Comitato Etico per le Sperimentazioni Cliniche relative alle Terapie Avanzate
giovedì 17 luglio ore 16:30 – 18:30

ORDINE DEL GIORNO

1. Comunicazioni del Presidente
2. Approvazione verbale n. 5/2025 seduta del 24 giugno e n. 6/2025 seduta del 08 luglio
3. Studi in valutazione

EU CT number	IN/SM	TITOLO	DATA DECISIONE AIFA / SCADENZA PART I CONCLUSION	DATA SOTTOMISSIONE/NOTE
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	no conclusion/manca valutazione RFI sollevate (RISPOSTA 03/03/2025)
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	scadenza PART I conclusion 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	17/04/2025 RFI PARTE I CLINICA-RISPOSTA SPONSOR 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	16/05/2025 (non ci sono RFI di Parte I)
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	02/05/2025
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		15/05/2025

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)		04/06/2025
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 (Breakfree-SLE)		13/06/2025
2024-512898-27-00	SM-1	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)	07/04/2025	21/03/2025
BELLA-RR	EM-01	“STUDIO MULTICENTRICO RETROSPETTIVO SULL'USO DI BREXUCABTAGENE AUTOLEUCEL IN PAZIENTI ADULTI AFFETTI DA LEUCEMIA LINFOBLASTICA ACUTA B RECIDIVATA / REFRATTARIA: RISULTATI PRELIMINARI DAL NAMED-PATIENT PROGRAM KTE-X19-ALL (BELLA-RR)”		

4. Studi da assegnare

EU CT number	IN/SM	TITOLO	DATA DECISIONE AIFA / SCADENZA PART I CONCLUSION	CONDIZIONE SOSPENSIVA	DATA SOTTOMISSIONE/NOTE
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 no conclusion part II ma il provvedimento dice che è stata acquisita
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
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
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
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
2024-518972-30-00	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

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
2024-512504-19-00	SM-7				
2022-502907-31-00	SM-2			**ONLY PART I**	

5. Varie ed eventuali

Il Presidente
(Roberto Poscia)