

## Previous and ongoing clinical trials

JAN 2001	PI	Sevelamer study (APN initiated, Genzyme REN 002-01; EU/199/123/001-004)
JAN 2002- JAN 2005	PI	Basiliximab study (APN initiated, Novartis, Protokoll No: CCHI621A DE01)
JAN 2002- JAN 2004	PI	Oxalobacter studies I and II (Ixion Biotechnology Inc., CTIOx.0002 and CTIOx.0005)
JAN 2004- JAN 2006	PI	Symbiolact study (Symbiopharm, Protokoll No. SY 2003/110)
JAN 2004- OCT 2008	LKP	Alkaline Citrate Treatment to Lower the Risk of Nephrokalzinosis in Preterm Infants
JAN 2005	PI	Prospective study of diagnosis and treatment of post transplant lymphoproliferative disease after solid organ transplantation in children
JAN 2006- JAN 2007	PI	TWIST Study (Protokoll No.: FG-02-43-DE-016)
NOV 2007- SEP 2008	LKP	International Study to Evaluate the Efficacy and Safety of Oxalobact to Reduce Urinary Oxalate Excretion in Subjects with Primary Hyperoxaluria, Oxthera AB, OC01-DB
APR 2008- APR 2009	LKP	An Open Label (OL) Extension Study, Evaluating the Long Term Safety of OC, in Subjects with Primary Hyperoxaluria (PH) who participated in the Double Blind Study (DB) study
JUN 2008- JUN 2011	LKP	A Prospective Registry Study Observing the Safety and Patterns of Use of Darbepoetin Alfa in EU Pediatric Chronic Kidney Disease Patients Receiving or not Receiving Dialysis – a Post Approval Study
AUG 2008- NOV 2011	PI	An Open Label Multi-Center, multiple dose study to determine the optimum of intravenous Mircera for maintenance treatment of anemia in pediatric patients with chronic kidney disease on hemodialysis Dolphin Study (Dose Finding Trial of Pediatrics on Hemodialysis in Nephrology)
SEP 2009- SEP 2009	PI	A study to determine feasibility of procedures for 24 h urine collections and variability in urinary oxalate and creatinine excretion test results
SEP 2009- May 2010	PI	An Open Label, Multi-Center Controlled Trial Of Eculizumab in Adolescent Patients with Plasma Therapy-Resistant Atypical Hemolytic Uremic Syndrome (AHUS), Alexion
DEC 2009- ongoing	PI	The Cardiovascular Comorbidity in Children with Chronic Kidney Disease Study (4c)

JAN 2010- MAR 2011	PI	A Phase 2/3, Double blind, Randomized, Placebo Controlled, Multi-Center Study to evaluate the Efficacy and Safety of Oxabact to Reduce Urinary Oxalate in Subjects with Primary Hyperoxaluria. OC3-DB-01, Oxthera AB
AUG 2010- MAY 2012	PI	Randomized, multicenter cross-over study investigating the effect of bicarbonate-based solutions (Physioneal 35 vs.40) on protein metabolism in children and adolescents on chronic peritoneal dialysis
AUG 2010 ongoing	PI	International registry investigating the safety of bicarbonate solutions (Physioneal 35 vs.40) in children and adolescents on chronic peritoneal dialysis
DEC 2010- JUN 2013	LKP	Pilot trial on treatment of Patients with primary Hyperoxaluria type I with pyridoxal phosphate
JAN 2011 ongoing	PI	A Phase 3, Prospective, Randomized, Double-Blind, Placebo controlled Multi-Center Study to Evaluate the Pharmacokinetics, Safety and Efficacy of Paracalcitol Capsules in Decreasing Serum Intact Parathyroid Hormone Levels in Pediatric Subjects Ages 10 to 16 years with Moderate to Severe Chronic Kidney Disease
FEB 2011	PI	Register for Lupus Nephritis
FEB 2011	PI	Register for Purpura Schoenlein Henoch Nephritis (PSNH Register)
AUG 2011	PI	Renal Insufficiency Therapy in Children: Quality Assessment and Improvement
FEB 2012- JUN 2013	PI	Tolerability of up to 200 Days of Valganciclovir oral Solution or Tablets in Pediatric Kidney Transplant Recipients
FEB 2012	PI	A multicenter, randomized, open labelled study to steer immunosuppressive and antiviral therapy by measurement of virus (CMV, ADV, HSV) specific T cells in addition to determination of through levels of immunosuppressants in pediatric kidney and liver allograft recipients. An explorative study.
APR 2012- ongoing	PI	Early prospective therapy trial to delay renal failure in children with alport syndrome, Early Protect Study, GPN supported
APR 2012- FEB 2018	PI	Dia-Sport, Endurance orientated training program with children and adolescents on maintenance hemodialysis. HO DFG 1272/21-1
JUN 2012	PI	International Pediatric Peritoneal Biopsy Study
JUL 2013- NOV 2018	PI	International Registry for atypical haemolytic uremic syndrome
DEC 2013	PI	A phase 1/2, randomized, placebo controlled, double blind, multi-centre study to evaluate the efficacy and safety of OC5 to reduce urinary oxalate excretion in subjects with primary hyperoxaluria, ELIMOX
APR 2014 - ongoing	PI	A phase 2, open label, multi-centre study to evaluate the efficacy and safety of Oxabact® to reduce plasma oxalate in subjects with primary hyperoxaluria who are on dialysis
May 2015 - ongoing	PI	Initial treatment of idiopathic nephrotic syndrome in children with mycophenolate mofetil vs. prednisone: A randomized, controlled, multicenter study (INTENT Study)

NOV 2015 – Dec 2017	PI	An observational Study of Patients with Primary Hyperoxaluria Type 1, PHYOS
MAR 2016 – OCT 2016	PI	A Phase 1 Study of DCR-PH1 in Patients with Primary Hyperoxaluria Type 1 (PH1)
AUG 2016 - ongoing	PI	A Phase 1/2 , Single-Blind, Placebo-Controlled, Single– and Multiple-Ascending Dose Safety, Tolerability, Pharmacokinetic and Pharmacodynamics Study of Subcutaneously Administered ALN-GO1 in Healthy Adult Subjects , and Patients with Primary Hyperoxaluria Type 1
NOV 2016 - ongoing	PI	A multicenter, open label, uncontrolled study to evaluate the nutritional suitability, acceptability, and tolerability of Renastart
APR 2017 – AUG 2018	PI	A single Arm study to of ALXN1210 in complement inhibitor treatment naïve adult and adolescent patients with atypical haemolytic uremic syndrome (aHUS). ALXN1210-aHUS-311
JUN 2017 – MAR 2019	PI	A 24-week randomized, open-label, study to evaluate the safety and efficacy of Fesoterodine in subjects aged 6 to 17 years with symptoms of detrusor over-activity associated with a neurological condition (neurogenic detrusor overactivity).
AUG 2017 – NOV 2017	PI	Evaluation of potential predictors of disease progression of disease progression in patients with aHUS, including genetics, biomarkers and treatment.
SEPT 2017 – ongoing	PI	A phase III double-blind, randomised study to evaluate the long-term efficacy and safety of Oxabact® in patients with primary hyperoxaluria. OC5-DB-02
JAN 2018 – ongoing	PI	A Phase 3, open-label. Multicenter study of ALXN1210 in children and adolescents with Atypical-Hemolytic-Uremic syndrome (aHUS). ALXN1210-aHUS-312
MAR 2018 – December 2018	PI	A Phase 2, Multicenter, Open-Label, Extension Study to Evaluate the Long-Term Administration of ALN-Go1 in patients with Primary Hyperoxaluria Type 1 ALN-GO1-002
July 2018 - ongoing	PI	A Placebo-Controlled, Single-Blind, Single-Center Phase 1 Study in Normal Healthy Volunteers and Open-Label Multi-Center Study in Patients with Primary Hyperoxaluria to Evaluate the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of Single Ascending Doses of DCR-PHXC Solution for Injection (subcutaneous use). DCR-PHXC-101
NOV 2018 - ongoing	PI	Evaluate the Safety and Efficacy of ALLN-177 in Patients with Enteric Hyperoxaluria: A Phase III Randomized, Placebo-Controlled Study. ALLN-177-301.
NOV 2018 - ongoing		A Phase 2, Multicenter, Open-Label, Extension Study to Evaluate the Long-Term Administration of ALN-GO1 in Patients with Primary Hyperoxaluria Type 1

OCT 2018 - ongoing		PHYOX: A Safety and Tolerability Study of DCR-PHXC in Primary Hyperoxaluria Types 1 and 2
MAR 2019 – ongoing		Alynham Pharmaceuticals ILLUMINATE-A study: A Phase 3 Randomized, Double-Blind, Placebo-Controlled Study with and Extended Dosing Period to Evaluate the Efficacy and Safety of Lumasiran in Children and Adults with Primary Hyperoxaluria Type I.