



# Do kidney issues run in your family?

Learn more about the AMPLITUDE clinical research study

**For more information, please contact:**

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# Helping you on your kidney health journey

Thank you for taking the time to learn more about the AMPLITUDE clinical research study. Whether you have firsthand experience with kidney problems, or you know that kidney problems run in your family, you may be wondering what options are available and if a clinical research study is right for you.

This brochure provides a summary of the AMPLITUDE clinical research study, including why the study is taking place, who can take part, and what to expect if you decide to take part.

# Learn more about kidney health

The AMPLITUDE clinical research study is looking for people with APOLI-mediated kidney disease to take part. However, many people with this type of kidney disease don't know they have it. Before we get started on the details of the AMPLITUDE clinical research study, let's first take a look at some information around kidney health.

## What are the kidneys' main jobs?



Remove waste from the body to make urine



Balance fluids in the body



Control blood pressure



Promote strong and healthy bones

## What is chronic kidney disease?

Chronic kidney disease occurs when the kidneys don't work how they're supposed to. This can cause symptoms such as:



Weight loss and poor appetite



Swollen ankles, feet, or hands



Shortness of breath



Tiredness



Foamy urine

**Chronic kidney disease is progressive and can lead to kidney failure. People who experience kidney failure will need dialysis or a kidney transplant.**

# What is APOL1-mediated kidney disease?

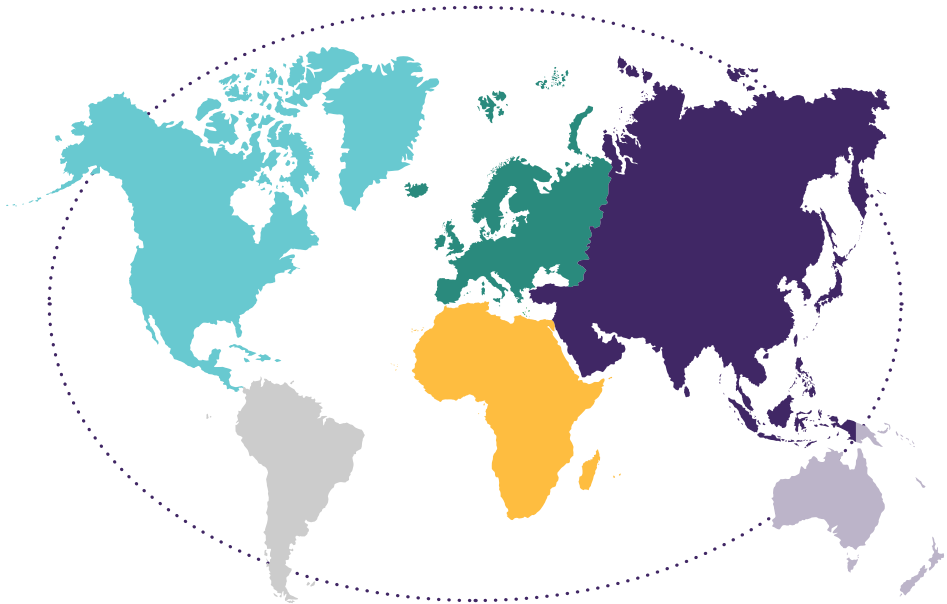
- There are different types of chronic kidney disease with different causes. **APOL1-mediated kidney disease** is caused by a substance in our body called the APOL1 protein.
- We all have the APOL1 protein, however some people have **differences in their DNA** (the genetic material in our body that carries information about how our body works) that means they carry a **changed type of APOL1 protein**, known as a variant. People with APOL1 variants are at a **higher risk** for chronic kidney disease.
- People share DNA with their blood relatives. This means APOL1-mediated kidney disease can affect many people **within the same family**.



# What is the APOL1 gene?

Several thousand years ago, the APOL1 variant genes evolved in people who lived in Western and Central Africa, to protect them from a parasitic disease known as African sleeping sickness.

**Today, people of African descent across the globe may carry these APOL1 variants.**



## Who is at risk of APOL1-mediated kidney disease?

Researchers have learned that variants of the APOL1 gene are more likely to occur in people of African descent, which may include but is not limited to, people who identify as African American, Black, Caribbean, African, and/or Latino (Cuban, Mexican, Puerto Rican, or South or Central American).

## How do I know if I carry the APOL1 variant?

The only way to find out is to examine your DNA, in a process called genetic testing



## What is genetic testing?

**Genetic testing is used to check differences in people's DNA.**

A doctor takes a small blood sample and saliva sample, and sends them to a laboratory to be tested.

If you decide to take part in the AMPLITUDE clinical research study, you will be asked to have a genetic test, to see if you carry the variant of the APOL1 protein being researched in the study.

This test used in the AMPLITUDE clinical research study is investigational which means it is not approved by health authorities and is not available outside of a clinical research study. If you would like to discuss the test more, or understand what the results of your test mean, an appointment can be made with a genetic counselor (at no cost to you).

**In order to join this clinical research study, a genetic test will be performed to confirm if you have APOL1-mediated kidney disease.**

# About the AMPLITUDE clinical research study

The AMPLITUDE clinical research study is exploring an investigational study drug, called VX-147, for people living with APOLI-mediated kidney disease. This will involve looking at **two different amounts** (doses) of the investigational study drug to help doctors and researchers decide which dose is safest and works the best. This study will measure if people who get the investigational drug have better outcomes than those who take the placebo.

**About 466 people will take part in this study around the world.**

## Why is this study important?

While there are treatments available to help with the symptoms of APOLI-mediated kidney disease, there are currently no approved treatments that address the underlying cause. As APOLI-mediated kidney disease is genetic, it can affect multiple people in the same family and be passed down to younger generations. This is why clinical research is so important: to try to find a potential treatment that addresses the underlying cause and potentially help create better treatments for those impacted by the disease.





# What can I expect in the AMPLITUDE clinical research study?

If you meet the requirements and choose to take part in the study, you will be given a full schedule with details about each study appointment. On the days when you have a scheduled appointment, you will either visit the study site, have a home health-service provider visit you, or have a mobile health visit.

There will be more appointments in the first few weeks as you begin the study. After that, on average, you will have an appointment scheduled about every 8 weeks.



## Some of the assessments and tests that may be included in your appointments include:



Questionnaires about yourself and your health



Physical exams (like weight and blood pressure measurements)



Heart assessments



Blood and urine samples



Pregnancy tests (if you are able to get pregnant)

# What happens if I decide I want to join the AMPLITUDE clinical research study?

- 1** Before joining the clinical research study, you will be given an informed consent form (ICF), which is a very detailed document with all of the information for you to review about the study. You will first need to sign the ICF, and the study doctor will perform some screening tests to see if you meet the requirements to be able to take part. This is called the 'screening period'.
- 2** If you meet the requirements and choose to take part, you will be randomly assigned to 1 of 3 groups. You will either receive dose 1 of the investigational study drug, dose 2 of the investigational study drug, or the placebo.
- 3** You will be given a schedule to follow, outlining when to take the investigational study drug, when and where you will have a study visit, and any other tests or procedures you will have.
- 4** The study doctor will be available to answer your questions and concerns throughout the study. If you wish to leave the clinical study, you can withdraw at any time by informing your study doctor.

**A placebo looks exactly like the investigational study drug, but doesn't have any effects. Placebos help researchers to compare the effectiveness of the investigational study drug.**



## Frequently asked questions

It's normal to have lots of questions about participating in clinical trials. Below are some common questions and answers that may help you to decide if clinical study participation is for you.

### **How much of my time will a clinical research study take up?**

While length of participation in the study may vary between participants, it's estimated that the entire AMPLITUDE clinical research study will run for up to 4 years.

### **What do you mean by 'investigational study drug'?**

The study drug is called 'investigational' because it has not yet been approved for treating chronic kidney disease by health authorities. This clinical research study will help researchers to understand the safety of VX-147, how well it works at treating APOL1-mediated chronic kidney disease, and help to increase scientific knowledge of chronic kidney disease.

### **How will I take the investigational study drug?**

The investigational study drug and placebo come in a tablet form and are taken by mouth once a day.

## Are there any risks to taking part in this clinical research study?

There may be some risks to taking part, either from side effects of the investigational study drug or from some of the assessments and procedures. Before you join the clinical research study, the study doctor will explain any potential risks or side effects, and your health will be closely monitored throughout the study.

## Will being in this clinical research study benefit me?

It is not known if taking part in this clinical research study will help you — your health may or may not get better. However, the information gained from this clinical research study will help researchers learn more about the investigational study drug, which may help people living with APOLI-mediated kidney disease in the future.

## Are there any costs involved with this clinical research study?

The tests or visits associated with the study come at no cost to you. You may be compensated for your study-related time and reimbursed for expenses needed to participate in the clinical research study, like transportation and meals. You will be given full details as to how much you may be reimbursed by a member of the study team.



Scan here

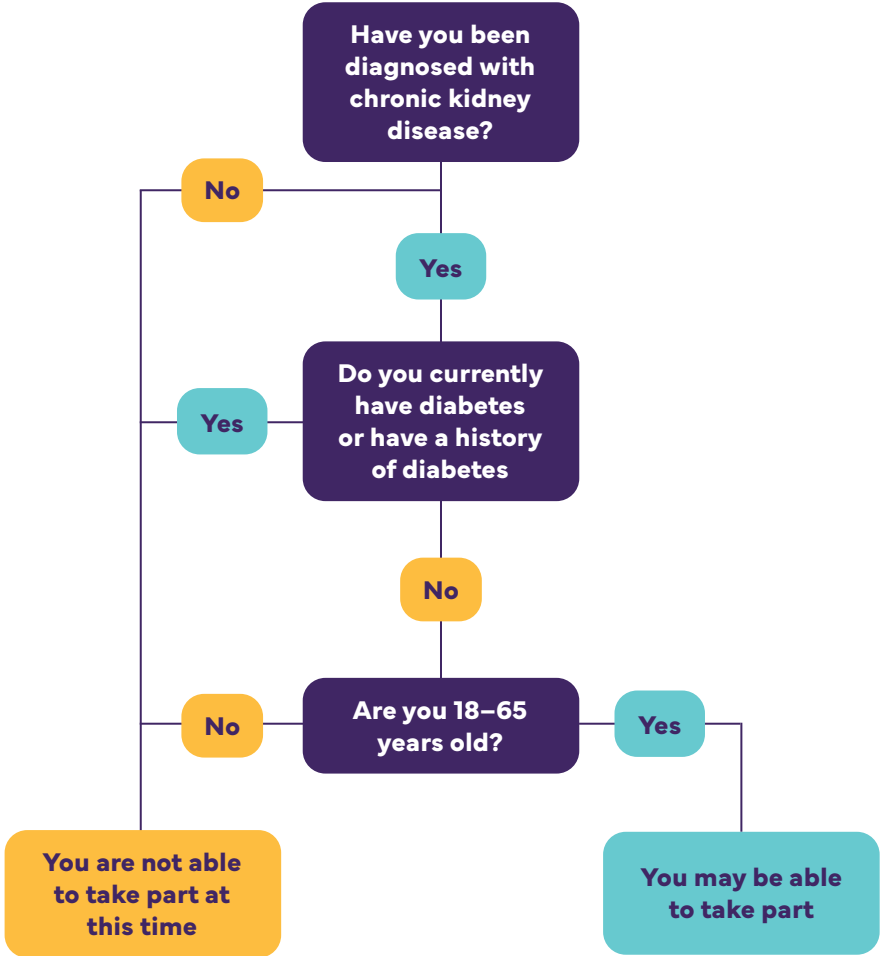
**Check out our website!**

**You can find more information over on our website at [amplitudeclinicalresearchstudy.com/research](https://amplitudeclinicalresearchstudy.com/research)**

**If you have any further questions or would like to know more about the AMPLITUDE clinical research study, please contact your doctor.**

# See if I qualify

To find out if you may be able to take part in the AMPLITUDE clinical research study, please use the flowchart below.



*Please note that this is not a complete list of criteria; you will need to answer some additional health-related questions and take part in some medical tests to confirm that you can join the clinical research study.*

**Interest in this clinical research study is welcomed from all people who meet the requirements to take part, regardless of sex, gender, sexual orientation, race, or religion.**

# What happens next?

## The decision is yours

Deciding to take part in a clinical research study is your decision. You can take as much time as you need to share any of the clinical study information with your loved ones or healthcare provider so that you can discuss your options and feel confident in your decision.

If you are considering joining the AMPLITUDE clinical research study, we would truly value your participation. Please talk to your doctor if you are interested in taking part.



## You can contact the AMPLITUDE clinical research study team through:

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646-317-0785

# Notes

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