Chronic Obstructive Pulmonary Disease 2024

# ICER SNAPSHOT

Reviewed by: COPD Foundation

*The COPD Foundation is not responsible for the final contents of ICER's Report or Snapshot, nor should their review be assumed to support any part of ICER's findings.*

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 ensifentrine for chronic obstructive pulmonary disease.

**The information included is up to date as of July 2024. New information about this therapy may become available, but is not captured here.**

## Let's Take a Look



## What is Chronic Obstructive Pulmonary Disease?

Chronic obstructive pulmonary disease (COPD) is a group of lung diseases that leads to airflow in the lungs being blocked and that can get worse over time. Symptoms include shortness of breath, fatigue, cough, wheezing, chest tightness, and mucus production.

COPD affects 16 million people in the United States and is the 6th leading cause of death. Smoking is the leading cause of COPD in the US, although other factors such as secondhand smoke exposure, chronic asthma, workplace exposure, and environmental factors can play a role. COPD leads to 1 million emergency room visits and 500,000 hospitalizations a year, resulting in medical costs of $24 billion per year.

# Impact on Patients and Families

## What ICER Learned from the Community

**Diagnosis with COPD carries a stigma** because of its association with cigarette smoking. This makes it less likely for people to report their smoking habits and to also blame themselves for their symptoms.

**Due to shortness of breath and fatigue,** some chores that require bending and lifting, such as making the bed, filling the dishwasher, or doing laundry, are very difficult or impossible.

With more severe disease, **equipment such as shower chairs and wheelchairs,** as well as **oxygen therapy,** may become necessary to help patients complete daily activities.

**Unpaid caregivers often have significant responsibilities** when caring for someone living with COPD, including medication management, physical chores, and emotional support.

Access to care can be **extremely difficult in rural areas**, particularly for patients who were dependent on oxygen that limit their ability to move or travel.

# Treatment of Focus & Clinical Context

Ensifentrine, made by Verona Pharma, is a new type of treatment that works by relaxing airway muscles in the lungs and reducing inflammation. Ensifentrine is a nebulized treatment which means it is a liquid medicine that is turned into mist so that a patient can inhale the treatment through a mouth piece or face mask. Ensifentrine is meant to be taken twice per day as an added maintenance treatment for COPD.

Ensifentrine (Ohtuvayre™) was approved by the FDA on June 26, 2024 as a maintenance treatment for patients with COPD.

## HOW DID CLINICAL TRIALS DEFINE COPD OUTCOME SEVERITY?

| MODERATE EXACERBATION | SEVERE EXACERBATION |
|---|---|
| Worsening of symptoms for more than 2 days that requires treatment with systemic corticosteroids and/or antibiotics | Worsening of symptoms leading to hospitalization |

# What Did Clinical Trials Show?

| | |
|---|---|
| **TRIAL NAMES** | **ENHANCE-1 and ENHANCE-2** |
| **PARTICIPANTS** | **Studied in 1,594 adults with moderate-to-severe COPD** |
| **TREATMENT GROUPS** | **Ensifentrine** vs. **Placebo** |

**RESULTS** Outcomes for patients receiving ensifentrine vs. placebo:

| **IMPROVED** air flow in the lungs | **IMPROVED** respiratory symptoms & overall health | **REDUCED** moderate to severe exacerbations |
|---|---|---|
| (In both ENHANCE-1 and ENHANCE-2) | (In ENHANCE-1 only) | (Pooled result for ENHANCE-1 and ENHANCE-2) |

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

## Helpful Clinical Terms

**Placebo:** An inactive treatment intended to hide whether a patient received the studied drug

**Exacerbation:** The worsening of COPD symptoms (increased shortness of breath, cough, and mucus amount)

**Pooled Result:** A statistical method to combine the results of multiple studies into a single result

## Safety of Ensifentrine

Those who received ensifentrine were slightly more likely to experience side effects such as back pain and hypertension compared to placebo. There were similar numbers of serious adverse events, discontinuation (stopping treatment) due to adverse events, and digestive-related issues for patients receiving ensifentrine or placebo.

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

## What We Still Don't Know

We still don't know 1) how well ensifentrine works in those taking multiple treatments, 2) ensifentrine's long-term safety and efficacy, and 3) how ensifentrine works in a broader population that includes nonsmokers, older patients, or those with mild or very severe COPD.



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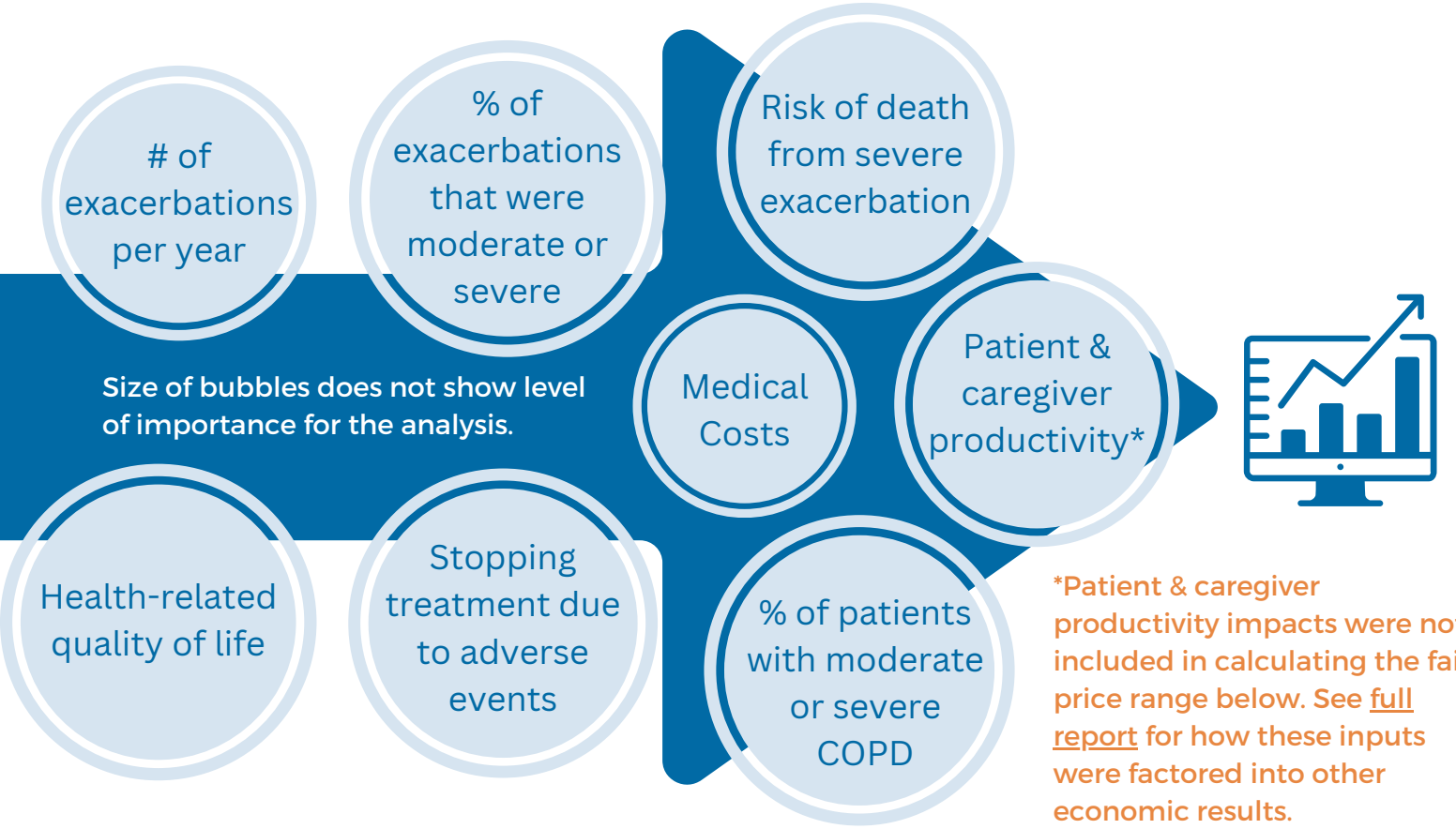
# How Did ICER Calculate a Fair Price?

Using economic modeling, we calculated the cost-effectiveness of ensifentrine based on number of exacerbations (increase in COPD symptoms) compared to usual care. See below for what types of information ICER considered to calculate a fair price range for this treatment.

## Population

Adult patients with moderate to severe COPD being treated with ensifentrine added on to current maintenance therapy or current maintenance therapy alone.

## Factors Included in ICER's Economic Analysis




*Patient & caregiver productivity impacts were not included in calculating the fair price range below. See full report for how these inputs were factored into other economic results.

# Fair Price Range for Ensifentrine

**$7,500 to $12,700**

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 ensifentrine is between $7,500 to $12,700. Verona Pharma's launch price for ensifentrine is $35,400 a year, which is much higher than ICER's fair price range.

# Key Policy Recommendations

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

**1** **Patient groups can continue to advocate for better oxygen access while payers (public and private health insurance plans) should change how they cover supplemental oxygen.**

Currently, more expensive forms of oxygen, which allow patients with severe forms of COPD to experience 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 increased access to oxygen and better oxygen systems.

**2** **Manufacturers should price treatments in a way that supports affordable access for all patients. For ensifentrine, the manufacturer has priced far above this level and therefore missed an opportunity to provide broad access and increased uptake of the drug.**

With a new way to treat COPD with limited side effects, there is likely to be significant interest in using ensifentrine for many patients with COPD. Given the large COPD population, the manufacturer of ensifentrine had an important opportunity to support broad access by setting the price in fair alignment with the proven benefits for patients. In pricing above this fair range, payers are likely to limit access and patients may find it difficult to access ensifentrine.

## 3 Manufacturers should include a more diverse patient population in clinical trials.

Clinical trials should reflect the affected populations as closely as possible. For example, the racial and ethnic make-up, and smoking history. Trials should include those who have never smoked, who represent an increasing percentage of the COPD population and who are excluded from COPD clinical trials.

## 4 Expand the set of outcome measures for studies of COPD interventions in order to capture the broader effects of treatment on patients' lives.

The FDA currently focuses on lung function, exacerbations, and death as outcomes for drug approvals. While these are core measures for COPD, they do not fully capture the ways that treatments may help patients. The FDA should seek to include all additional outcome measures, including more patient-centered outcome measures, in development programs for treatments for people living with COPD.

# Impact of Patient Engagement

Patients emphasized that **even with medication**, there remained a **high symptom burden** as well as physical activity limitations, **highlighting the unmet need for this population.**

The COPD Foundation estimated that on average, caregivers of patients with COPD provide **20 hours of care per week**. This input directly **informed ICER's economic model.**

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