Therapy Fact Sheet

Iloprost (Ventavis®) and Pulmonary Arterial Hypertension (PAH)

Key facts

- PAH is a fatal disease where blood pressure in the lungs is abnormally high. It affects an estimated 52 people per million. If left untreated, the median survival for patients with idiopathic PAH (IPAH) is only 2.8 years.
- Ventavis® (iloprost) is currently the only EMA-approved inhaled treatment for PAH.
- Ventavis® gives immediate and long-term relief of symptoms, with improved quality of life for patients with PAH. Data indicate a positive impact on survival.
- By treatment with Ventavis® for up to 24 months an estimated survival rate of 91% compared to an expected survival of 63% could be achieved in patients with idiopathic PAH.
- Ventavis® treatment has been shown to slow or reverse clinical deterioration with an improvement in NYHA functional class in 25% of patients treated with Ventavis® alone and 34% of patients treated with Ventavis® in addition to the endothelin receptor antagonist bosentan.

Pulmonary Arterial Hypertension (PAH)

PAH is a fatal disease where blood pressure in the arteries of the lungs is abnormally high. PAH presents in a number of forms, including idiopathic pulmonary arterial hypertension (IPAH), also known as primary pulmonary hypertension (PPH), which has no apparent cause; familial or heritable PAH; and a number of forms of PAH associated with specific underlying causes (such as drugs, toxins, congenital heart defects, connective tissue disease, HIV infection, schistosomiasis, etc). The various forms of PAH share common morphological changes to the endothelium of the arteries of the lungs, causing remodeling of the tissue, vasoconstriction and thrombosis. As a result of these changes, the blood vessels in the lungs constrict, making it more difficult for the heart to pump blood through to the lungs. This in turn causes right heart overload with symptoms such as shortness of breath particularly upon physical exercise (exertional dyspnea), fatigue, dizziness, fainting and edema, and can lead to heart failure and death. If left untreated, the median survival for patients suffering from IPAH is only 2.8 years following diagnosis.

Awareness and Prevalence

PAH is a life-threatening disease that strikes people in the prime of their lives, with a peak among women who are 20 to 40 years of age although men, children and the elderly are also affected. It affects an estimated 52 people per million. Epidemiological data for PAH are difficult to confirm, however, as there are no international registries tracking incidence and prevalence. Under-diagnosis is common due to low
awareness and difficulty in accurately diagnosing the condition. Right heart catheterization (RHC) is the
gold-standard procedure for accurate diagnosis of pulmonary hypertension (PH), evaluating the blood
pressure in the right side of the heart and pulmonary arteries.

**Ventavis®: Treating PAH**

Ventavis® is the first and currently only European Medicines Agency (EMA)-approved, inhaled therapy for
the treatment of PAH. It was approved throughout Europe in 2003 for treatment of patients with idiopathic
and heritable PAH in NYHA functional class III, and in the United States in 2004 for PAH in functional
class III or IV.

Ventavis® is a stable analogue of the naturally occurring prostacyclin and leads to dilation of the narrowed
blood vessels, therefore allowing more blood to flow through the arteries of the lung, and lowering blood
pressure within the pulmonary artery. This leads to an improved supply of oxygen to the body and
reduced strain on the heart, enabling the heart to function more effectively and allowing patients to
breathe more easily.11

The recently published AIR-23 and the STEP4 study have underlined previous evidence that Ventavis®
gives immediate and long-term relief of symptoms, with improved quality of life for patients with PAH. Data
indicate a positive impact on survival.3,4,5 In AIR-2, by treatment with Ventavis® for up to 24 months an
estimated survival rate of 91% compared to an expected survival of 63% could be achieved in patients
with idiopathic PAH with significantly improved clinical symptoms.3 In an earlier study, where a total of 76
patients with PAH were treated with inhaled iloprost for up to five years, survival in the study population
was also higher than expected without treatment. Overall survival was 79% and 51% compared to a
predicted survival of 68% and 46% after one and three years of treatment respectively.6 In the STEP
study, Ventavis® treatment was also shown to slow or reverse clinical deterioration: 34% of patients
treated with Ventavis® in addition to the endothelin receptor antagonist bosentan experienced an
improvement in NYHA functional class.4 This was also shown in a previous study where 25% of patients
treated with Ventavis® monotherapy improved in NYHA functional class.5
Inhalation Makes Patients’ Lives Easier

Ventavis® is provided as a nebulizer solution. Inhaling aerosolized Ventavis® from a nebulizer into the lungs targets the diseased pulmonary vessels directly. The intermittent inhaled administration of Ventavis gives dual benefits. One is the selective deposition of Ventavis® in the lung - guaranteeing rapid effect, good tolerability and a sustained improvement in physical exercise capacity and quality of life. The second is intrapulmonary selectivity that ensures vasodilation only in well ventilated areas of the lung where the gas exchange is taking place. This approach also avoids the potential dangers associated with invasive, indwelling intravenous catheters, which are necessary for continuous intravenous administration of prostanoids.

Ventavis® can currently be administered through a choice of nebulizers, offering patients fast and effective delivery of their medication. These new generation nebulizers adapt to the patient’s breathing pattern to ensure that the correct dose is delivered every time. They are small, light and battery-operated, which means they can be carried anywhere and be used anytime, leading to increased compliance.

References