

CENSIMENTO NEW DRUGS: CATEGORIA PATOLOGIE NON ONCOEMATOLOGICHE						
STUDIO	FARMACO	FASE	PATOLOGIA	CENTRO COORDINATORE	ALTRI CENTRI PARTECIPANTI	SPONSOR
DIFETTI DELLA COAGULAZIONE						
BoehringerIngelheim n. 1160.106. Open-label, randomized, parallel-group, active-controlled, multi-centre, non-inferiority study of dabigatranetexilate versus standard of care for venous thromboembolism treatment in children from birth to less than 18 years of age: The DIVERSITY study	Dabigatranetexilate	IIb/III	Venous thromboembolism	Ospedale Pediatrico Bambin Gesù, Roma	IRCCS Fondazione Ospedale Maggiore, Milano; A.O. U. Policlinico "Paolo Giaccone", Palermo; Università degli Studi "La Sapienza", Roma; Ospedale Pediatrico Bambin Gesù, Roma; Ospedale Infantile Regina Margherita, Torino	BoehringerIngelheim
BoehringerIngelheim n. 1160.108 : Open label, single arm safety prospective cohort study of dabigatranetexilate for secondary prevention of venous thromboembolism in children from 0 to less than 18 years	Dabigatranetexilate	III	Secondary prevention of venous thromboembolism	Ospedale Pediatrico Bambin Gesù, Roma	A.O. U.. Policlinico "Paolo Giaccone", Palermo; Ospedale Pediatrico Bambin Gesù, Roma; Ospedale Infantile Regina Margherita, Torino	BoehringerIngelheim

BoehringerIngelheim n. 1321.7 - Studio clinico in aperto, non controllato, per valutare la sicurezza di una singola dose di Idarucizumab, somministrato per via endovenosa in pazienti pediatrici arruolati negli studi in corso di fase IIb/III con DabigatranEtexilato per il trattamento e la prevenzione secondaria del tromboembolismo venoso	Idarucizumab	IIb/III	Pazienti pediatrici arruolati negli studi in corso di fase IIb/III con DabigatranEtexilato per il trattamento e la prevenzione secondaria del tromboembolismo venoso	Ospedale Pediatrico Bambin Gesù, Roma	A.O. U. Policlinico "Paolo Giaccone", Palermo; Università degli Studi "La Sapienza", Roma; Ospedale Pediatrico Bambin Gesù, Roma; Ospedale Infantile Regina Margherita, Torino	BoehringerIngelheim
A Multicenter Phase III Uncontrolled Open-label Trial to Evaluate Safety and Efficacy of BAY81-8973 in Children With Severe Hemophilia A Under Prophylaxis Therapy (NCT01311648)	BAY81-8973	III	Severe Hemophilia A Under Prophylaxis Therapy	Fondazione IRCCS Ca' Granda - Ospedale Maggiore Policlinico, Milano	Ospedale Pediatrico Bambin Gesù, Roma; Policlinico Universitario Gemelli, Roma; U.O.C. Clinica Pediatrica "B. Trambusti" Bari, A.O. di Padova	Bayer
Baxalta. A Phase 3, Prospective, Multicentre, Uncontrolled, Open-Label Clinical Study To Determine The Efficacy, Safety, And Tolerability of rvwf With Or Without ADVATE In The Treatment And Prevention Of Bleeding Episodes, The Efficacy And Safety Of rvwf In Elective Surgeries, And The Pharmacokinetics (PK) Of rvwf In Children Diagnosed With Severe von Willebrand Disease.	Rvwf With Or Without ADVATE	III	Treatment And Prevention Of Bleeding Episodes	Fondazione IRCCS CA' Granda Ospedale Maggiore Policlinico, Milano	A.O.U. Careggi, Firenze; A.O. Pediatrica SantobonoPausillipon, Napoli; Ospedale Bambino Gesù, Roma	Baxalta

An Open-label Single-arm Multicentre Non-controlled Phase 3a Trial Investigating Safety and Efficacy of N8-GP in Prophylaxis and Treatment of Bleeding Episodes in Previously Untreated Paediatric Patients With Severe Haemophilia A	Turoctocog alfa pegol	IIIa	Untreated Paediatric Patients With Severe Haemophilia A	A.O.U. Careggi, Firenze	A.O.U. Careggi, Firenze; Ospedale Infantile Regina Margherita, Torino	Novo Nordisk A/S
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STUDIO	FARMACO	FASE	PATOLOGIA	CENTRO COORDINATORE	ALTRI CENTRI PARTECIPANTI	SPONSOR
IMMUNODEFICIENZE						
A phase I/II clinical trial of hematopoietic stem cell gene therapy for the Wiskott-Aldrich Syndrome	CD34+ autologhe ingegnerizzate con vettore lentivirale	I/II	Sindrome di Wiskott Aldrich	U.O. Immunoematologia Pediatrica, Ospedale San Raffaele, Milano	No	Orchard Ltd
A Single Arm, Open Label Clinical Study of Haematopoietic Stem Cell Gene Therapy with Cryopreserved Autologous CD34+ Cells Transduced with Lentiviral Vector encoding WAS cDNA in Subjects with Wiskott-Aldrich Syndrome (WAS)	CD34+ autologhe ingegnerizzate con vettore lentivirale	III	Sindrome di Wiskott Aldrich	U.O. Immunoematologia Pediatrica, Ospedale San Raffaele, Milano	No	Orchard Ltd
A phase I/II clinical Trial of hematopoietic stem cell gene therapy for the treatment of Metachromatic Leukodystrophy	CD34+ autologhe ingegnerizzate con vettore lentivirale	I/II	Leucodistrofia metacromatica	U.O. Immunoematologia Pediatrica, Ospedale San Raffaele, Milano	No	Orchard Ltd

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 (MLD)	CD34+ autologhe ingegnerizzate con vettore lentivirale	III	Leucodistrofia metacromatica	U.O. Immunoematologia Pediatrica, Ospedale San Raffaele, Milano	No	Orchard Ltd
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	CD34+ autologhe ingegnerizzate con vettore lentivirale	I/II	Talassemia Beta	U.O. Immunoematologia Pediatrica, Ospedale San Raffaele, Milano	No	Ospedale San Raffaele
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 human alpha-L-iduronidase gene for the treatment of patients affected by Mucopolysaccharidosis Type I, Hurler variant	CD34+ autologhe ingegnerizzate con vettore lentivirale	I/II	MPSI Hurler	U.O. Immunoematologia Pediatrica, Ospedale San Raffaele, Milano	No	Ospedale San Raffaele
A multicentric exploratory non randomised non controlled prospective open label phase II study evaluating safety and efficacy of IBU, G-CSF and Plerixafor as a stem cell mobilisation regimen in patient affected by XCGD	Ibuprofene, plerixafor, lenogastrom, pantoparazolo	II	XCGD	U.O. Immunoematologia Pediatrica, Ospedale San Raffaele, Milano	Ospedale Bambino Gesù, Roma	Ospedale San Raffaele

STUDIO	FARMACO	FASE	PATOLOGIA	PI/CENTRO COORDINATORE	ALTRI CENTRI PARTECIPANTI	SPONSOR
PATOLOGIE DEL GLOBULO ROSSO						
An Open Label Extension Study of GBT440 Administered Orally to Patients with Sickle Cell Disease who have participated in GBT440 Clinical Trials	GBT440	III	Sickle Cell Disease	Azienda Ospedaliera di Padova	No	Global Blood Therapeutics
A phase 2, Multicenter, Open-Label Study to Assess Appropriate Dosing and to Evaluate Safety of Crizanlizumab, with or without Hydroxyurea/Hydroxycarbamide, in Sequential, Descending Age Groups of Pediatric Sickle Cell Disease Patients with Vaso-Occlusive Crisis	Crizanlizumab	II	Pediatric Sickle Cell Disease Patients with Vaso-Occlusive Crisis	Azienda Ospedaliera di Padova	A.O. di Padova; Ospedale San Luigi, Torino; Azienda Policlinico Di Modena	Novartis Pharmaceuticals