Comitato Etico per le Sperimentazioni Cliniche relative alle Terapie Avanzate
martedì 08 luglio ore 16:30 – 18:30

ORDINE DEL GIORNO

1. Comunicazioni del Presidente
2. Revisione Gruppi di Lavoro
3. Revisione procedura valutazione studi clinici
4. Studi in valutazione

EU CT number	IN/SM	TITOLO	DATA DECISIONE AIFA / SCADENZA PART I CONCLUSION	CONDIZIONE SOSPENSIVA	Data Ingresso	Scadenza RFI/CONCLUSION PART II

2024-518797-13-00	IN	The CALiPSO-1 Study: A Study of CNTY-101, a CD19-targeted CAR iNK Cell Product, in Participants with Refractory B cell-mediated Autoimmune Diseases	17/04/2025		31/01/2023	06/03/2025
2024-518797-13-00	SM-2 PART II	The CALiPSO-1 Study: A Study of CNTY-101, a CD19-targeted CAR iNK Cell Product, in Participants with Refractory B cell-mediated Autoimmune Diseases	07/07/2025	**Y**		01/07/2025
2024-513349-35-00	SM-1	A Phase 3 Study to Evaluate the Safety and Efficacy of a Single Dose of CTX001 in Pediatric Subjects With Transfusion-Dependent β-Thalassemia	14/05/2025	**Y**		07/04/2025
2024-514641-12-00	SM-1	A Phase 3b Study to Evaluate Efficacy and Safety of a Single Dose of Autologous CRISPR Cas9 Modified CD34+ Human Hematopoietic Stem and Progenitor Cells (CTX001) in Subjects with Transfusion-Dependent β-Thalassemia or Severe Sickle Cell Disease	14/05/2025	**Y**		14/04/2025
2024-513978-22-00	SM-1	A Phase 3 Study to Evaluate the Safety and Efficacy of a Single Dose of CTX001 in Pediatric Subjects With Severe Sickle Cell Disease	04/06/2025	**Y**		24/04/2025
2024-513683-25-00	IN	Randomized, controlled, multi-center phase II clinical trial for the treatment of patellofemoral osteoarthritis with nasal chondrocyte-based tissue engineered cartilage implantation vs current standard of care (ENCANTO)	13/06/2025	**Y**		03/06/2025

5. Procedure da assegnare

EU CT number	IN/SM	TITOLO	DATA DECISIONE AIFA / SCADENZA PART I CONCLUSION
2024-517335-46-00	IN	A Phase 2 Open-label Randomized Study of V940 in Combination With BCG Versus BCG Monotherapy in Participants With High-risk Non-muscle Invasive Bladder Cancer (INTerpath-011)	23/04/2025
2023-503952-28-00	SM-2	A Randomised, Open label, Controlled, Multicentre, Phase 2 Trial of First line Treatment with Mesenchymal Stromal Cells MC0518 Versus Best Available Therapy in Paediatric Participants with Steroid refractory Acute Graft versus host Disease After Allogeneic Stem Cell Transplantation (BALDER Trial)	16/04/2025
2024-515279-37-00	IN	A Phase 3, Randomized, Open-Label, Multicenter Study to Compare the Efficacy and Safety of BMS-986393, a GPRC5D-directed CAR-T Cell Therapy, Versus Standard Regimens in Adult Participants with Relapsed or Refractory and Lenalidomide-refractory Multiple Myeloma	02/04/2025
2023-503652-27-00	SM-6	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)	12/06/2025
2022-500746-16-00	SM-8	A Phase 2b, Randomized, Double-masked, Multicenter, Dose-ranging, Sham-controlled Clinical Trial to Evaluate Intravitreal JNJ-81201887 (AAVCAGsCD59) Compared to Sham Procedure for the Treatment of Geographic Atrophy (GA) Secondary to Age-related Macular Degeneration (AMD)	14/07/2025
2023-510380-34-00	SM-5	A Phase II, multi-part, three-year, randomized, open-label, assessor-blinded, active-controlled, multicenter study to evaluate the efficacy and safety of rapcabtagene autoleucel versus rituximab treatment in participants with severe refractory diffuse cutaneous systemic sclerosis	04/08/2025
2023-510150-17-00	SM-4	A Phase 2, adaptive, randomized, open-label, assessor-blinded active-controlled study to evaluate the efficacy and safety of rapcabtagene autoleucel versus Standard of Care in patients suffering from systemic lupus erythematosus (SLE) with active, refractory lupus nephritis (LN).	08/09/2025

2023-503281-23-00	SM-3	A seamless Phase I/II trial with an initial open-label dose escalation part and a subsequent randomised, double-blind, placebo-controlled expansion part to evaluate the safety, tolerability, and efficacy of a single dose of BI 3720931, an inhaled lentiviral vector gene therapy, in adult people with cystic fibrosis who are ineligible for CFTR modulators (LenticlairTM 1)	30/06/2025
2022-501346-30-00	SM-22	A Randomized, Open-Label, Phase 3 Trial to Compare the Efficacy and Safety of Idecabtagene Vicleucel with Lenalidomide Maintenance Versus Lenalidomide Maintenance Therapy Alone in Adult Participants with Newly Diagnosed Multiple Myeloma Who Have Suboptimal Response After Autologous Stem Cell Transplantation	30/06/2025
2022-501489-24-00	SM-8	An Adaptive Phase 3, Randomized, Open-Label, Multicenter Study to Compare the Efficacy and Safety of Axicabtagene Ciloleucel versus Standard of Care Therapy as First-Line Therapy in Subjects with High-Risk Large B-Cell Lymphoma (ZUMA-23)	14/07/2025
2022-502873-40-00	SM-2	A Randomized, Double-blind, Placebo-controlled, Multicenter, Seamless, Adaptive, Safety, Dose-finding, and Phase 3 Clinical Study of UX701 AAV-mediated Gene Transfer for the Treatment of Wilson Disease	
2024-517335-46-00	SM-1	A Phase 2 Open-label Randomized Study of V940 in Combination With BCG Versus BCG Monotherapy in Participants With High-risk Non-muscle Invasive Bladder Cancer (INTerpath-011)	
2024-519278-37-00	IN	A Phase 2, Multicenter, Open-Label Study of CC-97540 (BMS-986353), CD19-Targeted NEX-T CAR T Cells, in Participants with Active SLE (Including Lupus Nephritis) with Inadequate Response to Glucocorticoids and at Least 2 Immunosuppressants (Breakfree-SLE)	

6. Varie ed eventuali

Il Presidente
(Roberto Poscia)