About PNH

- Paroxysmal Nocturnal Haemoglobinuria (PNH) is a very rare, life-threatening bone marrow disease affecting the blood and major organs.
- PNH affects 3-4 people per million population or about 90 patients in all of Canada.
- PNH is not hereditary – it is an acquired illness that occurs when a patient’s oxygen-carrying red blood cells are destroyed by a protein called complement, an element of the body’s immune system.
- People with PNH lack naturally occurring complement inhibitors which would normally protect red blood cells from being destroyed by complement.
- The destruction of the red blood cells by complement (also known as haemolysis) can lead to life-threatening blood clots, kidney disease, pulmonary hypertension and other catastrophic consequences.
- PNH develops without warning and can occur in men and women of all races, backgrounds and ages.
- The average age of onset of PNH is in the early 30s, although approximately 10 per cent of all patients first develop symptoms at 21 years of age or younger.
- Scientific studies have shown that without treatment, approximately one-third of PNH patients do not survive more than five years and about half die within 10 years from the time of diagnosis.

Symptoms

- Symptoms of PNH can include:
  - Blood clots, also known as thrombosis
  - Kidney disease
  - Pulmonary hypertension
  - Shortness of breath
  - Abdominal/muscular pain
  - Fatigue
  - Anaemia
  - Intermittent episodes of dark-colored urine, also known as haemoglobinuria
  - Impaired quality of life
- Symptoms of PNH can be non-specific to the disease and take years to diagnose.

Diagnosis

- Flow cytometry is a specialized blood test that confirms a PNH diagnosis.
- The condition often goes unrecognized, with delays in diagnosis ranging from one to more than 10 years.
Treatment

- A bone marrow transplant (BMT) is the only cure for PNH, however well-matched donors are often difficult to find, and the surgery carries a significant risk with half the patients dying and at least another third suffering from rejection complications.
- Other supportive measures include blood transfusions, corticosteroids, anticoagulants, folic acid or iron supplements.
- Soliris is the first and only treatment for PNH that effectively prevents the premature destruction of red blood cells and thereby significantly reduces the development of life-threatening complications such as blood clots (strokes, heart attacks) and kidney failure.
- Soliris allows a patient’s life expectancy to return to that of a healthy person.
- Soliris is administered by infusion every 14 days by a licensed health care professional at a health care clinic, physician’s office or infusion clinic.
- Approved by Health Canada for the treatment of PNH in January 2009, Soliris is not yet funded by any provincial drug programs and, as a result, most patients have no access to this life-saving treatment.