R., Wiszniewski W.K., Fevang B., Aukrust P., Tjønnfjord G.E. , Gedde-Dahl T., Hjorth-Hansen H. , Dybedal I., Jørgense S.F., Abrahamsen T.G., Øverland T., Skogen V., Osnes L.T., Kulseth M.A. , Prescott T.E., Rustad C.F. , Heimdal K.R. , Belmont J.W. , Rider N., Chinen J., Cao T., Smith E. , Caldirola M.S., Bezrodnik L., Lugo Reyes S.O. , Espinosa Rosales F.J., Guererro D., Pedroza L.A., Poli C.M., Franco J.L., Trujillo Vargas C.M., Aldave Becerra J.C., Wright N., Issekutz T.B., Issekutz A.C., Abbott J., Caldwell J.W., Bayer D., Chan A., Aiuti A., Holmberg E., Karaca E., Yesil G., Artac H., Bayram Y., Atik M.M., Eldomery M.K.I., Ehlayel M.S., Jolles S., Bertuch A.A., Hanson C.I., Zhang, V.W. , Wong L.J., M. Walkiewich, Yang Y., Eng C., Boerwinkle E.A., Gibbs R.A., Shearer W.T., Lyle R., Orange J.S., and Lupski J.R.(2017). Primary immunodeficiency diseases – genomic approaches delineate heterogeneous

- Mendelian disorders. *J Allergy Clin Immunol.* 139: 232-245. Epub July 16,2016. IF (2016) 13.081
137. Brigida I, Chiriaco M, Di Cesare S, Cittaro D, Di Matteo G, Giannelli S, Lazarevic D, Zoccolillo M, Stupka E, Jenkner A, Francalanci P, Livadiotti S, Morawski A, Ravell J, Lenardo M, Cancrini C, Aiuti A, Finocchi A. (2017). Large deletion of MAGT1 gene in a patient with classic Kaposi Sarcoma, CD4 lymphopenia, and EBV infection. *J Clin Immunol* 37:32-35. Epub October 21, 2016. IF (2016) 3.253
138. Speckmann C, Doerken S, Aiuti A, Albert MH, Al-Herz W, Allende LM, Scarselli A, Avcin T, Perez-Becker R, Cancrini C, Cant A, Di Cesare S, Finocchi A, Fischer A, Gaspar HB, Ghosh S, Gennery A, Gilmour K, González-Granado LI, Martinez-Gallo M, Hambleton S, Hauck F, Hoenig M, Moshous D, Neven B, Niehues T, Notarangelo L, Picard C, Rieber N, Schulz A, Schwarz K, Seidel MG, Soler-Palacin P, Stepensky P, Strahm B, Vraetz T, Warnatz K, Winterhalter C, Worth A, Fuchs S, Uhlmann A, Ehl S; PCID-study of the Inborn Errors Working Party of the EBMT. (2017). A prospective study on the natural history of patients with profound combined immunodeficiency (P-CID): an interim analysis. *J Allergy Clin Immunol.* 139: 1302-1310. Epub 2016 Sep 19. IF (2016) 13.081
139. Sauer AV, Hernandez RJ, Fumagalli F, Bianchi V, Poliani PL, Dallatomasina C, Riboni E, Politi LS, Tabucchi A, Carlucci F, Casiraghi M, Carriglio N, Cominelli M, Forcellini CA, Barzaghi F, Ferrua F, Minicucci F, Medaglini S, Leocani L, la Marca G, Notarangelo LD, Azzari C, Comi G, Baldoli C, Canale S, Sessa M, D'Adamo P, Aiuti A. (2017). Alterations in the brain adenosine metabolism cause behavioral and neurological impairment in ADA-deficient mice and patients. *Sci Rep* 7:40136. IF (2016) 4.259
140. Carriglio N., Klapwijk J., Hernandez R.J., Vezzoli M., Chanut F., Lowe R., Elena D., Nord M., Albertini P., Cristofori P., Richards J., Staton H., Appleby J., Aiuti A.,and Sauer A.V. (2017). Good laboratory practice preclinical safety studies for GSK2696273 (MLV vector-based ex vivogene therapy for adenosine deaminase deficiency severe combined immunodeficiency) in NSG mice. *Hum Gene Ther Clin Dev.* 28:17-27. IF (2016) 2.490
141. Zonari E., Desantis G., Petrillo C., Boccalatte F.E., Lidonnici M.R., Kajaste-Rudnitski A., Aiuti A., Ferrari G., Naldini L., and Gentner B. (2017). Efficient ex vivo engineering and expansion of highly purified human hematopoietic stem and progenitor cell populations for gene therapy. *Stem Cell Reports.* 8: 977-990. Epub March 13,2017. IF (2016) 7.338
142. Aiuti A., Roncarolo M.G. and Naldini L. (2017). Gene therapy for ADA-SCID, the first marketing approval of an ex vivo gene therapy in Europe: paving the road for the next generation of advanced therapy medicinal products. *EMBO Mol Med.* 9: 737-740. Epub April 10, 2017. IF (2016) 9.249
143. Chiriaco M., Brigida I., Ariganello P., Di Cesare S., Di MAtteo G., Taus F., Cittaro D., Lazarevic D., Scarselli A., Santilli V., Attardi E., Stupka E., Giannelli S., Fraziano M., Finocchi A., Rossi P., Aiuti A., Palma P. and Cancrini C. (2017). The case of an APDS patient: defects in maturation and function and decreased in vitro anti-mycobacterial activity in the myeloid compartment. *Clin Immunol.* 178, 20-28. Epub 2015 December 28. I.F. (2016) 3.990
144. Penati R.* , Fumagalli F.* , Calbi V., Bernardo M.E., and Aiuti A. (2017).Gene therapy for lysosomal storage disorders: recent advances for metachromatic leukodystrophy and mucopolysaccharidosis I. (2017). *J Inherit Metab Dis.* 40:543-554. * Epub 2017 May 30. *Equal contribution. IF (2016) 3.970
145. Basso-Ricci L., Scala S., Milani R., Migliavacca M., Rovelli A., Bernardo M.E., Ciceri F., Aiuti A., and Biasco L. (2017). Multiparametric Whole Blood Dissection: A one-shot comprehensive picture of the human hematopoietic system. *Cytometry A.* 91: 952-965. Epub June 13, 2017. IF (2016) 3.222
146. Starc N., Ingo D., Conforti A., Rossella V., Tomao L., Pitisci A., De Mattia F., Brigida I., Algeri M., Montanari M., Giuseppe Palumbo G., Merli P., Rossi P., Aiuti A., Locatelli F., and Bernardo M.E. (2017). Biological and functional characterization of bone marrow-

- derived mesenchymal stromal cells from patients affected by primary immunodeficiency. *Sci Rep.* 7:8153. IF (2016) 4.259
147. Azario I., Pievani A., Del Priore F., Antolini L., Santi L., Corsi A., Cardinale L., Sawamoto K., Kubaski F., Gentner B., Bernardo M.E., Valsecchi M.G., Riminucci M., Tomatsu S., Aiuti A., Biondi A., and Serafini M. (2017) Neonatal umbilical cord blood transplantation halts skeletal disease progression in the murine model of MPS-I. *Sci Rep.* 7: 9473. IF (2016) 4.259
148. Maass P.G., Glažar P., Memczak S., Dittmar G., Hollfinger I., Schreyer L., Sauer A.V., Toka O., Aiuti A., Luft F.C., and Rajewsky N. (2017). A map of human circular RNAs in clinically relevant tissues. *J Mol Med (Berl).* 95:1179-1189. IF (2016) 4.686
149. Ferrua F., and Aiuti A. (2017). Twenty-five years of gene therapy for ADA-SCID: from bubble babies to an approved drug. *Hum Gene Ther.* 28:972-981. IF (2016) 4.187
150. Maccari M.E., Scarselli A., Di Cesare S., Floris M., Angius A., Deodati A., Chiriaco M., Cambiaso P., Corrente S., Colafati G.S., Utz P.J., Angelini F., Fierabracci A., Aiuti A., Carsetti R., Rosenberg J.M., Cappa M., Rossi P., Bacchetta R., and Cancrini C. (2017). Severe Toxoplasma gondii infection in a member of a NFKB2-deficient family with T and B cell dysfunction. *Clin Immunol.* 183:273-277. IF (2016) 3.990
151. Barzaghi F., Amaya Hernandez L.C., Neven B., Ricci S., Kucuk Z.Y., Bleesing J., Nademi Z., Slatter M.A., Ulloa E.R., Shcherbina A., Roppelt A., Worth A., Silva J., Aiuti A., Murguia-Favela L., Speckmann C., Carneiro-Sampaio M., Fernandes J.F., Baris S., Ozen A., Karakoc-Aydiner E., Kiykim A., Schulz A., Steinmann S., Notarangelo L.D., Gambineri E., Lionetti P., Shearer W.T., Forbes L., Martinez C., Moshous D., Blanche S., Fisher A., Ruemmele F.M., Tissandier C., Ouachee-Chardin M., Rieux-Lauzier F., Cavazzana M., Qasim W., Lucarelli B., Albert M.H., Kobayashi I., Alonso L., Diaz De Heredia C., Kanegane H., Lawitschka A., Seo J.J., Gonzalez-Vicent M., Diaz M.A., Goyal R.K., Sauer M.G., Yesilipek A., Kim M., Yilmaz-Demirdag Y., Bhatia M., Khlevner J., Richmond Padilla E.J., Martino S., Montin D., Neth O., Molinos-Quintana A., Valverde-Fernandez J., Broides A., Pinsk V., Ballauf A., Haerynck F., Bordon V., Dhooge C., Garcia-Lloret M.L., Bredius R.G., Kałwak K., Haddad E., Seidel M.G., Duckers G., Pai S.Y., Dvorak C.C., Ehl S., Locatelli F., Goldman F., Gennery A.R., Cowan M.J., Roncarolo M.G., Bacchetta R.; PIDTC and IEWP of EBMT. (2017). Long-term follow up of IPEX syndrome patients after different therapeutic strategies: an international multicenter retrospective study. *J Allergy Clin Immunol.* Epub 2017 Dec. 11. IF (2016) 13.081
152. Cicalese M.P., Ferrua F., Castagnaro L., Rolfe K., De Boever E., Reinhardt R.R., Appleby J., Roncarolo M.G., and Aiuti A. (2018). Gene therapy for adenosine deaminase deficiency: a comprehensive evaluation of short-and medium-term safety. *Mol Ther.* Epub 2018 Jan 4. IF (2016) 15.093
153. Calbi V., Fumagalli F., Consiglieri G., Penati R., Acquati S., Redaelli D., Attanasio V., Facchini M., Cicalese M.P., Migliavacca M., Barzaghi F., Ferrua F., Assanelli A., Silvani P., Zoccolillo M., Chidini G., Chiesa R., Arora R., Ciotti F., Sarzana M., Antonioli G., Baldoli C., Morena F., Martino S., Ardissino G.L., Natali Sora M.G., Naldini L., Ciceri F., Aiuti A., and Bernardo M.E. (2018). Use of Defibrotide to help prevent post-transplant endothelial injury in a genetically predisposed infant with metachromatic leukodystrophy undergoing hematopoietic stem cell gene therapy. *Bone Marrow Transplant.* Epub 2018 Jan 29. IF (2016) 3.874
154. Chiriaco M., Casciano F., Di Matteo G., Gentner B., Claps A., Cotugno N., Argenio P., Rossi P., Aiuti A., and Finocchi A. (2018). Impaired X-CGD T cell compartment is gp91phox-NADPH oxidase independent. *Clin Immunol.* Epub 2018 Feb 2. IF (2016) 3.990
155. Sereni L., Castiello M.C., Marangoni F., Anselmo A., di Silvestre D., Motta S., Draghici E., Mantero S., Thrasher A.J., Giliani S., Aiuti A., Mauri P., Notarangelo L.D., Bosticardo M., and Villa A. (2018). Autonomous role of Wiskott-Aldrich Syndrome platelet deficiency in

- inducing autoimmunity and inflammation. *J Allergy Clin Immunol.* Epub 2018 Feb. 5. IF (2016) 13.081
156. Cervantes-Luevano K.E., Caronni N., Castiello M.C., Fontana E., Piperno G., Naseem A., Uva P., Bosticardo M., Marcovecchio G.E., Notarangelo L.D., Cicalese M.P., Aiuti A., Villa A., and Benvenuti F. (2018). Neutrophils drive type-I interferon production and autoantibodies in Wiskott-Aldrich syndrome. *J Allergy Clin Immunol.* Epub 2018 Feb. 12. IF (2016) 13.081
157. Migliavacca M, Assanelli A, Ponzoni M, Pajno R, Barzaghi F, Giglio F, Ferrua F, Frittoli M, Brigida I, Dionisio F, Nicoletti R, Casiraghi M, Roncarolo MG, Doglioni C, Peccatori J, Ciceri F, Cicalese MP, Aiuti A. (2018). First occurrence of plasmablastic lymphoma in Adenosine Deaminase-Deficient Severe Combined Immunodeficiency Disease patient and review of the literature. *Front Immunol.* 2018 Feb 2;9:113. IF (2016) 6.429
158. Maccari ME, Abolhassani H, Aghamohammadi A, Aiuti A, Aleinikova O, Bangs C, Baris S, Barzaghi F, Baxendale H, Buckland M, Burns SO, Cancrini C, Cant A, Cathébras P, Cavazzana M, Chandra A, Conti F, Coulter T, Devlin LA, Edgar JDM, Faust S, Fischer A, Prat MG, Hammarström L, Heeg M, Jolles S, Karakoc-Aydiner E, Kindle G, Kiykim A, Kumararatne D, Grimbacher B, Longhurst H, Mahlaoui N, Milota T, Moreira F, Moshous D, Mukhina A, Neth O, Neven B, Nieters A, Olbrich P, Ozen A, Schmid JP, Picard C, Prader S, Rae W, Reichenbach J, Rusch S, Savic S, Scarselli A, Scheible R, Sediva A, Sharapova SO, Shcherbina A, Slatter M, Soler-Palacin P, Stanislaski A, Suarez F, Tucci F, Uhlmann A, van Montfrans J, Warnatz K, Williams AP, Wood P, Kracker S, Condliffe AM, Ehl S. (2018). Disease Evolution and Response to Rapamycin in Activated Phosphoinositide 3-Kinase δ Syndrome: The European Society for Immunodeficiencies-Activated Phosphoinositide 3-Kinase δ Syndrome Registry. *Front Immunol.* 2018 Mar 16;9:543. IF (2016) 6.429
159. Stirnadel-Farrant H, Kudari M, Garman N, Imrie J, Chopra B, Giannelli S, Gabaldo M, Corti A, Zancan S, Aiuti A, Cicalese MP, Batta R, Appleby J, Davinelli M, Ng P. (2018). Gene therapy in rare diseases: the benefits and challenges of developing a patient-centric registry for Strimvelis in ADA-SCID. *Orphanet J Rare Dis.* 2018; 13:49. IF (2016): 3.478

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

Publications on Italian journals:

1. Aiuti A., Rinaldi M., La Porta C., Fantoni A. e Citarella F. (1990). Production of Hageman factor (human FXII) in simian cells by transient expression of its cDNA. *Rend. Accademia Lincei.* 9, 1, 91-97.
2. Forte P., Aiuti A., Nisini R., D'Amelio R. e Fantoni A. (1991). Produzione in vitro di peptidi virus specifici. In: "Oligonucleotidi sintetici nello studio della biologia molecolare di HIV-1", Ferrara. Editori: R. Gambari e C. Nastruzzi; p. 35-40.
3. Biselli R., Nisini R., Matricardi P.M., Aiuti A. e D'Amelio R. (1992). Clonotypic analysis in different physiopathological conditions. *Immunol. Clin.* XI, 175-183.
4. Fantoni A., Forte P., Aiuti A. e Pozzi L. (1993). Topi transgenici. In: Attuali metodi in genetica molecolare. Ed. C. Morandi, M. Mottes, P.F. Pignatti, M.G. Romanelli, A.Turco. P. 44-45
5. Scala E., Aiuti A., Ansotegui I.J. e Paganelli R. (1993.) Identificazione dei geni responsabili di tre forme di immunodeficienza primitiva legata al cromosoma X. Analisi

- critica delle nuove acquisizioni pubblicate. Giorn. Ital. di Allerg. e Immun. Clin. 3, 287-294.
6. Aiuti A., Notarangelo L., Dolcini F., Casorati G., Bernardi M., Ficara F., Lietti G., Porta F., Ferrari G., e Bordignon C. (1998). Terapia genica e malattia ereditaria. In: "Medicina Perinatale '98", Bari, Ed. A. Mautone, L. Selvaggi. P. 134-136.
 7. Deola S., and Aiuti A. (2005). Gene therapy. A matter of realism. (Terapia genica. Una questione di realismo). Journal of Medicine and The Person 3, 54-56.
 8. Aiuti F., Aiuti A., Calza L., Chiodo F., De Santis W., D'Ettorre G., Emmi L., Isgrò A., Luzi G., Maggi E., Marziali M., Mezzaroma I., Montroni M., Muscaritoli M., Paganelli R., Pandolfi F., Starnino S., Sirianni M.C., Spadarò G., e Vullo V. (2006). Linee Guida per la diagnosi e terapia dell'Immunodeficienza Comune Variabile. It J. Allergy Clin Immunol, 16 (Suppl. Al. N.1), 1-30.
 9. Aiuti A., Cappelli B., Biffi A., Marktel S., and Roncarolo M.G. (2009). Gene therapy in pediatrics. Minerva Pediatr. 61, 775-778.
 10. Aiuti A. (2009). Pediatric testing and primary immunodeficiencies. Minerva Pediatr. 61, 785-787.

Book chapters:

1. Aiuti A., Bordignon C. "Gene Therapy for Severe combined Immunodeficiencies". (1999) in: Gene Therapy: Principles and Applications. Ed T. Blankenstein. Birkhauser. Pages 105-122.
2. Aiuti A. "Gene therapy with hematopoietic stem cells: new perspectives for the treatment of blood-borne genetic disorders". (2007) in: XXXIII Seminario sulla Evoluzione Biologica e i Grandi Problemi della Biologia. Accademia Nazionale dei Lincei. Pages 79-84.
3. Aiuti A. "Terapia Genica" (2007) in: Enciclopedia Italiana di Scienze, Lettere ed Arti. XXI Secolo. VII Appendice. Pages 351-355. Istituto della Enciclopedia Italiana fondata da Giovanni Treccani, Roma.
4. Sauer A., Cassani B., and Aiuti A. "Primary Immunodeficiencies" (2009) in: Guide to Human Gene Therapy. Chapter 13. Editor Roland W. Herzog, Sergei Zolotuklin. Publisher World Scientific. First edition.
5. Brigida I., and Aiuti A. "Immune reconstitution after gene therapy for adenosine deaminase severe combined immunodeficiency (ADA-SCID)" (2012) in: The CliniBook: Clinical gene transfer. Ed. Cohen-Hagenauer. EDK Paris.
6. Ravelli A., Aiuti A., Dallapiccola B., Petralia P. "Il reclutamento dei ricercatori clinici negli Istituti di ricovero e cura a carattere scientifico" (2016) in: Il futuro della ricerca clinica (pediatrica) – Problemi, prospettive, proposte. Società Italiana di Ricerca Pediatrica (SIRP) Onlus. Pag. 71-83. Giannini Editore, Napoli.
7. Cicalese M.P., and Aiuti A. "Gene therapy for primary immunodeficiencies" in: Management of infections in the immunocompromised host. Editor Brahm Segal. Springer International Publishing AG, Cham (in press).

BIOGRAPHICAL SKETCH

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

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 Committee 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 “Il Foresight training course” (Pavia, September 2 to 4, 2009)
- AIFA Annual Meeting on Independent Research (Rome, October 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 integration 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 Teleangiectasia, 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
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., Tuszyński 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
54. Booth C., Hershfieeld M., Notarangelo L., Buckley R., Hoenig M., Mahlaoui N., Cavazzana-Calvo M., Aiuti A., and Gaspar H. B. (2007). Management options for adenosine deaminase deficiency; 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
56. Cattoglio C., Facchini G., Sartori D., Antonelli A., Miccio A., Cassani B., Schmidt M., von Kalle C., Howe S., Thrasher A. J., Aiuti A., Ferrari G., Recchia A., and Mavilio F. (2007). Hot spots of retroviral integration in human CD34+ hematopoietic cells. *Blood* 110, 1770-1778. IF 10.370

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., Vena 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
59. Husain M., Grunebaum E., Naqvi A., Atkinson A., Ngan B. Y., Aiuti A., and Roifman C. M. (2007). Burkitt's lymphoma in a patient with adenosine deaminase deficiency-severe combined immunodeficiency treated with polyethylene glycol-adenosine deaminase. *J Pediatr* 151, 93-95. IF 3.991
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
61. Benninghoff U., Cattaneo F., Aiuti A., Flores-D'Arcais A., Gelmetti C., Viscardi M., Callegaro L., Mirolo M., Ambrosi A., Roncarolo M. G., and Bacchetta R. (2008). Clinical improvement and normalized Th1 cytokine profile in early and long-term interferon-alpha treatment in a suspected case of hyper-IgE syndrome. *Pediatr Allergy Immunol* 19, 564-568. IF 2.454
62. Cassani B., Mirolo M., Cattaneo F., Benninghoff U., Hershfield M., Carlucci F., Tabucchi A., Bordignon C., Roncarolo M.G., and Aiuti A. (2008). Altered intracellular and extracellular signaling leads to impaired T-cell functions in ADA-SCID patients. *Blood* 111, 4209-19. IF 10.896
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
64. Aiuti A., Brigida I., Ferrua F., Cappelli B., Chiesa R., Marktel S., and Roncarolo M.G. (2009). Hematopoietic stem cell gene therapy for adenosine deaminase deficient-SCID. *Immunol Res* 44, 150-159. IF 2.364
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
66. Aiuti A., and Roncarolo M.G. (2009). Ten years of gene therapy for primary immune deficiencies. *Hematology Am Soc Hematol Educ Program*. 2009, 682-689. IF 1.333
67. Bosticardo M., Marangoni F., Aiuti A., Villa A., and Roncarolo M.G. (2009). Recent advances in understanding the pathophysiology of Wiskott-Aldrich syndrome. *Blood* 113, 6288-6295. IF 10.896
68. Cassani B., Montini E., Maruggi G., Ambrosi A., Mirolo M., Selleri S., Biral E., Frugnoli I., Hernandez-Trujillo V., Di Serio C., Roncarolo M. G., Naldini L., Mavilio F., and Aiuti A. (2009). Integration of retroviral vectors induces minor changes in the transcriptional activity of T cells from ADA-SCID patients treated with gene therapy. *Blood* 114, 3546-3556. IF 10.896
69. Chiesa R., Cappelli B., Crocchiolo R., Frugnoli I., Biral E., Noe A., Evangelio C., Fossati M., Roccia T., Biffi A., Finizio V., Aiuti A., Broglia M., Bartoli A., Ciceri F., Roncarolo M. G., and Marktel S. (2010). Unpredictability of iv Busulfan pharmacokinetics in children

- undergoing hematopoietic stem cell transplant for advanced beta thalassemia: Limited toxicity with a dose adjustment policy. *Biol Blood Marrow Transplant.* 16, 622-628. Epub Dec 4, 2009. IF 3.732
70. Gabriel R., Eckenberg R., Paruzynski A., Bartholomae C. C., Nowrouzi A., Arens A., Howe S. J., Recchia A., Cattoglio C., Wang W., Faber K., Schwarzwaelder K., Kirsten R., Deichmann A., Ball C. R., Balaggan K. S., Yanez-Munoz R. J., Ali R. R., Gaspar H. B., Biasco L., Aiuti A., Cesana D., Montini E., Naldini L., Cohen-Haguenauer O., Mavilio F., Thrasher A. J., Glimm H., von Kalle C., Saurin W., and Schmidt M. (2009). Comprehensive genomic access to vector integration in clinical gene therapy. *Nat Med.* 15, 1431-1436. IF 27.136
71. Gaspar H. B., Aiuti A., Porta F., Candotti F., Hershfield M. S., and Notarangelo L. D. (2009). How I treat ADA deficiency. *Blood* 114, 3524-3532. IF 10.896
72. Locci M., Draghici E., Marangoni F., Bosticardo M., Catucci M., Aiuti A., Cancrini C., Marodi L., Espanol T., Bredius R. G., Thrasher A. J., Schulz A., Litzman J., Roncarolo M. G., Casorati G., Dellabona P., and Villa A. (2009). The Wiskott-Aldrich syndrome protein is required for iNKT cell maturation and function. *J Exp Med* 206, 735-742. IF 15.162
73. Marangoni F., Bosticardo M., Charrier S., Draghici E., Locci M., Scaramuzza S., Panaroni C., Ponzoni M., Sanvito F., Doglioni C., Liabeuf M., Gjata B., Montus M., Siminovitch K., Aiuti A., Naldini L., Duprè L., Roncarolo M. G., Galy A., and Villa A. (2009). Evidence for long-term efficacy and safety of gene therapy for Wiskott-Aldrich syndrome in preclinical models. *Mol Ther* 17, 1073-1082. IF 5.896
74. Sauer A.V., and Aiuti A. (2009) New insights into the pathogenesis of adenosine deaminase-severe combined immunodeficiency and progress in gene therapy. *Curr Opin Allergy Clin Immunol* 9, 496-502. IF 3.497
75. Sauer A. V., Mrak E., Hernandez R. J., Zucchi E., Cavani F., Casiraghi M., Grunebaum E., Roifman C. M., Cervi M. C., Ambrosi A., Carlucci F., Roncarolo M. G., Villa A., Rubinacci A., and Aiuti A. (2009). ADA-deficient SCID is associated with a specific microenvironment and bone phenotype characterized by RANKL/OPG imbalance and osteoblast insufficiency. *Blood* 114, 3216-3226. IF 10.896
76. Trifari S., Scaramuzza S., Catucci M., Ponzoni M., Mollica L., Chiesa R., Cattaneo F., Lafouresse F., Calvez R., Vermi W., Medicina D., Castiello M.C., Marangoni F., Bosticardo M., Doglioni C., Caniglia M., Aiuti A., Villa A., Roncarolo M.G., and Dupré L. (2010). Revertant T lymphocytes in a patient with Wiskott-Aldrich syndrome: Analysis of function and distribution in lymphoid organs. *J Allergy Clin Immunol.* 125, 439-448.e8. IF 9.273
77. Cappelli B., and Aiuti A. (2010). Gene therapy for adenosine deaminase deficiency. *Immunol Allergy Clin. North Amer.* 30, 249-260. IF 2.959
78. Cancrini C., Ferrua F., Scarselli A., Brigida I., Romiti M.L., Barera G., Finocchi A., Roncarolo M.G., Caniglia M., and Aiuti A. (2010). Role of reduced intensity conditioning in T-cell and B-cell immune reconstitution after HLA-identical bone marrow transplantation in ADA-SCID. *Haematologica.* 95, 1778-1782. IF 6.532
79. Ferrua F., Brigida I., and Aiuti A. (2010). Update on gene therapy for adenosine deaminase-deficient severe combined immunodeficiency. *Curr Opin Allergy Clin Immunol* 10, 551-556. IF 3.431
80. Cohen-Haguenauer O., Creff N., Cruz P., Tunc C., Aiuti A., Baum C., Bosch F., Blomberg P., Cichutek K., Collins M., Danos O., Dehaut F., Federspiel M., Galun E., Garritsen H., Hauser H., Hildebrandt M., Klatzmann D., Merten O., Montini E., O'Brien T., Panet A., Rasooly L., Scherman D., Schmidt M., Schweitzer M., Tiberghien P., Vandendriessche T., Ziehr H., Ylä-Herttuala S., von Kalle C., Gahrton G., and Carrondo M. (2010). Relevance of an Academic GMP Pan-European Vector Infra-structure (PEVI). *Curr Gene Ther.* 10, 414-422. IF 4.482
81. Biasco L., Ambrosi A., Pellin D., Bartholomae C., Brigida I., Roncarolo M.G., Di Serio C., von Kalle C., Schmidt M., and Aiuti A. (2010) Integration profile of retroviral vector in

- gene therapy treated patients is cell-specific according to gene expression and chromatin conformation of target cell. *EMBO Mol Med.* 3, 89-101. IF 10.333
82. Selleri S., Brigida I., Casiraghi M., Scaramuzza S., Cappelli B., Cassani B., Ferrua F., Aker M., Slavin S., Scarselli A., Cancrini C., Marktel S., Roncarolo M.G., and Aiuti A. (2011). In vivo T-cell dynamics during immune reconstitution after hematopoietic stem cell gene therapy in adenosine deaminase severe combined immune deficiency. *J Allergy Clin Immunol.* 127, 1368-1375. IF 11.003
83. Grunebaum E., Chung C.T., Dadi H., Kim P., Brigida I., Ferrua F., Cicalese M.P., Aiuti A., and Roifman C.M. (2011). Purine metabolism, immune reconstitution, and abdominal adipose tumor after gene therapy for adenosine deaminase deficiency. *J Allergy Clin Immunol.* 127, 1417-1419. IF 11.003
84. Bosticardo M., Draghici E., Schena F., Sauer A.V., Fontana E., Castiello M.C., Catucci M., Locci M., Naldini L., Aiuti A., Roncarolo M.G., Poliani P.L., Traggiai E., and Villa A. (2011) Lentiviral-mediated gene therapy leads to improvement of B-cell functionality in a murine model of Wiskott-Aldrich syndrome. *J Allergy Clin Immunol.* 127, 1376-1384. IF 11.003
85. Angelino G., Natali G.L., Falappa P., Folgori L., Moretti R., Cantarutti N., Di Matteo G., Chiriaco M., Rossi P., Roos D., Aiuti A., and Finocchi A. (2011) Successful treatment with percutaneous transhepatic alcoholization of a liver abscess in a child with chronic granulomatous disease. *Pediatr Infect Dis J.* 30, 819-820. IF 3.064
86. Deichmann A., Brugman M.H., Bartholomae C.C., Schwarzwaelter K., Verstegen M.M., Howe S.J., Arens A., Ott M.G., Hoelzer D., Seger R., Grez M., Hacein-Bey-Abina S., Cavazzana-Calvo M., Fischer A., Paruzynski A., Gabriel R., Glimm H., Abel U., Cattoglio C., Mavilio F., Cassani B., Aiuti A., Dunbar C.E., Baum C., Gaspar H.B., Thrasher A.J., von Kalle C., Schmidt M., and Wagemaker G. (2011). Insertion sites in engrafted cells cluster within a limited repertoire of genomic areas after gammaretroviral vector gene therapy. *Mol Ther.* 19, 2031-2039. IF 6.873
87. Cancrini C., Scarselli A., Scaramuzza S., Chiriaco M., Di Cesare S., Di Matteo G., Romiti M.L., Palma P., De Felice L., Palumbo G., Pinto R.M., De Vito R., Racioppi L., Livadiotti S., Fischer A., Rossi P., Caniglia M., and Aiuti A. (2011). Early-onset monocyte-B-natural killer-dendritic cells' deficiency successfully treated with hematopoietic stem cell transplantation. *J Allergy Clin Immunol.* 128, 897-900. IF 11.003
88. Catucci M., Prete F., Bosticardo M., Castiello M.C., Draghici E., Locci M., Roncarolo M.G., Aiuti A., Benvenuti F., and Villa A. (2012). Dendritic cell functional improvement in a preclinical model of lentiviral-mediated gene therapy for Wiskott-Aldrich syndrome. *Gene Ther.*, 19, 1150-1159. Epub 2011 Dec 22. IF 4.538
89. Sauer A.V., Brigida I., Carriglio N., Jofra Hernandez R., Scaramuzza S., Clavenna D., Sanvitto F., Poliani P.L., Gagliani N., Carlucci F., Tabucchi A., Roncarolo M.G., Traggiai E., Villa A., and Aiuti A. (2012). Alterations in the adenosine metabolism and CD39/CD73 adenosinergic machinery cause loss of Treg cell function and autoimmunity in ADA-deficient SCID. *Blood.* 119, 1428-1439. IF 9.898
90. Mariani S.A., Brigida I., Kajaste-Rudnitski A., Vicenzi E., Aiuti A., and Poli G. (2012). HIV-1 envelope dependent post-entry restriction of CXCR4-using viruses in children but not adult derived CD4+ T lymphocytes. *Blood.* 119, 2013-2023. IF 9.898
91. Biasco L., Baricordi C., and Aiuti A. (2012). Retroviral integrations in gene therapy trials. *Mol. Ther.* 20, 709-716. IF 6.873
92. Sauer A.V., Morbach H., Brigida I., Ng Y.S., Aiuti A., and Meffre E. (2012). Defective B cell tolerance due to adenosine deaminase deficiency is corrected by gene therapy. *J Clin Investig.* 122, 2141-2152. IF 13.069
93. Corrigan-Curay J., Cohen-Haguenuer O., O'Reilly M., Ross S.R., Fan H., Rosenberg N., Somia N., King N., Friedmann T., Dunbar C., Aiuti A., Naldini L., Baum C., von Kalle C., Kiem H.P., Montini E., Bushman F., Sorrentino B.P., Carrondo M., Malech H., Gahrton G.,

- Shapiro R., Wolff L., Rosenthal E., Jambou R., Zaia J., and Kohn D.B. (2012). Challenges in Vector and Trial Design using Retroviral vectors for Long Term Gene Correction in Hematopoietic Stem Cell Gene Therapy: Summary of a Symposium Sponsored by the NIH Office of Biotechnology Activities and the EC DG-research NoE for the Advancement of Clinical Gene Transfer and Therapy. *Mol Ther.* 20, 1084-1094. IF 6.873
94. Vago L., Oliveira G., Bondanza A., Noviello M., Solati C., Ghio D., Brigida I., Greco R., Lupo Stanghellini M.T., Peccatori J., Fracchia S., Del Fiacco M., Traversari C., Aiuti A., Del Maschio A., Bordignon C., Ciceri F., and Bonini C. (2012). T cell suicide gene therapy prompts thymic renewal in adults after hematopoietic stem cell transplantation. *Blood* 120, 1820-30. IF 9.898
95. Hassan A., Booth C., Brightwell A., Allwood Z., Veys P., Rao K., Hoenig M., Friedrich W., Gennery A.R., Slatter M., Bredius R., Finocchi A., Cancrini C., Aiuti A., Lanfranchi A., Porta F., Ridella M., Graham Steward C., Filipovich A., Marsh R., Bordon V., Al-Muhsen S., Al-Mousa H., Alsum Z., Al-Dhekri H., Al Ghonaium A., Speckmann C., Fischer A., Mahlaoui N., Nichols K. E., Grunebaum E., Al Zaharani D., Roifman C., Boelens J.J., Davies E. G., Cavazzana-Calvo M., Notarangelo L., and Gaspar H.B. (2012). Outcome of hematopoietic stem cell transplantation for adenosine deaminase-deficient severe combined immunodeficiency. *Blood* 120, 3615-24. IF 9.898
96. Aiuti A., Bacchetta R., Seger R., Villa A., Cavazzana-Calvo M. (2012) Gene therapy for primary immunodeficiencies: Part 2 *Curr. Opin. Immunol.*, 24, 585-591. IF 9.522
97. Cavazzana-Calvo M., Fischer A., Hacein-Bey-Abina S., Aiuti A. (2012) Gene therapy for primary immunodeficiencies: Part 1. *Curr. Opin. Immunol.* 24, 580-584. IF 9.522
98. Sauer A.V., Brigida I., Carriglio N., Aiuti A. (2012) Autoimmune dysregulation and purine metabolism in adenosine deaminase deficiency. *Front. Immunol.* 3, 265. IF N.A.
99. Scaramuzza S., Biasco L., Ripamonti A., Castiello M.C., Loperfido M., Draghici E., Jofra Hernandez R., Benedicenti F., Radrizzani M., Salomoni M., Ranzani M., Bartholomae CC., Vicenzi E., Finocchi A., Bredius R., Bosticardo M., Schmidt M., von Kalle C., Montini E., Biffi A., Roncarolo M.G., Naldini L., Villa A., and Aiuti A. (2013). Preclinical safety and efficacy on human CD34+ cells transduced with lentiviral vector for the treatment of Wiskott-Aldrich Syndrome. *Mol Ther.* 21, 175-184. Epub 2012 Feb 28. IF 7.149
100. Prete F., Catucci M., Labrada M., Gobessi S., Castiello M.C., Bonomi E., Aiuti A., Vermi W., Cancrini C., Metin A., Hambleton S., Bredius R., Notarangelo L.D., Van der Burg M., Kalinke U., Villa A., and Benvenuti F. (2013). Wiskott-Aldrich syndrome protein-mediated actin dynamics control type-I interferon production in plasmacytoid dendritic cells. *J. Exp. Med.*, 210, 355-374. IF 13.214
101. Goudy K., Aydin D., Barzaghi F., Gambinieri E., Vignoli M., Doglioni C., Ponzoni M., Cicalese M.P., Assanelli A., Tommasini A., Brigida I., Dellepiane R.M., Martino S., Olek S., Aiuti A., Ciceri F., and Roncarolo M.G. (2013). Human IL2RA null mutation mediates immunodeficiency with lymphoproliferation and autoimmunity. *Clin Immunol.*, 146, 248-261. IF 3.771
102. Salfa I., Cantarutti N., Angelino G., Matteo G.D., Capo V., Farinelli G., Cancrini C., Aiuti A., Palma P., and Finocchi A. (2013). Serratia marcescens osteomyelitis in a newborn with chronic granulomatous disease. *Pediatric Infect Dis J.*, 32: 926. IF 3.569
103. Aiuti A., Biasco L., Scaramuzza S., Ferrua F., Cicalese M.P., Baricordi C., Dionisio F., Calabria A., Giannelli S., Castiello M.C., Bosticardo M., Evangelio C., Assanelli A., Casiraghi M., Di Nunzio S., Callegaro L., Benati C., Rizzardi P., Pellin D., Di Serio C., Schmidt M., Van Kalle C., Gardner J., Mehta N., Nedeva V., Dow D.J., Galy A., Miniero R., Finocchi A., Metin A., Banerjee P., Orange J., Galimberti S., Valsecchi M.G., Biffi A., Montini E., Villa A., Ciceri F., Roncarolo M.G., and Naldini L. (2013). Lentivirus based gene therapy of hematopoietic stem cell in Wiskott-Aldrich Syndrome. *Science* 341(6148):1233151. Epub 2013 Jul 11. I.F. 31.207

104. Biffi A., Montini E., Lorioli L., Cesani M., Fumagalli F., Plati T., Baldoli C., Martino S., Calabria A., Canale S., Benedicenti F., Vallanti G., Biasco L., Leo S., Kabbara N., Zanetti G., Rizzo W.B., Mehta N., Cicalese M.P., Casiraghi M., Boelens J.J., Del Carro U., Dow D.J., Schmidt M., Assanelli A., Neduva V., Di Serio C., Stupka E., Gardner J., Van Kalle C., Bordignon C., Ciceri F., Rovelli A., Roncarolo M.G., Aiuti A., Sessa M. and Naldini L. (2013). Therapeutic benefit in metachromatic leukodystrophy by lentiviral hematopoietic stem cell gene therapy. *Science* 341(6148):1233158. Epub 2013 Jul 11. I.F. 31.207
105. Aiuti A., Cossu G., de Felipe P., Galli M.C., Narayanan G., Renner M., Stahlbom A., Schneider C.K., and Voltz-Girolt C. (2013). The Committee for Advanced Therapies' (CAT) reflection paper on management of clinical risks deriving from insertional mutagenesis. *Hum Gene Ther Clin Dev.* Jul 18. 24:47-54. I.F. 4.019
106. Castiello M.C., Bosticardo M., Pala F., Catucci M., Chamberlain N., van Zelm M.C., Driessen G.J., Pac M., Bernatowska E., Scaramuzza S., Aiuti A., Sauer A.V., Traggiai E., Meffre E., Villa A., van der Burg M. (2014). Wiskott-Aldrich Syndrome protein deficiency perturbs the homeostasis of B-cell compartment in humans. *J Autoimmun.* 50, 42-50. I.F. 8.41
107. Angelino G., Caruso R., D'Argenio P., Calò Carducci F.I., Pascone R., Lanciotti M., Cancrini C., Palma P., Aiuti A., Rossi P., and Finocchi A. (2014). Etiology, clinical outcome, and laboratory features in children with neutropenia: analysis of 104 cases. *Pediatr Allergy Immunol.* 25: 283-289. Epub 2013 Dec 10. IF 3.376
108. Brigida I., Sauer A.V., Ferrua F., Giannelli S., Scaramuzza S., Pistoia V., Castiello M.C., Barendregt B.H., Cicalese M.P., Casiraghi M., Brombin C., Puck J., Müller K., Notarangelo L.D., Montin D., van Montfrans J.M., Roncarolo M.G., Traggiai E., van Dongen J.J.M., van der Burg M., and Aiuti A. (2014). B-cell development and functions and therapeutic options in adenosine deaminase deficient patients. *J Allergy Clin Immunol* 133:799-806. Epub 2014 Feb 5. IF 11.248
109. Finocchi A., Claps A., Serafinelli J., Saifa I., Longo D., Di Matteo G., Aiuti A., and Rossi P. (2014). Chronic granulomatous disease presenting with *Salmonella* brain abscesses. *Pediatr Infect Dis J* 33: 525-528. IF 3.135
110. Farinelli G., Capo V., Scaramuzza S., and Aiuti A. (2014). Lentiviral vectors for the treatment of primary immunodeficiencies. *J Inherit Metab Dis.* 37: 525-533. Epub 2014 Mar 12. IF 4.07
111. Cancrini C., Puliafito P., Digilio M.C., Soresina A., Martino S., Ruga E.M., Rondelli R., Consolini R., Milanesi O., Cardinale F., Finocchi A., Romiti M.L., Martire B., Corsello G., Bacchetta R., Albano V., Pignata C., Azzari C., Carotti A., Specchia F., Montin D., Cirillo E., Cocchi G., Trizzino A., Bossi G., Aiuti A., Pietrogrande M.C., Marino B., Ugazio A.G., Plebani A. and Rossi P. for the Italian Network for Primary Immunodeficiencies (IPINET). (2014). Clinical features of a cohort of 22q11.2 deletion syndrome patients: an Italian multicenter study. *J of Pediatr* 164, 1475-1480.e2. Epub 2014 Mar 20. IF 4.05
112. Chiriaco M., Farinelli G., Capo V., Di Matteo G., Zonari E., Scaramuzza S., Sergi L., Migliavacca M., Hernandez R.J., Bombelli F., Giorda E., Kajaste-Rudnitski A., Trono D., Grez M., Rossi P., Finocchi A., Naldini L., Gentner B., and Aiuti A. (2014). Dual-regulated lentiviral vector for gene therapy of X-linked chronic granulomatosis. *Mol Therapy.* 22, 1472-1483. Epub 2014 May 29. IF. 6.82
113. Sauer A.V., Di Lorenzo B., Carriglio N., and Aiuti A. (2014). Progress in gene therapy for primary immunodeficiencies using lentiviral vectors. *Curr Opin Allergy Clin Immunol.* 14, 527-534. IF 3.659
114. Bosticardo M., Ferrua F., Cavazzana M., and Aiuti A. (2014). Gene therapy for Wiskott Aldrich Syndrome. *Curr Gene Ther.* 14:413-421. Epub 2014 Sep 17. IF 4.096
115. Biasco L., Scala S., Basso Ricci L., Dionisio F., Baricordi C., Calabria A., Giannelli S., Cieri N., Barzaghi F., Pajno R., Al-Mousa H., Scarselli A., Cancrini C., Bordignon C., Roncarolo

- M.G., Monti E., Bonini C., and Aiuti A. (2015). In vivo tracking of T cells in humans unveils decade-long survival and activity of genetically modified T memory stem cells. *Sci Transl Med.* 7: 273ra13. I.F. 14.414
116. Castiello M.C., Scaramuzza S., Pala F., Ferrua F., Uva P., Brigida I., Sereni L., van den Burg M., Ottaviano G., Albert M.H., Roncarolo M.G., Naldini L., Aiuti A., and Bosticardo M. (2015). B-cell reconstitution after lentiviral vector-mediated gene therapy in patients with Wiskott-Aldrich syndrome. *J Allergy Clin Immunol.* 136, 692-702. Epub 2015 March 16. I.F. 11.248
117. Cotugno N., Finocchi A., Cagigi A., Di Matteo G., Chiriaco M., Di Cesare S., Rossi P., Aiuti A., Palma P., and Douagi I. (2015). Defective B-cell proliferation and maintenance of long-term memory in patients with chronic granulomatous disease. *J Allergy Clin Immunol.* 135, 753-761. Epub 2014 Aug 29. IF 11.248
118. Lorioli L., Cicalese M.P., Silvani P., Assanelli A., Salvo I., Mandelli A., Fumagalli F., Fiori R., Ciceri F., Aiuti A., Sessa M., Roncarolo M.G., Lanzani C., and Biffi A. (2015). Abnormalities of acid-base balance and predisposition to metabolic acidosis in Metachromatic Leukodystrophy patients. *Mol Genet Metab.* 115:48-52. Epub 2015 March 6. I.F. 2.827
119. Cicalese M.P., and Aiuti A. (2015). Clinical applications of gene therapy for primary immunodeficiencies. *Hum Gene Ther* 26: 210-219. I.F. 3.623
120. Scarselli A., Di Cesare S., Capponi C., Romiti M.L., Di Matteo G., Simonetti A., Palma P., Finocchi A., Lucarelli B., Pinto R.M., Rana I., Palumbo G., Caniglia M., Rossi P., Carsetti R., Cancrini C., and Aiuti A. (2015). Longitudinal evaluation of immune reconstitution and B-cell function after hematopoietic cell transplantation for primary immunodeficiency. *J Clin Immunol.* 35:373-383. Epub 2015 April 15. I.F. 2.654
121. Di Cesare S., Puliafito P., Ariganello P., Marcovecchio G.E., Mandolesi M., Capolino R., Digilio M.C., Aiuti A., Rossi P., and Cancrini C. (2015). Autoimmunity and regulatory T cells in 22q11.2 deletion syndrome patients. *Pediat Allergy Immunol* 26:591-594. Epub 2015 June 9.I.F.3.397
122. Pala F., Morbach H., Castiello M.C., Schickel J.N., Scaramuzza S., Chamberlain N., Cassani B., Glauzy S., Romberg N., Candotti F., Aiuti A., Bosticardo M., Villa A., and Meffre E. (2015). Lentiviral-mediated gene therapy restores B cell tolerance in Wiskott-Aldrich syndrome patients. *J Clin Invest.* 125: 3941-3951. I.F. 13.262
123. Oliveira G., Ruggiero E., Stanghellini M.T., Cieri N., D'Agostino M., Fronza R., Lulay C., Dionisio F., Mastaglio S., Greco R., Peccatori J., Aiuti A., Ambrosi A., Biasco L., Bondanza A., Lambiase A., Traversari C., Vago L., von Kalle C., Schmidt M., Bordignon C., Ciceri F., and Bonini C. (2015). Tracking genetically engineered lymphocytes long-term reveals the dynamics of T cell immunological memory. *Sci Transl Med.* 7: 317ra198. I.F. 15.843
124. Scarselli A., Di Cesare S., Di Matteo G., De Matteis A., Ariganello P., Romiti M.L., Cascioli S., De Vito R., Bertaina A., Locatelli F., Gaspar H.B., Aiuti A., Rossi P., Gilmour K., and Cancrini C. (2015). Combined immunodeficiency due to JAK3 mutation in a child presenting with skin granuloma. *J Allergy Clin Immunol.* S0091-6749 Epub 2015 November 3. I.F. 11.476
125. Migliavacca M., Assanelli A., Ferrua F., Cicalese M.P., Biffi A., Frittoli M., Silvani P., Chidini G., Calderini E., Mandelli A., Camporesi A., Milani R., Farinelli G., Nicoletti R., Ciceri F., Bernardo M.E., and Aiuti A.(2016). Pioglitazone as a novel therapeutic approach in Chronic Granulomatous Disease. *J Allergy Clin Immunol.* 137: 1913-1915. Epub 2016 April 4. IF (2015) 12.485
126. Brigida I., Scaramuzza S., Lazarevic D., Cittaro D., Ferrua F., Leonardelli L., Alessio M., Forma O., Lanzani C., Viarengo G., Ciceri F., Jankovic M., Pesce F., Aiuti A., and Cicalese M.P. (2016). A novel genomic inversion in Wiskott-Aldrich-associated autoinflammation. *J Allergy Clin Immunol.* 138: 619-622.e.7. Epub 2016 April 22. IF (2015) 12.485
127. Cicalese M.P., Ferrua F., Castagnaro L., Pajno R., Barzaghi F., Giannelli S., Dionisio F., Brigida I., Bonopane M., Casiraghi M., Tabucchi A., Carlucci F., Grunebaum E., Adeli M.,

- Bredius R.G., Puck J.M., Stepensky P., Tezcan I., Rolfe K., De Boever E., Reinhardt R.R., Appleby J., Ciceri F., Roncarolo M.G., and Aiuti A.(2016). Update on the safety and efficacy of retroviral gene therapy for immunodeficiency due to adenosine deaminase deficiency. *Blood*. 128: 45-54. Epub April 29, 2016. IF (2015) 11.841
128. Biasco L., Pellin D., Scala S., Dionisio F., Basso-Ricci L., Leonardelli L., Scaramuzza S., Baricordi C., Ferrua F., Cicalese M.P., Giannelli S., Neduva V., Dow D.J., Schmidt M., Von Kalle C., Roncarolo M.G., Ciceri F., Vicard P., Wit E., Di Serio C., Naldini L., and Aiuti A. (2016). In vivo tracking of human hematopoiesis reveals patterns of clonal dynamics during early and steady-state reconstitution phases. *Cell Stem Cell*. 19: 107-119. Epub May 25, 2016. IF (2015) 22.387
129. Sessa M*, Lorioli L*, Fumagalli F, Acquati S, Redaelli D, Baldoli C, Canale S, Lopez ID, Morena F, Calabria A, Fiori R, Silvani P, Rancoita PM, Gabaldo M, Benedicenti F, Antonioli G, Assanelli A, Cicalese MP, Del Carro U, Natali Sora MG, Martino S, Quattrini A, Montini E, Di Serio C, Ciceri F, Roncarolo MG, Aiuti A, Naldini L, and Biffi A.(2016). Lentiviral haemopoietic stem-cell gene therapy in early-onset metachromatic leukodystrophy: an ad-hoc analysis of a non-randomised, open-label, phase 1/2 trial. *Lancet*. 388: 476-487. Epub June 8, 2016. IF (2015) 44.002 * equal contribution
130. Magnani CF, Turazzi N, Benedicenti F, Calabria A, Tenderini E, Tettamanti S, Giordano Attianese GM, Cooper LJ, Aiuti A, Montini E, Biondi A, and Biagi E. (2016). Immunotherapy of acute leukemia by chimeric antigen receptor-modified lymphocytes using an improved Sleeping Beauty transposon platform. *Oncotarget*. 7: 51581-51597. Epub June 13, 2016. IF (2015) 5.008
131. Aiuti A., and Naldini L. (2016). Safer conditioning for blood stem cell transplants. *Nat Biotechnol* 34: 721-723. IF (2015) 43.113
132. Farinelli G., Hernandez RJ, Rossi A, Ranucci S, Sanvito F, Migliavacca M, Brombin C, Pramov A, Serio CD, Bovolenta C, Gentner B, Bragonzi A, and Aiuti A. (2016). Lentiviral vector gene therapy protects XCGD mice from Acute Staphylococcus aureus pneumonia and inflammatory response. *Mol Ther*. 24: 1873-1880. Epub July 26, 2016. IF (2015) 6.938
133. Ingo D.M., Redaelli D., Rossella V., Perini O., Santoleri L., Ciceri F., Aiuti A., and Bernardo M.E. (2016). Bone marrow-derived CD34- fraction: A rich source of mesenchymal stromal cells for clinical application. *Cytotherapy*. 18: 1560-1563. Epub October 16, 2016. IF (2015) 3.625
134. Bertolotti M., Farinelli G., Galli M., Aiuti A., and Sitia R. (2016). AQP8 transports NOX2-generated H₂O₂ across the plasma membrane to promote signaling in B cells. *J Leukoc Biol*. 100: 1071-1079. Epub June 2, 2016. IF (2015) 4.165
135. Bernardo M.E., and Aiuti A. (2016). The role of conditioning in hematopoietic stem cell gene therapy. *Hum Gene Ther*. 27:741-748. Epub August 16, 2016. IF (2015) 4.062
136. Stray-Pedersen A. *, Sørmo Sorte H. *, Gambin T. , Samarakoon P. , Chinn I.K., Coban Akdemir Z.H. , Erichsen H.C., Forbes L.R. , Gu S. , Yuan B., Jhangiani S.N., Muzny D.M. , Rødningen O.K., Sheng Y., Nicholas S.K. , Noroski L.M., Seeborg F.O., Davis C. , Canter D., Mace E.M., Vece T., Allen C.E., Abhyankar H.A. , Boone P., Beck C.R., Wiszniewski W.K., Fevang B., Aukrust P., Tjønnfjord G.E. , Gedde-Dahl T., Hjorth-Hansen H. , Dybedal I., Jørgense S.F., Abrahamsen T.G., Øverland T., Skogen V., Osnes L.T., Kulseth M.A. , Prescott T.E., Rustad C.F. , Heimdal K.R. , Belmont J.W. , Rider N., Chinen J., Cao T., Smith E. , Caldirola M.S., Bezrodnik L., Lugo Reyes S.O. , Espinosa Rosales F.J., Guererro D., Pedroza L.A., Poli C.M., Franco J.L., Trujillo Vargas C.M., Aldave Becerra J.C., Wright N., Issekutz T.B., Issekutz A.C., Abbott J., Caldwell J.W., Bayer D., Chan A., Aiuti A., Holmberg E., Karaca E., Yesil G., Artac H., Bayram Y., Atik M.M., Eldomery M.K.I., Ehlayel M.S., Jolles S., Bertuch A.A., Hanson C.I., Zhang, V.W. , Wong L.J., M. Walkiewich, Yang Y., Eng C., Boerwinkle E.A., Gibbs R.A., Shearer W.T., Lyle R., Orange J.S., and Lupski J.R.(2017). Primary immunodeficiency diseases – genomic approaches delineate heterogeneous

- Mendelian disorders. *J Allergy Clin Immunol.* 139: 232-245. Epub July 16,2016. IF (2016) 13.081
137. Brigida I, Chiriaco M, Di Cesare S, Cittaro D, Di Matteo G, Giannelli S, Lazarevic D, Zoccolillo M, Stupka E, Jenkner A, Francalanci P, Livadiotti S, Morawski A, Ravell J, Lenardo M, Cancrini C, Aiuti A, Finocchi A. (2017). Large deletion of MAGT1 gene in a patient with classic Kaposi Sarcoma, CD4 lymphopenia, and EBV infection. *J Clin Immunol* 37:32-35. Epub October 21, 2016. IF (2016) 3.253
138. Speckmann C, Doerken S, Aiuti A, Albert MH, Al-Herz W, Allende LM, Scarselli A, Avcin T, Perez-Becker R, Cancrini C, Cant A, Di Cesare S, Finocchi A, Fischer A, Gaspar HB, Ghosh S, Gennery A, Gilmour K, González-Granado LI, Martinez-Gallo M, Hambleton S, Hauck F, Hoenig M, Moshous D, Neven B, Niehues T, Notarangelo L, Picard C, Rieber N, Schulz A, Schwarz K, Seidel MG, Soler-Palacin P, Stepensky P, Strahm B, Vraetz T, Warnatz K, Winterhalter C, Worth A, Fuchs S, Uhlmann A, Ehl S; PCID-study of the Inborn Errors Working Party of the EBMT. (2017). A prospective study on the natural history of patients with profound combined immunodeficiency (P-CID): an interim analysis. *J Allergy Clin Immunol.* 139: 1302-1310. Epub 2016 Sep 19. IF (2016) 13.081
139. Sauer AV, Hernandez RJ, Fumagalli F, Bianchi V, Poliani PL, Dallatomasina C, Riboni E, Politi LS, Tabucchi A, Carlucci F, Casiraghi M, Carriglio N, Cominelli M, Forcellini CA, Barzaghi F, Ferrua F, Minicucci F, Medaglini S, Leocani L, la Marca G, Notarangelo LD, Azzari C, Comi G, Baldoli C, Canale S, Sessa M, D'Adamo P, Aiuti A. (2017). Alterations in the brain adenosine metabolism cause behavioral and neurological impairment in ADA-deficient mice and patients. *Sci Rep* 7:40136. IF (2016) 4.259
140. Carriglio N., Klapwijk J., Hernandez R.J., Vezzoli M., Chanut F., Lowe R., Elena D., Nord M., Albertini P., Cristofori P., Richards J., Staton H., Appleby J., Aiuti A.,and Sauer A.V. (2017). Good laboratory practice preclinical safety studies for GSK2696273 (MLV vector-based ex vivogene therapy for adenosine deaminase deficiency severe combined immunodeficiency) in NSG mice. *Hum Gene Ther Clin Dev.* 28:17-27. IF (2016) 2.490
141. Zonari E., Desantis G., Petrillo C., Boccalatte F.E., Lidonnici M.R., Kajaste-Rudnitski A., Aiuti A., Ferrari G., Naldini L., and Gentner B. (2017). Efficient ex vivo engineering and expansion of highly purified human hematopoietic stem and progenitor cell populations for gene therapy. *Stem Cell Reports.* 8: 977-990. Epub March 13,2017. IF (2016) 7.338
142. Aiuti A., Roncarolo M.G. and Naldini L. (2017). Gene therapy for ADA-SCID, the first marketing approval of an ex vivo gene therapy in Europe: paving the road for the next generation of advanced therapy medicinal products. *EMBO Mol Med.* 9: 737-740. Epub April 10, 2017. IF (2016) 9.249
143. Chiriaco M., Brigida I., Ariganello P., Di Cesare S., Di MAtteo G., Taus F., Cittaro D., Lazarevic D., Scarselli A., Santilli V., Attardi E., Stupka E., Giannelli S., Fraziano M., Finocchi A., Rossi P., Aiuti A., Palma P. and Cancrini C. (2017). The case of an APDS patient: defects in maturation and function and decreased in vitro anti-mycobacterial activity in the myeloid compartment. *Clin Immunol.* 178, 20-28. Epub 2015 December 28. I.F. (2016) 3.990
144. Penati R.* , Fumagalli F.* , Calbi V., Bernardo M.E., and Aiuti A. (2017).Gene therapy for lysosomal storage disorders: recent advances for metachromatic leukodystrophy and mucopolysaccharidosis I. (2017). *J Inherit Metab Dis.* 40:543-554. * Epub 2017 May 30. *Equal contribution. IF (2016) 3.970
145. Basso-Ricci L., Scala S., Milani R., Migliavacca M., Rovelli A., Bernardo M.E., Ciceri F., Aiuti A., and Biasco L. (2017). Multiparametric Whole Blood Dissection: A one-shot comprehensive picture of the human hematopoietic system. *Cytometry A.* 91: 952-965. Epub June 13, 2017. IF (2016) 3.222
146. Starc N., Ingo D., Conforti A., Rossella V., Tomao L., Pitisci A., De Mattia F., Brigida I., Algeri M., Montanari M., Giuseppe Palumbo G., Merli P., Rossi P., Aiuti A., Locatelli F., and Bernardo M.E. (2017). Biological and functional characterization of bone marrow-

- derived mesenchymal stromal cells from patients affected by primary immunodeficiency. *Sci Rep.* 7:8153. IF (2016) 4.259
147. Azario I., Pievani A., Del Priore F., Antolini L., Santi L., Corsi A., Cardinale L., Sawamoto K., Kubaski F., Gentner B., Bernardo M.E., Valsecchi M.G., Riminucci M., Tomatsu S., Aiuti A., Biondi A., and Serafini M. (2017) Neonatal umbilical cord blood transplantation halts skeletal disease progression in the murine model of MPS-I. *Sci Rep.* 7: 9473. IF (2016) 4.259
148. Maass P.G., Glažar P., Memczak S., Dittmar G., Hollfinger I., Schreyer L., Sauer A.V., Toka O., Aiuti A., Luft F.C., and Rajewsky N. (2017). A map of human circular RNAs in clinically relevant tissues. *J Mol Med (Berl).* 95:1179-1189. IF (2016) 4.686
149. Ferrua F., and Aiuti A. (2017). Twenty-five years of gene therapy for ADA-SCID: from bubble babies to an approved drug. *Hum Gene Ther.* 28:972-981. IF (2016) 4.187
150. Maccari M.E., Scarselli A., Di Cesare S., Floris M., Angius A., Deodati A., Chiriaco M., Cambiaso P., Corrente S., Colafati G.S., Utz P.J., Angelini F., Fierabracci A., Aiuti A., Carsetti R., Rosenberg J.M., Cappa M., Rossi P., Bacchetta R., and Cancrini C. (2017). Severe Toxoplasma gondii infection in a member of a NFKB2-deficient family with T and B cell dysfunction. *Clin Immunol.* 183:273-277. IF (2016) 3.990
151. Barzaghi F., Amaya Hernandez L.C., Neven B., Ricci S., Kucuk Z.Y., Bleesing J., Nademi Z., Slatter M.A., Ulloa E.R., Shcherbina A., Roppelt A., Worth A., Silva J., Aiuti A., Murguia-Favela L., Speckmann C., Carneiro-Sampaio M., Fernandes J.F., Baris S., Ozen A., Karakoc-Aydiner E., Kiykim A., Schulz A., Steinmann S., Notarangelo L.D., Gambineri E., Lionetti P., Shearer W.T., Forbes L., Martinez C., Moshous D., Blanche S., Fisher A., Ruemmele F.M., Tissandier C., Ouachee-Chardin M., Rieux-Lauzier F., Cavazzana M., Qasim W., Lucarelli B., Albert M.H., Kobayashi I., Alonso L., Diaz De Heredia C., Kanegane H., Lawitschka A., Seo J.J., Gonzalez-Vicent M., Diaz M.A., Goyal R.K., Sauer M.G., Yesilipek A., Kim M., Yilmaz-Demirdag Y., Bhatia M., Khlevner J., Richmond Padilla E.J., Martino S., Montin D., Neth O., Molinos-Quintana A., Valverde-Fernandez J., Broides A., Pinsk V., Ballauf A., Haerynck F., Bordon V., Dhooge C., Garcia-Lloret M.L., Bredius R.G., Kałwak K., Haddad E., Seidel M.G., Duckers G., Pai S.Y., Dvorak C.C., Ehl S., Locatelli F., Goldman F., Gennery A.R., Cowan M.J., Roncarolo M.G., Bacchetta R.; PIDTC and IEWP of EBMT. (2017). Long-term follow up of IPEX syndrome patients after different therapeutic strategies: an international multicenter retrospective study. *J Allergy Clin Immunol.* Epub 2017 Dec. 11. IF (2016) 13.081
152. Cicalese M.P., Ferrua F., Castagnaro L., Rolfe K., De Boever E., Reinhardt R.R., Appleby J., Roncarolo M.G., and Aiuti A. (2018). Gene therapy for adenosine deaminase deficiency: a comprehensive evaluation of short-and medium-term safety. *Mol Ther.* Epub 2018 Jan 4. IF (2016) 15.093
153. Calbi V., Fumagalli F., Consiglieri G., Penati R., Acquati S., Redaelli D., Attanasio V., Facchini M., Cicalese M.P., Migliavacca M., Barzaghi F., Ferrua F., Assanelli A., Silvani P., Zoccolillo M., Chidini G., Chiesa R., Arora R., Ciotti F., Sarzana M., Antonioli G., Baldoli C., Morena F., Martino S., Ardissino G.L., Natali Sora M.G., Naldini L., Ciceri F., Aiuti A., and Bernardo M.E. (2018). Use of Defibrotide to help prevent post-transplant endothelial injury in a genetically predisposed infant with metachromatic leukodystrophy undergoing hematopoietic stem cell gene therapy. *Bone Marrow Transplant.* Epub 2018 Jan 29. IF (2016) 3.874
154. Chiriaco M., Casciano F., Di Matteo G., Gentner B., Claps A., Cotugno N., Argenio P., Rossi P., Aiuti A., and Finocchi A. (2018). Impaired X-CGD T cell compartment is gp91phox-NADPH oxidase independent. *Clin Immunol.* Epub 2018 Feb 2. IF (2016) 3.990
155. Sereni L., Castiello M.C., Marangoni F., Anselmo A., di Silvestre D., Motta S., Draghici E., Mantero S., Thrasher A.J., Giliani S., Aiuti A., Mauri P., Notarangelo L.D., Bosticardo M., and Villa A. (2018). Autonomous role of Wiskott-Aldrich Syndrome platelet deficiency in

- inducing autoimmunity and inflammation. *J Allergy Clin Immunol.* Epub 2018 Feb. 5. IF (2016) 13.081
156. Cervantes-Luevano K.E., Caronni N., Castiello M.C., Fontana E., Piperno G., Naseem A., Uva P., Bosticardo M., Marcovecchio G.E., Notarangelo L.D., Cicalese M.P., Aiuti A., Villa A., and Benvenuti F. (2018). Neutrophils drive type-I interferon production and autoantibodies in Wiskott-Aldrich syndrome. *J Allergy Clin Immunol.* Epub 2018 Feb. 12. IF (2016) 13.081
157. Migliavacca M, Assanelli A, Ponzoni M, Pajno R, Barzaghi F, Giglio F, Ferrua F, Frittoli M, Brigida I, Dionisio F, Nicoletti R, Casiraghi M, Roncarolo MG, Doglioni C, Peccatori J, Ciceri F, Cicalese MP, Aiuti A. (2018). First occurrence of plasmablastic lymphoma in Adenosine Deaminase-Deficient Severe Combined Immunodeficiency Disease patient and review of the literature. *Front Immunol.* 2018 Feb 2;9:113. IF (2016) 6.429
158. Maccari ME, Abolhassani H, Aghamohammadi A, Aiuti A, Aleinikova O, Bangs C, Baris S, Barzaghi F, Baxendale H, Buckland M, Burns SO, Cancrini C, Cant A, Cathébras P, Cavazzana M, Chandra A, Conti F, Coulter T, Devlin LA, Edgar JDM, Faust S, Fischer A, Prat MG, Hammarström L, Heeg M, Jolles S, Karakoc-Aydiner E, Kindle G, Kiykim A, Kumararatne D, Grimbacher B, Longhurst H, Mahlaoui N, Milota T, Moreira F, Moshous D, Mukhina A, Neth O, Neven B, Nieters A, Olbrich P, Ozen A, Schmid JP, Picard C, Prader S, Rae W, Reichenbach J, Rusch S, Savic S, Scarselli A, Scheible R, Sediva A, Sharapova SO, Shcherbina A, Slatter M, Soler-Palacin P, Stanislaski A, Suarez F, Tucci F, Uhlmann A, van Montfrans J, Warnatz K, Williams AP, Wood P, Kracker S, Condliffe AM, Ehl S. (2018). Disease Evolution and Response to Rapamycin in Activated Phosphoinositide 3-Kinase δ Syndrome: The European Society for Immunodeficiencies-Activated Phosphoinositide 3-Kinase δ Syndrome Registry. *Front Immunol.* 2018 Mar 16;9:543. IF (2016) 6.429
159. Stirnadel-Farrant H, Kudari M, Garman N, Imrie J, Chopra B, Giannelli S, Gabaldo M, Corti A, Zancan S, Aiuti A, Cicalese MP, Batta R, Appleby J, Davinelli M, Ng P. (2018). Gene therapy in rare diseases: the benefits and challenges of developing a patient-centric registry for Strimvelis in ADA-SCID. *Orphanet J Rare Dis.* 2018; 13:49. IF (2016): 3.478

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

Publications on Italian journals:

1. Aiuti A., Rinaldi M., La Porta C., Fantoni A. e Citarella F. (1990). Production of Hageman factor (human FXII) in simian cells by transient expression of its cDNA. *Rend. Accademia Lincei.* 9, 1, 91-97.
2. Forte P., Aiuti A., Nisini R., D'Amelio R. e Fantoni A. (1991). Produzione in vitro di peptidi virus specifici. In: "Oligonucleotidi sintetici nello studio della biologia molecolare di HIV-1", Ferrara. Editori: R. Gambari e C. Nastruzzi; p. 35-40.
3. Biselli R., Nisini R., Matricardi P.M., Aiuti A. e D'Amelio R. (1992). Clonotypic analysis in different physiopathological conditions. *Immunol. Clin.* XI, 175-183.
4. Fantoni A., Forte P., Aiuti A. e Pozzi L. (1993). Topi transgenici. In: Attuali metodi in genetica molecolare. Ed. C. Morandi, M. Mottes, P.F. Pignatti, M.G. Romanelli, A.Turco. P. 44-45
5. Scala E., Aiuti A., Ansotegui I.J. e Paganelli R. (1993.) Identificazione dei geni responsabili di tre forme di immunodeficienza primitiva legata al cromosoma X. Analisi

- critica delle nuove acquisizioni pubblicate. Giorn. Ital. di Allerg. e Immun. Clin. 3, 287-294.
6. Aiuti A., Notarangelo L., Dolcini F., Casorati G., Bernardi M., Ficara F., Lietti G., Porta F., Ferrari G., e Bordignon C. (1998). Terapia genica e malattia ereditaria. In: "Medicina Perinatale '98", Bari, Ed. A. Mautone, L. Selvaggi. P. 134-136.
 7. Deola S., and Aiuti A. (2005). Gene therapy. A matter of realism. (Terapia genica. Una questione di realismo). Journal of Medicine and The Person 3, 54-56.
 8. Aiuti F., Aiuti A., Calza L., Chiodo F., De Santis W., D'Ettorre G., Emmi L., Isgrò A., Luzi G., Maggi E., Marziali M., Mezzaroma I., Montroni M., Muscaritoli M., Paganelli R., Pandolfi F., Starnino S., Sirianni M.C., Spadarò G., e Vullo V. (2006). Linee Guida per la diagnosi e terapia dell'Immunodeficienza Comune Variabile. It J. Allergy Clin Immunol, 16 (Suppl. Al. N.1), 1-30.
 9. Aiuti A., Cappelli B., Biffi A., Marktel S., and Roncarolo M.G. (2009). Gene therapy in pediatrics. Minerva Pediatr. 61, 775-778.
 10. Aiuti A. (2009). Pediatric testing and primary immunodeficiencies. Minerva Pediatr. 61, 785-787.

Book chapters:

1. Aiuti A., Bordignon C. "Gene Therapy for Severe combined Immunodeficiencies". (1999) in: Gene Therapy: Principles and Applications. Ed T. Blankenstein. Birkhauser. Pages 105-122.
2. Aiuti A. "Gene therapy with hematopoietic stem cells: new perspectives for the treatment of blood-borne genetic disorders". (2007) in: XXXIII Seminario sulla Evoluzione Biologica e i Grandi Problemi della Biologia. Accademia Nazionale dei Lincei. Pages 79-84.
3. Aiuti A. "Terapia Genica" (2007) in: Enciclopedia Italiana di Scienze, Lettere ed Arti. XXI Secolo. VII Appendice. Pages 351-355. Istituto della Enciclopedia Italiana fondata da Giovanni Treccani, Roma.
4. Sauer A., Cassani B., and Aiuti A. "Primary Immunodeficiencies" (2009) in: Guide to Human Gene Therapy. Chapter 13. Editor Roland W. Herzog, Sergei Zolotuklin. Publisher World Scientific. First edition.
5. Brigida I., and Aiuti A. "Immune reconstitution after gene therapy for adenosine deaminase severe combined immunodeficiency (ADA-SCID)" (2012) in: The CliniBook: Clinical gene transfer. Ed. Cohen-Hagenauer. EDK Paris.
6. Ravelli A., Aiuti A., Dallapiccola B., Petralia P. "Il reclutamento dei ricercatori clinici negli Istituti di ricovero e cura a carattere scientifico" (2016) in: Il futuro della ricerca clinica (pediatrica) – Problemi, prospettive, proposte. Società Italiana di Ricerca Pediatrica (SIRP) Onlus. Pag. 71-83. Giannini Editore, Napoli.
7. Cicalese M.P., and Aiuti A. "Gene therapy for primary immunodeficiencies" in: Management of infections in the immunocompromised host. Editor Brahm Segal. Springer International Publishing AG, Cham (in press).