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ALICE BERTAINA

EDUCATION

2010–2013

PhD in Immunology and Biotechnology, Tor Vergata University, Rome, Italy

February 2010–November 2010

Post-graduate fellowship in Hematopoietic Stem Cell Transplantation (basic and applied research), Bambino Gesù Children’s Hospital, Rome, Italy

November 2005–November 2010

Residency in Pediatrics University of Pavia, Pavia, Italy

Thesis title: “*NK-alloreactivity and outcome of pediatric patients with acute leukemia given an HLA-haploidentical stem cell transplantation*” under Prof.

Franco Locatelli supervision

Top grade 50/50 with Honors

November 1998–December 2005

Medical Degree University of Pavia, Pavia, Italy

Thesis title: “*Human Metapneumovirus II in acute pediatric respiratory infection: epidemiology, clinical picture and diagnosis*”

Top grade 110/110, Cum Laude

September 1993–September 1998

High School Diploma

Liceo Classico ‘G.D. Cassini’, Sanremo, Italy

WORK EXPERIENCE

August 2020–ongoing

Section Chief, Section of Stem Cell Transplantation and Regenerative Medicine, Division of Hematology/Oncology/Stem Cell Transplantation and Regenerative Medicine, Lucile Packard Children’s Hospital, Stanford University School of Medicine

August 2020–ongoing

Co-Director, Bass Center for Childhood Cancer and Blood Diseases, Lucile Packard Children’s Hospital, Stanford University School of Medicine

April 2019–ongoing

Medical Director of Inpatient Services, Division of Stem Cell Transplantation and Regenerative Medicine, Lucile Packard Children’s Hospital, Stanford University School of Medicine

September 2017–ongoing

Platform Leader, Blood Stem Cell Engineering and Transplantation, Center for Definitive and Curative Medicine (CDCM)

September 2017-ongoing

Associate Professor of Pediatrics, Stanford University School of Medicine, Medical Center Line, Department of Pediatrics, Division of Stem Cell Transplantation and Regenerative Medicine

October 2013-September 2017

Head of Stem Cell Transplant Unit, Department of Hematology and Oncology, Bambino Gesù Children's Hospital, Rome, Italy

March 2010- October 2013

Physician assistant and researcher in hematology/oncology and allogeneic stem cell transplantation, Department of Hematology and Oncology, Bambino Gesù Children's Hospital, Rome, Italy

November 2008-March 2010

Residency in Pediatrics, Pediatric Hematology and Oncology, Fondazione IRCCS Policlinico San Matteo, Pavia, Italy

2004-2008

Fellowship, medical assistance and research activities, Department of Pediatrics Fondazione IRCCS Policlinico San Matteo, Pavia, Italy

2004

Medical assistance and research activities

- Collaboration on the study entitled: "*Emerging respiratory viral infections: Influenzavirus, Coronavirus, Metapneumovirus*"
- Collaboration on the study entitled: "*Severity of infection from respiratory syncytial virus*"
- Collaboration on the multicenter study entitled: "*Clinical-virological study of the circulation of various strains of respiratory viruses in the pediatric population*" virus and the TLR4 polymorphism"

2002-2003

Medical assistance

Ergonomic Service, Division of Cardiology, Fondazione IRCCS Policlinico San Matteo, Pavia

ACADEMIC RANK

Dr. Bertaina is currently Associate Professor in the Division of Hematology, Oncology and Stem Cell Transplantation, Department of Pediatrics, Stanford University in the Medical Center Line (MCL) track.

In December 2016, Dr. Bertaina applied to the national competition for the Professorship in Italy and on April 2017 obtained the habilitation as Associate Professor.

RESEARCH EXPERIENCE

GRANTS

PAST FUNDING:

1. *'Study of epigenetic pathways regulating drug response in pediatric patients with acute myeloid leukemia'*
Role: PI
Period: January 2014 – December 2015
Funding Institution: Italian Ministry of Health (Ricerca Corrente)
2. *'MicroRNA and cytokines: identification of graft-versus-host disease biomarkers (GvHD) in children given allogeneic hematopoietic stem cell transplantation'*
Role: PI
Period: January 2017 – December 2017
Funding Institution: Italian Ministry of Health (Ricerca Corrente)
3. *'Gamma/delta and their reconstitution in children with leukemia given T-cell depleted stem cell transplantation'*
Role: PI
Period: January 2015 – January 2018
Funding Institution: AIRC (Associazione Italiana per la Ricerca contro il Cancro)
4. *'Role of the BMP4 signaling pathway in the pathogenesis of Juvenile Myelomonocytic Leukemia and childhood Myelodysplastic Syndromes by utilizing a new human patient-specific disease model to develop novel therapeutic approaches'*
Role: PI
Period: January 2016 – January 2019
Funding Institution: Ministry of Health (ricerca finalizzata, giovani ricercatori)

CURRENT FUNDING

1. *'Generating a novel humanized mouse model of Juvenile myelomonocytic leukemia (JMML) by knockout of NF1 using CRISPR/Cas9 genome editing'*
Role: Principal Investigator
Period: January 2020-January 2022
Funding Institution: Hyundai Hope on Wheels
2. *'Transposition of native chromatin for fast and sensitive epigenomic profiling of open chromatin, DNA-binding proteins and nucleosome position (ATAC-seq) in JMML'*
Role: Principal Investigator
Period: January 2019-January 2020
Funding Institution: Maternal and Child Health Research Institute
3. *'Transposition of native chromatin for fast and sensitive epigenomic profiling of open chromatin, DNA-binding proteins and nucleosome position (ATAC-seq) in JMML'*
Role: Principal Investigator
Period: January 2019-January 2020
Funding Institution: Children's Leukemia Research Association
4. *'Optimizing TCR $\alpha\beta$ + / CD19+ -depleted haploidentical HSCT for ALL using donor-derived genome-edited CAR T cells'*
Role: Principal Investigator

Period: February 2020- February 2022

Funding Institution: Reach Grant, Alex's Lemonade Stand Foundation

5. *'Adaptive immunotherapy to reduce leukemia relapse in children after $\alpha\beta$ T-cell depleted haploidentical hematopoietic stem cell transplantation ($\alpha\beta$ haplo-HSCT)'*

Role: PI

Period: November 2020-November 2022

Funding Institution: Bass Center for Childhood Cancer and Blood Disorder Innovations in Leukemia Research Award

6. *'CD8+ memory donor-derived addback after $\alpha\beta$ T-cell haploidentical HSCT to reduce leukemia relapse in children'*

Role: PI

Period: October 2020-October 2021

Funding Institution: The Cancer League

7. *'A new program of adoptive immunotherapy after alpha beta T-cell haplo-identical hematopoietic stem cell transplantation (HSCT) to reduce leukemia relapse in children'*

Role: PI

Period: October 2020-September 2022

Funding Institution: V Foundation

CLINICAL TRIAL

Dr. Bertaina has extensive experience in phase I/II studies according to GCP rules in pediatric patients with hematological/oncological malignancies and non-malignant disorders.

PUBLICATIONS (2015-present)

Peer reviewed (original work)

1. Girmenia C, Rossolini GM, Piciocchi A, **Bertaina A**, Pisapia G, Pastore D, Sica S, Severino A, Cudillo L, Ciceri F, Scimè R, Lombardini L, Viscoli C, Rambaldi A; Gruppo Italiano Trapianto Midollo Osseo (GITMO); Gruppo Italiano Trapianto Midollo Osseo GITMO. Infections by carbapenem-resistant *Klebsiella pneumoniae* in SCT recipients: a nationwide retrospective survey from Italy. *Bone Marrow Transplant.* 2015 Feb;50(2):282-8. doi: 10.1038/bmt.2014.231. **Dr. Bertaina transplanted patients, treated infected patients, collected data of children coming from Bambino Gesù' Children's Hospital and contributed to analyze data.*
2. Locatelli F, Masetti R, Rondelli R, Zecca M, Fagioli F, Rovelli A, Messina C, Lanino E, **Bertaina A**, Favre C, Giorgiani G, Ripaldi M, Ziino O, Palumbo G, Pillon M, Pession A, Rutella S, Prete A. Outcome of children with high-risk acute myeloid leukemia given autologous or allogeneic hematopoietic cell transplantation in the aieop AML-2002/01 study. *Bone Marrow Transplant.* 2015 Feb;50(2):181-8. doi: 10.1038/bmt.2014.246. Erratum in: *Bone Marrow Transplant.* 2015 Feb;50(2):320. **Dr. Bertaina transplanted patients, collected data of children coming from Bambino Gesù' Children's Hospital and contributed to analyze and interpret data.*
3. Stabile H, Nisti P, Morrone S, Pagliara D, **Bertaina A***, Locatelli F, Santoni A, Gismondi A. Multifunctional human CD56 low CD16 low natural killer cells are the prominent subset in

bone marrow of both healthy pediatric donors and leukemic patients. *Haematologica*. 2015 Apr;100(4):489-98. doi: 10.3324/haematol.2014.116053. **Dr. Bertaina transplanted patients, collected samples of both healthy donors and leukemic patients, collected clinical data and largely contributed to analyze data and to edit manuscript.*

4. Airoldi I*, **Bertaina A***, Prigione I, Zorzoli A, Pagliara D, Cocco C, Meazza R, Loiacono F, Lucarelli B, Bernardo ME, Barbarito G, Pende D, Moretta A, Pistoia V, Moretta L, Locatelli F. $\gamma\delta$ T-cell reconstitution after HLA-haploidentical hematopoietic transplantation depleted of TCR- $\alpha\beta$ + /CD19+ lymphocytes. *Blood*. 2015 Apr 9;125(15):2349-58. doi: 10.1182/blood-2014-09-599423. * equal first authors.
5. Della Chiesa M, Moretta L, Muccio L, **Bertaina A***, Moretta F, Locatelli F, Moretta A. Haploidentical Haematopoietic Stem Cell Transplantation: Role of NK Cells and Effect of Cytomegalovirus Infections. *Curr Top Microbiol Immunol*. 2016;395:209-24. doi: 10.1007/82_2015_450. Review. **Dr. Bertaina contributed to analyze the literature and to write the manuscript.*
6. Vella S, Pomella S, Leoncini PP, Colletti M, Conti B, Marquez VE, Strillacci A, Roma J, Gallego S, Milano GM, Capogrossi MC, **Bertaina A***, Ciarapica R, Rota R. MicroRNA- 101 is repressed by EZH2 and its restoration inhibits tumorigenic features in embryonal rhabdomyosarcoma. *Clin Epigenetics*. 2015 Aug 6;7(1):82. doi: 10.1186/s13148-015-0107-z. **Dr. Bertaina participated in the study design, discussion, and manuscript writing.*
7. Pietra G, Vitale C, Pende D, **Bertaina A***, Moretta F, Falco M, Vacca P, Montaldo E, Cantoni C, Mingari MC, Moretta A, Locatelli F, Moretta L. Human natural killer cells: news in the therapy of solid tumors and high-risk leukemias. *Cancer Immunol Immunother*. 2015 Aug 20. Review. **Dr. Bertaina contributed to analyze the literature and to write the manuscript.*
8. Di Nardo M, Merli P, Cecchetti C, Pasotti E, **Bertaina A***, Locatelli F. Progressive increase in D-dimer levels during extracorporeal membrane oxygenation can predict membrane oxygenator failure in children given hematopoietic stem cell transplantation? *J Crit Care*. 2016 Feb;31(1):262-3. doi: 10.1016/j.jcrc.2015.09.010. **Dr. Bertaina participated in study design, transplanted patients, collected samples and clinical data, extensively contributed in analyzing data and writing manuscript.*
9. Gawronski O, Ciofi Degli Atti ML, Di Ciommo V, Cecchetti C, **Bertaina A***, Tiozzo E, Raponi M; Stem Cell Transplant Unit BedsidePEWS Study Group. Accuracy of Bedside Paediatric Early Warning System (BedsidePEWS) in a Pediatric Stem Cell Transplant Unit. *J Pediatr Oncol Nurs*. 2015 Oct 24. pii: 1043454215600154. **Dr. Bertaina participated in study design, transplanted patients, collected clinical data, extensively contributed in analyzing data and writing manuscript.*
10. Scarselli A, Di Cesare S, Di Matteo G, De Matteis A, Ariganello P, Romiti ML, Cascioli S, De Vito R, **Bertaina A***, Locatelli F, Gaspar HB, Aiuti A, Rossi P, Gilmour K, Cancrini C. Combined immunodeficiency due to JAK3 mutation in a child presenting with skin granuloma. *J Allergy Clin Immunol*. 2015 Nov 3. pii: S0091-6749(15)01355-X. doi: 10.1016/j.jaci.2015.09.017. **Dr. Bertaina transplanted this young girl, collected data and largely contributed in editing the manuscript.*
11. Buontempo F, Orsini E, Lonetti A, Cappellini A, Chiarini F, Evangelisti C, Evangelisti C, Melchionda F, Pession A, **Bertaina A***, Locatelli F, Bertacchini J, Neri LM, McCubrey JA, Martelli AM. Synergistic cytotoxic effects of bortezomib and CK2 inhibitor CX-4945 in acute

lymphoblastic leukemia: turning off the prosurvival ER chaperone BIP/Grp78 and turning on the pro-apoptotic NF- κ B. *Oncotarget*. 2016 Jan 12;7(2):1323-40. doi: 10.18632/oncotarget.6361. **Dr. Bertaina collected samples and clinical data, contributed to data analysis and to edit manuscript.*

12. Muccio L, **Bertaina A**, Falco M, Pende D, Meazza R, Lopez-Botet M, Moretta L, Locatelli F, Moretta A, Della Chiesa M. Analysis of memory-like Natural Killer cells in Human Cytomegalovirus-infected children undergoing $\alpha\beta$ +T- and B-cell depleted HSCT for hematological malignancies. *Haematologica*. 2015 Dec 11. pii: haematol.2015.134155.
13. Moretta F, Petronelli F, Lucarelli B, Pitisci A, **Bertaina A***, Locatelli F, Mingari MC, Moretta L, Montaldo E. The generation of human innate lymphoid cells is influenced by the source of hematopoietic stem cells and by the use of G-CSF. *Eur J Immunol*. 2016 Feb 3. doi: 10.1002/eji.201546079. **Dr. Bertaina enrolled and transplanted all patients, collected samples and clinical data, contributed to study design and participated in writing the manuscript.*
14. Girmenia C, Annino L, Mariotti B, Fanci R, Minotti C, Spadea A, Carotti A, Piedimonte M, Chierichini A, Cerchiara E, Caselli D, Cupelli L, Arcioni F, **Bertaina A***, Ribersani M, Proia A, Mengarelli A, Perriello V, Torelli GF, Di Gioia M, Del Principe MI, Cassetta MI, Fallani S, Novelli A. Posaconazole oral suspension primary prophylaxis in acute leukemia and allogeneic stem cell transplant patients: can it be used without measurement of plasma concentration? *Med Mycol*. 2016 Feb 11. pii: myw001. **Dr. Bertaina contributed to study design, enrolled and transplanted all pediatric patients coming from Bambino Gesù' Children's Hospital, collected samples and clinical data and contributed to edit manuscript.*
15. Alicata C, Pende D, Meazza R, Canevali P, Loiacono F, **Bertaina A***, Locatelli F, Nemat-Gorgani N, Guethlein LA, Parham P, Moretta L, Moretta A, Bottino C, Norman PJ, Falco M. Hematopoietic stem cell transplantation: Improving alloreactive Bw4 donor selection by genotyping codon 86 of KIR3DL1/S1. *Eur J Immunol*. 2016 Jun;46(6):1511- doi: 10.1002/eji.201546236. **Dr. Bertaina contributed to study design, enrolled and transplanted patients, collected samples and clinical data and participate in analyzing results and editing manuscript.*
16. Moretta L, Pietra G, Vacca P, Pende D, Moretta F, **Bertaina A**, Mingari MC, Locatelli F, Moretta A. Human NK cells: From surface receptors to clinical applications. *Immunol Lett*. 2016 May 13. pii: S0165-2478(16)30083-9. doi: 10.1016/j.imlet.2016.05.007. **Dr. Bertaina contributed to analyze the literature and to write the manuscript.*
17. Vacca P, Montaldo E, Croxatto D, Moretta F, **Bertaina A***, Vitale C, Locatelli F, Mingari MC, Moretta L. NK Cells and Other Innate Lymphoid Cells in Hematopoietic Stem Cell Transplantation. *Front Immunol*. 2016 May 18;7:188. doi: 10.3389/fimmu.2016.00188. eCollection 2016. Review. **Dr. Bertaina contributed to analyze the literature and to write the manuscript.*
18. Leoncini PP*, **Bertaina A***, Papaioannou D, Flotho C, Masetti R, Bresolin S, Menna G, Santoro N, Zecca M, Basso G, Nigita G, Veneziano D, Pagotto S, D'Ovidio K, Rota R, Dorrance A, Croce CM, Niemeyer C, Locatelli F, Garzon R. MicroRNA fingerprints in juvenile myelomonocytic leukemia (JMML) identified miR-150-5p as a tumor suppressor and potential target for treatment. *Oncotarget*. 2016 Aug 23;7(34):55395-55408. doi: 10.18632/oncotarget.10577. **These two authors equally contributed as first author.*

19. Li Pira G, Malaspina D, Girolami E, Biagini S, Cicchetti E, Conflitti G, Broglia M, Ceccarelli S, Lazzaro S, Pagliara D, Meschini A, **Bertaina A***, Montanari M, Locatelli F. Selective depletion of alfa/beta T cells and B cells for Human Leukocyte Antigen- Haploidentical hematopoietic stem cell transplantation. A three-years follow-up of procedure efficiency. *Biol Blood Marrow Transplant.* 2016 Aug 9. Pii: S1083- 8791(16)30281-6. 10.1016/j.bbmt.2016.08.006. **Dr. Bertaina largely contributed to study design, transplanted all patients, collected clinical data and participated in analaizyng end interpret the results.*
20. De Felice F, Grapulin L, Musio D, Pomponi J, Di Felice C, Iori AP, **Bertaina A***, Tombolini V. Treatment complications and long-term outcomes of Total Body Irradiation in patietnts with acute lymphoblastic leukemia: a single institute experience. *Anticancer Res.* 2016 Sep; 36(9):4859-64. **Dr. Bertaina transplanted all patients, collected clinical data and participated in editing the manuscript.*
21. Toma P, **Bertaina A**, Castagnola E, Colafati GS, D'Andrea ML, Finocchi A, Lucidi V, Matsronuzzi A, Granata C. Fungal infections of the lung in children. *Pediatr Radiol.* 2016 Dec;46(13):1856-1865.
22. **Bertaina A**, Zorzoli A, Petretto A, Barbarito G, Inglese E, Merli P, Lavarello C, Brescia LP, De Angelis B, Tripodi G, Moretta L, Locatelli F and Airoidi I. Zoledronic acid boosts $\gamma\delta$ -T-cell activity in children given hematopoietic transplantation. *Oncoimmunology.* 2016 Sep 27;6(2):e1216291. doi: 10.1080/2162402X.2016.1216291. eCollection 2017.
23. Lonetti A, Cappellini A, **Bertaina A***, Locatelli F, Pession A, Buontempo F, Evangelisti C, Evangelisti C, Orsini E, Zambonin L, Neri LM, Martelli AM, Chiarini F. Improving nelarabine efficacy in T cell acute lymphoblastic leukemia by targeting aberrant PI3K/AKT/mTOR signaling pathway. *J Hematol Oncol.* 2016 Oct 24;9(1):114. **Dr. Bertaina collected samples and clinical data, contributed to data analysis and to edit manuscript.*
24. **Bertaina A**, Vinti L, Strocchio L, Gaspari S, Caruso R, Algeri M, Coletti V, Gurnari C, Romano M, Cefalo MG, Girardi K, Trevisan V, Bertaina V, Merli P, Locatelli F. The combination of bortezomib with chemotherapy to treat relapsed/refractory acute lymphoblastic leukaemia of childhood. *Br J Haematol.* 2017 Jan 24. doi: 10.1111/bjh.14505. [Epub ahead of print]
25. Slack J, Albert MH, Balashov D, Belohradsky B, **Bertaina A***, Bleesing J, Booth C, Büchner J, Buckley RH, Chardin M, Deripapa E, Drabko K, Eapen M, Feuchtinger T, Finocchi A, Gaspar HB, Ghosh S, Gillio A, Gonzalez-Granado LI, Grunebaum E, Guengoer T, Heilmann C, Helminin M, Higuchi K, Imai K, Kalwak K, Kanazawa N, Karasu G, Kucuk ZY, Laberko A, Lange A, Mahlaoui N, Meisel R, Moshous D, Muramatsu H, Parikh S, Pasic S, Schmid I, Schuetz C, Schultz KR, Shaw PJ, Slatter MA, Sykora K-W, Tamura S, Taskinen M, Wawer A, Wolska-Kuśnierz B, Cowan MJ, Fischer A, Gennery AR on behalf of the Inborn Errors Working Party of the European Society for Blood and Marrow Transplantation and the European Society for Immunodeficiencies, Stem Cell Transplant for Immunodeficiencies in Europe (SCETIDE), the Center for International Blood and Marrow Transplant Research, and the Primary Immunodeficiency Treatment Consortium. Outcome of Haematopoietic Stem Cell Transplantation for DNA-Double Strand Breakage Repair Disorders: An EBMT/ESID IEWP, SCETIDE and CIBMTR/PIDTC Survey. Jan 2017. *JACI. In press.* **Dr. Bertaina collected clinical data, contributed to data analysis and to edit manuscript.*
26. Li Pira G, Di Cecca S, Biagini S, Girolami E, Cicchetti E, Bertaina V, Quintarelli C, Caruana I, Lucarelli B, Merli P, Pagliara D, Brescia LP, **Bertaina A***, Montanari M, Locatelli F.

Preservation of Antigen-Specific Functions of $\alpha\beta$ T Cells and B Cells Removed from Hematopoietic Stem Cell Transplants Suggests Their Use As an Alternative Cell Source for Advanced Manipulation and Adoptive Immunotherapy. *Front Immunol.* 2017 Mar 23;8:332. doi: 10.3389/fimmu.2017.00332. eCollection 2017. *Dr. Bertaina transplanted all patients, collected data and largely contributed to interpret data.

27. Girmenia C, Annino L, **Bertaina A***, Mariotti B, Caselli D, Fanci R, Barberi W, Marchesi F, Carotti A, Ferrari A, Cerchiara E, Cupelli L, Arcioni F, Ribersani M, Proia A, Cartoni C, Girardi K, Venditti A, Cassetta MI, Fallani S, Novelli A. Voriconazole treatment in adults and children with hematological diseases: can it be used without measurement of plasma concentration? *Medical Micology. In press.* *Dr. Bertaina collected clinical data, contributed to data analysis and to edit manuscript.
28. Locatelli F, Merli P, Pagliara D, Li Pira G, Falco M, Pende D, Rondelli R, Lucarelli B, Brescia LP, Masetti R, Milano GM, Bertaina V, Algeri M, Pinto RM, Strocchio L, Meazza R, Grapulin L, Handgretinger R, Moretta A, **Bertaina A***, Moretta L*. Outcome of children with acute leukemia given HLA-haploidentical HSCT after $\alpha\beta$ T-cell and B-cell depletion. *Blood.* 2017 Aug 3;130(5):677-685. doi: 10.1182/blood-2017-04-779769. Epub 2017 Jun 6. * equal contribution as a last author.
29. Locatelli F, Bernardo ME, **Bertaina A**, Rognoni C, Comoli P, Rovelli A, Pession A, Fagioli F, Favre C, Lanino E, Giorgiani G, Merli P, Pagliara D, Prete A, Zecca M. Efficacy of two different doses of rabbit anti-T-lymphocyte globulin to prevent graft-versus-host disease in children with haematological malignancies transplanted from an unrelated donor: a multicentre, randomised, open-label, phase 3 trial. *Lancet Oncol.* 2017 Aug;18(8):1126-1136. doi: 10.1016/S1470-2045(17)30417-5. Epub 2017 Jul 10.
30. Leoncini PP, Vitullo P, Di Florio F, Tocco V, Cefalo MG, Pitisci A, Girardi K, Niemeyer C, Locatelli F, **Bertaina A**. Whole Genome MBD-seq reveals different CpG methylation patterns in Azacytidine-treated Juvenile Myelomonocytic Leukaemia (JMML) patients. *Br J Haematol.* 2017 Aug 2. doi: 10.1111/bjh.14876.
31. Evangelisti C, Cappellini A, Oliveira M, Fragoso R, Barata JT, **Bertaina A**, Locatelli F, Simioni C, Neri LM, Chiarini F, Lonetti A, Buontempo F, Orsini E, Pession A, Manzoli L, Martelli AM, Evangelisti C. PHOSPHATIDYLINOSITOL 3-KINASE INHIBITION POTENTIATES GLUCOCORTICOID RESPONSE IN B-CELL ACUTE LYMPHOBLASTIC LEUKEMIA. *J Cell Physiol.* 2017 Aug 4. doi: 10.1002/jcp.26135.
32. Girmenia C, **Bertaina A**, Piciocchi A, Carotti, Algarotti A, Busca A, Cattaneo C, Raiola AM, Guidi S, Iori AP, Candoni A, Irrera G, Milone G, Marcacci G, Scimè R, Musso M, Cudillo L, Sica S, Castagna L, Corradini P, Marchesi F, Pastore D, Alessandrino EP, Annaloro C, Ciceri F, Santarone S, Nassi L, Farina C, Viscoli C, Rossolini GM, Bonifazi F, Rambaldi A. Gruppo Italiano Trapianto di Midollo Osseo (GITMO) and Associazione Microbiologi Clinici Italiani (AMCLI). Incidence, risk factors and outcome of pre engraftment Gram- negative bacteremia after allogeneic and autologous hematopoietic stem cell transplantation: an Italian prospective multicenter survey. *Clinical Infectious Diseases. Clin Infect Dis.* 2017 Nov 13;65(11):1884-1896. doi: 10.1093/cid/cix690.
33. Prieto C, López-Millán B, Roca-Ho H, Stam RW, Romero-Moya D, Rodríguez-Baena FJ, Sanjuan-Pla A, Ayllón V, Ramírez M, Bardini M, De Lorenzo P, Valsecchi MG, Stanulla M, Iglesias M, Ballerini P, Carcaboso ÁM, Mora J, Locatelli F, **Bertaina A**, Padilla L, Rodríguez-Manzaneque JC, Bueno C, Menéndez P. NG2 antigen is involved in leukemia invasiveness

and central nervous system infiltration in MLL-rearranged infant B-ALL. *Leukemia*. 2017 Sep 25. doi: 10.1038/leu.2017.294.

34. Locatelli F, Merli P, **Bertaina A**. Rabbit anti-human T-lymphocyte globulin and hematopoietic transplantation. *Oncotarget*. 2017 Sep 14;8(57):96460-96461. doi: 10.18632/oncotarget.20878. eCollection 2017 Nov 14.
35. Cifaldi C, Chiriaco M, Di Matteo G, Di Cesare S, Scarselli A, De Angelis P, Rea F, Angelino G, Pastore M, Ferradini V, Pagliara D, Cancrini C, Rossi P, **Bertaina A**, Finocchi A. Novel XIAP mutation in VEOIBD child successfully treated with HLA-haploidentical HSCT after removal of $\alpha\beta^+$ T and B cells. *Front. Immunol.* doi: 10.3389/fimmu.2017.01893
36. Pasquini L, Rossi Espagnet MC, Napolitano A, Longo D, **Bertaina A**, Visconti E, Tomà P. Dentate nucleus T1 hyperintensity: is it always gadolinium all that glitters? *Radiol Med*. 2018 Jun;123(6):469-473. doi: 10.1007/s11547-017-0846-3. Epub 2018 Jan 27.
37. Gabelli M, Zecca M, Messina C, Carraro E, Buldini B, Rovelli A, Fagioli F, **Bertaina A**, Lanino E, Favre C, Rabusin M, Prete A, Ripaldi M, Barberi W, Porta F, Caniglia M, Santarone S, D'Angelo P, Basso G, and Locatelli F. Hematopoietic stem cell transplantation for isolated extramedullary relapse of acute lymphoblastic leukemia in children. *Bone Marrow Transplant*. 2018 Jun 13. doi: 10.1038/s41409-018-0259-5.
38. Muccio L, Falco M, **Bertaina A**, Locatelli F, Frassoni F, Sivori S, Moretta L, Moretta A, Della Chiesa M. Late development of Fc ϵ R γ neg adaptive NK cells upon HCMV reactivation in UCBT recipients. *Front. Immunol.*, 15 May 2018 | <https://doi.org/10.3389/fimmu.2018.01050>.
39. Bono M, Pende D, Bertaina A, Moretta A, Della Chiesa M, Sivori S, Zecca M, Locatelli F, Moretta L, Bottino C, Falco M. Analysis of KIR3DP1 polymorphism provides relevant information on centromeric KIR gene content. *Journal of Immunology*. *J Immunol*. 2018 Sep 1;201(5):1460-1467. doi: 10.4049/jimmunol.1800564. Epub 2018 Aug 1.
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41. van der Stoep MYEC, Zwaveling J, **Bertaina A**, Locatelli F, Guchelaar HJ, Lankester AC, Moes DJAR. Population pharmacokinetics of treosulfan in paediatric patients undergoing hematopoietic stem cell transplantation. *Br J Clin Pharmacol*. 2019 Sep;85(9):2033-2044. doi:10.1111/bcp.13995. Epub 2019 Jul 1.
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6. **Bertaina A**, Roncarolo MG. Graft Engineering and Adoptive Immunotherapy: New Approaches to Promote Immune Tolerance After Hematopoietic Stem Cell Transplantation. *Front Immunol*. 2019 Jul 10;10:1342. doi: 10.3389/fimmu.2019.01342. eCollection 2019. Review.
7. Yazdanifar M, Barbarito G, **Bertaina A***, Airoidi I*. $\gamma\delta$ T Cells: The Ideal Tool for Cancer Immunotherapy. *Cells*. 2020 May 24;9(5):1305. doi: 10.3390/cells9051305. PMID: 32456316
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Chapters

- In 2018, Dr. Bertaina co-authored the chapter titled 'Haploidentical HSCT' in the 7th Edition of The EBMT Handbook, Hematopoietic Stem Cell Transplantation and Cellular Therapies.
- In 2012, Dr. Bertaina contributed to the chapter titled '*HSCT in children with myelodysplastic syndromes*' the 6th Edition of The EBMT Handbook, a text consulted by transplanters over the world.
- Dr. Bertaina contributed to writing the chapter entitled '*Haploidentical-Haematopoietic Stem Cell Transplantation: role of NK cells and effect of cytomegalovirus infections*' of the Natural Killer Cells Book, Current Topics on Microbiology and Immunology, Volume 395, Springer, <http://www.springer.com/series/82>.

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- **Poster presentation, EBMT 2020**, entitled 'The role of donor KIR genotyping in Pediatric Hematopoietic Stem Cell Transplantation for Non-Malignant Disorders'. Mariano L, Zhang MB, Kristovich K, Agarwal-Hashmi R, Roncarolo MG, Bertaina A* and Fernandez-Vina M*.
 - **Poster presentation** at European Society for Blood and Marrow Transplantation (EBMT) (March 20, 2020) Entitled 'Supportive care during pediatric hematopoietic stem cell transplantation: beyond infectious diseases.'
 - **Poster presentation, ASTCT 2019**, entitled 'Early Epigenetic Immune Quantification Following Alpha/Beta T-Cell/CD19 B-Cell Depleted Haploidentical Stem Cell Transplant Correlates with CD4+T Cell Recovery at Day+100' Mayers, M., Schulze, J., Barbarito, G., Lakshmanan, U., Parkman, R., Weinberg, K. I., Chu, J., Agarwal, R., Roncarolo, M., Sachsenmaier, C., Bacchetta, R., Bertaina, A.
 - **Poster presentation, ASTCT 2019**, entitled 'Alpha/beta T-Cell/CD19 B-Cell Depleted Haploidentical Stem Cell Transplantation: A New Platform for Curing Rare and Monogenic Disorders'. Bertaina, A., Bacchetta, R., Lewis, D. B., Grimm, P. C., Shah, A. J., Agarwal, R., Concepcion, W., Czechowicz, A., Bhatia, N., Lahiri, P., Weinberg, K. I., Parkman, R., Porteus, M., Roncarolo, M.
 - **Poster presentation, ISCT 2019**, entitled 'Gamma/delta T cells: a potent immunotherapy tool for children with high-risk acute leukemia'. Barbarito, G., Patil, R., Favaro, P., Sinha, R., Zorzoli, A., Klein, C. A., Airoidi, I., Bertaina, A.
 - **Oral Communication, 18th Annual Meeting of the Federation of Clinical Immunology Societies (FOCIS 2018), June 20th-23rd, San Francisco, CA, USA**, entitled 'BPX-501 cells infusion after alpha/beta T-cell and B-cell depleted haploidentical transplant in children with primary immunodeficiencies: high rate of cure with low risk of acute and chronic GvHD'.
 - The abstract entitled 'Outcome of children with acute leukemia given allogeneic HSCT either from an unrelated donor (UD-HSCT) or from an HLA-partially matched relative after $\alpha\beta$ -T cell/B-cell depletion ($\alpha\beta$ haplo-HSCT)' was accepted for **Oral presentation** at the 43rd Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT 2017) taking place on March 26–29 2017 in Marseille, France.
 - **Oral Communication, 2016 JMML International Symposium** entitled 'Identification of deregulated microRNAs in Juvenile Myelomonocytic Leukemia (JMML)'.
 - **Poster presentation # 2345** at the 21st EHA Meeting, Copenhagen, June 2016, entitled 'The combination of bortezomib and chemotherapy is an effective treatment to re-induce remission in relapse/refractory B-cell precursor or T-cell acute lymphoblastic leukemia'.
 - **Oral presentation # 3789** at the 21st EHA Meeting, Copenhagen, June 2016, entitled 'Infusion of BPX-501 (donor T-cells transduced with the iC9 suicide gene) after $\alpha\beta$ T-cell depleted haplo-HSCT in children with acute leukemia: preliminary results of a phase I-II trial'.
 - **Poster presentation #P264** at the 42nd Annual Meeting of EBMT, Valencia, Spain, entitled 'Treosulfan-based conditioning regimen for children with chronic granulomatous disease'.
 - **Poster presentation #166** at the BMT Tandem Meeting 2016, Honolulu, entitled 'Clinical outcome and immune recovery after Adoptive Infusion of BPX-501 Cells (donor T cells transduced with iC9 suicide gene) in children with Wiskott-Aldrich Syndrome given alfa/beta T-cell depleted HLA-haploidentical Stem Cell Transplantation.

- **Poster presentation #450** at the *BMT Tandem Meeting 2016*, Honolulu, entitled 'Clinical outcome after Adoptive Infusion of BPX-501 Cells (donor T cells transduced with iC9 suicide gene) in children with Thalassemia Major given alfa/beta T-cell depleted HLA- haploidentical Stem Cell Transplantation.
- **Poster presentation** at the *57th ASH Annual Meeting (2015)* entitled 'MicroRNA-150 regulates STAT5b levels in JMML'.
- **Oral Communication** at the *57th ASH Annual Meeting (2015, Abstract #195)* entitled 'Children with Acute Leukemia Given Hematopoietic Stem Cell Transplantation (HSCT) from an HLA-Compatible Sibling, or an Unrelated Donor (UD) or an HLA-Haploidentical Relative after Alpha/Beta T-Cell Depletion Have a Comparable Outcome'.
- **Oral Communication** at the *7th Symposium on MDS, JMML and Bone Marrow Failure (2015, Aarhus, Denmark)* entitled 'Identification of deregulated microRNAs in Juvenile Myelomonocytic Leukemia (JMML)'.
- **Oral Communication** at the *41st EBMT Annual Meeting (2015, Abstract O067)* entitled 'HLA haploidentical hematopoietic stem cell transplantation after removal of $\alpha\beta$ + T lymphocytes for children with non-malignant disorders: a fulfilled promise'.

INVITED PRESENTATIONS

- CTTC January 2021 webinar- Cell Therapy Transplant Canada- Lecture entitled: Recent Advances in $\alpha\beta$ T-Cell/CD19 B-Cell Depleted Haploidentical HSCT for Children with Either Malignant or Non-Malignant Disorders.
- 2020 International JMML Symposium, Decembr 3rd, 2020, Presentation 'Alpha/beta T-cell depleted haploidentical transplants for JMML'.
- HAPLO Meeting in Orlando, FL December 5, 2019, "Alpha/beta T-cell depleted haploidentical stem cell transplant in children: a platform for immunotherapy".
- 7th Annual PBMT Educational Meeting, New Orleans, May 2019, Lecture entitled 'Cord Blood vs Haplo-HSCT in children with malignant disorders: the conundrum'.
- EBMT-Annual Meeting 2019, Frankfurt, March 2019, Lecture entitled 'How do I treat and predict long term outcomes of steroid-refractory acute GvHD'.
- 3rd Annual Center for Definitive and Curative Medicine Symposium, Stanford, March 19th, 2019, Lecture entitled " $\alpha\beta$ T-cell/CD19 B-cell depleted Haploidentical Stem Cell Transplant in children: a new platform for adoptive immunotherapy'.
- CCRI Seminar, Children's National, Washington, March 2019, Lecture " $\alpha\beta$ T-cell/CD19 B-cell depleted Haploidentical Stem Cell Transplant in childhood: a fulfilled promise?'
- EBMT-Annual Meeting 2019, Frankfurt, March 2019, Medscape event, Lecture 'New Frontiers for patient care: the intersection of inherited disease, gene therapy and HSCT'.
- TCT | Transplantation & Cellular Therapy Meetings of ASBMT and CIBMTR (TCT Meetings) Session entitled "Optimizing Haploidentical Transplants", February 23, 2019
- St. Jude Ground Rounds, Memphis, February 7th, 2019, Lecture entitled " $\alpha\beta$ T-cell/CD19 B-cell depleted Haploidentical Stem Cell Transplant in childhood: a fulfilled promise?'
- ISCT, Annual Meeting 2018, Montreal, Lecture entitled 'Haploidentical Transplant in the Era of Graft Engineering: What's New in Pediatrics'.

- 11th PDWP, IDWP, IEWP & 6th Pediatric Nurse Group Meeting, Verona, 7th-9th June 2018, Lecture entitled 'Acute GVHD: incidence, treatment and outcome'.
- EBMT-Pediatric Disease Working Party Meeting, Verona, June 2018, Lecture entitled 'Acute GvHD: incidence, treatment and outcome'.
- EWOG Annual Meeting, Frankfurt, June 2018, Lecture entitled 'Transposition of native chromatin for fast and sensitive epigenomic profiling of open chromatin in JMML'.
- 10th Stem Cell Clonality and Genome Stability Retreat Chicago, May 15th 2018, Lecture entitled 'Graft Engineering for Haploidentical Transplants'.
- International Society for Cellular Therapy 2018 Annual Meeting, Montreal, May 4th 2018, Lecture entitled 'Haploidentical Transplant in the Era of Graft Engineering: What's New in Pediatrics'.
- Stanford Cancer Immunotherapy and Blood and Marrow Transplantation Symposium 2018: Update on current advances in BMT and Cancer Immunotherapy, Stanford, April 20th 2018, Lecture entitled 'Hematopoietic Cell Transplantation in Hemoglobinopathies'.
- Stanford Immunology Retreat 2018, Asilomar, CA, Lecture entitled 'Ex-vivo expansion of $\gamma\delta$ T-cells as a new tool of immunotherapy for patients with acute leukemia'.
- ASCGT, Chicago 2018, 10th Stem Cell Clonality and Genome Stability Retreat, Lecture entitled 'Graft Engineering for Haploidentical Transplants'.
- International Meeting on Childhood MDS and SAA (EWOG MDS/SAA), Rome, September 28th 2017, Lecture entitled 'JMML and microRNA'.
- National Annual Meeting GITMO (Gruppo Italiano per il Trapianto di Midollo), Milano, May 5th, 2017, Lecture entitled 'MicroRNA, cytokines and GvHD in pediatric patients: not only miR155'.
- 43rd Annual Meeting of the EBMT in Marseille, France, March 26-29, 2017. Lecture entitled 'Outcome in children with GVHD III-IV: of a PDWP retrospective study and treatment of severe acute GVHD focusing on MSC'.
- Autologous and allogeneic transplantation: stem cells, GvHD and indications. February 16th, 2017, Rome, Policlinico Gemelli, Lecture entitled 'GvHD prophylaxis and treatment in children'.
- Oral Communication at the 45^o Congresso Nazionale SIE, Firenze, entitled 'Allogeneic Hematopoietic Stem Cell Transplantation in pediatric patients with non-malignant disorders'.
- Inborn errors working party group (IEWP) of EBMT, November 2016, Ghent, Belgium. Lecture entitled 'T-cell depleted haplo-HSCT in children with erythroid disorders'.
- Scientific Symposium 'Guidelines on Fanconi Anemia', Fondi, October 2016. AIRFA (Associazione Italiana per la Ricerca sull'anemia di Fanconi). Lecture entitled 'Hematopoietic stem cell transplantation and alternative treatments in Fanconi Anemia'.
- Scientific Meeting 'La vita dopo il Midollo', Padova, October 2016. Lecture entitled 'HSCT tomorrow: the future of transplant'.

- Il Punto sulle Emoglobinopatie, Catania, June 24th, 2016. Lecture entitled 'Hematopoietic stem cell transplantation in hemoglobinopathies'.
- National Annual Meeting GITMO (Gruppo Italiano per il Trapianto di Midollo), Bologna, May 5th, 2016, Lecture entitled 'Haploidentical hematopoietic stem cell transplantation in children: non-malignant disorders'.
- Scientific Meeting on Thalassemia Major, Matera, March 6th, 2016. Lecture entitled 'Hematopoietic stem cell transplantation and gene-therapy'.
- Interim Meeting of the I-BFM SCT Committee Milan, 26-27 January 2016, 'Children with Acute Leukemia given Hematopoietic Stem Cell Transplantation (HSCT) from an HLA-Compatible Sibling, or an Unrelated Donor (UD) or an HLA-Haploidentical Relative after α/β T-Cell Depletion Have a Comparable Outcome'.
- I-BFM Board Meeting, Frankfurt March 4th, 2015, Conditioning strategies for alternative donors (UCBT and haplo-HSCT), the Rome experience.
- Stem Cells, Cancer, Immunology and Aging, Genoa, February 2015. Lecture entitled 'NK cells in the evolution of transplant approaches'.
- Meeting entitled 'Focus on alfa/beta T-cell depletion', Bologna, February 2015. Lecture entitled 'New strategies of transplant in children with malignant and non-malignant disorders'.

Other Presentations/Teaching:

- Seminars in Immunology, October 29th, 2019, Lecture entitled: Alpha/beta T-cell depleted haploidentical stem cell transplant in children: a new platform for immunotherapy.
- CIRM, San Jose University, October 2019, Lecture: $\alpha\beta$ T-cell/CD19 B-cell depleted Haploidentical Stem Cell Transplant in childhood: a platform for adoptive Immunotherapy?
- Faculty Teaching to Pediatric Hematology and Oncology/BMT Fellowship class, Stanford, May 2018.
- Faculty Teaching to Pediatric BMT Nurses class, Stanford, April 2018.
- 17° CORSO POST-UNIVERSITARIO IN IMMUNOLOGIA CLINICA - 2° AUTOIMMUNITY AND IMMUNODEFICIENCY DAY, Verona, October 14th, 2016 Title: *Novel approaches to hematopoietic transplant from HLA-haploidentical donor in primary immunodeficiencies.*
- Training Live. Fungal infections in children. Rome, Ospedale Pediatrico Bambino Gesù', December 2015.

HONORS

- 2019: Lorry Lokey Faculty Scholar, Stanford University School of Medicine (2019).
- 2018: One of three exceptional young physician scientists awarded by the Italian Scientists and Scholars of North America Foundation with the '*Paola Campese Award for Research in Leukemias*'. I was recognized for my work to ensure that every child who needs a stem cell transplant can find a suitable donor.

- 2015: Dr. Bertaina was invited by the President of Italian Republic, to participate in the *Annual National Ceremony for Cancer Research*, at the Palazzo del Quirinale.
- 2016: Abstract Travel Award 'Identification of deregulated microRNAs in JMML', *2016 International JMML Symposium*, San Diego.

MEMBERSHIPS

- AAP- American Academy of Pediatrics
- ASBMT- American Society of Bone Marrow Transplantation
- ASH – American Society of Hematology
- ISCT- International Society of Cell Therapy
- EBMT – European Society for Blood and Marrow Transplantation
- MCHRI- Maternal & Child Health Research Institute
- AIEOP – Associazione Italiana di Ematologia ed Oncologia Pediatrica
- GITMO – Italian Group for Bone Marrow Transplantation
- SPR – Society for Pediatric Research

NATIONAL and INTERNATIONAL COMMITTEE SERVICE

- Chair, **Stanford Center for Cell Therapy Safety Monitoring Committee** (2020-Present)
- Member of the committee for the **2021 Transplantation & Cellular Therapy Meeting of ASTCT and CIBMTR Peds Day Session** Themes, Talks, and Speakers
- Member of the **BMT-CTN Pediatric Hematologic Malignant Diseases State of the Science Committee** (2020-present)
- Member of the **PTCT Think Tank leadership group** (2020-present)
- Member of the **ISCT Stem Cell Engineering Scientific Committee** (2020-2023)
- **Member of Infections Control Committee, Lucille Packard Children's Hospital, Stanford University, CA, USA, January 2018-present**
- **Board Member, EBMT PDWP (European Society for Blood and Marrow Transplantation Pediatric Disease Working Party).** 2014 – present
- **Member, Comitato per il Controllo delle Infezioni Ospedaliere (CCIO)** at the Ospedale Pediatrico Bambino Gesù, Rome. 2011 – 2017
- **Head, Hematopoietic Stem Cell Transplantation Working Party Group of AIEOP (Italian Association of Pediatric Hematology and Oncology).** 2015 – 2017
- **Member, GITMO project** entitled '3 Steps', aiming at defining strategies of prophylaxis and treatment of veno-occlusive disease in transplant recipients. 2016 – present
- **Member, Inborn Errors Working Group, EBMT.** Member of a closed meeting organized to prepare guidelines/recommendations for conditioning and graft sources in various inborn errors for both haplo-approaches, namely TCR $\alpha\beta$ depletion and T-replete BM combined with post-transplant cyclophosphamide. At the end of the Meeting, the board decided to employ graft manipulation, conditioning regimen and GvHD prophylaxis published by Dr. Bertaina (Bertaina A. Blood 2014) for children with non-malignant disorders receiving a T-cell depleted haploidentical transplant. This reference will be in the next guidelines of the Inborn Errors Working Group of EBMT. 2016 – present
- **Chair, Paediatric transplantation Session, EBMT 43rd Annual Meeting, Marseille, France, March 26-29, 2017.**

PEER REVIEWED JOURNAL REVIEWER:

- Blood
- Clinical Cancer Research
- Haematologica
- Biology and Blood Marrow Transplantation
- Bone Marrow Transplantation
- Frontiers in Immunology
- Journal of Experimental Medicine
- Oncoimmunology
- Plos One

January 5th, 2021
Alice Bertaina, MD,
PhD