

N	EudraCT Number	Trial Type	Trial Phase	Sponsor	Investigator	Titel and Design	Trial Population	Age	R goal	R	R success	Recruiting	Duration
17	2020-003384-25	SIT	III	Ultragenyx Pharmaceutical Inc.	Prof. Dr. med. Thomas Opladen	A Phase 3, Randomized, Double-Blind, Placebo-Controlled Study of Adeno-Associated Virus Serotype 8 (AAV8)-Mediated Gene Transfer of Human Ornithine Transcarbamylase (OTC) in Patients with Late-Onset OTC Deficiency	Adolescents and adult subjects with Late-Onset OTC Deficiency	>12 years	2	1		Yes	Since 01/2023
16	2021-000497-28	SIT	III	PTC Therapeutics	Prof. Dr. med. Thomas Opladen	A Phase 3 Open-Label Extension Study of PTC923 in Phenylketonuria ("PTC923-MD-004-PKU")	Male or female subjects of any age with Phenylketonuria	> 0 months	1-2	1	100%	Yes	Since 11/2022
15	2021-002071-19	SIT	III	Bayer AG	Prof. Dr. med. Elke Wühl	A 6 month multicenter, randomized, double-blind, placebo-controlled study to evaluate the efficacy, safety and PK/PD of an age- and body weight-adjusted oral finerenone regimen, in addition to an ACEI or ARB, for the treatment of children, 6 months to <18 years of age, with chronic kidney disease and proteinuria ("FIONA" Study)	Children and Adolescents with chronic kidney disease and proteinuria	6 months to <18 years	3			Yes	Since 06/2022
14	2020-004381-19	SIT	III	Neurocrine Biosciences Inc.	Prof. Dr. med. Markus Bettendorf	A Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Safety and Efficacy of Crinicerfont (NBI-74788) in Pediatric Subjects with Classic Congenital Adrenal Hyperplasia, Followed by Open-Label Treatment	Children and adolescents with Classic congenital Adrenal Hyperplasia	6 – 17 years	15	1	7%	No	Since 08/2022
13	2021-000474-29	SIT	III	PTC Therapeutics	Prof. Dr. med.	A Phase 3 Study of PTC923 in Subjects with Phenylketonuria ("PTC923-MD-003-PKU")	Male or female subjects of any	> 0 months	2	3	150%	No	Since 07/2022

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					Thomas Opladen		age with Phenylketonuria						
12	2020-002798-92	SIT	III	Provention Bio	Dr. med. Kristine Chobanyan-Jürgens	A Multicenter, Multinational Extension of Study PRV-031-001 to Evaluate the Long-Term Safety of Teplizumab (PRV-031), a Humanized, FcR Non-Binding, Anti-CD3 Monoclonal Antibody, in Children and Adolescents with Recent-Onset Type 1 Diabetes Mellitus ("PROTECT Extension Study")	Children and Adolescents with Recent-Onset Type 1 Diabetes Mellitus	> 9 years	4-5	5	100%	Roll-over from the PROTECT-Study	Since 05/2022
11	2021-001083-16	SIT	II	Dicerna Pharmaceuticals Inc.	Prof. Dr. med. Burkhard Tönshoff	A Phase 2 Open-Label Multicenter Study to Evaluate the Efficacy, Safety, and Pharmacokinetics of Nedosiran in Pediatric Patients from Birth to 11 Years of Age with Primary Hyperoxaluria and Relatively Intact Renal Function ("PHYOX8" Study)	Children with Primary Hyperoxaluria and Relatively Intact Renal Function	0-11 years	2			Yes	Since 03/2022
10	2020-000561-16	SIT	II	Novartis Pharma AG	Prof. 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	3	2	150 %	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" Study)	Children and Adolescents with Primary Hyperoxaluria	≥ 6 years	1	1	100 %	Yes	Since 12/2020

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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" Study)	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; Dr. med. Kristine Chobanyan-Jürgens	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" Study)	Children and Adolescents with Type 1 Diabetes	8 to 17 years	3	5	167 %	No	Since 09/2020
6	2019-002817-21	SIT	III	Janssen 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" Study)	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

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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	15	75 %	No	12/2019-07/2022
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
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” Study)	Children and adults with Duchenne Muscular Dystrophy	≥ 10 years	3	4	133 %	No	04/2019-03/2021

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