

**Comitato Etico per le Sperimentazioni Cliniche relative alle Terapie Avanzate**  
**O.d.G Seduta del 24/02/2026**

*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 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  |
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| CTIS ITALY - RMS<br>2024-514501-57-00<br>SUBSTANTIAL<br>MODIFICATION SM-3<br>(Part I + Part II) | SGT-003-101<br>Interventistico con<br>farmaco - Profit<br>Phase I/II<br>Promotore: Solid<br>Biosciences Inc.<br>Mononational       | A Phase 1/2, Multicenter, Open-Label Study to Investigate the Safety, Tolerability, and Efficacy of a Single Intravenous Dose of SGT-003 in Ambulant Males with Duchenne Muscular Dystrophy   |
| CTIS ITALY - MS<br>2022-503140-41-00<br>SUBSTANTIAL<br>MODIFICATION SM-13<br>(Part I + Part II) | IOV-MEL-301<br>Interventistico con<br>farmaco - Profit<br>Phase III<br>Promotore: lovance<br>Biotherapeutics Inc.<br>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 |
| CTIS ITALY - MS<br>2023-505043-39-00<br>SUBSTANTIAL<br>MODIFICATION SM-7<br>(Part I + Part II)  | SRP-9001-305<br>Interventistico con<br>farmaco - Profit<br>Phase III<br>Promotore: Serepta<br>Therapeutics Inc.<br>Multinational   | A Phase 3, Multinational, Long-Term Follow-Up Study to Evaluate Safety and Efficacy in Subjects Who Have Previously Received SRP-9001 in a Clinical Study   |
| CTIS ITALY - MS<br>2024-514137-38-00<br>SUBSTANTIAL<br>MODIFICATION SM-4<br>(Part I + Part II)  | CYTB323L12201<br>Interventistico con<br>farmaco - Profit<br>Phase II<br>Promotore: Novartis<br>Pharma AG<br>Multinational          | A Phase 2, randomized, open-label, controlled study to evaluate the efficacy and safety of rapcabtagene autoleucel versus comparator in participants with severe refractory idiopathic inflammatory myopathies  |
| CTIS ITALY - RMS<br>2023-506348-17-00<br>SUBSTANTIAL<br>MODIFICATION SM-3<br>(Part I + Part II) | M-2021-389<br>Interventistico con<br>farmaco - Profit<br>Phase II<br>Promotore: Miltenyi<br>Biomedicine GmbH<br>Multinational      | A single-arm, multi-center, open-label Phase II study to determine the safety and efficacy of MB-CART2019.1 in pediatric subjects with relapsed/refractory (r/r) mature B-cell neoplasms who have relapsed after one or more prior therapies, including subjects with primary refractory disease  |

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| CTIS ITALY - MS<br>2024-512029-10-00<br>ADDED MEMBER STATE AM-<br>2<br>(Part I + Part II)      | 000423<br>Interventistico con<br>farmaco - Profit<br>Phase III<br>Promotore: Ferring<br>Pharmaceuticals A/S<br>Multinational                | A phase 3b, Randomized, Controlled Trial of Nadofaragene Firadenovec vs. Observation in Subjects with Intermediate Risk Non-Muscle Invasive Bladder Cancer (IR NMIBC)  |
| CTIS ITALY - MS<br>2025-523660-21-00<br>INITIAL<br>(Part I + Part II)                          | C0371017<br>Interventistico con<br>farmaco - Profit<br>Phase III<br>Promotore: Pfizer Inc.<br>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  |
| CTIS ITALY - MS<br>2025-522647-17-00<br>INITIAL<br>(Part I + Part II)                          | Interventistico con<br>farmaco - Profit<br>Promotore: Hmg<br>Biologics S.r.l.<br>Multinational  | A two-part study: open label, dose escalation phase aimed to evaluate the safety and tolerability of two different doses of allogeneic fibroblasts for the treatment of chronic refractory wounds and identifying the best dose for subsequent cohort expansion phase aimed to assess efficacy and confirm safety and tolerability       |
| CTIS ITALY - MS<br>2025-522949-22-00<br>INITIAL<br>(Part I + Part II)                          | SGT-003-301<br>Interventistico con<br>farmaco - Profit<br>Phase III<br>Promotore: Solid<br>Biosciences Inc<br>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<br>2024-517580-23-00<br>SUBSTANTIAL<br>MODIFICATION SM-3<br>(Part I + Part II) | VALO-001<br>Interventistico con<br>farmaco - Profit<br>Phase I<br>Promotore: Valo<br>Therapeutics Oy<br>Multinational                       | A Phase I, open-label, non-randomized study to evaluate the safety and immune activity of PeptiCRAd-1, a tumor-specific peptide coated conditionally replicating adenovirus, in combination with immune checkpoint inhibitor pembrolizumab in patients with injectable solid tumors in indications known to express NY-ESO-1 and MAGE-A3 |
| CEN ATMP<br>RSO - 3770<br>INITIAL<br>(Part I + Part II)  | QOL-ONE PRO-CT<br>Osservazionale con<br>farmaco - No Profit<br>Promotore: Associazione<br>Culturale e di Ricerca<br>QOL-ONE<br>Mononational | A Real-World Observational Study on Patient-Reported Outcomes in Allogeneic Stem Cell Transplantation (Allo-SCT) and CAR-T Therapy   |
| CTIS ITALY - RMS<br>2024-511028-15-00<br>SUBSTANTIAL<br>MODIFICATION SM-3<br>(Part II)         | STEMS2<br>Interventistico con<br>farmaco - No Profit<br>Phase II<br>Promotore: Ospedale<br>San Raffaele S.r.l.<br>Mononational              | A phase 2, randomized, patient and assessor blind, multicenter study comparing fetal neural stem cells transplantation to sham procedure in progressive multiple sclerosis (STEMS2)  |

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| CTIS ITALY - MS<br>2025-523285-25-00<br>INITIAL<br>(Part I + Part II)                           | D8311C00001<br>Interventistico con<br>farmaco - Profit<br>Phase III<br>Promotore: AstraZeneca<br>AB<br>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<br>2025-523448-10-00<br>INITIAL<br>(Part I + Part II)                           | KTE-C19-104<br>Interventistico con<br>farmaco - Profit<br>Phase II<br>Promotore: Gvhd<br>Resistant to ruxolitinib<br>and steroids<br>Multinational                               | 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  |
| CTIS ITALY - MS<br>2023-505177-32-00<br>SUBSTANTIAL<br>MODIFICATION SM-7<br>(Part I + Part II)  | V940-004<br>Interventistico con<br>farmaco - Profit<br>Phase II<br>Promotore: Merck Sharp<br>& Dohme LLC<br>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<br>2023-504909-37-00<br>SUBSTANTIAL<br>MODIFICATION SM-3<br>(Part I + Part II)  | 1504-0003<br>Interventistico con<br>farmaco - Profit<br>Phase I/II<br>Promotore: Boehringer<br>Ingelheim International<br>GmbH<br>Multinational                                  | A clinical trial to evaluate the long-term safety and durability of efficacy of BI 3720931, an inhaled lentiviral vector gene therapy, after single dose administration in a previous clinical trial in people with cystic fibrosis rolled-over from a previous clinical trial with BI 3720931 (Lenticlair™-ON). |
| CTIS ITALY - RMS<br>2024-514053-30-00<br>SUBSTANTIAL<br>MODIFICATION SM-1<br>(Part I + Part II) | IRST153.09<br>Interventistico con<br>farmaco - No Profit<br>Phase II<br>Promotore: Istituto<br>Romagnolo Per Lo<br>Studio Dei Tumori Dino<br>Amadori IRST S.r.l.<br>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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| CTIS ITALY - MS<br>2025-521145-24-01<br>INITIAL<br>(Part I + Part II)                          | CAB-201-002<br>Interventistico con<br>farmaco - Profit<br>Phase I/II<br>Promotore: Cabaletta<br>Bio Inc.<br>Multinational  | 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      |
| CTIS ITALY - MS<br>2023-505658-17-00<br>SUBSTANTIAL<br>MODIFICATION SM-6<br>(Part I + Part II) | V940-005<br>Interventistico con<br>farmaco - Profit<br>Phase I/II<br>Promotore: Merck Sharp<br>& Dohme LLC<br>Multinational  | A Phase 1/2 Study of V940 Plus Pembrolizumab With or Without Enfortumab Vedotin in Muscle-Invasive Urothelial Carcinoma (MIUC) (INTERpath-005)   |
| CTIS ITALY - MS<br>2023-503652-27-00<br>SUBSTANTIAL<br>MODIFICATION SM-9<br>(Part I)           | V940-001<br>Interventistico con<br>farmaco - Profit<br>Phase III<br>Promotore:<br>Merck Sharp & Dohme<br>LLC<br>Multinational  | A Phase 3, Randomized, Double-blind, Placebo- and Active-Comparator-Controlled Clinical study of Adjuvant V940 (mRNA-4157) Plus Pembrolizumab Versus Adjuvant Placebo Plus Pembrolizumab in Participants with High-risk Stage II-IV Melanoma (INTERpath-001) |
| CEN ATMP<br>RSO - 4260<br>INITIAL  | DISH-SCC<br>Osservazionale senza<br>farmaco e dispositivo -<br>No Profit<br>Promotore: Fondazione<br>Policlinico Universitario<br>Agostino Gemelli IRCCS<br>Mononational | Development of immunotherapy strategies in head and neck squamous cell carcinoma   |