



## Comitato Etico per le Sperimentazioni Cliniche relative alle Terapie Avanzate

**O.d.G. Seduta del 14/04/2026**

*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 dliberata 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 2024-513349-35-00 SUBSTANTIAL MODIFICATION SM-4 (Part I)	VX21-CTX001-141 Interventistico con farmaco - Profit Phase III Promotore: Vertex	A Phase 3 Study to Evaluate the Safety and Efficacy of a Single Dose of CTX001 in Pediatric Subjects With Transfusion-Dependent $\beta$ -Thalassemia

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	Pharmaceuticals Inc. Multinational	
CEN ATMP RSO - 4260 INITIAL	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
CTIS ITALY - MS 2025-523660-21-00 INITIAL (Part I + 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

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CTIS ITALY - MS 2023-503666-23-00 SUBSTANTIAL MODIFICATION SM-6 (Part I + Part II)	RGX-314-3101/M23-409 Interventistico con farmaco - Profit Phase III Promotore: Abbvie Deutschland GmbH & Co. KG Multinational	A Randomized, Partially Masked, Controlled, Phase 3 Clinical Study to Evaluate the Efficacy and Safety of RGX-314 Gene Therapy in Participants with nAMD (ASCENT)
CTIS ITALY - MS 2025-523285-25-00 INITIAL (Part I + Part II)	D8311C00001 Interventistico con farmaco - Profit Phase III Promotore: AstraZeneca AB Multinational	A Phase III Open-label, Randomised, Multicentre Study Comparing AZD0120, a Dual-Targeting Autologous Chimeric Antigen Receptor T-cell (CAR-T) Therapy Directed Against BCMA and CD19, versus Standard Regimens in Participants with Relapsed Refractory Multiple Myeloma (DURGA-4)
CTIS ITALY - MS 2025-523448-10-00 INITIAL (Part I + Part II)	KTE-C19-104 Interventistico con farmaco - Profit Phase II Promotore: Gvhd Resistant to ruxolitinib	Efficacy of cell therapy with allo-MSCs in patients with gvhd resistant to ruxolitinib second line therapy: a Phase IIB Clinical Trial - MSC-GRR2 Study

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	and steroids Multinational	
CTIS ITALY - MS 2023-504786-23-00 SUBSTANTIAL MODIFICATION SM-15 (Part I + Part II)	VX21-522-001 Interventistico con farmaco - Profit Phase I/II Promotore: Vertex Pharmaceuticals Inc. Multinational	A Phase 1/2 Dose-escalation Study Evaluating the Safety, Tolerability, and Efficacy of VX-522 in Subjects 18 Years of Age and Older With Cystic Fibrosis and a CFTR Genotype Not Responsive to CFTR Modulator Therapy
CTIS ITALY - RMS 2024-514053-30-00 SUBSTANTIAL MODIFICATION SM-1 (Part I + Part II)	IRST153.09 Interventistico con farmaco - No Profit Phase II Promotore: Istituto Romagnolo Per Lo Studio Dei Tumori Dino Amadori IRST S.r.l. Mononational	Sequential immunochemotherapy treatment with Pembrolizumab plus Dendritic Cell (DC) Vaccine followed by Trifluridine/Tipiracil plus Bevacizumab in refractory mismatch-repair-proficient (pMMR) or microsatellite-stable (MSS) metastatic colorectal cancer

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<p>CTIS ITALY - MS 2023-506267-33-00 SUBSTANTIAL MODIFICATION SM-8 (Part I + Part II)</p>	<p>90014496LYM1001 Interventistico con farmaco - Profit Phase I/II Promotore: Janssen - Cilag International Multinational</p>	<p>A Phase 1b Multicenter, Open-label, Study of JNJ-90014496, an Autologous CD19/CD20 Bi-specific CAR-T Cell Therapy in Adult Participants with Relapsed or Refractory B-cell Non-Hodgkin Lymphoma</p>
<p>CTIS ITALY - MS2025-521145- 24-01INITIAL(Part I + Part II)</p>	<p>CAB-201- 002Interventistico con farmaco - ProfitPhase I/II Promotore: Cabaletta Bio Inc.Multinational</p>	<p>A Phase 1/2, Open-Label Study to Evaluate the Safety and Efficacy of Autologous CD19-specific Chimeric Antigen Receptor T cells (CABA-201) in Subjects with Active Idiopathic Inflammatory Myopathy or Active Juvenile Idiopathic Inflammatory Myopathy</p>
<p>CTIS ITALY - MS 2022-503112-17-00 SUBSTANTIAL MODIFICATION SM- 4 (Part I + Part II)</p>	<p>SRP-9003-301 Interventistico con farmaco - Profit Phase III Promotore: Sarepta Therapeutics Inc. Multinational</p>	<p>A Phase 3 Multinational, Open-label, Systemic Gene Delivery Study to Evaluate the Safety and Efficacy of SRP-9003 in Subjects with Limb Girdle Muscular Dystrophy 2E/R4</p>

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CTIS ITALY - MS 2025-524337-11-00 INITIAL (Part I + Part II)	U1111-1330-3381 Interventistico con farmaco - Profit Phase III Promotore: Celgene Corporation Multinational	A Phase 3, Randomized, Open-label, Multicenter Study to Compare the Efficacy and Safety of BMS-986353, CD19-targeted NEX-T CAR T Cells, Versus Standard of Care in Participants with Active Systemic Sclerosis (Breakfree-SSc)
CTIS ITALY - RMS 2025-522643-18-00 INITIAL (Part I + Part II)	V940-014 Interventistico con farmaco - Profit Phase III Promotore: Merck Sharp & Dohme LLC Multinational	A Phase 3, Randomized, Placebo-Controlled Study of Adjuvant Intismeran Autogene Plus Subcutaneous Pembrolizumab and Berahyaluronidase Alfa (MK-3475A) or Intismeran Autogene Monotherapy Versus Placebo in Participants With Completely Resected High-Risk Stage I Non-Small Cell Lung Cancer (INTerpath-014)
CTIS ITALY - MS 2022-501607-27-00 SUBSTANTIAL MODIFICATION SM-13 (Part II)	NA Interventistico con farmaco - Profit Phase I/II Promotore: Cellectis Multinational	Open-label dose-finding and dose-expansion study to evaluate the safety, expansion, persistence, and clinical activity of UCART20x22 in subjects with relapsed or refractory B-cell Non-Hodgkin Lymphoma (B-NHL)

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CTIS ITALY - MS 2025-522275-28-00 INITIAL (Part I + Part II)	OTL-200-11 Interventistico con farmaco - Profit Phase IV Promotore: Orchard Therapeutics (Europe) Ltd Multinational	Sample Collection Study to Monitor the Risk of Malignancy Due to Insertional Oncogenesis in Early Onset Patients with Metachromatic Leukodystrophy Treated with OTL-200 in the Clinical Development Program
CTIS ITALY - RMS 2025-524635-39-00 INITIAL (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 2024-514596-18-00 ADDED MEMBER STATE AM- 2 (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 - MS 2023-506327-29-00 SUBSTANTIAL MODIFICATION SM-5 (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)
CTIS ITALY - RMS 2025-522160-32-00 SUBSTANTIAL MODIFICATION SM-1 (Part I + Part II)	BTHAL-FT007-01 Interventistico con farmaco - Profit Phase II Promotore: Fondazione Telethon Ets Mononational	A single arm, open label, multicenter, single-dose, phase 2b clinical study evaluating efficacy and safety of gene therapy using autologous CD34+ hematopoietic stem cells transduced with the GLOBE lentiviral vector using an improved transduction protocol in subjects with transfusion-dependent beta-thalassemia.
CEN ATMPRSO - 3770INITIAL(Part I + Part II)	QOL-ONE PRO- CTOsservazionale con farmaco - No ProfitPromotore: 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

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CTIS ITALY - RMS 2023-505511-20-00 SUBSTANTIAL MODIFICATION SM-2 (Part II)	FT04CARCIK Interventistico con farmaco - No Profit Phase I/II Promotore: Fondazione Tettamanti Mononational	Phase I/II Trial to Determine Persistence, Safety and Clinical Activity of Allogeneic CIK Cells Transduced with a Transposon CD19-chimeric Antigen Receptor (CARCIK-CD19) in Adult and Pediatric Patients with Relapsed/Refractory B-cell Non-Hodgkin Lymphoma and B-cell Chronic Lymphocytic Leukemia