Summary of Results for Laypersons

Astellas is grateful to everyone who took part in this clinical study. Thank you.

What was the Study Called?

A Phase 1 Study to Evaluate the Pharmacokinetics of Roxadustat in Subjects with Different Degrees of Renal Function.

Why was this Study Needed?

Healthy kidneys filter the blood and they also produce a hormone that tells the soft tissue within the bone (bone marrow) to make red blood cells. Patients with chronic kidney disease often have a lack of enough red blood cells (kidney anemia). When kidneys stop working it is called end-stage renal disease (or ESRD for short). These patients need kidney dialysis or a kidney transplant to survive. Dialysis is a treatment to filter out wastes and extra salt and fluid from the blood. Roxadustat (also known as FG-4592 and ASP1517) is an oral medicine for the treatment of kidney anemia.

This was a phase 1 study. These studies look at what the body does to the study medicine and what the study medicine does to the body. Phase 1 studies often involve healthy participants. These studies may also involve patients with certain health conditions. This study included healthy participants who had normal kidneys. And it included patients with kidneys that did not work well or their kidneys had stopped working (dialysis was used). The main question this study helped answer was how roxadustat was taken up, broken down, distributed through the body and removed from the body. This was looked at in healthy participants. And also in patients with kidneys that did not work well or they were on dialysis.

It was also important to find out what unwanted effects these patients and participants had from roxadustat.

The study started in December 2016 and ended in December 2017. When the study ended, the sponsor of this study (Astellas) reviewed all the study information and created a report of the results. This is a summary of that report.

What Kind of Study was This and Who Took Part in it?

This was an “open-label” study. This means that each patient or healthy participant and the study doctors knew which study medicine that person took (roxadustat).

Everyone in this study had body weight that was between 45 to 160 kg. Some participants were healthy women and men aged 40 to 75 years with kidneys that worked normally. Some patients were women and men aged 18 to 75 years with kidneys that did not work well or their kidneys had stopped working. The patients with kidneys that did not work well were able to stop medicines for their kidney for 2 weeks before the study. Patients whose kidneys had stopped working had been on dialysis for at least 4 months before the study.

During the study, the study doctor did a check-up of the patients and healthy participants at several study visits. At the first visit, they were checked to see if they could be in the study. Those who could be in the study took a single tablet of roxadustat.

This study took place at 2 clinics in 2 countries (Germany and the United Kingdom). 34 participants were in the study. 12 were healthy participants and 22 were patients with kidney disease.

	Number of Participants and Patients (out of 34)
Age Group	
Aged 64 years or younger	23
Aged 65 years or older	11
Sex	
Men	21
Women	13
Clinic Location	
European Union Countries (*at the time of the study*)	34
Germany	28
The United Kingdom	6

What Were the Study Results?

The main question this study helped answer was how roxadustat was taken up, broken down, distributed through the body and removed from the body. This was looked at in healthy participants whose kidneys worked well. And also in patients whose kidneys did not work well or were on dialysis. To do that, this study looked at the overall amount of roxadustat in the blood after a single dose. The study also looked at the time it takes for roxadustat in the blood to decline by half. And it looked at the effect of dialysis on the study medicine.

Patients with kidneys that didn't work well and patients on dialysis had double the amount of roxadustat in their blood after 1 dose, compared to healthy participants. And the amount of roxadustat in the blood took longer to decline by half as the kidneys got worse. Roxadustat declined by half in about 10 hours in healthy participant's blood. Patients whose kidneys did not work well took about 15 hours. And it was about 15 to 16 hours in patients on dialysis.

Most of the dose of roxadustat was not cleared by 4 hours of dialysis.

What Adverse Reactions did Patients Have?

A lot of research is needed to know whether a medicine causes a medical problem. So when new medicines are being studied researchers keep track of all medical problems that participants have while they are in the study. These medical problems are called "adverse events" and are recorded whether or not they might be caused by the treatment taken. An "adverse reaction" is any medical problem or "adverse event" that is judged by the study doctor to be possibly caused by a medicine or treatment used in the study.

The table below shows the adverse reactions experienced by healthy participants and patients in this study.

Adverse Reaction	**Roxadustat (out of 34)**
Any adverse reaction	11 (32.4%)
Headache or head pain	5 (14.7%)
Diarrhea	4 (11.8%)
Nausea or the urge to vomit	2 (5.9%)
Fast heartbeat	1 (2.9%)
Skin rash with the presence of macules (flat discolored area) and papules (raised bump)	1 (2.9%)
Urinary tract infection	1 (2.9%)

An adverse reaction is considered "serious" when it is life-threatening, causes lasting problems or needs hospital care. There were no patients or participants who experienced serious adverse reactions in this study.

Where Can I Learn More About This Study?

This document is a short summary of the main results from this study and reflects the information available as of August 2019. You can find this summary and more information about this study online at http://www.astellasclinicalstudyresults.com.

Please remember that researchers look at the results of many studies to find out how well medicines work and which adverse reactions they might cause. This summary only shows the results of this 1 study. Your doctor may help you understand more about the results of this study.

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