

Follow-up of PNH patients after initial workup – routine visit (every 3-4 months) for all patients, regardless of Soliris treatment

Frequency determined by treatment, disease severity and local support

History and physical exam

Monitor the “6 P’s”:

1. Pep: rating fatigue
2. Paroxysms: have there been any episodes of increased hemolysis or of hemoglobinuria
3. Pallor: anemia, transfusions
4. Pulmonary: dyspnea
5. Pain: esophageal spasm, chest/abdominal pain, need for narcotic analgesia
6. Penis: erectile dysfunction (if applicable)

Rationale for test/evaluation

- Physical symptoms will help determine management strategy

Hematology

1. FLAER/RBC flow
2. CBC
3. Reticulocytes
4. LDH
5. DAT
6. D-dimer
7. Serum ferritin

Rationale for test/evaluation

- Flow cytometry required to monitor any expansion of the PNH clone
- CBC to track anemia and other cytopenias
- Reticulocytes and LDH to detect active hemolysis
- DAT to confirm that hemolysis is not autoimmune
- D-dimer to assess thrombotic risk
- Serum ferritin to assess potential iron overload or deficiency – if normal, it can be tested less frequently (e.g., every 6 months)

Renal

1. Electrolytes
2. Creatinine, estimated CrCl
3. Microalbumin
4. Urinalysis (routine & microscopic)

Rationale for test/evaluation

- Important to compare markers of renal function across visits to assess any deterioration

Other

1. BNP
2. Previous Soliris treatment history (if any) and outcomes

Rationale for test/evaluation

- If BNP stable/normal, ongoing monitoring can be less frequent (e.g., annually)

Follow-up – additional evaluations at routine visit for patients on Soliris

History and physical exam

An additional “2 P’s”:

1. Pyrexia: history of fever/infections
2. Prophylaxis: history of penicillin or other antibiotics for meningococcal prophylaxis, requirement for new/renewed prescription

Rationale for test/evaluation

- Patients on Soliris should report any infections/fevers they experience
- Prophylaxis recommended with Soliris treatment even if patient vaccinated

Additional evaluations – to be done at least once in patients on Soliris

Other

1. Anti-meningococcal titres (once available)

Rationale for test/evaluation

- Antimeningococcal titres coming soon to Canada; will help assess whether prior vaccination provided effective protection

Additional evaluations – annual visit (all patients, whether on Soliris or not)

Other

1. 2-D echocardiogram

Rationale for test/evaluation

- Monitor cardiac function and assess for pulmonary hypertension

Additional evaluations – patients with small/asymptomatic clone

Hematology

1. Evaluate clone size every 6-12 months

Rationale for test/evaluation

- Monitor any changes in clone size and how they correlate to development of symptoms