Clinical Study Results

This summary reports the results of only one study. Researchers must look at the results of many types of studies to understand if a study medication works, how it works, and if it is safe to prescribe to patients. The results of this study might be different than the results of other studies that the researchers review.

Sponsor:	Pfizer Inc.
Medicine Studied:	Recifercept (PF-07256472)
Protocol Number:	C4181008
Dates of Study:	24 December 2021 to 30 March 2023
Title of this Study:	A Study to Learn About The Long-Term Safety of Reciferceipt and How Well it Works in Children With Achondroplasia [A Phase 2 Open Label Extension Study to Assess the Long-Term Safety, Tolerability, Pharmacokinetics and Efficacy of Recifercept in Children With Achondroplasia]
Date of this Report:	04 December 2023

– Thank You –

If you or your child participated in this study, Pfizer, the Sponsor, would like to thank you for your participation.

This summary will describe the study results. If you or your child have any questions about the study or the results, please contact the doctor or staff at your study site.

Why was this study done?

What is achondroplasia?

Achondroplasia is a rare genetic disorder that causes problems with how children's bones and joints grow.

People with achondroplasia can have short arms and legs and other bone complications. Achondroplasia is the most common type of bone disorder seen in children.

What is recifercept?

Recifercept is an injectable medicine that was tested in this study for the treatment of achondroplasia. The use of recifercept in this study is investigational, which means it is not approved for the treatment of achondroplasia.

Recifercept is designed to help boost growth in children.

What was the purpose of this study?

Before this study, recifercept was studied in children with achondroplasia that were aged 3 months to below 11 years old. The study was referred to as Study C4181005.

The purpose of this study was to learn if recifercept was safe in children with achondroplasia when taken for a long period of time. This study included children aged 15 months to 12 years who have participated in Study C4181005.

Researchers wanted to know:

- **What medical problems did participants have during the study?**

What happened during the study?

How was the study done?

Researchers tested recifercept on a group of participants aged 15 months to 12 years old who completed the Study C4181005.

Researchers wanted to learn about any medical problems participants had during this study.

Participants received 1 of the 3 doses of recifercept as a subcutaneous (SC) injection given under the skin up to 24 months or until a tested dose was identified:

- Low dose: 1 mg/kg once a week
- Medium dose: 2 mg/kg two times a week
- High dose: 1.5 mg/kg once in a day

The dose of recifercept was given based on the child's weight.

This was an open-label study. This means that the children or their parents/caregivers and the researchers knew the treatment being given.

Figure 1 below shows what happened during the study.

Screening	Treatment	Follow-up
 Children with achondroplasia aged 15 months to 12 years old who completed the earlier C4181005 Study entered into this study.	 Participants continued their assigned dose from the earlier study. Participants continued to receive 1 of 3 doses: • **Low dose:** 1 mg/kg once a week • **Medium dose:** 2 mg/kg 2 times a week • **High dose:** 1.5 mg/kg once in a day	 **28 to 35 days after the last dose** Study doctors or team called the participants' parents/caregivers to ask how the participants were feeling and medications they were taking.

Researchers also planned to check the long-term effect of recifercept on growth, but it was not done because the Sponsor stopped the study early.

Where did this study take place?

The Sponsor took place at 10 locations in 7 countries.

When did this study take place?

The study started on 24 December 2021 and ended on 30 March 2023.

Who participated in this study?

The study included children with achondroplasia aged 15 months to 12 years old who have completed Study C4181005.

A total of 35 participants started the study. Of these:

- 15 were boys and 20 were girls.
- All were between the ages of 3 years and 12 years.

All 35 participants who started the study did not finish the treatment period because:

- 4 participants left before the study was by their parents/caretakers choice.

- 31 participants did not finish the study over as the Sponsor decided to stop the study.

How long did the study last?

Study participants were in the study for about 12 months. The entire study took about 15 months to complete.

The Sponsor decided to end the study earlier than planned in November 2022. This was because the results from Study C4181005 did not show the desired change in height compared to the those seen in children who had not received any treatment for achondroplasia from another study. Study C4181005 was not stopped due to any safety concerns with recifercept.

When the study ended in March 2023, the Sponsor began reviewing the information collected. The Sponsor then created a report of the results. This is a summary of that report.

What were the results of the study?

What medical problems did participants have during the study?

The researchers recorded any medical problems the participants had during the study. Participants could have had medical problems for reasons not related to the study (for example, caused by an underlying disease or by chance). Or, medical problems could also have been caused by a study treatment or by another medicine the participant was taking. Sometimes the cause of a medical problem is unknown. By comparing medical problems across many treatment groups in many studies, doctors try to understand what effects a study medication might have on a participant.

Of the 35 participants, 20 participants (57%) in this study had at least 1 medical problem.

- 9 out of 16 participants (56%) in the low dose recifercept group
- 11 out of 17 participants (65%) in the medium dose recifercept group
- None of the participants (0%) in the high dose recifercept group

No participant left the study because of a medical problem they had during the study.

Below are instructions on how to read Table 1.

Instructions for Understanding Table 1.

- The **1st** column of Table 1 lists medical problems that were commonly during the study. All medical problems reported by 3 or more participants in any group are listed.
- The **2nd** column tells how many of the 16 participants who were given the low dose of recifercept reported each medical problem. Next to this number is the percentage of the 16 participants who had the medical problem.
- The **3rd** column tells how many of the 17 participants who were given the medium dose of recifercept reported each medical problem. Next to this number is the percentage of the 17 participants who had the medical problem.
- Using these instructions, you can see that,
  - 3 out of the 16 participants (19%) who were given the low dose of recifercept reported COVID-19.

- 4 out of the 17 participants (24%) taking the medium dose recifercept reported inflammation of nose.

The most common medical problems – those seen in 3 or more participants in any group – are described in Table 1 below.

Table 1. Commonly reported medical problems (reported by 3 or more participants)

Medical Problem	Recifercept 1 mg/kg (16 Participants)	Recifercept 2 mg/kg (17 Participants)
COVID-19	3 out of 16 participants (19%)	0 out of 17 participants (0%)
Cough	3 out of 16 participants (19%)	0 out of 17 participants (0%)
Infection of nose and throat	3 out of 16 participants (19%)	1 out of 17 participants (6%)
Inflammation of the nose	1 out of 16 participants (6%)	4 out of 17 participants (24%)

Did study participants have any serious medical problems?

A medical problem is considered “serious” when it is life-threatening, needs hospital care, or causes lasting problems.

No participant had serious medical problems or died during the study.

Where can I learn more about this study?

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

For more details on your study protocol, please visit:

www.pfizer.com/research/research_clinical_trials/trial_results	Use the protocol number **C4181008**

The full scientific report of this study is available online at:

www.clinicaltrials.gov	Use the study identifier **NCT05116046**
www.clinicaltrialsregister.eu	Use the study identifier **2021-003149-39**

Please remember that researchers look at the results of many studies to find out which medicines can work and are safe for patients.

Again, if you and your child participated in this study, **thank you** for volunteering.
We do research to try to find the best ways to help patients, and you helped us to do that!