

CTC-A No.	eudraCTNo	Registration No.	Studienname	Betroffene SE	PI	Behandlungs- und Forschungszentrum	Art der Studie
22-319	2022-002435-66		Phase/II trial to assess the safety and efficacy of Venetoclax in addition to sequential conditioning with Fludarabine / Amsacrine / Ara-C (FLAMSA) + Treosulfan for allogeneic blood stem cell transplantation in patients with MDS, CMML or sAML	MDS, CMML, AML	Edgar Jost	Hämato	national, Multizentrisch, External
22-321			Outcomes for patients living with primary biliary cholangitis	Primär biliäre Cholangitis	Karsten Große	Gastro	national, Multizentrisch, External
22-341	2022-501484-40-00		A multicenter, randomized, double-blind, parallel group, placebo-controlled study to evaluate the efficacy and safety of iptacopan on top of supportive care including SGLT2 inhibitors in patients with primary IgA nephropathy	IgA-Nephritis	Marcus Möller	Nephro	National Monozentrisch, External
22-344			A Multi-Part, Randomized, Double-Blind, Placebo-Controlled Phase 2 Clinical Study of the Safety and Efficacy of CGT9486 in Subjects with Nonadvanced Systemic Mastocytosis	Systemische Mastozytose	Jens Panse	Hämato	national, Multizentrisch, External
22-346			Juvenile Arthritis - Methotrexate / Biologics long-term Observation (JIA biologic register)	Juvenile idiopathische Arthritis	Thomas Rauen	Nephro	national, Multizentrisch, External
22-350	2020-001181-10	DRKS00024085	Age-adjusted high-dose chemotherapy followed by autologous stem cell transplantation or conventional chemotherapy with R-MP as first-line treatment in elderly primary CNS lymphoma patients – a randomized phase III trial	Primäres Lymphom des Zentralnervensystems	Jens Panse	Hämato	national, Multizentrisch, External
22-352	2022-003069-39		VENETOCLAX AFTER TKI TO TARGET PERSISTING STEM CELLS IN CML (VARIANT)	CML	Martina Crysandt	Hämato	national, Multizentrisch, External
22-353			MAGROLimab plus Induction Chemotherapy in newly diagnosed "ELN 2022 intermediate or adverse-risk" AML or high risk MDS patients intended to undergo allogeneic stem cell transplantation. A Phase 1/2, Single-Arm, Open-Label Study (MAGROLIC)	AML, MDS	Martina Crysandt	Hämato	national, Multizentrisch, External
22-363			A PHASE II, RANDOMIZED, DOUBLE-BLIND, PLACEBO-CONTROLLED, DOSE-FINDING STUDY TO EVALUATE THE SAFETY, BIOMARKERS, AND EFFICACY OF TOMINERSEN IN INDIVIDUALS WITH PRODROMAL AND EARLY MANIFEST HUNTINGTON'S DISEASE BN42489	Chorea Huntington	Kathrin Reetz	Neuro	national, Multizentrisch, External
22-381	2021-005744-29		A Randomized, Open-Label, Multicenter, Global, Phase 2 Trial to Assess the Efficacy and Safety of Epcoritamab (GEN3013; DuoBody®-CD3xCD20) Plus Lenalidomide or Ineligible Subjects With Diffuse Large B-cell Lymphoma	Diffus großzelliges B-Zell-Lymphom	Mareike Tometten	Hämato	national, Multizentrisch, External
22-382			The European Rare Kidney Disease Registry	Seltene Nierenerkrankungen	Jürgen Floege	Nephro	International, Multizentrisch, External
22-409	2022-002746-40		A Phase 2, Open-label Study Evaluating Efficacy and Safety of VX-864 in Subjects With Alpha-1 Antitrypsin Deficiency Who Have the PIZZ Genotype, Over 48 Weeks	Alpha-1-Antitrypsin-Mangel	Pavel Strnad	Gastro	national, Multizentrisch, External

22-414		Phase 3 study to Evaluate the Efficacy and Safety of Fazirsiran in Participants with alpha 1 Antitrypsin Deficiency Associated Liver Disease	Alpha-1-Antitrypsin-Mangel	Pavel Strnad	Gastro	national, Multizentrisch, External
22-416	2022-001627-32	A phase 3 randomized, double-blind study of ivalumab (VAY736) versus placebo in addition to eltrombopag in patients with primary immune thrombocytopenia (ITP) who had an insufficient response or relapsed after first line steroid treatment (VAYHIT2)	Immunthrombozytopenie (ITP)	Martina Crysandt	Hämatologie	national, Multizentrisch, External
23-040	2021-000803-20	AN OPEN-LABEL, 2-ARM, MULTICENTER, RANDOMIZED PHASE 3 STUDY TO EVALUATE THE EFFICACY AND SAFETY OF ELRANATAMAB (PF-06863135) + DARATUMUMAB + LENALIDOMIDE VERSUS DARATUMUMAB + LENALIDOMIDE + DEXAMETHASONE IN TRANSPLANT-INELIGIBLE PARTICIPANTS WITH NEWLY-DIAGNOSED MULTIPLE MYELOMA	Multipl. Myelom	Deniz Gezer	Hämatologie	International, Multizentrisch, External
23-044		Diagnostik, Therapie und Verlauf der Neuromyelitis optica: eine nicht-interventionelle Kohortenstudie der Neuromyelitis optica Studiengruppe (NEMOS)	Neuromyelitis optica	Simone Tauber	Neuro	International, Multizentrisch, External
23-077	2020-001428-33	Eine randomisierte, doppelblinde, placebokontrollierte Dosisfindungsstudie der Phase 2a zur Beurteilung der Sicherheit, Verträglichkeit und Pharmakokinetik von PLN-74809 bei Teilnehmern mit primär sklerosierender Cholangitis (PSC) und Verdacht auf Leberfibrose (INTEGRIS-PSC)	Primär sklerosierende Cholangitis	Pavel Strnad	Gastro	national, Multizentrisch, External
23-092	2023-503497-21	A Phase 3, Open-Label Extension Study to Evaluate the Long-Term Safety and Efficacy of Fazirsiran in Participants With Alpha-1 Antitrypsin Deficiency-Associated Liver Disease	Alpha-1-Antitrypsin-Mangel	Pavel Strnad	Gastro	national, Multizentrisch, External
23-102	2018-004250-17	SIOP High Risk Medulloblastoma: An international prospective trial on clinically high-risk medulloblastoma in patients older than 3 years	Medulloblastom	Udo Konny	Tumor	national, Multizentrisch, External
23-104		Multicentric, observational Cohort to evaluate rAvulizumab real world usage in patients with aHUS	Hämolytisch-urämisches Syndrom, atypische Form	Anja Mühlfeld	Nephro	national, Multizentrisch, External
23-118	2023-503952-28-00	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)	Akute Graft versus host-Krankheit	Udo Konny	Tumor	national, Multizentrisch, External
23-142	DRKS00028171	Defizite und Verbesserungspotential in der psychosozialen und palliativen Versorgung von ALS-Patient*innen in Deutschland	ALS	Robert Brunkhorst	Neuro	national, Multizentrisch, External
23-157		Multiorgan complexity in Friedreich Ataxia (FACROSS)	Friedreich-Ataxie	Kathrin Reetz	Neuro	national, Multizentrisch, External
23-171		A PHASE III, MULTICENTER, RANDOMIZED, DOUBLE-BLIND, PLACEBO-CONTROLLED STUDY TO EVALUATE THE EFFICACY AND SAFETY OF RO7434656, AN ANTISENSE INHIBITOR OF COMPLEMENT FACTOR B, IN PATIENTS WITH PRIMARY IgA NEPHROPATHY AT HIGH RISK OF PROGRESSION	IgA-Nephritis	Claudia Seikrit	Nephro	International, Multizentrisch, External

23-172	2022-502514-86-00	A Phase 1, Open-Label, Multicenter Study of INCA033989 Administered as a Monotherapy or in Combination With Ruxolitinib in Participants With Myeloproliferative Neoplasms	Myeloproliferative Neoplasie	Steffen Koschmieder	Hämato	national, Multizentrisch, External
23-181		Hypogammaglobulinämie unter Ocrelizumab – prädisponierende Faktoren und Risiken einer VZV-Reaktivierung	Hypogammaglobulinämie	Amelie Bohn	Neuro	national, Multizentrisch, External
23-227		A Phase 3 open-label, controlled, randomised, multi-centre trial comparing imlifidase and standard-of-care with standard-of-care alone in the treatment of severe anti-GBM antibody disease (Goodpasture disease)	Anti-GBM-Syndrom	Anja Mühlfeld	Nephro	national, Multizentrisch, External
23-257		A Phase III, single arm, multicentre study to evaluate the efficacy and safety of ropeginterferon alfa-2b in essential thrombocythaemia patients who are intolerant or refractory to or not eligible for other cytoreductive treatments	Essentielle Thrombozythämie	Steffen Koschmieder	Hämato	national, Multizentrisch, External
23-271	NCT05183646	A pivotal Phase 3, multicenter, randomized, double-blind, placebo-controlled study of the efficacy and safety of DMX-200 in patients with focal segmental glomerulosclerosis (FSGS) who are receiving an angiotensin II receptor blocker (ARB)	FSGS	Marcus Möller	Nephro	national, Multizentrisch, External
23-299		Einfluss der Covid-19 Pandemie auf die Versorgung von Patienten mit Seltenen Erkrankungen	Seltene Erkrankungen allgemein	Martin Mücke	Geschäftsstelle	National Monozentrisch, Internal
23-302		A randomized phase III trial assessing iberdomide versus iberdomide plus isatuximab maintenance therapy post autologous hematopoietic stem-cell transplantation in patients with newly diagnosed multiple myeloma Eine Randomisierte Phase 3-Studie zur Untersuchung einer Erhaltungstherapie mit Iberdomid versus Iberdomid plus Isatuximab nach autologer hämatopoetischer Blutstammzelltransplantation für Patienten mit neudiagnostiziertem Multiplem Myelom	Multiples Myelom	Deniz Gezer	Hämato	national, Multizentrisch, External
23-304	2022-002850-13	A Phase 3, Randomized, Open-label, Active-Comparator-Controlled Clinical Study to Evaluate the Safety and Efficacy of Bomedemstat (MK-3543/IMG-7289) versus Best Available Therapy (BAT) in Participants With Essential Thrombocythemia who have an Inadequate Response to or are Intolerant of Hydroxyurea	Essentielle Thrombozythämie	Steffen Koschmieder	Hämato	international, Multizentrisch, External