

Original Articles in Scientific international Journals

1. Gene therapy for transfusion-dependent β -thalassemia. *N Engl J Med*. 2018. Accepted for publication.
2. Gene transfer into hematopoietic stem cells reduces HLH manifestations in a murine model of Munc13-4 deficiency. Soheili T, Durand A, Sepulveda FE, Rivière J, Lagresle-Peyrou C, Sadek H, de Saint Basile G, Martin S, Mavilio F, **Cavazzana M**, André-Schmutz I. *Blood Adv*. 2017 Dec 21;1(27):2781-2789. doi: 10.1182/bloodadvances.2017012088. eCollection 2017 Dec 26.
3. Successful haematopoietic stem cell transplantation in a case of pulmonary alveolar proteinosis due to GM-CSF receptor deficiency. Frémond ML, Hadchouel A, Schweitzer C, Berteloot L, Bruneau J, Bonnet C, Cros G, Briand C, Magnani A, Pochon C, Delacourt C, **Cavazzana M**, Moshous D, Fischer A, Blanche S, Blic J, Neven B. *Thorax*. 2017 Dec 20. pii: thoraxjnl-2017-211076. doi: 10.1136/thoraxjnl-2017-211076
4. Long-term follow up of IPEX syndrome patients after different therapeutic strategies: an international multicenter retrospective study. Barzaghi F, Amaya Hernandez LC, Neven B, Ricci S, Cucuk ZY, Blessing J, Nademi Z, Slatter MA, Ulloa ER, Shcherbina A, Roppelt A, Worth A, Silva J, Aiuti A, Murguia-Favela L, Speckmann C, Carneiro-Sampaio M, Fernandes JF, Baris S, Ozen A, Karakoc-Aydiner E, Kiykim A, Schulz A, Steinmann S, Notarangelo LD, Gambineri E, Lionetti P, Shearer WT, Forbes L, Martinez C, Moshous D, Blanche S, Fisher A, Ruummele FM, Tissandier C, Ouachee-Chardin M, Rieux-Laucat F, **Cavazzana M**, Qasim W, Lucarelli B, Albert MH, Kobayashi I, Alonso L, Diaz De Heredia C, Kanegane H, Lawitschka A, Seo JJ, Gonzalez-Vicent M, Diaz MA, Goyal RK, Sauer MG, Yesilipek A, Kim M, Yilmaz-Demirdag Y, Bhatia M, Khlevner J, Richmond Padilla EJ, 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 ML, Bredius RG, Kafwak K, Haddad E, Seidel MG, Duckers G, Pai SY, Dvorak CC, Ehl S, Locatelli F, Goldman F, Gennery AR, Cowan MJ, Roncarolo MG, Bacchetta R; PIDTC and IEWP of EBMT. *J Allergy Clin Immunol*. 2017 Dec 11. pii: S0091-6749(17)31893-6. doi: 10.1016/j.jaci.2017.10.041.
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6. Auer F, Ingenhag D, Pinkert S, Kracker S, Hacein-Bey-Abina S, **Cavazzana M**, Gombert M, Martin-Lorenzo A, Lin MH, Vicente-Dueñas C, Sánchez-García I, Borkhardt A, Hauer J. Activation-induced cytidine deaminase prevents pro-B cell acute lymphoblastic leukemia by functioning as a negative regulator in Rag1 deficient pro-B cells. *Oncotarget*. 2017 Sep 7;8(44):75797-75807. doi: 10.18632/oncotarget.20563.
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