Comitato Etico per le Sperimentazioni Cliniche relative alle Terapie Avanzate

Martedì 24 giugno ore 16:30 – 18:30

ORDINE DEL GIORNO

1. Comunicazioni del Presidente

2. Approvazione del verbale n.3/2025 della seduta del 19.06.2025

3. Procedure in valutazione urgente approvate con condizione sospensiva (Segreteria).

AUTORIZZATE A CONDIZIONE SOSPENSIVA				
EU CT number	**IN/SM**	**TITOLO**	**DATA DECISIONE AIFA**	**Scadenza RFI/CONCLUSION PART II**
2024-519317-66-00	IN	(ESMERALD) A phase-2A, singlecentre, prospective, uncontrolled, open label, single arm, exploratory clinical study to evaluate the safety and efficacy of cutaneous AUP1602C with foam dressing as a treatment for non-healing neuroischemic diabetic foot ulcers	15/05/2025	06/03/2025
2023-507041-28-00	SM-5	Long-term Follow-up Study for Interventional Studies Treated	30/04/2025	24/03/2025
2023-506327-29-00	SM-1	A Phase 3 Randomized Doubleblind 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 Platinumbased Doublet Chemotherapy (INTerpath-009)	14/05/2025	07/04/2025
2024-511188-26-00	SM-1	A Phase 3, Randomized, OpenLabel Study to Compare the Efficacy and Safety of Anitocabtagene Autoleucel Versus Standard of Care Therapy in Participants With Relapsed/Refractory Multiple Myeloma	14/05/2025	07/04/2025
2023-507260-40-00	SM-2	A Two-Part Open-Label Study of REGV131-LNP1265, A CRISPR/CAS9-Based Coagulation Factor IX Gene Insertion Therapy in Participants with Hemophilia B	19/05/2025	14/04/2025
2023-506348-17-00	SM-1	A single-arm, multi-center, openlabel Phase II study to determine the safety and efficacy of MBCART2019.1 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	19/05/2025	14/04/2025
2023-507220-23-00	SM-5	MAGNITUDE: A Phase 3, Multinational, Multicenter, Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Efficacy and Safety of NTLA-2001 in Participants with Transthyretin Amyloidosis with Cardiomyopathy (ATTR-CM)	22/05/2025	28/04/2025
2023-504923-20-00	SM-3	A Phase 3, Randomized, Doubleblind, Placebo- and ActiveComparator-Controlled Clinical Study of Adjuvant V940 (mRNA4157) Plus Pembrolizumab Versus Adjuvant Placebo Plus Pembrolizumab in Participants With Resected Stage II, IIIA, IIIB (N2) Non-small Cell Lung Cancer (INTerpath-002)	09/05/2025	08/05/2025
2022-502673-41-01	SM-1	A Multicenter, Randomized, Double-blind, Placebo-Controlled Phase II Study to Investigate the Efficacy and Safety of CYP-001 in Combination with Corticosteroids vs Corticosteroids Alone for the Treatment of High-Risk Acute Graft Versus Host Disease	22/05/2025	19/05/2025
2024-513683-25-00	IN	Randomized, controlled, multicenter 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	02/05/2025
2023-503652-27-00	SM-6	A Phase 3, Randomized, Doubleblind, Placebo- and ActiveComparator-Controlled Clinical study of Adjuvant V940 (mRNA4157) Plus Pembrolizumab Versus Adjuvant Placebo Plus Pembrolizumab in Participants with High-risk Stage II-IV Melanoma (INTerpath-001)	12/06/2025	09/06/2025
2023-505177-32-00	SM-5	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)	17/06/2025	16/06/2025

4. Ulteriori procedure da assegnare e valutare

AUTORIZZATE IN NO CONCLUSION					
EU CT number	**IN/SM**	**TITOLO**	**DATA DECISIONE**	**CONDIZIONE SOSPENSIVA**	**Scadenza RFI/CONCLUSION PART II**
2024-512714-18-00	IN	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	25/03/2025		24/03/2025
2023-505177-32-00	SM-4	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)	25/03/2025		24/03/2025
2022-503140-41-00	SM-10	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	28/03/2025	**ONLY PART II**	24/03/2025
2023-504786-23-00	SM-4	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	01/04/2025	**ONLY PART II**	31/03/2025
2024-515279-37-00	IN	A Phase 3, Randomized, Open-Label, Multicenter Study to Compare the Efficacy and Safety of BMS-986393, a GPRC5Ddirected CAR-T Cell Therapy, Versus Standard Regimens in Adult Participants with Relapsed or Refractory and Lenalidomiderefractory Multiple Myeloma	02/04/2025		31/03/2025
2024-512504-19-00	AM-1	Phase I open-label, dose escalation trial of BI 1831169 monotherapy and in combination with an anti-PD-1 mAb in patients with advanced or metastatic solid tumors.	03/04/2025		02/04/2025
2024-512898-27-00	SM-1	An open-label phase 1/2 study to evaluate the safety, biological response and efficacy of a single dose of Temferon (autologous CD34+-enriched hematopoietic stem and progenitors cells genetically modified with human Interferon-α2) in patients with metastatic renal cell carcinoma	07/04/2025		21/03/2025
2024-518369-92-00	IN	A Long-term Follow-up Study for Subjects Previously Treated with Autologous ex vivo Lentiviral Hematopoietic Stem and Progenitor Cell Gene Therapy for Wiskott-Aldrich Syndrome (WAS)	08/04/2025		31/03/2025
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 Allogeneic Stem Cell Transplantation (BALDER Trial) versus host Disease After	16/04/2025		21/03/2025
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		06/03/2025
2024-514006-31-00	IN	An open-label, multi-center, phase I/II study to assess safety, disease progression, and cellular kinetics following YTB323 administration in participants with non-active Progressive Multiple Sclerosis (PMS)	23/04/2025		17/03/2025
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		17/03/2025
2022-503112-17-00	SM-2	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	23/04/2025		24/03/2025

NO CONCLUSION NON AUTORIZZATE E PROCEDURE IN CORSO				
EU CT number	**IN/SM**	**TITOLO**	**DATA DECISIONE AIFA / SCADENZA PART I CONCLUSION**	**Scadenza RFI/CONCLUSION PART II**
2024-518972-30-00	IN	A phase I/II open label study to assess safety, feasibility and efficacy of ex vivo expanded, autologous haematopoietic stem and progenitor cell populations that contain CD34+ cells transduced with a lentiviral vector encoding the TCIRG1 cDNA in children with autosomal recessive osteopetrosis caused by mutations in the TCIRG1 gene.	**30/06/2025**	16/05/2025
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	23/06/2025
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	30/06/2025
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	01/07/2025
2022-500746-16-00	SM-8		14/07/2025	09/06/2025
2022-501489-24-00	SM-8	An Adaptive Phase 3, Randomized, OpenLabel, 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	03/07/2025
2023-510380-34-00	SM-5	A Phase II, multi-part, three-year, randomized, open-label, assessorblinded, 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	19/06/2025
2023-510150-17-00	SM-4	A Phase 2, adaptive, randomized, openlabel, 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	19/06/2025
2023-506327-29-00	SM-2	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)		11/07/2025
2022-502873-40-00	SM-2	A Randomized, Double-blind, Placebocontrolled, Multicenter, Seamless, Adaptive, Safety, Dose-finding, and Phase 3 Clinical Study of UX701 AAVmediated Gene Transfer for the Treatment of Wilson Disease		17/07/2025
2023-504786-23-00	SM-10	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		30/06/2025
2024-517335-46-00	SM-1			04/08/2025
2025-521349-25-00	IN	A Phase 3, Randomized, Double-Masked, Active-Controlled Trial of a Single Intravitreal Injection of 4D-150 in Adults with Macular Neovascularization Secondary to Age-Related Macular Degeneration (4FRONT-2)		01/09/2025
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 (BreakfreeSLE)		29/08/2025

5. Revisione/adozione Regolamento del CEN ATMP (Tutti i componenti)
6. Varie ed eventuali