N	EudraCT Number	Trial Type	Trial Phase	Sponsor	Investigator	Titel and Design	Trial Population	Age	R goal	R	R success	Recruiting	Duration
10	2020-000561-16	SIT	II	Novartis Pharma AG	PD Dr. med. Steffen Syrbe	EPIK-P2: A Phase II double-blind study with an upfront, 16-week randomized, placebo-controlled period, to assess the efficacy, safety and pharmacokinetics of alpelisib (BYL719) in pediatric and adult patients with PIK3CA-related overgrowth spectrum (PROS)	Pediatric and adult patients with PIK3CA-related overgrowth spectrum (PROS)	≥ 6 years	2			Yes	Since 10/2021
9	2018-003099-10	SIT	III	Dicerna Pharmaceuticals Inc.	Prof. Dr. med. Burkhard Tönshoff	An Open-Label Roll-Over Study to Evaluate the Long-Term Safety and Efficacy of DCR-PHXC Solution for Injection (subcutaneous use) in Patients with Primary Hyperoxaluria ("PHYOX3")	Children and Adolescents with Primary Hyperoxaluria	≥ 6 years	1	1	100 %	No	12/2020-08/2021
8	2018-003098-91	SIT	II	Dicerna Pharmaceuticals Inc.	Prof. Dr. med. Burkhard Tönshoff	A Phase 2 Placebo-Controlled, Double-Blind, Multicenter Study to Evaluate the Efficacy, Safety, and Tolerability of DCR-PHXC Solution for Injection (subcutaneous use) in Patients with Primary Hyperoxaluria ("PHYOX2")	Children and Adolescents with Primary Hyperoxaluria (Type 1 or Type 2)	≥ 6 years	1	1	100 %	No	06/2020-12/2020
7	2018-004926-26	SIT	III	Provention Bio, Inc.	Dr. med. Jürgen Grulich-Henn	A Phase 3, Randomized, Double-Blind, Multinational, Placebo-Controlled Study to Evaluate Efficacy and Safety of Teplizumab (PRV-031), a Humanized, FcR Non-Binding, anti-CD3 Monoclonal Antibody, in Children and Adolescents with Newly Diagnosed Type 1 Diabetes (T1D) ("PROTECT")	Children and Adolescents with Type 1 Diabetes	8 to 17 years	3	5	167 %	No	Since 09/2020

SIT: Sponsor-Initiated Trial (clinical trials with sponsors from pharmaceutical industry), IIT: Investigator-Initiated Trial, OS: Observation Study, R – Recruitment, recruited
Green lettering: Currently recruiting trials, blue lettering: All other trials

N	EudraCT Number	Trial Type	Trial Phase	Sponsor	Investigator	Titel and Design	Trial Population	Age	R goal	R	R success	Recruiting	Duration
6	2019-002817-21	SIT	III	Jannsen Cilag, GmbH	Prof. Dr. med. Matthias Gorenflo	A Randomized, Multicenter, Double-Blind, Placebo-Controlled, Parallel-Group, Event-Driven, Group-Sequential Study with Open-Label Extension Period to Assess the Efficacy and Safety of Selexipag as Add-On Treatment to Standard of Care in Children Aged ≥2 to <18 years with Pulmonary Arterial Hypertension ("SALTO")	Children with Pulmonary Arterial Hypertension	2 to 18 years	3			Yes	Since 09/2020
5	N.A.	OS	N.A.	Albert-Ludwigs-University Freiburg (Medical Faculty)	Dr. med. Andreas Ziegler	SMArtCARE: Longitudinal data collection in patients with Spinal Muscular Atrophy (SMA)	Children with Spinal Muscular Atrophy	Birth to 18 years	open	65	ongoing	Yes	Since 04/2020
4	N.A.	IIT (OS)	N.A.	Biogen GmbH (Sponsor Research Agreement)	Dr. med. Andreas Ziegler	Metabolic Profiling of Neuromuscular Diseases (MetabNMD) – subproject SMA	Children and adults with Spinal Muscular Atrophy	Birth to 99 years	300	Ca. 200	ongoing	Yes	Since 05/2020
3	2019-001759-38	IIT	I	Ruprecht-Karls University Heidelberg (Medical Faculty)	Prof. Dr. med. Matthias Gorenflo	Pharmacokinetics of a microdosed cocktail containing rivaroxaban, apixaban and edoxaban in children with congenital heart defects	Children with Congenital Heart Defects	2 to 6 years	20	11	55 %	Yes	Since 12/2019
2	2020-000561-16	SIT	II	Kaleido Biosciences	Prof. Dr. med. Thomas Opladen	A Phase 2, Open-label Study to Evaluate the Efficacy and Safety of KB195 in Subjects with a Urea Cycle Disorder with Inadequate Control on Standard of Care	Adults with Urea Cycle Disorder	18 to 65 years	1	1	100 %	No	03/2020-11/2021

SIT: Sponsor-Initiated Trial (clinical trials with sponsors from pharmaceutical industry), IIT: Investigator-Initiated Trial, OS: Observation Study, R – Recruitment, recruited
Green lettering: Currently recruiting trials, blue lettering: All other trials

N	EudraCT Number	Trial Type	Trial Phase	Sponsor	Investigator	Titel and Design	Trial Population	Age	R goal	R	R success	Recruiting	Duration
1	2018-003099-10	SIT	III	Santhera Pharmaceuticals (Switzerland) Limited	Prof. Dr. med. Thomas Opladen	A Phase III Double-blind, Randomized, Placebo-Controlled Study assessing the Efficacy, Safety and Tolerability of Idebenone in Patients with Duchenne Muscular Dystrophy Receiving Glucocorticoid Steroids (“SIDEROS”)	Children and adults with Duchenne Muscular Dystrophy	≥ 10 years	3	4	133 %	No	04/2019-03/2021

SIT: Sponsor-Initiated Trial (clinical trials with sponsors from pharmaceutical industry), IIT: Investigator-Initiated Trial, OS: Observation Study, R – Recruitment, recruited
Green lettering: Currently recruiting trials, blue lettering: All other trials