Suggested Questions to Ask When Considering Participation in a Clinical Trial

The following clinical trial (or clinical "study") questions, intended for U.S. patients or caregivers of patients, were developed based on educational resources from the U.S. Department of Health and Human Service's Office for Human Research Protections and the experiences of Alport syndrome patients and family members.

The Research and Drug Therapy Being Studied:

- What exactly is being studied in this clinical trial (including the name of the drug/therapy being studied)?
- Who is the sponsor of the clinical trial (the company funding the study)?
- What does the clinical trial sponsor hope to learn and who might benefit from the results of the study?
- Is Alport syndrome a "therapeutic target" of the trial? More particularly, if the new drug is approved by the FDA, will it be approved specifically as a treatment for Alport syndrome?
- Did experts in Alport syndrome provide feedback related to pre-clinical research associated with this study?
- Do any Alport syndrome experts currently serve on the advisory board for this study?
- When is the clinical trial expected to be completed?
- How will the results of the clinical trial be shared with participating patients?
- How many Alport syndrome patients in total will be studied in this clinical trial?

Patient Participation:

- What are the criteria for patients to qualify to participate in the clinical trial? *Inclusion/exclusion criteria examples may include age, body mass index, level of protein in urine, estimated glomerular function (eGFR), urine protein creatinine ratio (UPCR), genetic type of Alport syndrome, and existence of other medical conditions.*
- What kinds of procedures or tests would be part of the clinical trial?
- Will patients be given the results of any tests or procedures during the study?
- Do patients have to be on current standard of care (taking a prescribed ACE or ARB medication for Alport patients)? If ACEs/ARBs are not tolerated, can patients still participate? Can patients be taking an SGLT2i (such as Farxiga)? Are there any medications trial participants must stop taking?
- Are all patients being given the drug being studied, or is there a placebo group?
- Has the drug been studied in humans before or only in animal models?
- How long do patients have to participate in the clinical trial?
- Where is the closest participating study location?

- How many times do patients have to visit the study site in person?
- Can some visits be done virtually?
- If a patient's current doctor is not participating as part of the study, how will that doctor be able to follow the patient's progress?
- If a patient does well in the study and has stable or improved laboratory results, can the patient remain on the clinical trial drug while it is being reviewed by the U.S. Food and Drug Administration (be given "off-label extension" to the drug) as that review process can take an unknown amount of time?

Risks for Patients:

- What is known about the risks of the drug being studied?
- Are there any known discomforts or negative side effects that have been experienced by humans that have been given this drug so far?
- What is being done to minimize risks, discomfort, or negative side effects?

Confidentiality:

- How will patient data from individuals be kept confidential and private?
- How will overall patient data collected for the clinical study be shared and with whom?

Financial Considerations:

- Will it cost patients anything out of pocket to participate in the clinical trial?
- If a patient experiences negative effects directly related to study participation that requires medical attention, who would be financially responsible for any necessary medical treatment?
- If the closest clinical trial site requires travel, will the study sponsor reimburse patients for travel expenses such as gas mileage, airfare, and lodging if an overnight stay is needed?
- Is the study sponsor offering financial compensation for patient participants?

Other Questions:

- Who should patients contact if they have questions or concerns about participating in the clinical trial?
- If a patient requires medical treatment in an emergency room or other hospital setting and the treating physician needs information about the study drug the patient is on, who should be contacted to supply these important details?
- What happens if a patient decides to quit participating in the clinical trial? Are there any consequences for the patient or for the clinical study?

For information about clinical trials currently open for Alport syndrome patients in the U.S, visit: https://alportsyndrome.org/for-patients/clinical-trials/current-past-clinical-trials/ or email info@alportsyndrome.org.