A clinical study of sotatercept in children with pulmonary arterial hypertension (MK-7962-008)

Protocol Title: A Phase 2 Open-label Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of Sotatercept (MK-7962) in Children from 1 to Less Than 18 Years of Age With PAH on Standard of Care

Why is this study needed?

Researchers are looking for other ways to treat children with **pulmonary arterial hypertension (PAH)**. In PAH, the blood vessels in the lungs become thick and narrow which makes it harder for blood to flow. This causes high blood pressure in the lungs and overworks the heart. PAH can make it hard to breathe and be active.

The **standard treatment** for children with PAH includes one or multiple medicines. However, these may not fully work or treat the symptoms of PAH in some children. **Sotatercept** is a study medicine designed to treat PAH.

The goal of this study is to learn about the safety of sotatercept and how well children tolerate it. Researchers also want to learn what happens to sotatercept in a child's body.

Who will take part in this study?

About 42 children with PAH will be in this study. They will be between ages 1 to less than 18 years old when they join the study, and:

- Take standard treatments for PAH
- Have had a test to see how well the heart is pumping (called a right heart catheterization)
- Do not have certain types of heart disease or other certain health conditions

What treatments are being given during the study?

All the children in this study will receive **sotatercept** under the skin as a subcutaneous (SC) injection once every 3 weeks for 6 months. All the children will receive sotatercept while they continue their standard treatment for PAH.

Children who complete 6 months of treatment will also have the option to continue receiving sotatercept for either up to 6 years or until they reach 18 years of age.

How is this study designed?

Children will be assigned to one of these groups based on their age:

- **Group 1**: 12 years to less than 18 years
- **Group 2**: 6 years to less than 12 years
- **Group 3**: 2 years to less than 6 years
- **Group 4**: 1 year to less than 2 years

Researchers will start by giving sotatercept to children in Group 1 (the oldest group). Researchers will check for any safety concerns and look at the level of sotatercept in the body over time before giving sotatercept to the next age group. This will also help researchers adjust the dose of sotatercept if needed.

Both the children in the study and the researcher will know which study treatment a child is getting since all children receive sotatercept (open-label study).

During the study, children will give urine and blood samples, have an imaging test that sees how well the heart works (called an **echocardiogram**), have physical examinations, and answer questions about how they are feeling and their ability to carry out daily tasks. Some children who are able may have a test to measure how far they can walk in 6 minutes and other imaging tests.

A child may be in this study for up to about 7 years.

What are the goals of this study and how will they be measured?

Main goals	How they will be measured
To learn about the **safety** of sotatercept and how well children **tolerate** it over 6 months	The number of children who: • Have an **adverse event (AE)—**an AE is a health problem that happens or worsens during the study • Stop treatment due to an AE
	During the study, researchers will also measure: • The number of red blood cells (the cells that carry oxygen in the body) • The number of platelets (the cells that help blood to clot) • Blood pressure • Immunogenicity (if a treatment triggers an immune system response against the treatment)
To learn what happens to different doses of sotatercept in a child's body over 6 months	During the study, researchers will measure the amount of sotatercept in children's blood samples at different time points

Other goals	How they will be measured
To learn about the **effects** of sotatercept on PAH over 6 months	During the study, researchers will measure changes in: • Echocardiogram images • **Quality of life** measured by children's answers to sets of questions, including questions about their overall health and ability to carry out daily tasks • The level of **NT-proBNP** in the blood—**NT-proBNP** is a protein that the heart makes when it is strained or damaged • **WHO functional class (FC) system**—**WHO FC** is a system to classify how severe a person's PAH is
	For children in **Groups 1 and 2**, researchers will also measure changes in: • How far they can walk in 6 minutes (**6-minute walking test**) • **Cardiac MRI images** for certain children**—**a **cardiac MRI** is a specialized imaging test that takes pictures of the heart

What are the possible benefits and risks?

Children may or may not benefit from the treatment received during the study. This study has an external group of experts that will oversee the overall risk and benefit. If this group of experts decides that the study treatment is not safe or does not show benefit, the study can be stopped.

More information about the benefits and risks is in the protocol.