1. Adrenocorticotropic Hormone (ACTH) for Frequently Relapsing and Steroid Dependent Nephrotic Syndrome: ATLANTIS Study

**Purpose:** In childhood nephrotic syndrome, the kidneys leak protein, causing body swelling and a variety of possible complications such as infection, blood clots, and kidney failure. The first-line treatment for nephrotic syndrome is corticosteroids. Many children respond to prednisone treatment, but the disease comes back (relapses) when the prednisone is stopped or the dose is reduced. Children with frequently relapsing or steroid dependent nephrotic syndrome are at risk for toxicity from frequent exposure to corticosteroids.

Currently, the standard treatment for frequently relapsing and steroid dependent nephrotic syndrome involves a variety of medications that suppress the immune system, which can produce serious side effects. We propose a study to examine the effects of a different medication, ACTH, on nephrotic syndrome. ACTH is a hormone naturally found in the body. Recently, in adult studies, ACTH has been shown to be effective for the treatment of nephrotic syndrome. It has also been shown to have mild and reversible side effects. ACTH is potentially an attractive therapeutic alternative for the treatment of frequently relapsing and steroid dependent nephrotic syndrome in children. Our study will randomly assign patients with frequently relapsing or steroid dependent nephrotic syndrome to either ACTH treatment or no treatment. This will allow us to study the effects of ACTH on this disease and its side effects, by comparing how patients do on ACTH treatment versus no treatment. We hypothesize that ACTH gel is superior to no treatment in maintaining remission in children with frequently relapsing or steroid dependent nephrotic syndrome.

[https://clinicaltrials.gov/ct2/show/NCT02132195](https://clinicaltrials.gov/ct2/show/NCT02132195)

**Recruitment status:** Enrolling

**Sponsor:** Mallinckrodt Pharmaceuticals

**Site PI:** Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA and Dr. Chia-shi Wang, Assistant Professor, Division of Pediatric Nephrology, Emory University, Atlanta, GA

**Study coordinator:** Margo Kamel, PhD

2. An Observational, Non-Interventional, Multi-Center, Multi-National Study of Patients with Atypical Hemolytic-Uremic Syndrome (aHUS Registry) (Study Number: M11-001)
Purpose: The registry is an observational, non-interventional, multi-center, multi-national, study that has been designed to capture safety and effectiveness data specific to the use of eculizumab in aHUS patients, as well as to compile data on the long term manifestations of TMA complications of aHUS. The registry will enroll aHUS patients treated not with eculizumab. It is anticipated that patients will be followed at least for 5 years. Data collected in the registry will be reported to the FDA and the EMA.

http://clinicaltrials.gov/ct2/show/NCT01522183

Recruitment status: open to enrollment

Sponsor: Alexion Pharmaceuticals

Site PI: Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

Site coordinator: Ling Iem, BS

3. Evaluation of Potential Predictors of Disease Progression in Patients with aHUS, Including Genetics, Biomarkers, and Treatment (EVIDENCE) (STUDY Number: ECU-aHUS-403)

Purpose: This is a prospective, open-label study with no patient randomization. Treatment for atypical hemolytic uremic syndrome (aHUS) will remain observational and at the discretion of the treating physician. The purpose of this study is to assess disease manifestations of complement mediated thrombotic microangiopathy (TMA) and potential clinical predictors of disease manifestations and progression in patients with aHUS with or without eculizumab treatment in the clinical setting.

https://clinicaltrials.gov/ct2/show/NCT02614898

Recruitment Status: Enrolling

Sponsor: Alexion Pharmaceuticals

Site PI: Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

Site Coordinator: Ling Iem, BS

4. Chronic Kidney Disease in Children Prospective Cohort Study (CKiD III)

Purpose: This is an observational study of children with chronic kidney disease. The primary goals of this study are to determine the risk factors for decline in kidney function and to define
how a progressive decline in kidney function impacts neurocognitive function and behavior; the risk factors for cardiovascular disease; and growth failure and its associated morbidity.

http://clinicaltrials.gov/ct/show/NCT00327860?order=1

Recruitment status: Enrolling

Sponsor: National Institute of Health (NIH)

Site PI: Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

Site coordinator: Ling Iem, BS and Margo Kamel, PhD

5. Childhood Nephrotic Syndrome Observational Study (CNOS)

Purpose: Childhood onset nephrotic syndrome is a condition that affects the kidneys. It causes them to leak protein from the blood into the urine. The purpose of this study is to improve our understanding of the causes, effects, and treatment response of childhood nephrotic syndrome. We are also hoping to find out more information about how nephrotic syndrome progresses in different people and how or why that happens.

Recruitment status: Enrolling

Sponsor: Investigator Initiated

Site PI: Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

Site coordinator: Matthew Bennett, BS and Priscilla Quach, BS

6. Executive Function in Children with Hypertension

Purpose: Studies in young adults indicate that primary hypertension is associated with decreased performance on neurocognitive testing compared with normotensive controls, particularly in the domains of attention, working memory, and executive function. These cognitive deficits can improve in adults when hypertension is subsequently well-controlled, indicating that the neurocognitive deficits seen in hypertensives may represent an early manifestation of hypertensive target organ damage of the brain. The goal of the current proposal is to investigate the relationship between primary hypertension and executive function as an emerging target of hypertensive damage in children. The overall hypothesis is that children with primary hypertension have evidence for central nervous system target organ damage, as manifested by decreased executive function.

Recruitment status: closed to enrollment

Sponsor: NIH
7. Immune Monitoring and Assay Development in Kidney Transplant Recipients (IMP)

**Purpose:** Currently, a kidney biopsy is the only way to determine whether a patient with a kidney transplant has rejection of their kidney. The goals of this study are to develop and study urine and blood tests that can determine if a patient is rejecting a transplanted kidney. This will hopefully decrease the need to perform kidney biopsies and allow for earlier diagnosis of rejection.

**Recruitment Status:** Enrolling

**Sponsor:** Investigator Initiated

**Sponsor: PI's:** Dr. Roshan George and Dr. Pamela Winterberg, Division of Pediatric Nephrology, Emory University, Atlanta, GA

**Site coordinator:** Brian Lee, BS

8. A Molecular Pathogenesis-Driven Approach for Diagnosis and Treatment of Complement-Based Renal Diseases (KidCOM)

**Purpose:** The purpose of this research is to build a registry of patients with aHUS and MPGN. MPGN is a rare disease that can cause problems with how the kidney filters blood and waste. Currently, little is known about how to treat MPGN. There are three different types of MPGN; MPGN I, MPGN II/Dense Deposit Disease (MPGN II/DDD), and MPGN III. Although we will look at all types of MPGN, we are focused on MPGN II/DDD because it is most closely linked to the immune system.

Hemolytic Uremic Syndrome (HUS) is another rare immune disease that affects blood supply to the kidneys, and impairs their function. There are two types of HUS; typical HUS, and atypical HUS. Typical HUS, which is usually diagnosed in childhood, is due to a bacterial infection in the stomach and intestines. Atypical HUS (aHUS) is a hereditary disease that is associated with recurrent episodes.

**Recruitment status:** Enrolling

**Collaborators:** Dr. Christoph Licht at The Hospital for Sick Children, Toronto, Ontario, Canada; Dr. William Smoyer at The Research Institute at Nationwide Children's Hospital, Columbus, Ohio; Dr. Patrick Brophy at University of Iowa Hospitals and Clinics, Iowa City, Iowa.

**Sponsors:** Foundation for Children with Atypical HUS, and Optherion, Inc.

Version date 10/07/2016
9. Pediatric Lupus Nephritis Registry (Lupus Registry)

**Purpose:** This study will examine the incidence, causes, clinical course, and outcomes in children with lupus kidney disease. After informed consent and assent, medical records will be reviewed and history, physical exam findings, laboratory, radiology, hospital events, and medication information will be recorded into a multi-center registry. Longitudinal data will be collected for at least 5 years after entry into the registry.

**Recruitment status:** Enrolling

Participating site with Children’s Hospital of Chicago

Site PI: Dr. Donald Batisky, Director of Hypertension Program, Division of Pediatric Nephrology, Emory University, Atlanta, GA

Site coordinator: Brian Lee, BS

10. Nephrotic Syndrome Study Network (NEPTUNE)

**Purpose:** The purpose of this study is to find markers of Nephrotic Syndrome (kidney disease with too much protein in the urine). We are particularly interested in diseases called Focal and Segmental Glomerulosclerosis (FSGS), Minimal Change Disease (MCD), and Membranous Nephropathy (MN). By collecting health information and laboratory samples, our goal is to learn more about these kidney diseases and find better ways to prevent and treat people with these kidney diseases.

http://clinicaltrials.gov/ct2/show/NCT01240564?

**Recruitment status:** Enrolling

**Sponsor:** National Institutes of Health (NIH)-National Institute of Diabetes and Digestive and Kidney Disease (NIDDK)

Site PI: Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

Site coordinators: Brian Lee, BS and Helina Iyob-Tessema, BS

11. A Phase II Randomized, Placebo-Controlled, Double-Blind, Parallel Arms with Switchover, Pilot Study to Evaluate the Efficacy and Safety of Intravenous Abatacept in Treatment Resistant Nephrotic Syndrome (Focal Segmental Glomerulosclerosis/Minimal Change)
**Purpose:** The purpose of this study is evaluate if abatacept is effective and safe in decreasing the level of protein loss in the urine in patients with excessive loss of protein in the urine (nephrotic syndrome) due to either focal segmental glomerulosclerosis (FSGS) or minimal change disease (MCD). Candidates must have a prior kidney biopsy with either diagnosis. Another kidney biopsy will not be required as part of the study. Candidates must have failed or be intolerant of prior therapy for their kidney disease. The failed or intolerant therapy must include corticosteroids and at least one other drug. Candidates can be adults and children over the age of 6. Abatacept will be administered by venous infusion every 4 weeks.

[https://clinicaltrials.gov/ct2/show/study/NCT02592798](https://clinicaltrials.gov/ct2/show/study/NCT02592798)

**Recruitment status:** Enrolling

**Sponsor:** Bristol-Myers Squibb

**Site PI:** Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

**Site coordinator:** Brian Lee, BS

**12. Bovine Serum Albumin-Related Membranous Nephropathy**

**Purpose:** Bovine serum albumin (BSA) is one of the cow’s milk and beef proteins that can escape from the intestinal barrier and induce formation of anti-BSA antibodies. Modern day foods are subjected to a variety of processing conditions that may modify food proteins, and thus could change their digestion and allow their passage into the blood stream. This study will examine individuals with a diagnosis of idiopathic membranous nephropathy and investigate the involvement of cationic BSA and its relationship to the subject’s clinical presentation, past medical history including dietary history, family history, and clinical course.

**Recruitment status:** Enrolling

**Sponsor:** Investigator Initiated

**Site PI:** Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

**Site coordinator:** Brian Lee, BS

**13. CureGN: Cure Glomerulonephropathy Network (CureGN)**

**Purpose:** There are several different types of glomerular diseases, such as minimal change disease (MCD), focal segmental glomerulosclerosis (FSGS), membranous nephropathy (MN), and immunoglobulin A nephropathy (IgAN). Over time, these diseases may cause kidney damage. These kidney diseases are rare and because of that, it is difficult for individual researchers to gather a large enough number of people to effectively study underlying causes, identify markers of disease, and identify and evaluate new therapies. The purpose of CureGN is
to gather a group of patients with glomerular disease to create a source of information and blood and urine samples, so that researchers can easily and effectively study glomerular disease.

**Recruitment Status: Enrolling**

**Sponsor:** National Institute of Health (NIH)

**Site PI:** Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

**Site coordinator:** Margo Kamel, PhD and Helina Iyob Tessema, BS

14. "A Phase 3b, Multicenter, Open-label, Randomized Withdrawal Trial of the Effects of Titrated Oral SAMSCA® (Tolvaptan) on Serum Sodium, Pharmacokinetics, and Safety in Children and Adolescent Subjects Hospitalized With Euvolemic or Hypervolemic Hyponatremia" (OTSUKA)

**Purpose:** Otsuka Pharmaceutical Development & Commercialization, Inc. (OPDC), is studying an investigational drug called tolvaptan (“Study Drug”). Tolvaptan (Samsca®) is a drug approved for use in the United States (2009) in patients with certain types of hyponatremia (low amount of sodium or salt in the blood) due to syndrome of inappropriate antidiuretic hormone. Tolvaptan (Samsca®) is approved in the European Union (2009) for treatment of a specific type of hyponatremia due to SIADH. Tolvaptan (Samsca®) has been approved by the Japanese Ministry of Health, Labour, and Welfare (2010) for the treatment of volume overload in heart failure when used in combination with other approved drugs.

Tolvaptan is still being studied to see if it can be used to treat problems associated with various causes of hyponatremia. A low amount of sodium in the blood may be due to abnormal hormone levels, medication that your child has to take, or another disease that your child may have. Low blood sodium levels may result in nausea, vomiting, and muscle discomfort. It may cause weakness and slow, abnormal, or poor thinking. Low blood sodium may cause abnormal behavior, seizures or fits, and coma or unconsciousness. It may cause lack of emotion or slowed breathing. Tests taken during the study will determine how useful tolvaptan will be in treating low blood sodium. The study doctor has determined that your child has low blood sodium levels. Your child is invited to take part in this research study.

The reason for this study is to find out the potential benefits and safety of tolvaptan in the pediatric and adolescent population. About 100 participants will be joining in this study globally or regionally in approximately 50 centers. It is expected that participation will last approximately 21 days for each participant.

https://clinicaltrials.gov/ct2/show/NCT02012959
Recruitment Status: Enrolling

Sponsor: Otsuka Pharmaceuticals

Site PI: Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

Site coordinator: Margo Kamel, PhD

15. A Phase 3b, Multicenter, Extension Follow-up Trial to Evaluate the Long-term Safety of Children and Adolescent Subjects With Euvolemic or Hypervolemic Hyponatremia Who Have Previously Participated in a Trial of Titrated Oral SAMSCA® (Tolvaptan)

**Purpose:** The objective of this trial is to provide 6 months of safety follow-up for children and adolescents with dilutional (euvolemic or hypervolemic) hyponatremia who have previously participated in a tolvaptan hyponatremia trial, and to assess the efficacy of tolvaptan in increasing serum sodium for those subjects who receive optional continuing tolvaptan treatment of variable duration (up to 6 months).

[https://clinicaltrials.gov/ct2/show/NCT02020278](https://clinicaltrials.gov/ct2/show/NCT02020278)

Recruitment Status: Not yet open to enrollment

Sponsor: Otsuka Pharmaceuticals

Site PI: Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

Site Coordinator: Margo Kamel, PhD


**Purpose:** This is a phase 3, 32-week, multicenter, single arm, open-label extension study. All enrolled subjects will be administered cinacalcet daily during the treatment period in addition to standard of care treatment which can include therapy with Vitamin D sterols, calcium supplementation, and phosphate binders.

[https://clinicaltrials.gov/ct2/show/NCT02341417](https://clinicaltrials.gov/ct2/show/NCT02341417)

Recruitment Status: Enrollment closed

Sponsor: Amgen, Inc.
Site PI: Dr. Stephanie Jernigan, Division of Pediatric Nephrology, Emory University, Atlanta, GA

Site coordinator: Ling Iem, BS

17. LN Antibodies

**Purpose:** This study will examine the ability of novel antibodies or other biomarkers to predict 1) findings on kidney biopsy, 2) disease progression, or 3) response to therapy in children with lupus nephritis (LN). The intent is to enroll subjects who have or will also enroll in the MWPNC’s Pediatric LN Registry (however, involvement in the registry is not required). After obtaining informed consent and assent, medical records including history, exam findings, laboratory radiology and biopsy findings, treatment information, and outcomes will be prospectively reviewed and recorded. There are 2 levels of involvement for this study. The short term goal of this study will be to measure antibody titers against basement membrane (BM) antigens in the plasma and urine at onset of LN and again 6-mo into therapy. Results would be compared with the currently available clinical indicators of outcome and treatment response. The long-term goal of this study will be to establish a bio-repository of blood and urine samples (level 1) and kidney tissue (level 2) to assess the utility of additional autoantibodies or other biomarkers in MWPNC pediatric LN cohort, and to facilitate collaboration with larger efforts for biomarker identification in SLE that would otherwise not have access to pediatric samples.

**Recruitment Status:** Enrolling

**Participating site with Midwest Pediatric Nephrology Consortium (MWPNC)**

Site PI: Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

Site Coordinator: Brian Lee, BS

18. Use of Metformin in the Treatment of Patients with Congenital NDI (Nephrogenic Diabetes Insipidus)

**Purpose:**

Nephrogenic diabetes insipidus (NDI) is a genetic disease. Patients with this disease make large amounts of urine because their kidneys don’t hold on to water. The large amount of urine means that patients need to urinate very frequently. They are also at increased risk for dehydration if they don’t drink enough. The large amount of urine can sometimes damage their bladders and kidneys. There are some medicines that may help these patients urinate less, but they are not very effective. There is evidence in animal studies that a medication called metformin may help patients with NDI urinate less. Metformin is a medication currently used to treat patients with
diabetes and other conditions. We are going to test metformin in patients with NDI. Patients will be invited to the Emory Clinical Research Center for a baseline urine measurement. They will return home and stop their usual medicines to treat NDI. Then another 24 hour urine collection which they will take to the nearest quest laboratory. Then they will resume metformin 500mg twice a day for 3 weeks. 24-hour urine will be collected once a week for 3 weeks in order to measure the volume of the urine and the urine concentration. After three weeks the medications will be discontinued.

**Recruitment Status:** Enrolling

**Sponsor:** Investigator Initiated

**Site PI:** Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

**Site Coordinator:** Margo Kamel, PhD

19. **An Open-label, Randomised, Active-controlled, Parallel Group, Multicentre, Phase 3 Study to Investigate the Safety and Efficacy of PA21 (Velphoro®) and Calcium Acetate (Phoslyra®) in Paediatric and Adolescent CKD Patients with Hyperphosphataemia**

**Purpose:** This a Phase 3, Open-label, Randomised, Active-controlled, Parallel Group, Multicentre Study to Investigate the Safety and Efficacy of PA21 (Velphoro®) and Calcium Acetate (Phoslyra®) in Paediatric and Adolescent CKD Patients with Hyperphosphataemia. The aim of this Phase 3 clinical study is to demonstrate similar efficacy of PA21 (Velphoro) in paediatric and adolescent patients with CKD, and to provide safety and dosing information for this patient population. The Phoslyra (comparator) group provides information for a descriptive comparison of PA21 against a commonly used calcium-based phosphate binder (calcium acetate).

[https://clinicaltrials.gov/ct2/show/NCT02688764](https://clinicaltrials.gov/ct2/show/NCT02688764)

**Recruitment Status:** Enrolling

**Sponsor:** Vifor Fresenius Medical Care Renal Pharma

**Site PI:** Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

**Site Coordinator:** Helina Iyob-Tessema, BS

20. **Cystinosis: Clinical Outcomes in a Contemporary Group of American Patients**

**Purpose:** We will be conducting a retrospective study to define the clinical outcomes of a contemporary group of nephropathic cystinosis patients in the United States.
Recruitment Status: Enrolling

Sponsor: Raptor Pharmaceuticals INC

Site PI: Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

Study Coordinator: Margo Kamel, PhD

21. Practice Patterns and Outcomes of ACTHar use in Children with Nephrotic Syndrome

Purpose: This study is a multi-center registry that collects information on children with childhood nephrotic syndrome who have been treated with a drug called ACTHar. ACTHar use in children is limited so a data registry will help guide medical decisions in the future and will help to design future research studies.

Recruitment Status: Enrolling

Sponsor: North American Pediatric Transplant Case Study

Site PI: Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

Site Coordinator: Margo Kamel, PhD

22. cNeptune (Children’s Nephrotic Syndrome Study Network)

Purpose: In response to a request for applications by the National Institutes of Health, Office of Rare Diseases (NIH, ORD) for the creation of Rare Disease Clinical Research Consortia, a number of affiliated universities joined together with The NephCure Foundation the NIDDK, the ORDR, and the University of Michigan in collaboration towards the establishment of a Nephrotic Syndrome (NS) Rare Diseases Clinical Research Consortium.

Through this consortium the investigators hope to understand the fundamental biology of these rare diseases and aim to bank long-term observational data and corresponding biological specimens for researchers to access and further enrich.

Recruitment Status: Enrolling

Sponsor: National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)

Site PI: Dr. Larry Greenbaum, Director, Division of Pediatric Nephrology, Emory University, Atlanta, GA

Version date 10/07/2016
23. Urological and Renal Disease Engaging Adolescents in Adherence Collaborative Trial (U-REAACT)

**Purpose:** The overarching goal of this five-year, phase II, randomized clinical trial is to improve poor long-term health outcomes in both adolescents and young adults (AYA) with either a kidney transplant (KT) or spina bifida (SB), respectively. More specifically, this study will focus on decreasing premature allograft loss in subjects with kidney transplant (KT) due to medication nonadherence and kidney damage in subjects with SB due to urinary non-continenence. To achieve these goals, this study will implement a real-time feedback system, Way to Health (WTH), that will provide education and support, increase awareness and incentivize positive health behavior, in addition to standard of care. Further, this study will investigate the mechanisms of behavior change by examining the role of financial incentives, positive feedback and the relationship between the two. The study will compare two cohorts of KT and SB subjects, which will undergo varied levels of financial incentives and positive feedback. Data from KT and SB subjects will be analyzed jointly and separately. This innovative mobile health (mhealth) strategy will improve our current measures of adherence and increase our understanding of factors that influence adherence for two AYA populations, KT and SB subjects, respectively. The study will contribute novel insight to inform the design of future interventions targeting persistence of behavior change and can be used in other centers and for other chronic disease groups.

The study intervention will use the WTH web-based platform to support AYA with KT or SB as they navigate their daily treatment burdens. This will be achieved via bi-directional text messaging, including the sending of reminders and positive feedback by WTH and the messaging of pictures of medication or catheter in hand at time of treatment by the participant. This intervention will assess sustainability of this novel bi-directional messaging system and the impact of providing education and support, increasing awareness and incentivizing positive health behavior in real-time.

**Recruitment Status:** Planning Stage

**Sponsor:** National Institute of Health (NIH) and Children’s Hospital of Philadelphia (CHOP)

**Site PI:** Dr. Roshan George

**Site Coordinator:** Helina Iyob-Tessema, BS

24. A Phase 2/3 Trial of the Efficacy and Safety of Bardoxolone Methyl in Patients With Alport Syndrome (CARDINAL)

**Purpose:** This international, multi-center, Phase 2/3 trial will study the safety, tolerability, and efficacy of bardoxolone methyl in qualified patients with Alport syndrome. The Phase 2 portion
of the trial will be open-label and enroll up to 30 patients. The Phase 3 portion of the trial will be double-blind, randomized, placebo-controlled and will enroll up to 180 patients.

**Recruitment Status: Start Up Phase**

**Sponsor: Reata Pharmaceuticals**

**Site PI: Dr. Greenbaum**

**Site Coordinator: Brian Lee, BS**

**25. Study of Weekly RG-012 Injections in Patients with Alport Syndrome (HERA)**

**Purpose:** This will be a randomized, double-blind, placebo-controlled, multi-center, Phase 2 study conducted in subjects with Alport syndrome at multiple investigative centers.

**Recruitment Status: Planning**

**Sponsor: Regulus Therapeutics Inc.**

**Site PI: Dr. Greenbaum**

**Site Coordinator: Brian Lee, BS**

**26. ATHENA: Natural History of Disease Study in Alport Syndrome Patients**

**Purpose:** There is limited published clinical data about the natural history of renal disease in Alport syndrome. The RG012-01 study will collect data to characterize the progression of renal dysfunction in Alport syndrome patients.

Patients with a confirmed diagnosis of Alport syndrome who have qualifying GFR will be considered for enrollment. The sequential sampling of subjects' urine and/or blood will allow an assessment of the rate of change of established clinical endpoints, such as GFR and/or the rate of change of other renal biomarkers (proteinuria and β-2 microglobulin) in subjects whose renal function is steadily declining. The identification of surrogate markers that track the decline of renal function and could correlate with time to end-stage renal disease (ESRD) is a key goal of the natural history study.

**Recruitment Status: Planning**

**Sponsor: Regulus Therapeutics Inc.**

**Site PI: Dr. Greenbaum**

**Site Coordinator: Brian Lee, BS**