PULMONARY HYPERTENSION ASSOCIATION OF CANADA

L'ASSOCIATION D'HYPERTENSION PULMONAIRE DU CANADA

Pulmonary Arterial Hypertension (PAH) Fact Sheet

What is pulmonary arterial hypertension (PAH)?

- PAH is a form of pulmonary hypertension (PH), a condition characterized by high blood pressure in the arteries that carry blood to the lungs.[i] It is a rare, serious, progressive, and potentially fatal chronic disease. There is no known cure[ii].
- It is estimated that more than 2,000 Canadians have been diagnosed with PAH, but as many as 4,000 may be affected by the condition.[iii]
- In PAH, the arteries of the lungs become narrowed and scarred, resulting in continuous high blood pressure in the lungs.[iv]
- PAH can strike people of all backgrounds, ages, and genders.
- PAH shares many symptoms with other diseases. This often leads to misdiagnosis and lengthy delays in diagnosis.[v]
- Physical symptoms can include:[vi]
    - Persistent or unexplained shortness of breath (especially upon exertion)
    - Fatigue/loss of energy
    - Swollen ankles and legs (also called edema)
    - Chest pain
    - Bluish lips, hands, and feet
    - Dizziness upon activity, including walking or climbing stairs
    - Fainting (also called syncope)
- If untreated, Canadians diagnosed with PAH live an average of two to three years. If treated, Canadians with PAH survive an average of five to seven years after diagnosis, but this has not improved in the past decade. [vii]

Existing treatment options

- PAH is a complex and progressive disease that requires specialized PAH doctors to prescribe treatments that offer the best short- and long-term outcomes for their patients.
- 10 PAH treatments are approved in Canada to slow disease progression and alleviate symptoms. These treatments act on three different biological pathways, all aiming to help blood vessels relax so blood flows more easily through them to the lungs:[viii]
- These treatments may be administered alone or in combination (dual or triple therapy).[ix]
- Other non-PH-specific treatments may include calcium channel blockers, diuretics ("water pills"), blood thinners, and supplemental oxygen.[x]
- If all else fails, transplantation of the lungs, or both the heart and lungs, may be considered.[xi]

- A majority of patients and caregivers agree that more treatment options – particularly ones that address additional, different pathways – are necessary, as current options are inadequate to prevent disease progression.[xii]

Sotatercept: a new treatment option

- Innovative new therapies now available in Canada or under investigation in clinical trials bring hope to PAH patients for better outcomes and improved quality of life.
- In August 2024, Health Canada approved **sotatercept** (Winrevair™), a new class of medication with a novel mechanism that potentially addresses disease progression. Sotatercept is the first activin signalling inhibitor therapy for the long-term treatment of Group 1 PAH.
- In November 2024, Canada's Drug Agency recommended that sotatercept be publicly funded in combination with standard PAH therapy for adult patients in WHO Functional Class II or III if prescribed by clinicians with PAH expertise and patients are not at low risk.[xiii]
- In Québec in December 2024, INESSS recommended against public funding for sotatercept despite recognizing its therapeutic benefit.[xiv]
- Sotatercept is used alongside whatever therapies a PAH patient is currently taking. You can take sotatercept whether you are taking one, two, or three existing therapies, so it provides a new avenue for therapy for people already on triple therapies. The effect was consistent whether people were taking one, two, or three other therapies.[xv]
- It can have significant benefits for people's functionality and mortality risk.
    - In a stage 3 clinical trial, people who were symptomatic but not severely ill who took sotatercept (plus their existing therapies) for 24 weeks significantly improved their 6-minute walk distance compared to people who just continued their existing therapies and received a placebo.[xvi]
    - The risk of death or nonfatal clinical worsening events was 84% lower with sotatercept than with placebo.[xvii]

Access to treatment

- Without public funding for sotatercept, PAH physicians are restricted from exercising their clinical judgment, which is essential to the proper treatment of PAH, and patients are unable to access optimal treatment for their disease.
- We hope that publicly funded access to sotatercept will be quickly negotiated through the pan-Canadian Pharmaceutical Alliance (pCPA), as any delay affects patients who may urgently require this treatment option.

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