

**Research Sponsor:** Novartis

**Treatment Studied:** QAW039 (fevipiprant)

**Protocol Number:** CQAW039A2107

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## ***Thank you!***

Thank you to the participants who took part in the clinical trial for the drug QAW039, also known as fevipiprant. This drug is not yet approved for doctors to prescribe. All of the participants helped researchers learn more about how QAW039 moves through the body.

Novartis sponsored this trial and believes it is important to share the results of the trial with participants and the public. An independent non-profit organization called CISCRP prepared this summary of the trial results. We hope it helps participants understand their important role in medical research.

If you participated in the trial and have questions about the results, please speak with the trial doctor or staff at your trial site.

## **What has happened since the trial ended?**

The whole trial took just over 1 year to finish. The trial started in July 2017 and ended in August 2018.

The trial included 45 participants in Germany and the United States. After the trial ended, the sponsor reviewed the data and created a report of the results. This is a summary of that report.

## Why was the research needed?

QAW039 was being developed as a possible treatment for asthma. The purpose of this trial was to help researchers find the right dose for patients who have kidney disease. Before a trial drug can be approved for patients to take, researchers do clinical trials to find out how safe it is, and how it works.

One of the ways the body removes drugs from the blood is through the kidneys. The kidneys filter waste, some drugs, and extra water out of the blood to make urine. So, when the kidneys are not working normally, the amount of some drugs in the blood can increase. If this happens, a different dose of the drug is often needed.

Researchers know that some QAW039 leaves the body after passing through the kidneys. Because some people with asthma also have kidney disease, it is important to know how QAW039 moves through the body for people with kidney disease.

In this trial, the researchers wanted to learn if participants with different severities of kidney disease need a different dose of QAW039 compared to healthy participants without kidney disease. The most serious kidney disease is called “end stage” kidney disease. In this stage, the kidneys cannot filter the blood enough anymore. People with end stage kidney disease need their blood filtered with a medical device. This treatment is called dialysis. There are other forms of kidney disease called mild, moderate, and severe kidney disease.

The main questions the researchers wanted to answer in this trial were:

- How much QAW039 got into the blood?
- Did the severity of kidney disease affect how much QAW039 got into the blood?
- Did dialysis affect how much QAW039 got into the blood?
- How much QAW039 left the body in urine?
- What medical problems did the participants have?

To answer the questions in this trial, the researchers asked for the help of participants with end stage kidney disease who were on dialysis. The researchers also asked for help from participants with less severe kidney disease and from healthy participants with no kidney disease. The men and women in the trial were 38 to 74 years old when they joined. None of the participants had asthma.

## What kind of trial was this?

This trial was “open-label”. This means that the participants, the trial doctors, and the trial staff knew what the participants were taking.

In this trial, all the participants took QAW039.





## What happened during the trial?

**Before the treatment started**, the trial doctors did tests to make sure the participants could take part in the trial. This part happened for about 1 month before participants started treatment.

**During treatment**, the participants took QAW039 as a pill by mouth. Doses were measured in milligrams, also called mg. There were 4 treatment groups:

- Participants in Group 1 had end stage kidney disease and were on dialysis. Each participant in this group took QAW039 two times:
  - First, the participants took 1 dose of QAW039 after having dialysis.
  - Then, there was a period of at least 7 days that the participants did not take any QAW039.
  - Next, the participants took another dose of QAW039 before starting dialysis.
- Participants in all other groups took 1 dose of QAW039.

The chart below shows the treatments the participants took in this trial.

	Group 1	Group 2	Group 3	Group 4
	• 8 participants	• 8 participants	• 8 participants	• 21 participants
	• End stage kidney disease	• Moderate or severe kidney disease	• Mild kidney disease	• No kidney disease
	• 450 mg QAW039	• 450 mg QAW039	• 450 mg QAW039	• 450 mg QAW039
	• 2 times: once after dialysis and once before dialysis	• 1 time	• 1 time	• 1 time

**After treatment ended**, the participants visited their trial site 1 time about 1 week after their last dose.

Throughout the trial, the doctors:

- checked the participants' overall health
- asked the participants what medicines they were taking
- took blood and urine samples from the participants

## What were the results of the trial?

This is a summary of the overall results of your trial, not your individual results. The results presented here are for a single trial. Other trials may provide new information or different results. You should not make medical decisions based on the results of a single trial. Always talk to a doctor before making any changes to your medications or treatment plans.

More information can be found about this trial on the websites listed at the end of this summary.

### How much QAW039 got into the blood?

The researchers wanted to know how much QAW039 got into the blood after the participants took QAW039. To find out, the trial doctors took blood samples at different times before and after the participants took QAW039. The researchers studied:

- the highest level of QAW039 in the blood after participants took it
- the average level of QAW039 in the blood over 68 hours, or about 3 days, after participants took it

Knowing how much QAW039 got in the blood helps researchers decide what doses to give participants in future trials.

### Average highest level of QAW039 in the blood

The researchers measured the average highest level of QAW039 in the blood of each participant. They combined the results from each participant to find out the average highest level of QAW039 in the blood.

Overall, the researchers found that the average highest level of QAW039 in the blood was similar between all treatment groups.

### Average levels of QAW039 in the blood

The researchers measured the average levels of QAW039 in the blood of each participant. They combined the results from each participant to find out the average levels of QAW039 in the blood over 68 hours.

Overall, the researchers found that the average levels of QAW039 in the blood of the participants with mild kidney disease were similar to levels seen in participants without kidney disease.

These levels were about 2 times higher in participants with moderate, severe, or end stage kidney disease.

### **Did the severity of kidney disease affect how much QAW039 got into the blood?**

The researchers wanted to find out if the severity of the participants' kidney disease affected how much QAW039 got into their blood. To answer this question, the researchers compared the average level and average highest level of QAW039 in the blood in each treatment group.

Overall, the researchers found that the severity of kidney disease did not affect the average highest level of QAW039 in the blood.

They found that the average level of QAW039 in the blood was higher in the participants who had more severe kidney disease. Researchers planned to use the results of this trial along with the results from other trials to decide which dose is right for patients with severe kidney disease.

### **Did dialysis affect how much QAW039 got into the blood?**

Yes. The researchers wanted to find out if dialysis affected the levels of QAW039 in the blood of the participants with end stage kidney disease. To do this, the researchers measured the average levels of QAW039 in the blood when the participants took QAW039 just before starting dialysis compared to when they took QAW039 after finishing dialysis.

Overall, the researchers found that:

- In the participants with end stage kidney disease, the average level and the average highest level of QAW039 in the blood were about 1.5 times lower when the participants took QAW039 before starting dialysis.
- Researchers think this means that the dialysis was working the way a healthy kidney would work to remove QAW039 from the body.

### **How much QAW039 left the body in urine?**

The researchers wanted to find out how much QAW039 was removed by the kidneys and left the body through urine. To do this, the trial doctors collected urine for some time after the participants took QAW039.

Overall, the researchers found that the participants with more severe kidney disease had less QAW039 removed from the body through urine compared to the participants without kidney disease. The amount of QAW039 that left the body through the urine was:

- 5.7% of the dose in the participants with moderate or severe kidney disease
- 8.2% of the dose in the participants with mild kidney disease
- 14.9% of the dose in the healthy participants

When people are on dialysis, they only produce a small amount of urine. Since participants with end stage kidney disease were on dialysis, they did not have any urine samples available and the researchers were not able to learn how much QAW039 left their bodies through urine.

## What medical problems did the participants have?

Medical problems that happen in clinical trials are called “adverse events”. An adverse event is any unwanted sign or symptom that participants have during a trial. An adverse event is considered “serious” when it is life-threatening, causes lasting problems, or the participants need hospital care.

These problems may or may not be caused by the trial drug. A lot of research is needed to know whether a drug causes a medical problem. During a trial, all medical problems are reported and written down, whether or not they are caused by the trial drug. So, when new drugs are being studied, researchers keep track of all medical problems that participants have.

This section is a summary of the adverse events that happened during this trial.

### How many participants had adverse events?

In this trial, 15.6% of the total participants had adverse events. This was 7 of the 45 participants. None of the participants had serious adverse events during the trial.

None of the participants stopped getting trial treatment because of an adverse event.

The table below shows how many participants in each treatment group had adverse events during this trial.

Adverse events during this trial					
	End stage kidney disease (Out of 8 participants)	Moderate or severe kidney disease (Out of 8 participants)	Mild kidney disease (Out of 8 participants)	No kidney disease (Out of 21 participants)	Total (Out of 45 participants)
How many participants had adverse events?	0.0% (0)	12.5% (1)	25.0% (2)	19.0% (4)	15.6% (7)

## What were the most common adverse events?

The most common adverse event in this trial was headache. This happened in 6.7% of the total participants. This was 3 of the 45 participants.

The table below shows all adverse events that happened in this trial.

Most common adverse events during this trial					
	End stage kidney disease (Out of 8 participants)	Moderate or severe kidney disease (Out of 8 participants)	Mild kidney disease (Out of 8 participants)	No kidney disease (Out of 21 participants)	Total (Out of 45 participants)
Headache	0.0% (0)	0.0% (0)	12.5% (1)	9.5% (2)	6.7% (3)
Back pain	0.0% (0)	12.5% (1)	0.0% (0)	0.0% (0)	2.2% (1)
Constipation	0.0% (0)	0.0% (0)	0.0% (0)	4.8% (1)	2.2% (1)
Dry skin	0.0% (0)	0.0% (0)	12.5% (1)	0.0% (0)	2.2% (1)
Thirst	0.0% (0)	0.0% (0)	12.5% (1)	0.0% (0)	2.2% (1)
Toothache	0.0% (0)	0.0% (0)	0.0% (0)	4.8% (1)	2.2% (1)

## What was learned from this trial?

The information described above helped the researchers learn more about how QAW039 moves through the body and how safe it is in both participants with kidney disease and healthy participants with no kidney disease. The amount of QAW039 in the blood depended on the severity of the participants' kidney disease. Participants with moderate, severe and end stage kidney disease had only slightly more QAW039 in their bodies compared to participants with mild kidney disease or no kidney disease. The researchers think this happened because other parts of the body besides the kidneys can help remove QAW039 from the body. Finding the right dose of QAW039 for patients with kidney disease was the main goal of this trial.

The results presented here are for a single trial. This summary shows only the main results from this one trial in a small number of participants. While this trial completed as planned, the results of another larger trial in asthma patients did not support further development of QAW039 as a treatment for asthma. QAW039 is no longer being studied as a potential treatment for asthma.

## Where can I learn more about this trial?

More information about the results of this trial can be found in the scientific results summary available on the Novartis Clinical Trial Results website ([www.novctrd.com](http://www.novctrd.com)). Once on the site, click **“Clinical trial results and trial summary for patients”** at the top right of the page. After accepting the terms, go to the bottom left of the page and click **“Search by study number”**. Type **“CQAW039A2107”** into the keyword search box and click **“Search”**.

If you have questions about the results, please speak with the trial doctor or staff at your trial site.

You can find other information about this trial on the website listed below:

- [www.clinicaltrials.gov](http://www.clinicaltrials.gov). Once you are on the website, type **“NCT03087942”** into the **“Other terms”** search box and click **“Search”**.

If more clinical trials are planned, they will be listed on the above public website or [www.novartisclinicaltrials.com](http://www.novartisclinicaltrials.com). Search for **“QAW039”**.

**Full trial title:** An open-label, single-dose, parallel group study to assess the pharmacokinetics of fevipirant (QAW039) in patients with End-stage Renal Disease on hemodialysis and optionally in patients with severe to moderate and mild renal impairment compared to matched healthy volunteers including a cross-over assessment in End-stage Renal Disease patients on the effect of dialysis on fevipirant pharmacokinetics

## Thank you!

Clinical trial participants belong to a large community of participants around the world. They help researchers answer important health questions and test new medical treatments.



The Center for Information & Study on Clinical Research Participation (CISCRP) is a non-profit organization focused on educating and informing the public about clinical research participation.

CISCRP  
One Liberty Square, Suite 1100  
Boston, MA 02109  
1-877-MED-HERO • [www.ciscrp.org](http://www.ciscrp.org)



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1-888-669-6682 (US);  
+41613241111 (EU)  
[novartisclinicaltrials.com](http://novartisclinicaltrials.com)