AGENZIA ITALIANA DEL FARMACO

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Gli studi vengono inseriti all'Ordine del Giorno esclusivamente nel momento in cui risulta disponibile l'intera documentazione validata e ritenuta idonea per avviare la fase di valutazione.
È importante sottolineare che l'inserimento in O.d.G. non garantisce che la valutazione venga completata o deliberata nella stessa seduta: esso rappresenta invece l'avvio formale dell'iter valutativo della procedura, che potrà richiedere ulteriori approfondimenti, integrazioni o successive discussioni in riunioni future.

INFO GENERALI	Info Studio	Titolo
CTIS ITALY - MS 2025-523845-90-00 ADDED MEMBER STATE AM-3 (Part I + Part II)	CRSP-AID-501 Interventistico con farmaco - No Profit Phase I/II Promotore: CRISPR Therapeutics AG Multinational	A Phase 1/2 Dose Evaluation Trial of the Safety and Preliminary Efficacy of AntiCD19 Allogeneic CRISPR-Cas9–Engineered T Cells (CTX112) in Adult Participants With Relapsed/Refractory Hematologic Autoimmune Disease
CEN ATMP RSO - 3770 INITIAL (Part I + Part II)	QOL-ONE PRO-CT Osservazionale con farmaco - No Profit Promotore: Associazione Culturale e di Ricerca QOL-ONE Mononational	A Real-World Observational Study on Patient-Reported Outcomes in Allogeneic Stem Cell Transplantation (Allo-SCT) and CAR-T Therapy
CTIS ITALY - MS 2026-525886-38-00 INITIAL (Part I + Part II)	ATSN-201-1 Interventistico con farmaco - Profit Phase I/II Promotore: Atsena Therapeutics Inc. Multinational	A Phase 1/2/3, Open-Label, Dose Escalation, Dose Expansion, and Randomized, Controlled Study to Evaluate the Safety and Efficacy of ATSN-201 Gene Therapy in Subjects with RS1-Associated X-linked Retinoschisis (LIGHTHOUSE)
CTIS ITALY - RMS 2025-524635-39-00 SUBSTANTIAL MODIFICATION SM-3 (Part I + Part II)	HIGM1-FT018-01 Interventistico con farmaco - Profit Phase I/II Promotore: San Raffaele Hospital Fondazione Telethon Ets Mononational	A Single Arm, Open Label, Single Center, Phase I/II Clinical Study evaluating Safety and Efficacy of gene therapy using Autologous CD4+ T-Cells Edited ExVivo at the CD40LG Locus by CRISPR/Cas9 and IDLV-based vector in Patients with Xlinked Hyper IgM Syndrome Type 1 (HIGM1).
CTIS ITALY - MS 2022-503140-41-00 SUBSTANTIAL MODIFICATION SM-14 (Part I + Part II)	IOV-MEL-301 Interventistico con farmaco - Profit Phase III Promotore: Iovance Biotherapeutics Inc. Multinational	A Phase 3, multicenter, randomized, open-label, parallel group, treatment study to assess the efficacy and safety of the lifileucel (LN-144, autologous tumor-infiltrating lymphocytes [TIL]) regimen in combination with pembrolizumab compared with pembrolizumab monotherapy in participants with untreated, unresectable or metastatic melanoma

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CEN ATMP RSO - 4260 INITIAL (Part I + Part II)	DISH-SCC Osservazionale senza farmaco e dispositivo - No Profit Promotore: Fondazione Policlinico Universitario Agostino Gemelli IRCCS Mononational	Development of immunotherapy strategies in head and neck squamous cell carcinoma
CEN ATMP RSO - 4408 INITIAL (Part I + Part II)	CART-T_PWH Osservazionale con farmaco - No Profit Promotore: Fondazione Policlinico Universitario A. Gemelli IRCCS Mononational	Sicurezza della terapia con CAR-T in persone con HIV e linfoma refrattario o recidivato: Studio retrospettivo multicentrico
CTIS ITALY - MS 2025-522949-22-00 SUBSTANTIAL MODIFICATION SM-1 (Part I + Part II)	SGT-003-301 Interventistico con farmaco - Profit Phase III Promotore: Solid Biosciences Inc Multinational	A Phase 3, Multicenter, Randomized, Double-Blind, Placebo-Controlled Study to Investigate the Efficacy of a Single Intravenous Dose of SGT-003 in Ambulant Males With Duchenne Muscular Dystrophy
CTIS ITALY - MS 2025-522207-15-01 SUBSTANTIAL MODIFICATION SM-8 (Part I + Part II)	039-101 Interventistico con farmaco - Profit Phase I/II Promotore: Aavantgarde Bio S.r.l. Multinational	An Open-label, Multicenter, Two Part, Ascending Dose Followed by a Controlled Trial to Assess the Safety and Efficacy of a Subretinal Administration of AAVB-039 in Participants with Stargardt Disease (STGD1) (CELESTE)
CTIS ITALY - MS 2024-514596-18-00 SUBSTANTIAL MODIFICATION SM-3 (Part I + Part II)	U1111-1325-1245 Interventistico con farmaco - Profit Phase I/II Promotore: Novartis Pharma AG Multinational	An open-label, multi-center, phase 1/2 study to assess safety, cellular kinetics and exploratory efficacy of rapcabtagene autoleucel in participants with difficult-to-treat rheumatoid arthritis and severe, refractory Sjogren's disease with organ involvement

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CTIS ITALY - RMS 2025-522821-37-00 INITIAL (Part I + Part II)	BETELGEUSE Interventistico con farmaco - No Profit Phase I Promotore: Azienda Ospedaliero-Universitaria Di Bologna IRCCS Istituto Di Ricerca E Di Cura A Carattere Scientifico Mononational	Basket trial for B-cEll driven severe auToimmunE and aLoimmune diseases (chronic Graft vErsUs host diseaSe) treated with anti-CD19 CAR T-cell thErapy
CTIS ITALY - RMS 2026-525847-34-00 INITIAL (Part I + Part II)	MPSVI-FT005-02 Interventistico con farmaco - Profit Phase I/II Promotore: Fondazione Telethon ETS Mononational	A Phase I/II Open-Label Extension of Study TIGEM1-MPSVI, Evaluating the Safety and Efficacy of FT005, an Adeno-Associated Viral Vector 8 to Deliver the Human ARSB Gene to the Liver of Subjects with Mucopolysaccharidosis type VI (MPS VI)
CTIS ITALY - MS 2023-506480-34-00 SUBSTANTIAL MODIFICATION SM-6 (Part II)	RP-A501-0123 Interventistico con farmaco - Profit Phase II Promotore: Merck Sharp & Dohme LLC Multinational	Gene Therapy for Danon Disease: A Phase 2 Study Evaluating the Efficacy and Safety of Intravenously Administered Adeno-Associated Virus Serotype 9 (rAAV9) Vector Containing the Human LAMP2 Isoform B Transgene (RP-A501; AAV9.LAMP2B) in Male Patients with Danon Disease
CTIS ITALY - MS 2025-524014-29-00 INITIAL (Part I + Part II)	D8312C00002 Interventistico con farmaco - Profit Phase III Promotore: AstraZeneca AB Multinational	A Phase III Open-Label, Randomised, Multicentre Study of Consolidation with a Single Administration of AZD0120, a Dual-Targeting Autologous Chimeric Antigen Receptor T-cell (CAR T) Therapy Directed Against BCMA and CD19, Compared with Continuous Standard Therapy in Participants with NDMM Who Are Ineligible to Receive ASCT as Initial Therapy (DURGA-5).
CTIS ITALY - MS 2024-512177-27-01 ADDED MEMBER STATE AM-3 (Part I + Part II)	000434 – ABLE-22 Interventistico con farmaco - Profit Phase III Promotore: Ferring Pharmaceuticals A/S Multinational	A phase 3, randomised, multi-center, open label trial to evaluate the safety and efficacy of intravesical nadofaragene firadenovec alone or in combination with chemotherapy (gemcitabine and docetaxel) or immunotherapy (pembrolizumab) in subjects with high-grade Bacillus Calmette-Guerin therapy (BCG) unresponsive non-muscle invasive bladder cancer (NMIBC)

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CTIS ITALY - MS 2024-512714-18-00 SUBSTANTIAL MODIFICATION SM-3 (Part II)	CYTB323N12101 Interventistico con farmaco - Profit Phase I/II Promotore: Novartis Pharma AG Multinational	An open-label, multi-center, phase 1/2 study to assess safety, efficacy, and cellular kinetics of YTB323 in participants with Relapsing Multiple Sclerosis with breakthrough disease activity during previous treatment with a highly efficacious therapy
CTIS ITALY - MS 2026-525367-40-00 INITIAL (Part I + Part II)	D8316C00001 (DURGA 6) Interventistico con farmaco - Profit Phase III Promotore: AstraZeneca AB Multinational	A Phase III, Open-label, Multicentre, Randomised Study Comparing Consolidation Treatment with AZD0120, an Autologous Dual Targeting Chimeric Antigen Receptor T-cell (CAR-T) Therapy Directed Against BCMA and CD19, versus Autologous Stem Cell Transplant (ASCT) in Participants with Newly Diagnosed Multiple Myeloma who are Transplant Eligible (TE NDMM) (DURGA-6)
CEN ATMP RSO - 4024 INITIAL (Part I + Part II)	iMMunoPro Osservazionale con farmaco - No Profit Promotore: AOU DI BOLOGNA POLICLINICO S.ORSOLAMALPIGHI Mononational	Multicenter, observational, prospective and retrospective Italian study on infections and anti-infective prophylaxis strategies in Relapsed/Refractory Multiple Myeloma patients undergoing immunotherapy with CAR-T cells or bispecific antibodies in real-life
CTIS ITALY - RMS 2026-525714-59-00 INITIAL (Part I + Part II)	SNIPER-LTFU Interventistico con farmaco - Profit Phase I/II Promotore: Eir Biotherapies S.r.l. Mononational	SNIPER-LTFU: Long-term follow-up study of patients administered with RR001 in the interventional Parent SNIPER study
CTIS ITALY - MS 2023-505177-32-00 SUBSTANTIAL MODIFICATION SM-8 (Part I + Part II)	V940-004 Interventistico con farmaco - Profit Phase II Promotore: Merck Sharp & Dohme LLC Multinational	A Phase 2, Randomized, Double-blind, Clinical Study of V940 (mRNA-4157) Plus Pembrolizumab (MK-3475) Versus Placebo Plus Pembrolizumab in the Adjuvant Treatment of Participants With Renal Cell Carcinoma (INTerpath-004)
CTIS ITALY - MS 2023-506327-29-00 SUBSTANTIAL MODIFICATION SM-6 (Part I + Part II)	V940-009 Interventistico con farmaco - Profit Phase III Promotore: Merck Sharp & Dohme LLC Multinational	A Phase 3 Randomized Double-blind Study of Adjuvant Pembrolizumab With or Without V940 in Participants With Resectable Stage II to IIIB (N2) NSCLC not Achieving pCR After Receiving Neoadjuvant Pembrolizumab With Platinum-based Doublet Chemotherapy (INTerpath-009)

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CTIS ITALY - MS 2025-523660-21-00 SUBSTANTIAL MODIFICATION SM-5 (Part II)	C0371017 Interventistico con farmaco - Profit Phase III Promotore: Pfizer Inc. Multinational	A Phase 3, Non-Investigational Product, Multi Country Cohort Study to Describe the Long-Term Safety and Effectiveness of a Prior Single-Dose Treatment With Investigative Giroctocogene Fitelparvovec or Fidanacogene Elaparvovec in Participants With Hemophilia A or Hemophilia B, Respectively