Clinical Study Results

Study Name

Title of the study:	An Open-label, Single-dose Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of Etelcalcetide (AMG 416) in Paediatric Subjects Aged 2 to less than 18 Years with Secondary Hyperparathyroidism (HPT) Receiving Maintenance Haemodialysis
Brief Title:	A Single-dose Study in Paediatric Patients Aged 2 to Less Than 18 Years with Secondary Hyperparathyroidism (sHPT) Receiving Haemodialysis
Protocol Number:	20140336
EU Trial Number	2015-005051-28
Other Identifiers	NCT02833857
Date of This Summary	03 December 2019

What does this summary cover?

This summary shows the main results from one clinical study. The results are only for this study. Other studies may find different results. Researchers and health authorities look at the results of many studies to decide which medicines work best and are safest for patients.

1. Who Sponsored This Study?

Amgen Inc.
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Amgen Inc. is the sponsor of the study who manufactured etelcalcetide, the investigational medicine included in the study. Amgen would like to thank everyone who participated in this study and feels it is important to share the results of this study.

2. General Information About the Clinical Trial

Where and when was the study done?

- This study took place in Belgium, Germany, United Kingdom, and United States.
- The study began in March 2017 and ended in October 2018.
- The study was completed as planned.

Why was the study done?

Chronic kidney disease (also called “CKD”) is a serious health problem where the kidneys are diseased or damaged. The kidneys are not able to control the amount of water flowing through the body or filter waste out of the blood like they should. CKD will continue to get worse over time. Once the kidneys fail, treatment includes hemodialysis. Hemodialysis uses a machine to filter the blood like healthy kidneys would. About 2 million people around the world need hemodialysis.

Patients with CKD often have a problem called secondary hyperparathyroidism (also called “SHPT”). SHPT is caused by too much of a certain hormone (called parathyroid hormone or “PTH”). PTH controls how much calcium is in the blood. Calcium is an important mineral our body needs for a healthy heart, bones, nervous system, and hormone control. PTH causes minerals, like calcium, to leave the bones and enter the blood. About 88% of hemodialysis patients will get SHPT.

Standard care for patients with SHPT may include vitamin D and medicines that remove extra salts and minerals from the blood if there is too much of it. Etelcalcetide is a medicine that is approved by the health agencies in some countries to treat SHPT in adults with CKD on hemodialysis. Etelcalcetide has not been tested or approved for children or teenagers with SHPT. When a medication is used in a study like this one, it is called an “investigational medicine.”

This was a phase 1 study conducted in participants with SHPT as part of the early process to develop new medicines for children and teenagers. Researchers looked at how this investigational medicine works in the body and the impact that it has in the body, including side effects. This study did not test if etelcalcetide improves health.

This was called an “open-label” study. It was “open-label” because all of the study doctors and participants knew the participants were taking etelcalcetide.

The main goal of this study was for researchers to learn more about the safety of etelcalcetide in children and teenagers with SHPT.

3. Who Was Included in This Study?

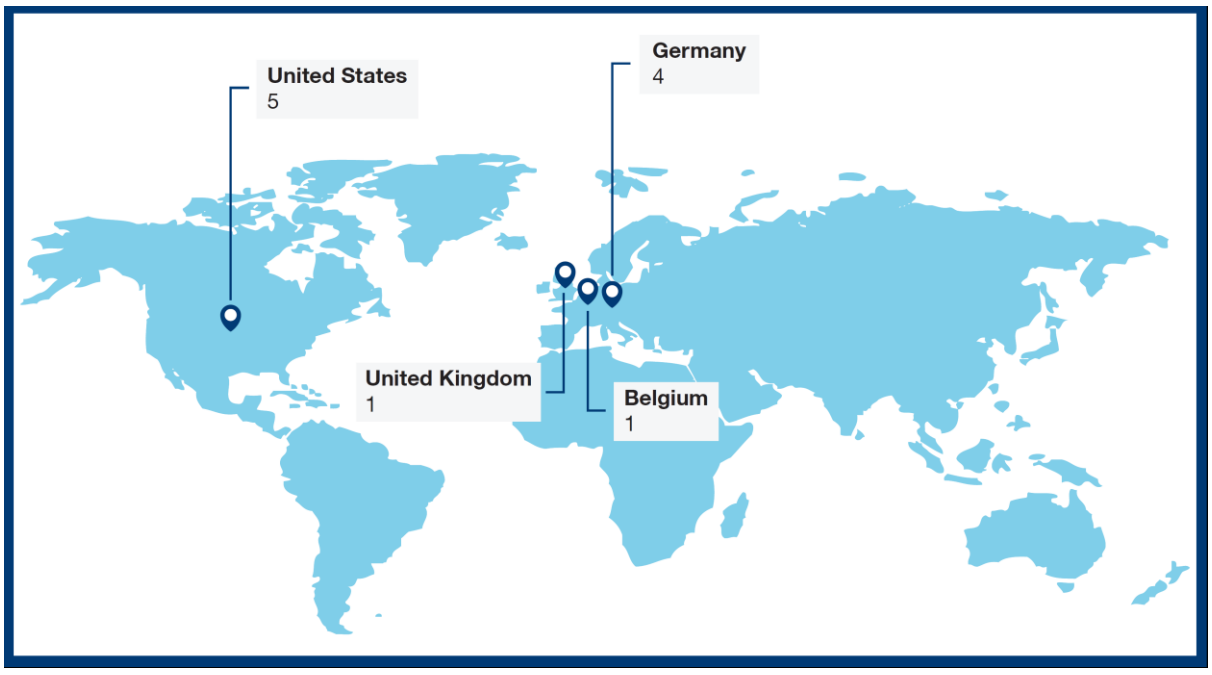
Who took part in the study?

This study included 11 children and teenagers with SHPT. Five (5) participants (46%) were boys and 6 participants (54%) were girls. They ranged in age from 3 to 15 years. Five (5) participants (46%) were younger than 12 years old. Six (6) participants (54%) were 12 years or older.

Participants were examined by a study doctor and chosen to be in the study if they:

- Had CKD and SHPT
- Were receiving hemodialysis for more than 30 days before the study started
- Were at least 2 years old, but younger than 18 years old

This study took place at 6 study centers across Belgium, Germany, United Kingdom, and United States. The numbers of participants in each country are shown below.



4. Which Medicines Were Studied?

All participants in this study received 1 dose of etelcalcetide. On day 1 of the study, the participants were given the etelcalcetide at the end of a hemodialysis treatment. The dose was 0.035 milligrams per kilogram of weight (mg/kg) and was injected into the participant's vein.

Over the next 30 days, participants visited their study doctors to see how they were doing after taking etelcalcetide. The study doctors also collected blood samples to learn more about what happens to etelcalcetide in the body.

5. What Were the Side Effects?

What is an adverse reaction (sometimes called a side effect)?

A lot of research is needed to know whether a medicine causes a side effect. All medicines can cause side effects, or unwanted medical problems that may happen when you take a medicine. In a clinical study, the study doctors record all unwanted medical problems that occur during the study including side effects that they believe are possibly caused by the investigational medicine each participant is receiving. These are also called “adverse reactions.”

What side effects related to the investigational medicine were seen?

A side effect was recorded as “serious” if it caused death, was life threatening, required the participant to stay in a hospital, or the study doctor thought it was clinically important enough to record as “serious”.

The table below shows how many participants had side effects that were considered related to treatment.

Treatment-Related Side Effects During the Study

	2 to 11 Years Old (5 participants)	12 to 17 Years Old (6 participants)
How many participants had serious side effects?	0 participants (0%)	0 participants (0%)
How many participants had non-serious side effects?	1 participant (20%)	1 participant (17%)
How many participants died from side effects?	0 participants (0%)	0 participants (0%)
How many participants stopped taking the study medicine because of side effects?	0 participants (0%)	0 participants (0%)

The table below shows the non-serious treatment-related side effects considered by the study doctor as related to treatment. In the 2 to 11 year old age group, 1 participant reported all 3 side effects.

Non-Serious Treatment-Related Side Effects During the Study

Non-serious side effect	2 to 11 Years Old (5 participants)	12 to 17 Years Old (6 participants)
Low levels of free calcium in the blood	0 participants (0%)	1 participant (17%)
Tingling or prickling "pins and needles" pain	1 participant (20%)	0 participants (0%)
Headache	1 participant (20%)	0 participants (0%)
Vomiting	1 participant (20%)	0 participants (0%)

This section only shows the side effects considered by the study doctor as related to treatment. No single clinical study can give a complete picture of the benefits and risks of a medicine. Information about other side effects may be available at the websites listed at the end of this summary.

6. What Were the Overall Results of the Study?

What other unwanted medical problems did participants have during the study?

A main goal of this study was to learn more about the safety of etelcalcetide in children and teenagers.

To learn more about safety, the researchers recorded all unwanted medical problems that happened during the study, including those that were not considered by the study doctor as related to treatment.

The table below shows the number of participants with unwanted medical problems during this study.

Participants With Unwanted Medical Problems During the Study

	2 to 11 years old (5 participants)	12 to 17 years old (6 participants)
How many participants had unwanted medical problems?	4 participants (80%)	2 participants (33%)

The table below shows all unwanted medical problems that happened during the study. Some participants had more than 1 unwanted medical problem.

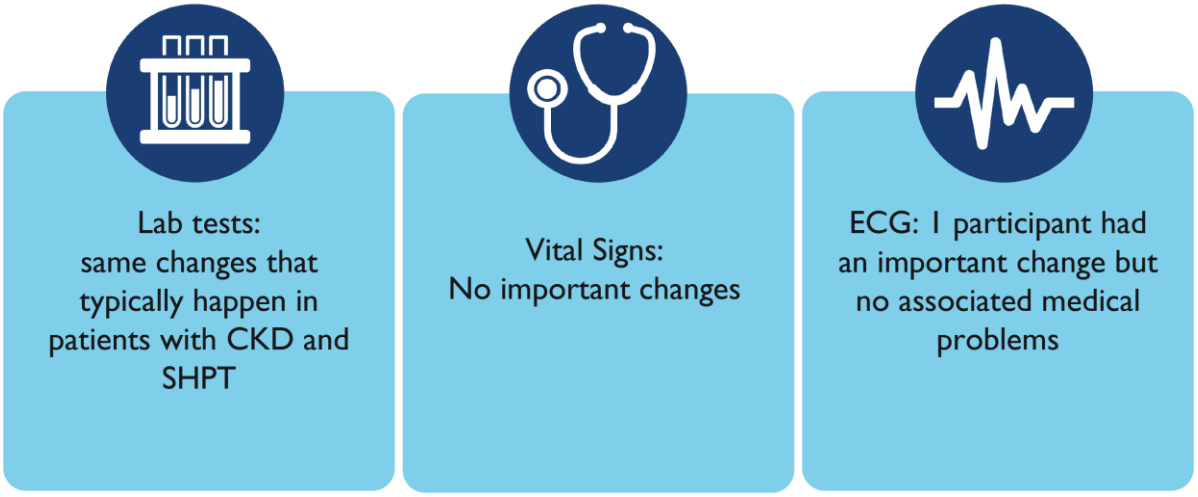
Unwanted Medical Problems During the Study

Unwanted Medical Problem	2 to 11 years old (5 participants)	12 to 17 years old (6 participants)
Headache	2 participants (40%)	0 participants (0%)
Feeling dizzy	0 participants (0%)	1 participant (17%)
Tingling or prickling "pins and needles" pain	1 participant (20%)	0 participants (0%)
Nausea	1 participant (20%)	0 participants (0%)
Vomiting	1 participant (20%)	0 participants (0%)
Low levels of free calcium in the blood	0 participants (0%)	2 participants (33%)
Needed a medical device called a catheter placed	0 participants (0%)	1 participant (17%)
Needed a surgery to have a feeding tube placed in the stomach	1 participant (20%)	0 participants (0%)
Low blood pressure	1 participant (20%)	1 participant (17%)

When participants came to study visits, their doctors did certain lab tests. The researchers looked for changes in these lab tests to learn more about the safety of etelcalcetide. The lab test changes that happened during this study were the same changes that typically happen in patients with CKD and SHPT.

At study visits, doctors measured the participants' vital signs, including body weight, body temperature, heart rate, and blood pressure. They also did a test called an electrocardiogram, or ECG, which measures the activity of the heart. The researchers looked for changes in vital signs and ECGs to learn more about the safety of etelcalcetide.

No important changes in vital signs happened during this study. One participant had an important change in ECG results, but did not have any medical problems associated with this change.



This study was completed as planned. All 11 participants completed treatment.

More results may be available at the websites listed at the end of this summary.

7. How Has This Study Helped Participants and Researchers?

What is important to know about these results?

These results are only for this clinical study, which looked at a sample of 11 children and teenagers with SHPT due to CKD. Not all participants in the study had the same results. The results for any individual participant could have been better or worse than the results for their group. Other studies may find different results. These results are not an explanation of what a treatment can and cannot do for an individual. Many studies are needed to show the benefits and risks of an investigational medicine.

This research may help patients and families in the future by helping doctors understand more about the safety of etelcalcetide in children and teenagers.

8. Are There Plans for Further Studies?

Additional studies with etelcalcetide in children and teenagers are ongoing.

If more clinical studies are done, they may be listed on public websites, such as those below. Search for study medicine name “etelcalcetide”, “Parsabiv”, or “AMG 416” on the websites below.

9. Where Can I Find More Information About This Study?

Amgen has committed to make research results available to the public. This summary has been provided as part of that commitment and should not be used for any other purpose. It should not be considered to make a claim for any product or to guide treatment decisions.

Some information in this summary may be different from the approved labeling for etelcalcetide. Your healthcare professional should refer to the full prescribing information for proper use of etelcalcetide.

To find out more about this study, check these websites:

- www.clinicaltrials.gov. Use the study identifier NCT02833857
- www.clinicaltrialsregister.eu. Use the study identifier 2015-005051-28

If you participated in the study and have questions about the study results, the doctor or staff at your study site may be able to answer them.