Pulmonary Arterial Hypertension 2024

ICER SNAPSHOT

Reviewed for accuracy by: The Pulmonary Hypertension Association

The ICER Snapshot is a summary designed to help patients and the broader community learn about the key results and recommendations from ICER's 2024 Final Evidence Report on sotatercept for pulmonary arterial hypertension (PAH).

The information included is up to date as of January 2024. New information about these therapies may become available, but is not captured here.

Let's Take a Look

What is Pulmonary Arterial Hypertension?

Impact on Patients and Families

Treatments: Benefits and Risks

Treatments: What's A Fair Price?

Policy Recommendations & Impact of Engagement

What is Pulmonary Arterial Hypertension?

Pulmonary arterial hypertension (PAH) is a rare illness that makes the blood vessels in the lungs narrower and hard for blood to pass through. This puts more pressure on the heart and makes it difficult to deliver oxygen from the lungs. Symptoms can include shortness of breath, fatigue, chest pain, dizziness, and fainting. Unfortunately, these symptoms can get worse over time and can significantly reduce how long a person lives.

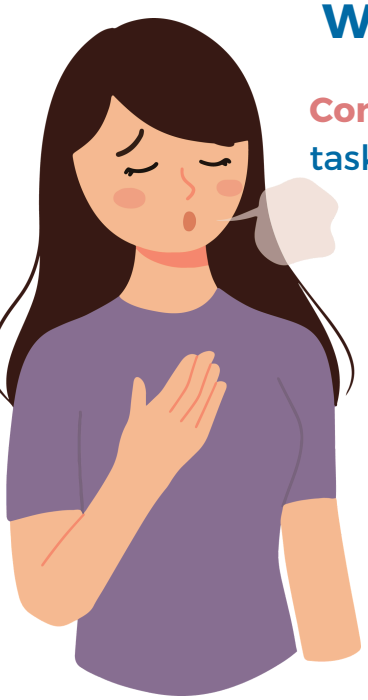


PAH affects more than 50,000 people in the United States. It usually occurs between the ages of 30 and 60 and is more common in women than men. People with PAH say it has a big impact on their lives because of the symptoms they experience, the high medical expenses, and the fear of dying young. An important treatment goal for PAH is to improve physical activity and reduce symptoms when being physically active.

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Impact on Patients and Families

What ICER Learned from the Community



Constant shortness of breath and fatigue can limit doing daily living tasks such as exercise, chores, hobbies, and attending social events.

Fatigue was reported as having the biggest impact on quality of life.

Partners and children may need to alter their daily lives to accommodate the limitations of **frequent symptoms or hospitalizations**.

Symptoms can lead to **substantial life changes** including the need to stop working, move to lower altitude, change family plans, and rely on others for help.

Currently available therapies are burdensome (daily pills and/or intravenous (IV) infusions) to manage in terms of administration and side effects, and create **significant financial and access-related burdens.**

Treatment of Focus & Clinical Context

SOTATERCEPT

Sotatercept is under FDA review as of January 2024.

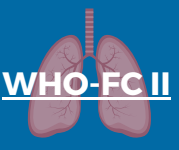
Developed by Merck & Co, Inc., sotatercept is a therapy that patients receive via injection under the skin every three weeks. It works in a new way compared to existing treatments and may improve blood flow and decrease pressure in the pulmonary arteries.

FUNCTIONAL CLASS

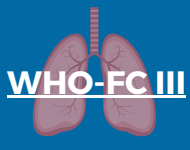
The World Health Organization (WHO) Functional Classification (FC) System, or **WHO-FC I through IV**, offers a way to group PAH patients based on their symptoms and disease progression.



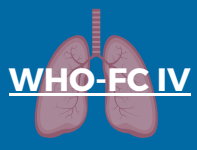
WHO-FC I — No symptoms



WHO-FC II — No symptoms at rest
Some discomfort with normal tasks



WHO-FC III — No symptoms at rest
Lots of discomfort with normal tasks



WHO-FC IV — Symptoms at rest
Symptoms get worse with normal tasks

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What Did Clinical Trials Show?

Sotatercept...

- **IMPROVED** physical activity & related symptoms
- **REDUCED** pressure in the arteries & signs of heart failure
- **REDUCED** risk of clinical worsening (hospitalizations, rescue therapy)

Based on...

The STELLAR Trial

323 patients who had WHO-FC II or III PAH for about 9 years and were on stable background therapy (1 to 3 therapies)

These represent some, but not all outcomes that were measured in the clinical trials.

Safety of Sotatercept

In the trial, patients treated with sotatercept had fewer serious or severe negative effects, and fewer effects that caused people to stop taking the drug, than patients on placebo. The side effects from sotatercept were mainly bleeding episodes, like minor nosebleeds and gum bleeds. There were also risks of small, widened blood vessels on the skin and a slight increase in hemoglobin levels.

ICER's report findings are NOT recommendations that support the use of sotatercept. Patients and families should always talk with their doctors to make shared decisions about treatment for PAH.

What We Still Don't Know

- How well sotatercept works in children and patients who were either newly diagnosed or have more severe PAH symptoms
- How well sotatercept works in patients with PAH related to connective tissue disease and other subgroups
- How safe and effective sotatercept is in the long-term
- If sotatercept can reduce the risk of dying
- Whether sotatercept can be given as the first drug of treatment in PAH

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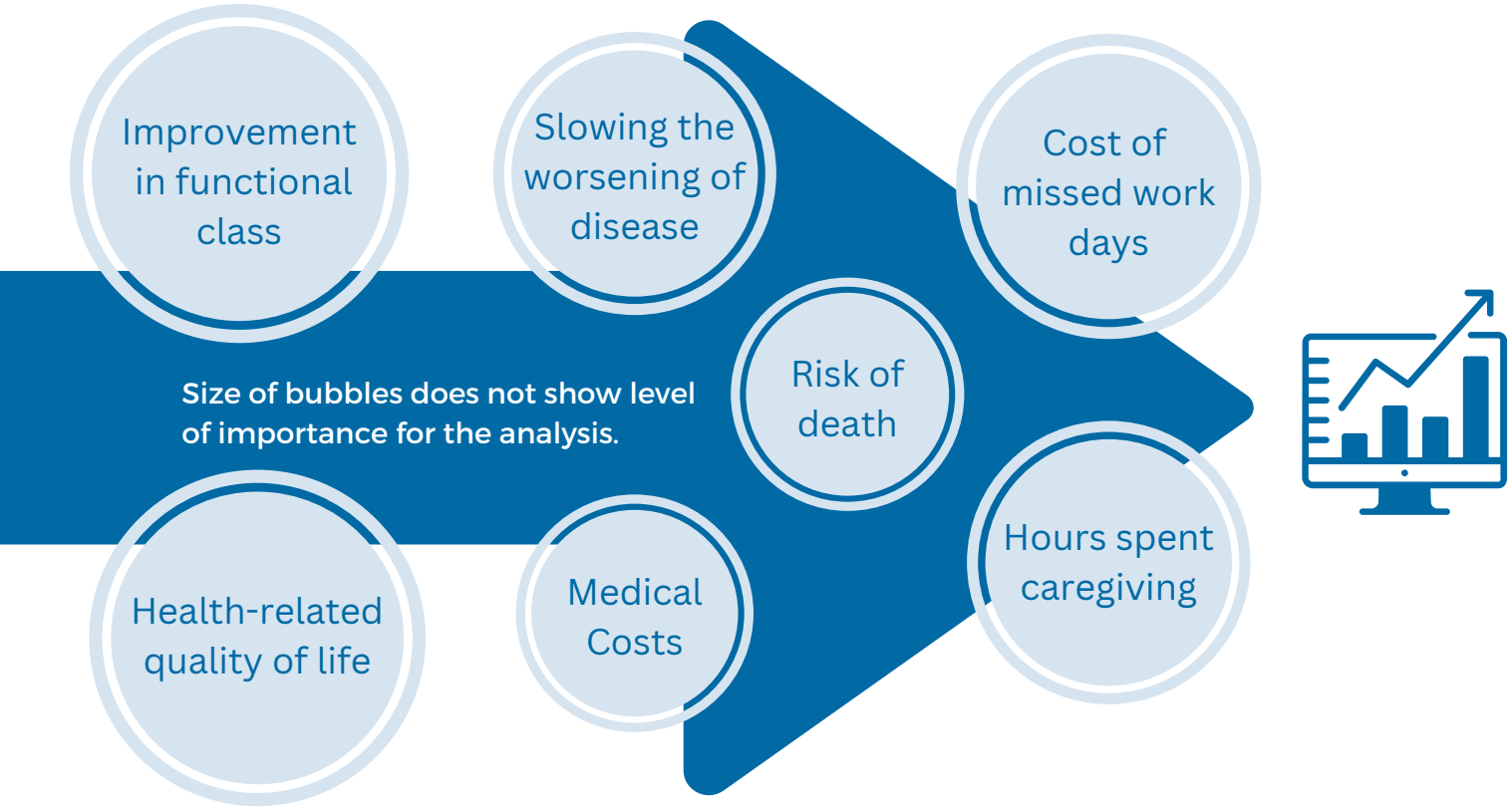
How Did ICER Calculate a Fair Price?

Using economic modeling, we calculated the cost-effectiveness of sotatercept based on improvement in functional class, compared to usual care. See below for what types of information ICER considered to calculate a fair price range for this treatement.

Population

Adults with WHO-FC II and III PAH who are on stable background therapy at baseline.

Factors Included in ICER's Economic Analysis



Fair Price Range for Sotatercept

$17,900 to $35,400*

A fair price is how much a treatment should cost based on how well it works for patients. Our economic analysis concluded that the fair-price range for sotatercept is between $17,900 to $35,400.

*Medical costs, missed work days, and hours spent caregiving were not included in calculating this fair price range. See full report for how these inputs were factored into other economic results.

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Key Policy Recommendations

The Policy Roundtable at the ICER public meeting informed several policy recommendations for pricing, access, guidelines, and future research in PAH. A few key recommendations are summarized below.

1 Patient groups can continue to advocate for better oxygen access while payers should change how they cover supplemental oxygen.

Currently, more expensive forms of oxygen, which allow patients with PAH improved mobility and a better quality of life, are not easily accessible. Payers should update their policies and practices to promote easier access to supplemental oxygen. Patient groups should also advocate for access to liquid oxygen when medically necessary.

2 Manufacturers and payers (public and private health insurance plans) should choose a price for sotatercept and design insurance policies that result in affordable access for all patients.

Insurance coverage policies should not create requirements for out-of-pocket spending that create major barriers to appropriate access for patients. High costs of new therapies may also worsen disparities in accessing treatment. Current treatments are already expensive, in part due to increased use of co-insurance, copay accumulator programs, and high deductible health plans.

3 Payers (public and private health insurance plans) should provide wraparound insurance coverage to ensure equal access to treatment.

Given that many patients with PAH will need to travel to obtain appropriate diagnosis and treatment, payers should consider including transportation costs under insurance coverage. All patients should have the fundamental right to fair access that is not undermined by geographical or income barriers.

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4 Manufacturers should work with payers, specialty pharmacies, and clinicians to rapidly transition sotatercept to home-based administration.

Since access to PAH specialty centers may be limited, home-based administration of sotatercept could help decrease disparities in access to treatment.

5 Researchers and regulators, in collaboration with manufacturers, clinicians, clinical specialty societies, and patient organizations, should focus on developing better quality of life measures for PAH.

Current patient-reported quality of life measures for PAH may not fully capture the burden of PAH and may not show small changes in quality of life. For example, although the PAH-SYMPACT questionnaire was developed specifically for PAH patients with patient input, patient experts who participated in the STELLAR trial reported that it was difficult to rate their symptoms, which may explain the minimal changes seen in the scale during the trial. Additionally, PAH-SYMPACT does not have a robust measure of caregiver burden.

Impact of Patient Engagement

The Pulmonary Hypertension Association provided ICER with **registry data** that better described the **demographics, epidemiology, and current treatment landscape** for PAH.

Collaboration with the PAH community allowed ICER to gain a **deep understanding of the lived experience** of individuals with PAH and their families.

Testimony from individuals living with PAH at the public meeting helped shape ICER's recommendations for policy makers to **reduce barriers to oxygen access.**

The Institute for Clinical and Economic Review (ICER) is an independent nonprofit organization that does research on how well new treatments work and what a fair price should be. Patients and families should always talk with their doctor to make shared decisions about the best treatment option for them.

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