CLINICAL TRIAL 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 medicine 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(s) Studied: Somatrogon (PF-06836922)

Protocol Number: C0311002

Dates of Trial: 7 February 2019 to 28 August 2020

Title of this Trial: A Phase 3, Randomized, Multicenter, Open-Label, Crossover Study Assessing Subject Perception of Treatment Burden With Use of Weekly Growth Hormone (Somatrogon) Versus Daily Growth Hormone (Genotropin®) Injections in Children With Growth Hormone Deficiency

[Patient Perception of Treatment Burden in Weekly Versus Daily Growth Hormone Injections in Children With GHD]

Date of this Report: 02 March 2021

– Thank You –

Pfizer, the Sponsor, would like to thank you and your child for participating in this clinical trial and provide you a summary of results representing everyone who participated. If you have any questions about the study or results, please contact the doctor or staff at your child's study site.

WHY WAS THIS STUDY DONE?

Growth hormone is a substance that is needed for normal growth. It is made by a small gland in the brain called the "pituitary gland". Some children have a condition called "growth hormone deficiency", which means that their pituitary gland is not making enough growth hormone. Children who are not treated for this condition may not grow and be as tall as other children of the same age.

Doctors may prescribe a medicine called Genotropin® (somatropin) that helps children with growth hormone deficiency to grow. Genotropin is known as a "recombinant human growth hormone", which means that it is identical to the natural human growth hormone, but is made by scientists to be used as a medicine. This treatment involves injections with a small needle under the skin, given each day.

Researchers have developed a recombinant human growth hormone called somatrogon. It is given once per week instead of each day. The purpose of this study was to learn more about the use of somatrogon in children with growth hormone deficiency. Researchers wanted to know:

- How much did a weekly treatment schedule with somatrogon interfere with the patient's life, compared to a daily treatment schedule with Genotropin?

The study also looked at the overall safety of somatrogon in children with growth hormone deficiency.

WHAT HAPPENED DURING THE STUDY?

This study compared the "treatment burden" (interference in child's life) of a weekly treatment schedule with somatrogon, compared to a daily treatment schedule with Genotropin. The study included children between the ages of 3 and 18 years. All children in the study had been diagnosed with growth hormone deficiency, had low levels of growth hormone, and were currently receiving daily treatment with Genotropin for at least 3 months.

Children were evaluated by the study doctor to make sure they met the criteria to participate in the study. This was known as the "screening period", which lasted up to 30 days. Next, eligible children were assigned to 1 of 2 treatment groups:

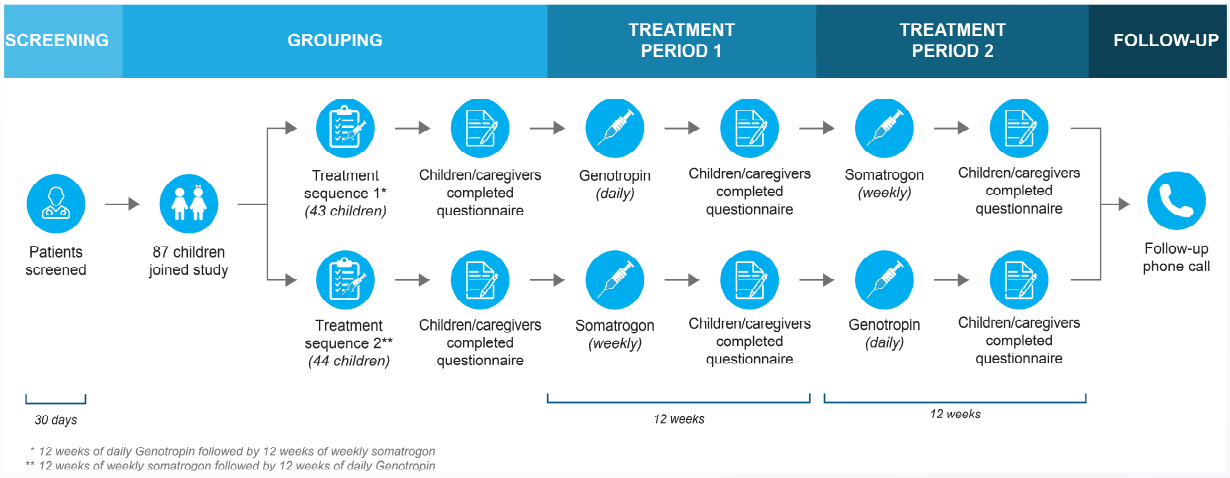
- Group A (43 children): 12 weeks of daily treatment with Genotropin, followed by 12 weeks of weekly treatment with somatrogon
- Group B (44 children): 12 weeks of weekly treatment with somatrogon, followed by 12 weeks of daily treatment with Genotropin

Children were assigned to each group by chance. Putting people into groups by chance is called randomization. This helps make it more likely that the groups will be more even to compare.

The children, their parents, and the study doctors knew who took somatrogon and who took Genotropin during the study. This is known as an "open-label" study.

Study treatments were given at home as injections with a small needle under the skin. Children were expected to participate in 7 study visits (over the phone or in person). At the beginning of the study and after each 12-week treatment period, the children and their parents/caregivers were asked to complete a questionnaire about the treatment burden of Genotropin or somatrogon. A follow-up phone visit was done 4 weeks after the last clinic visit.

The figure below shows what happened during this study.



Children were to be treated for 24 weeks. The entire study took about 1 ½ years to complete. The sponsor ran this study at 29 locations in 5 countries in Europe and the United States. It began on 7 February 2019 and ended on 28 August 2020. 72 boys (83%) and 15 girls (17%) joined the study. Children who joined the study were 3 to 17 years old.

Of the 87 children who started the study and received study treatment, 85 children (98%) completed the main part of the study. 2 children (2%) did not complete the study because of a medical problem or because they no longer met the requirements to participate in the study.

In August 2020, 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?

How much did a weekly treatment schedule with somatrogon interfere with the patient's life, compared to a daily treatment schedule with Genotropin?

To answer this question, the researchers used a questionnaire to assess the "treatment burden" (or how much the treatment interfered with the child's life) of weekly somatrogon or daily Genotropin. The children and their parents/caregivers were asked to complete the questionnaire at the beginning of the study, after the 12-week treatment period with somatrogon, and after the 12-week treatment period with Genotropin.

In this study, the children and their parents/caregivers reported that weekly somatrogon interfered less with their lives, compared to daily Genotropin. Based on these results, the researchers have decided that the results are not likely the result of chance. Weekly somatrogon may have a lower treatment burden than daily Genotropin.

This does not mean that everyone in this study had these results. Other studies may produce different results, as well. These are just some of the main findings of the study, and more information may be available at the websites listed at the end of this summary.

WHAT MEDICAL PROBLEMS DID CHILDREN HAVE DURING THE STUDY?

The researchers recorded any medical problems the children had during the study. Children 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 child 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 the side effects of an experimental drug might be.

58 out of 87 children (67%) in this study had at least 1 medical problem. 1 child (1%) left the study because of medical problems. The medical problems reported in at least 5% of children during either treatment are listed in the table below. The table shows the number and percent of children who experienced these medical problems during each treatment.

Most Common Medical Problems (Reported in At Least 5% of Children During Either Treatment)

Medical Problem	During Genotropin (86 Children Treated)	During Somatrogon (87 Children Treated)
Pain at injection site	11 (13%)	13 (15%)
Bruise at injection site	8 (9%)	4 (5%)
Common cold	5 (6%)	6 (7%)
Headache	5 (6%)	6 (7%)

WERE THERE ANY SERIOUS MEDICAL PROBLEMS?

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

No children in this study had serious medical problems, and no children died during this study.

WHERE CAN I LEARN MORE ABOUT THIS STUDY?

If you have questions about the results of your child's study, please speak with the doctor or staff at your child's study site. For more details on this study protocol, please visit:

www.clinicaltrials.gov	Use the study identifier **NCT03831880**
www.clinicaltrialsregister.eu	Use the study identifier **2018-000918-38**

Please remember that researchers look at the results of many studies to find out which medicines can work and are safe for patients. Additional studies with somatrogon in children with growth hormone deficiency are ongoing.

Again, **thank you** for volunteering. We do research to try to find the best ways to help patients, and you helped us to do that!