Study of Lademirsen (SAR339375) in Patients With Alport Syndrome

Status: Recruiting

Eligibility Criteria

Sex: All
Age: 18 Years to 55 Years old
This study is NOT accepting healthy volunteers
Inclusion criteria :

• Male or female

• Confirmed diagnosis of Alport syndrome 1. Clinical diagnosis (hematuria, family history, hearing loss, ocular change), AND 2. Genetic confirmation of Alport Syndrome in the subject or the family member, OR 3. Kidney biopsy showing glomerular basement membrane abnormalities (eg, significant thinning, thickening, irregularity or lucencies) consistent with Alport Syndrome.

• Age 18-55 years old

• eGFR > 35 ml/min/1.73m^2 and <90 mL/min/1.73m^2 (based on CKD-EPI) at screening

• Renal Function Criteria (patients must meet at least one of the following CRITERIA A, B or C):

• A)Decline in eGFR of â‰¥4 mL/min/1.73 m^2/year (eGFR slope <= -4) based on a linear regression slope analysis of â‰¥4 eGFR measurements within 3 years prior to the study and with a minimum of 2-year time span (the last, of the screening measurement, and first eGFR measurements should be separated by at least 2 years). eGFR should be calculated by using either the Chronic Kidney Disease Epidemiology Collaboration (CKD-EPI) creatinine equation.

• B) proteinuria (UPCR) >2000 mg/g (UACR>1000 mg/g)

• C) Age and sex adjusted eGFR (based on CKD-Epi; male 18-23 eGFR<90 mL/min/1.73m^2

• ACE inhibitor and/or ARB, the dosing regimen should be stable for at least 30 days prior to screening

• Sexually active female subjects of childbearing potential and sexually mature male subjects must agree to practice true abstinence in line with their preferred and usual lifestyle or to use two acceptable effective methods of contraception for the entire duration of the study and for at least 6 weeks after last dose.

• Negative drug screen for opiates, cocaine, heroin, phencyclidine, amphetamines (including ecstasy), barbiturates, benzodiazepines, and cannabinoids. At the Investigator's discretion, subjects prescribed benzodiazepines, cannabinoids, or opiates with positive results on a drug screen may be allowed.

• Negative screening results for hepatitis B surface antigen (HBsAg), hepatitis C virus (HCV) antibody, and human immunodeficiency virus (HIV) antibody

• Normal biological tests

• Able to understand all study procedures in the informed consent form (ICF) and willing to comply with all aspects of the protocol Exclusion criteria:

• Causes of chronic kidney disease aside from Alport syndrome (including but not limited to other heritable disorders leading to chronic kidney disease, diabetic nephropathy, hypertensive nephropathy, lupus nephritis, IgA nephropathy)

• ESRD as evidenced by ongoing dialysis therapy or history of renal transplantation

• Any clinically significant illness within 30 days before screening or surgical or medical condition (other than Alport syndrome) that could interfere with the subject's study compliance; confound the study results; impact subject safety; or significantly alter the absorption, distribution, metabolism, or excretion of drugs.

• Weight > 110 kg

• Any history of active malignancy within the last 1 year (history of localized basal cell or squamous cell carcinoma and cervical carcinoma in situ that has been excised/appropriately treated or a fully excised malignant lesion with a low probability of recurrence will not be considered exclusionary)

• Prior treatment with Bardoxolone within 90 days prior to screening

• History or presence of alcoholism or drug abuse within 2 years before screening or other concurrent social conditions that would potentially interfere with the subject's study compliance, at the discretion of the Investigator

• Participation in a recent investigational study and receipt of an investigational drug or investigational use of a licensed drug within 30 days or 5 half lives, whichever is longer, prior to screening

• History or presence of hypersensitivity or idiosyncratic, allergic, or other clinically significant reaction to the study drug (including placebo), inactive ingredients, or related compounds (eg, other oligonucleotide products)

• Any other condition or circumstance that, in the opinion of the Investigator, may make the subject unlikely to complete the study or comply with study procedures and requirements, or may pose a risk to the subject's safety and well-being The above information is not intended to contain all considerations relevant to a patient's potential participation in a clinical trial.

Conditions & Interventions

Interventions:
Drug: lademirsen (SAR339375), Drug: Placebo
Conditions:
Alport's Syndrome
Keywords:
Kidney disease, Nephritis, Hereditary nephritis, Hereditary kidney disease

More Information

Description: To assess the safety, tolerability, and efficacy of RG-012 in reducing the decline in renal function.
Contact(s): Amy Kowalski - mert0067@umn.edu
Phase: Phase 2
IRB Number:
System ID: NCT02855268