

# ALICE BERTAINA

## EDUCATION

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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

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September 2017

**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

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.

GRANTS

PAST FUNDING:

1. *'Generation of human Adenovirus (h-AdV)-specific immune effector cells for adoptive immunotherapy after allogeneic stem cell transplantation for pediatric malignancies'*  
Role: PI  
Period: January 2012 – December 2013  
Funding Institution: Italian Ministry of Health (Ricerca Corrente)
2. *'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)

CURRENT FUNDING

1. *'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)
2. *'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)
3. *'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)

CLINICAL TRIAL

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

**Peer reviewed (original work)**

1. Campanini G, Percivalle E, Baldanti F, Rovida F, **Bertaina A\***, Marchi A, Stronati M, Gerna G. Human respiratory syncytial virus (hRSV) RNA quantification in nasopharyngeal secretions identifies the hRSV etiologic role in acute respiratory tract infections of hospitalized infants. *J Clin Virol.* 2007 Jun;39(2):119-24. \*Dr. Bertaina enrolled majority of patients at the *Pediatric Clinic of Pavia. Collected all clinical data and participated in data analysis.*
2. Locatelli F, Testi AM, Bernardo ME, Rizzari C, **Bertaina A\***, Merli P, Pession A, Giralddi E, Parasole R, Barberi W, Zecca M. Clofarabine, cyclophosphamide and etoposide as single-course re-induction therapy for children with refractory/multiple relapsed acute lymphoblastic leukaemia. *Br J Haematol.* 2009 Nov;147(3):371-8. doi: 10.1111/j.1365-2141.2009.07882.x. \*Dr. Bertaina enrolled and treated majority of patients at the *Pediatric Haemato-Oncology Department of Pavia, collected clinical data and participated in editing the manuscript.*
3. Littera R, Orrù N, Vacca A, **Bertaina A\***, Caocci G, Mulargia M, Giardini C, Piras E, Mastronuzzi A, Vinti L, Orrù S, Locatelli F, Carcassi C, La Nasa G. The role of killer immunoglobulin-like receptor haplotypes on the outcome of unrelated donor hematopoietic SCT for thalassaemia. *Bone Marrow Transplant.* 2010 Nov;45(11):1618-24. doi: 10.1038/bmt.2010.24. Epub 2010 Feb 22. \*Dr. Bertaina enrolled majority of patients at the *Pediatric Haemato-Oncology Department of Pavia and collected clinical data.*
4. Caocci G, Baccoli R, Vacca A, Mastronuzzi A, **Bertaina A\***, Piras E, Littera R, Locatelli F, Carcassi C, La Nasa G. Comparison between an artificial neural network and logistic regression in predicting acute graft-vs-host disease after unrelated donor hematopoietic stem cell transplantation in thalassemia patients. *Exp Hematol.* 2010 May;38(5):426-33. doi: 10.1016/j.exphem.2010.02.012. \*Dr. Bertaina enrolled majority of patients at the *Pediatric Haemato-Oncology Department of Pavia and collected clinical data.*
5. Avanzini MA, Maccario R, Belloni C, Carrera G, **Bertaina A\***, Cagliuso M, La Rocca M, Valsecchi C, Mantelli M, Castellazzi AM, Quinti I, De Silvestri A, Marconi M. B lymphocyte subsets and their functional activity in the early months of life. *Int J Immunopathol Pharmacol.* 2010 Jan-Mar;23(1):247-54. \*Dr. Bertaina contributed to study design, enrolled patients, collected samples and clinical data and participated in analyzing data.
6. Bernardo ME, Ball LM, Cometa AM, Roelofs H, Zecca M, Avanzini MA, **Bertaina A**, Vinti L, Lankester A, Maccario R, Ringden O, Le Blanc K, Egeler RM, Fibbe WE, Locatelli F. Co-infusion of ex vivo-expanded, parental MSCs prevents life-threatening acute GVHD, but does not reduce the risk of graft failure in pediatric patients undergoing allogeneic umbilical cord blood transplantation. *Bone Marrow Transplant.* 2011 Feb;46(2):200-7. doi: 10.1038/bmt.2010.87. \*Dr. Bertaina participated in study design, enrolled and treated patients, collected clinical data and extensively contributed to data analysis.
7. Littera R, Orrù N, Caocci G, Sanna M, Mulargia M, Piras E, Vacca A, Giardini C, Orofino MG, Visani G, **Bertaina A\***, Giorgiani G, Locatelli F, Carcassi C, La Nasa G. Interactions

- between killer immunoglobulin-like receptors and their human leucocyte antigen Class I ligands influence the outcome of unrelated haematopoietic stem cell transplantation for thalassemia: a novel predictive algorithm. *Br J Haematol.* 2012 Jan;156(1):118-28. doi: 10.1111/j.1365-2141.2011.08923.x. *\*Dr. Bertaina enrolled majority of patients at the Pediatric Haemato-Oncology Department of Pavia and collected clinical data.*
8. Ferretti E, Montagna D, Di Carlo E, Cocco C, Ribatti D, Ognio E, Sorrentino C, Lisini D, **Bertaina A\***, Locatelli F, Pistoia V, Airoidi I. Absence of IL-12R $\beta$ 2 in CD33(+)CD38(+) pediatric acute myeloid leukemia cells favours progression in NOD/SCID/IL2R $\gamma$ C-deficient mice. *Leukemia.* 2012 Feb;26(2):225-35. doi: 10.1038/leu.2011.213. *\*Dr. Bertaina contributed to study design, collected samples and participated in data analysis.*
  9. Orrù S, Orrù N, Manolakos E, Littera R, Caocci G, Giorgiani G, **Bertaina A\***, Pagliara D, Giardini C, Nesci S, Locatelli F, Carcassi C, La Nasa G. Recipient CTLA-4\*CT60- AA genotype is a prognostic factor for acute graft-versus-host disease in hematopoietic stem cell transplantation for thalassemia. *Hum Immunol.* 2012 Mar;73(3):282-6. doi: 10.1016/j.humimm.2011.12.014. *\*Dr. Bertaina enrolled majority of patients at the Pediatric Haemato-Oncology Department of Pavia and collected clinical data.*
  10. Bernardo ME, Piras E, Vacca A, Giorgiani G, Zecca M, **Bertaina A\***, Pagliara D, Contoli B, Pinto RM, Caocci G, Mastronuzzi A, La Nasa G, Locatelli F. Allogeneic hematopoietic stem cell transplantation in thalassemia major: results of a reduced-toxicity conditioning regimen based on the use of treosulfan. *Blood.* 2012 Jul 12;120(2):473-6. doi: 10.1182/blood-2012-04-423822. *\*Dr. Bertaina participated in study design, enrolled the majority of patients at the Pediatric Haemato-Oncology Department of Pavia and Rome, performed transplantations, collected clinical data and extensively contributed to data analysis.*
  11. Li Pira G, Starc N, Conforti A, **Bertaina A\***, Rutella S, Locatelli F, Manca F. Lymphocyte proliferation specific for recall, CMV and HIV antigens in miniaturized and automated format. *J Immunol Methods.* 2012 Oct 31;384(1-2):135-42. *\*Dr. Bertaina collected clinical data and extensively contributed to data analysis.*
  12. Locatelli F, Pende D, Mingari MC, **Bertaina A\***, Falco M, Moretta A, Moretta L. Cellular and molecular basis of haploidentical hematopoietic stem cell transplantation in the successful treatment of high-risk leukemias: role of alloreactive NK cells. *Front Immunol.* 2013 Feb 1;4:15. doi: 10.3389/fimmu.2013.00015. *\*Dr. Bertaina contributed to write the manuscript.*
  13. Romania P, **Bertaina A\***, Bracaglia G, Locatelli F, Fruci D, Rota R. Epigenetic deregulation of microRNAs in rhabdomyosarcoma and neuroblastoma and translational perspectives. *Int J Mol Sci.* 2012 Dec 5;13(12):16554-79. doi: 10.3390/ijms131216554. Review. *\*Dr. Bertaina contributed to analyze the literature and to write the manuscript.*
  14. Caldarelli V, Gregoretta C, Paglietti MG, Pavone M, **Bertaina A\***, Chiarini Testa MB, Cutrera R. Late respiratory failure after hematopoietic stem cells transplant: just "lung failure"? *Minerva Anesthesiol.* 2013 Aug;79(8):969-70. *\*Dr. Bertaina treated the patients and contributed in the preparation of the manuscript.*

15. Manno EC, Salfa I, Palma P, **Bertaina A\***, Lombardi A, Moretta F, Coniglio ML, Sieni E, Aricò M, Finocchi A. Familial hemophagocytic lymphohistiocytosis type 3 diagnosed at school age: a case report. *J Pediatr Hematol Oncol.* 2014 Mar;36(2):e128-30. doi: 10.1097/MPH.0b013e318292bc7c. *\*Dr. Bertaina treated the patients and contributed in the preparation of the manuscript.*
16. Ball LM, Bernardo ME, Roelofs H, van Tol MJ, Contoli B, Zwaginga JJ, Avanzini MA, Conforti A, **Bertaina A\***, Giorgiani G, Jol-van der Zijde CM, Zecca M, Le Blanc K, Frassoni F, Egeler RM, Fibbe WE, Lankester AC, Locatelli F. Multiple infusions of mesenchymal stromal cells induce sustained remission in children with steroid-refractory, grade III-IV acute graft-versus-host disease. *Br J Haematol.* 2013 Nov;163(4):501-9. doi: 10.1111/bjh.12545. *\*Dr. Bertaina treated patients with MSCs, enrolled patients into the study and collected clinical data of Italian children. Dr. Bertaina largely contributed to analyze data.*
17. Di Nardo M, Li Pira G, Amodeo A, Cecchetti C, Giorda E, Ceccarelli S, Brescia LP, Pirozzi N, Rutella S, Locatelli F, **Bertaina A**. Adoptive immunotherapy with antigen-specific T cells during extracorporeal membrane oxygenation (ECMO) for adenovirus-related respiratory failure in a child given haploidentical stem cell transplantation. *Pediatr Blood Cancer.* 2014 Feb;61(2):376-9. doi: 10.1002/pbc.24753.
18. Hasegawa D, Bugarin C, Giordan M, Bresolin S, Longoni D, Micalizzi C, Ramenghi U, **Bertaina A\***, Basso G, Locatelli F, Biondi A, Te Kronnie G, Gaipa G. Validation of flow cytometric phospho-STAT5 as a diagnostic tool for juvenile myelomonocytic leukemia. *Blood Cancer J.* 2013 Nov 15;3:e160. doi: 10.1038/bcj.2013.56. *\*Dr. Bertaina collected samples and clinical data contributed the analysis. She contributed also to edit the manuscript.*
19. Buontempo F, Orsini E, Martins LR, Antunes I, Lonetti A, Chiarini F, Tabellini G, Evangelisti C, Evangelisti C, Melchionda F, Pession A, **Bertaina A\***, Locatelli F, McCubrey JA, Cappellini A, Barata JT, Martelli AM. Cytotoxic activity of the casein kinase 2 inhibitor CX-4945 against T-cell acute lymphoblastic leukemia: targeting the unfolded protein response signaling. *Leukemia.* 2014 Mar;28(3):543-53. doi: 10.1038/leu.2013.349. *\*Dr. Bertaina collected samples and clinical data, contributed to data analysis and to edit manuscript.*
20. Lonetti A, Antunes IL, Chiarini F, Orsini E, Buontempo F, Ricci F, Tazzari PL, Pagliaro P, Melchionda F, Pession A, **Bertaina A\***, Locatelli F, McCubrey JA, Barata JT, Martelli AM. Activity of the pan-class I phosphoinositide 3-kinase inhibitor NVP-BKM120 in T-cell acute lymphoblastic leukemia. *Leukemia.* 2014 Jun;28(6):1196-206. doi: 10.1038/leu.2013.369. *\*Dr. Bertaina collected samples and clinical data, contributed to data analysis and to edit manuscript.*
21. Della Chiesa M, Falco M, Muccio L, **Bertaina A\***, Locatelli F, Moretta A. Impact of HCMV Infection on NK Cell Development and Function after HSCT. *Front Immunol.* 2013 Dec 13;4:458. doi: 10.3389/fimmu.2013.00458. Review. *\*Dr. Bertaina contributed to analyze the literature and to write the manuscript.*
22. Della Chiesa M, Falco M, **Bertaina A\***, Muccio L, Alicata C, Frassoni F, Locatelli F, Moretta L, Moretta A. Human cytomegalovirus infection promotes rapid maturation of NK cells expressing activating killer Ig-like receptor in patients transplanted with NKG2C<sup>-/-</sup> umbilical cord blood. *J Immunol.* 2014 Feb 15;192(4):1471-9. doi: 10.4049/jimmunol.1302053. *\*Dr. Bertaina collected at the samples of patients coming from Bambino Gesù' Children's Hospital,*

*collected all the data, extensively contributed in analyzing data and to write the manuscript.*

23. Zecca M, Strocchio L, Pagliara D, Comoli P, **Bertaina A\***, Giorgiani G, Perotti C, Corbella F, Brescia L, Locatelli F. HLA-haploidentical T cell-depleted allogeneic hematopoietic stem cell transplantation in children with Fanconi anemia. *Biol Blood Marrow Transplant.* 2014 Apr;20(4):571-6. doi: 10.1016/j.bbmt.2014.01.015. *\*Dr. Bertaina participated in the design of the study, enrolled patients, performed transplantations, collected data and largely contributed to the analysis of the data.*
24. Di Nardo M, Locatelli F, Palmer K, Amodeo A, Lorusso R, Belliato M, Cecchetti C, Perrotta D, Picardo S, **Bertaina A\***, Rutella S, Rycus P, Di Ciommo V, Holzgraefe B. Extracorporeal membrane oxygenation in pediatric recipients of hematopoietic stem cell transplantation: an updated analysis of the Extracorporeal Life Support Organization experience. *Intensive Care Med.* 2014 May;40(5):754-6. doi: 10.1007/s00134-014-3240-9. *\*Dr. Bertaina extensively contributed in analyzing and interpreting data and writing the manuscript.*
25. Torelli GF, Peragine N, Raponi S, Pagliara D, De Propriis MS, Vitale A, **Bertaina A\***, Barberi W, Moretta L, Basso G, Santoni A, Guarini A, Locatelli F, Foà R. Recognition of adult and pediatric acute lymphoblastic leukemia blasts by natural killer cells. *Haematologica.* 2014 Jul;99(7):1248-54. doi: 10.3324/haematol.2013.101931. *\*Dr. Bertaina treated leukemia patients, collected pediatric samples and clinical data, extensively contributed to analyze data and to edit the manuscript.*
26. **Bertaina A**, Merli P, Rutella S, Pagliara D, Bernardo ME, Masetti R, Pende D, Falco M, Handgretinger R, Moretta F, Lucarelli B, Brescia LP, Li Pira G, Testi M, Cancrini C, Kabbara N, Carsetti R, Finocchi A, Moretta A, Moretta L, Locatelli F. HLA- haploidentical stem cell transplantation after removal of  $\alpha\beta+$  T and B cells in children with nonmalignant disorders. *Blood.* 2014 Jul 31;124(5):822-6. doi: 10.1182/blood-2014-03-563817.
27. Spartà AM, Bressanin D, Chiarini F, Lonetti A, Cappellini A, Evangelisti C, Evangelisti C, Melchionda F, Pession A, **Bertaina A\***, Locatelli F, McCubrey JA, Martelli AM. Therapeutic targeting of Polo-like kinase-1 and Aurora kinases in T-cell acute lymphoblastic leukemia. *Cell Cycle.* 2014;13(14):2237-47. doi: 10.4161/cc.29267. *\*Dr. Bertaina collected samples and clinical data, contributed to data analysis and to edit manuscript.*
28. Di Nardo M, Locatelli F, Di Florio F, Cecchetti C, Amodeo A, Rutella S, **Bertaina A**. Extracorporeal membrane oxygenation as a bridge to allogeneic T-cell depleted hematopoietic stem cell transplantation in infants with severe combined immune deficiency: is it feasible? *Intensive Care Med.* 2014 Oct;40(10):1600-1. doi: 10.1007/s00134-014-3432-3.
29. Oevermann L, Michaelis SU, Mezger M, Lang P, Toporski J, **Bertaina A**, Zecca M, Moretta L, Locatelli F, Handgretinger R. KIR B haplotype donors confer a reduced risk for relapse after haploidentical transplantation in children with ALL. *Blood.* 2014 Oct 23;124(17):2744-7. doi: 10.1182/blood-2014-03-565069. *\*Dr. Bertaina transplanted patients coming from Bambino Gesù' Children's Hospital, collected samples and clinical data, contributed to data analysis and to write the manuscript.*
30. Rutella S, Filippini P, Bertaina V, Li Pira G, Altomare L, Ceccarelli S, Brescia LP, Lucarelli B, Girolami E, Conflitti G, Cefalo MG, **Bertaina A\***, Corsetti T, Moretta L, Locatelli F. Mobilization of healthy donors with plerixafor affects the cellular composition of T-cell

- receptor (TCR)- $\alpha\beta$ /CD19-depleted haploidentical stem cell grafts. *J Transl Med.* 2014 Sep 2;12:240. doi: 10.1186/s12967-014-0240-z. \*Dr. Bertaina transplanted patients coming from Bambino Gesù' Children's Hospital, collected samples of donors, contributed to collect and interpret data analysis and to edit the manuscript.
31. Ciofi Degli Atti M, Bernaschi P, Carletti M, Luzzi I, García-Fernández A, **Bertaina A\***, Sisto A, Locatelli F, Raponi M. An outbreak of extremely drug-resistant *Pseudomonas aeruginosa* in a tertiary care pediatric hospital in Italy. *BMC Infect Dis.* 2014 Sep 10;14:494. doi: 10.1186/1471-2334-14-494. \*Dr. Bertaina treated all patients, collected clinical data and contributed to the analysis.
  32. Evangelisti C, Evangelisti C, Teti G, Chiarini F, Falconi M, Melchionda F, Pession A, **Bertaina A\***, Locatelli F, McCubrey JA, Beak DJ, Bittman R, Pyne S, Pyne NJ, Martelli AM. Assessment of the effect of sphingosine kinase inhibitors on apoptosis, unfolded protein response and autophagy of T-cell acute lymphoblastic leukemia cells; indications for novel therapeutics. *Oncotarget.* 2014 Sep 15;5(17):7886-901. \*Dr. Bertaina collected samples and clinical data, contributed to data analysis and to edit manuscript.
  33. 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.
  34. 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.
  35. 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.
  36. Airoidi 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. \*these two authors equally contributed as first name.
  37. 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.*
38. 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.*
39. 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.*
40. 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.*
41. 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.*
42. 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.*
43. 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- $\kappa$ 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.*
44. 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.
45. 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.*
46. 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.*
  47. 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):1511-7. 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.*
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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. \*This 2 authors equally contributed as a last author.

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2. Boncimino A, **Bertaina A**, Locatelli F. Cord blood transplantation in patients with hemoglobinopathies. *Transfus Apher Sci*. 2010 Jun;42(3):277-81. doi: 10.1016/j.transci.2010.03.006.
3. Locatelli F, **Bertaina A**. Reconstitution of repertoire of natural killer cell receptors after transplantation: just a question of time? *Bone Marrow Transplant*. 2010 Jun;45(6):968-9. doi: 10.1038/bmt.2010.116.
4. **Bertaina A**, Bernardo ME, Mastronuzzi A, La Nasa G, Locatelli F. The role of reduced intensity preparative regimens in patients with thalassemia given hematopoietic transplantation. *Ann N Y*

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6. Locatelli F, Vinti L, Palumbo G, Rossi F, **Bertaina A\***, Mastronuzzi A, Bernardo ME, Rutella S, Dellabona P, Giorgiani G, Moretta A, Moretta L. Strategies to optimize the outcome of children given T-cell depleted HLA-haploidentical hematopoietic stem cell transplantation. *Best Pract Res Clin Haematol.* 2011 Sep;24(3):339-49. doi: 10.1016/j.beha.2011.04.004. Epub 2011 Jul 7. Review. \*\*Dr. Bertaina contributed to analyze the literature and to write the manuscript.
7. Locatelli F, Bauquet A, Palumbo G, Moretta F, **Bertaina A**. Negative depletion of  $\alpha/\beta$ + T cells and of CD19+ B lymphocytes: a novel frontier to optimize the effect of innate immunity in HLA-mismatched hematopoietic stem cell transplantation. *Immunol Lett.* 2013 Sep-Oct;155(1-2):21-3. doi: 10.1016/j.imlet.2013.09.027. Review.
8. Locatelli F, Algeri M, Trevisan V, **Bertaina A**. Remestemcel-L for the treatment of graft versus host disease. *Expert Rev Clin Immunol.* 2017 Jan;13(1):43-56.
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10. **Bertaina A**, Pitisci A, Sinibaldi M, Algeri M. T Cell-Depleted and T Cell-Replete HLA-Haploidentical Stem Cell Transplantation for Non-malignant Disorders. *Curr Hematol Malig Rep.* 2017 Jan 23. doi: 10.1007/s11899-017-0364-3. [Epub ahead of print] Review.

## **Chapters**

- In 2012, Dr. Bertaina contributed to writing the chapter entitled '*HSCT in children with myelodysplastic syndromes*' of the 6<sup>th</sup> Edition of The EBMT Handbook, a text largely consulted by transplanters over the world.

## **ABSTRACTS**

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- 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 21<sup>st</sup> *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 21<sup>st</sup> *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 *57<sup>th</sup> ASH Annual Meeting (2015)* entitled ‘MicroRNA-150 regulates STAT5b levels in JMML’.
  - **Oral Communication** at the *57<sup>th</sup> 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 *7<sup>th</sup> 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 *41<sup>st</sup> 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’.
  - **Oral Communication** at the *40<sup>th</sup> EBMT Annual Meeting (2014, Abstract# 1915)* entitled ‘Outcome of children developing grade III-IV acute GvHD after allogeneic hematopoietic stem cell transplantation’.
  - **Oral Communication** at the *55<sup>th</sup> ASH Annual Meeting and Exposition (December 7-10, 2013, Abstract #60431)* entitled ‘Removal Of Alpha/Beta+ T Cells and Of CD19+ B Cells From The Graft Translates Into Rapid Engraftment, Absence Of Visceral Graft-Versus- Host Disease and Low Transplant-Related Mortality In Children With Acute Leukemia Given HLA-Haploidentical Hematopoietic Stem Cell Transplantation’.
  - **Oral Communication** at the *EBMT Annual Meeting 2013 (Abstract# 961)* entitled ‘HLA haploidentical stem cell transplantation after removal of alpha/beta+ T-lymphocytes and B-lymphocytes: a new transplant option for children with life-threatening, non-malignant disorders lacking a HLA-identical donor’.
  - **Poster presentation** at the *54<sup>th</sup> ASH Annual Meeting (December 2012)* entitled ‘HLA haploidentical stem cell transplantation after removal of  $\alpha\beta^+$  T lymphocytes and B lymphocytes is an effective treatment for children with life-threatening, non-malignant disorders’.
  - **Oral communication** at the *XXXVII Congresso Nazionale AIEOP (May 2012)* in the plenary session entitled ‘Trapianto aploidentico dopo procedura di deplezione dei Linfociti T alfa/beta positivi e B CD19+ in pazienti pediatrici con leucemia ad alto rischio’.

## INVITED PRESENTATIONS

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- National Annual Meeting GITMO (Gruppo Italiano per il Trapianto di Midollo), Milano, May 5<sup>th</sup>, 2017, Lecture entitled ‘MicroRNA, cytokines and GvHD in pediatric patients: not only miR155’.
- Autologous and allogeneic transplantation: stem cells, GvHD and indications. February 16<sup>th</sup>, 2017, Rome, Policlinico Gemelli, Lecture entitled ‘GvHD prophylaxis and treatment in children’.
- 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’.
- Oral Communication at the 45° Congresso Nazionale SIE, Firenze, entitled ‘Allogeneic Hematopoietic Stem Cell Transplantation in pediatrics patients with non-malignant disorders’.
- National Annual Meeting GITMO (Gruppo Italiano per il Trapianto di Midollo), Bologna, May 5<sup>th</sup>, 2016, Lecture entitled ‘Haploidentical hematopoietic stem cell transplantation in children: 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’.
- Il Punto sulle Emoglobinopatie, Catania, June 24<sup>th</sup>, 2016. Lecture entitled ‘Hematopoietic stem cell transplantation in hemoglobinopathies’.
- Scientific Meeting on Thalassemia Major, Matera, March 6<sup>th</sup>, 2016. Lecture entitled ‘Hematopoietic stem cell transplantation and gene-therapy’.
- 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’.
- Scientific Meeting ‘La vita dopo il Midollo’, Padova, October 2016. Lecture entitled ‘HSCT tomorrow: the futur of transplant’.
- EBMT-Pediatric Disease Meeting, Israel 2014, Lecture entitled ‘Biomarkers of acute renal injury: what to use in paediatric patients given HSCT?’.
- Acute Leukemia Working Party of EBMT, Marseille November 2013, Lecture entitled ‘Haplo-experience in children’.
- I-BFM Board Meeting, Frankfurt March 4<sup>th</sup>, 2015, Conditioning strategies for alternative donors (UCBT and haplo-HSCT), the Rome experience.
- 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  $\alpha/\beta$  T-Cell Depletion Have a Comparable Outcome’.

#### Other Presentations/Teaching:

- 17° CORSO POST-UNIVERSITARIO IN IMMUNOLOGIA CLINICA - 2° AUTOIMMUNITY AND IMMUNODEFICIENCY DAY , Verona, October 14<sup>th</sup>, 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.
- Update on antifungal treatment: talking with experts. Rome, September 2011. Teaching presentation entitled ‘Fungal infections in children given hematopoietic stem cell transplantation’.
- Lecture to nurses, Ospedale Pediatrico Bambino Gesù’, August 2011, Rome. Teaching presentation entitled ‘Acute leukemia in children’.
- Lecture to Resident in Pediatrics (Tor Vergata University, Ospedale Pediatrico Bambino Gesù’, Rome), April 2012, Rome. Teaching presentation entitled ‘HSCT from a HLA-partially matched family donor in pediatric patients affected by hematological disorders.
- Lecture to nurses, Ospedale Pediatrico Bambino Gesù’, November 2014, Rome. Teaching presentation entitled ‘Haploidentical stem cell transplantation in children’.

#### HONORS

- In October 2015, Dr. Bertaina was invited by the President of Italian Republic, to participate to the Annual National Ceremony for Cancer Research, which took place at the Palazzo del Quirinale.

#### PROFESSIONAL SOCIETY MEMBERSHIPS

- AIEOP – Associazione Italiana di Ematologia ed Oncologia Pediatrica
- GITMO – Italian Group for Bone Marrow Transplantation
- EBMT – European Society for Blood and Marrow Transplantation
- ASH – American Society of Hematology

#### NATIONAL COMMITTEE SERVICE

- **Member, Comitato per il Controllo delle Infezioni Ospedaliere (CCIO)** at the Ospedale Pediatrico Bambino Gesù’, Rome. 2011 – present
- **Board Member, EBMT PDWP (European Society for Blood and Marrow Transplantation Pediatric Disease Working Party)**. 2014 – present
- **Head, Hematopoietic Stem Cell Transplantation Working Party Group of AIEOP (Italian Association of Pediatric Hematology and Oncology)**. 2015 – present
- **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 recently 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 part of the next guidelines of the Inborn Errors Working Group of EBMT. 2016 – present

- **Chair, Paediatric transplantation Session, EBMT 43<sup>rd</sup> Annual Meeting, Marseille, France, March 26-29, 2017.**

PEER REVIEWED JOURNAL REVIEWER:

- Bone Marrow Transplantation
- Frontiers in Immunology

Alice Bertaina, MD, PhD