

Elenco delle 15 pubblicazioni più significative negli anni recenti del Prof. Franco Locatelli

1. Frangoul H, de la Fuente J, Chopra Y, Meisel R, Amrolia PJ, Algeri M, Sharma A, Cappellini MD, Corbacioglu S, Kattamis A, Lobitz S, de Montalembert M, Rondelli D, Sheth S, Steinberg MH, Walters MC, Boerner K, Liu T, Zairis S, Hobbs W, Grupp SA, **Locatelli F**. Exa-cel in Children with Transfusion-Dependent β -Thalassemia or Sickle Cell Disease. *N Engl J Med*. 2026 Jun 11.
2. **Locatelli F**, Buldini B, Pigazzi M, Rizzari C, Menna G, Fagioli F, Tumino M, Lo Nigro L, D'Angelo P, Cefalo MG, Paganelli V, Strocchio L, Becilli M, Spinelli M, Giagnuolo G, Saglio F, Santoro N, Micalizzi C, Zecca M, Masetti R, Merli P. Improved outcomes of the refined risk-stratification and risk-adapted therapy in children with acute myeloid leukemia: final results of the AIEOP AML 2013. *J Hematol Oncol*. 2026 Apr 20;19(1):35.
3. **Locatelli F**. More Options for Gene Editing in Hemoglobinopathies. *N Engl J Med*. 2026 Apr 2;394(13):1338-1340.
4. Becilli M, Metzler M, Bracaglia C, Nicolai R, Krickau T, Del Bufalo F, Rosignoli C, Marasco E, Naumann-Bartsch N, Messia V, Merli P, Pagliara D, Algeri M, Secinaro A, Cefalo MG, Diomedi Camassei F, Bertaina V, Sinibaldi M, Insalaco A, Aigner M, Leone G, Li Pira G, Gunetti M, De Angelis B, Völkl S, Hanssens L, Müller F, Mackensen A, Quintarelli C, Schett G, De Benedetti F, **Locatelli F**. Fresh-to-fresh autologous CD19-CAR T cells for pediatric patients with severe and refractory autoimmune disease: a case series from two european centers. **Nature Medicine** 2026 Feb 5.
5. **Locatelli F**, Pagliara D, De Ioris MA, Becilli M, Del Baldo G, Serra A, Mastronuzzi A, Cefalo MG, Li Pira G, Leone G, Bertaina V, Fabozzi F, Di Nardo M, Rosignoli C, D'Andrea ML, Crocoli A, Vennarini S, Sinibaldi M, Di Cecca S, Guercio M, Iaffaldano L, Lucarelli B, Algeri M, Merli P, Colafati GS, De Angelis B, Quintarelli C, Del Bufalo F. GD2-targeting CAR T cells in high-risk neuroblastoma: a phase 1/2 trial. **Nature Med**. 2025;31:3689-3699.
6. Quintarelli C, Del Bufalo F, De Ioris MA, Guercio M, Algeri M, Pagliara D, Silvestris DA, Di Nardo M, Sinibaldi M, Di Cecca S, Iaffaldano L, Manni S, Fustaino V, Garganese M, Colafati S, Bertaina V, Becilli M, Mastronuzzi A, Fabozzi F, Gunetti M, Iacovelli S, Bugianesi R, Macchia S, Li Pira G, Cefalo MG, Leone G, Del Baldo G, De Angelis B, **Locatelli F**. Donor-derived GD2-specific CAR T cells in relapsed or refractory neuroblastoma. **Nature Med** 2025;31:849-860.
7. **Locatelli F**, Lang P, Wall D, Meisel R, Corbacioglu S, Li AM, de la Fuente J, Shah AJ, Carpenter B, Kwiatkowski JL, Mapara M, Liem RI, Cappellini MD, Algeri M, Kattamis A, Sheth S, Grupp S, Handgretinger R, Kohli P, Shi D, Ross L, Bobruff Y, Simard C, Zhang L, Morrow PK, Hobbs WE, Frangoul H. Exagamglogene Autotemcel for Transfusion-Dependent β -Thalassemia. **N Engl J Med** 2024;390:1663-1676.
8. Frangoul H, **Locatelli F**, Sharma A, Bhatia M, Mapara M, Molinari L, Wall D, Liem RI, Telfer P, Shah AJ, Cavazzana M, Corbacioglu S, Rondelli D, Meisel R, Dedeken L, Lobitz S, de Montalembert M, Steinberg MH, Walters MC, Eckrich MJ, Imren S, Bower L, Simard C, Zhou W, Xuan F, Morrow PK, Hobbs WE, Grupp SA. Exagamglogene Autotemcel for Severe Sickle Cell Disease. **N Engl J Med** 2024;390:1649-1662.
9. Kwiatkowski JL, Walters MC, Hongeng S, Yannaki E, Kulozik AE, Kunz JB, Sauer MG, Thrasher AJ, Thuret I, Lal A, Tao G, Ali S, Thakar HL, Elliot H, Lodaya A, Lee J, Colvin RA, **Locatelli F**, Thompson AA. Betibeglogene autotemcel gene therapy in patients with transfusion-dependent, severe genotype β -thalassaemia (HGB-212): a non-randomised, multicentre, single-arm, open-label, single-dose, phase 3 trial. **Lancet**. 2024;404:2175-2186.
10. Oh BLZ, Shimasaki N, Coustan-Smith E, Chan E, Poon L, Lee SHR, Yeap F, Tan LK, Chai LYA, Le Bert N, Tan N, Bertoletti A, Chen SP, Del Bufalo F, Becilli M, **Locatelli F**, Yeoh AEJ, Campana D. Fratricide-resistant CD7-CAR T cells in T-ALL. **Nature Med** 2024;30:3687-3696.

11. Del Bufalo F, De Angelis B, Caruana I, Del Baldo G, De Ioris MA, Serra A, Mastronuzzi A, Cefalo MG, Pagliara D, Amicucci M, Li Pira G, Leone G, Bertaina V, Sinibaldi M, Di Cecca S, Guercio M, Abbaszadeh Z, Iaffaldano L, Gunetti M, Iacovelli S, Bugianesi R, Macchia S, Algeri M, Merli P, Galaverna F, Abbas R, Garganese MC, Villani MF, Colafati GS, Bonetti F, Rabusin M, Perruccio K, Folsi V, Quintarelli C, **Locatelli F**. GD2-CART01 for Relapsed or Refractory High-Risk Neuroblastoma. **N Engl J Med** 2023;388:1284-1295.
12. **Locatelli F**, Thompson AA, Kwiatkowski JL, Porter JB, Thrasher AJ, Hongeng S, Sauer MG, Thuret I, Lal A, Algeri M, Schneiderman J, Olson TS, Carpenter B, Amrolia PJ, Anurathapan U, Schambach A, Chabannon C, Schmidt M, Labik I, Elliot H, Guo R, Asmal M, Colvin RA, Walters MC. Betibeglogene Autotemcel Gene Therapy for Non- β^0/β^0 Genotype β -Thalassemia. **N Engl J Med** 2022;386:415-427.
13. Zeiser R, Polverelli N, Ram R, Hashmi SK, Chakraverty R, Middeke JM, Musso M, Giebel S, Uzay A, Langmuir P, Hollaender N, Gowda M, Stefanelli T, Lee SJ, Teshima T, **Locatelli F**. Ruxolitinib for Glucocorticoid-Refractory Chronic Graft-versus-Host Disease. **N Engl J Med** 2021;385:228-238.
14. **Locatelli F**, Zugmaier G, Rizzari C, Morris JD, Gruhn B, Klingebiel T, Parasole R, Linderkamp C, Flotho C, Petit A, Micalizzi C, Mergen N, Mohammad A, Kormany WN, Eckert C, Möricke A, Sartor M, Hrusak O, Peters C, Saha V, Vinti L, von Stackelberg A. Effect of Blinatumomab vs Chemotherapy on Event-Free Survival Among Children With High-risk First-Relapse B-Cell Acute Lymphoblastic Leukemia: A Randomized Clinical Trial. **JAMA**. 2021;325:843-854.
15. **Locatelli F**, Jordan MB, Allen C, Cesaro S, Rizzari C, Rao A, Degar B, Garrington TP, Sevilla J, Putti MC, Fagioli F, Ahlmann M, Dapena Diaz JL, Henry M, De Benedetti F, Grom A, Lapeyre G, Jacqmin P, Ballabio M, de Min C. Emapalumab in Children with Primary Hemophagocytic Lymphohistiocytosis. **N Engl J Med** 2020;382:1811-1822.