

Ongoing multisystemic assessments in patients with Morquio A³

| Assessments | Frequency |
|--|---|
| Musculoskeletal Standardised upper extremity function test, radiographs | <ul style="list-style-type: none"> • At diagnosis/baseline, annually |
| Respiratory FVC, MVV, respiratory rate, oxygen saturation, overnight sleep study | <ul style="list-style-type: none"> • At diagnosis/baseline, annually |
| Neurological Neurological exam Plain radiograph MRI scan CT scan | <ul style="list-style-type: none"> • At diagnosis/baseline, every visit (minimum, every 6 months) • At diagnosis, every 1 to 3 years • At diagnosis, annually • As clinically indicated |
| Cardiovascular Electrocardiogram Echocardiogram Heart rate | <ul style="list-style-type: none"> • At diagnosis, every 1 to 3 years, as clinically indicated • At diagnosis, every 2 to 3 years, as clinically indicated • At diagnosis, annually |
| Ophthalmological Refractive error and intraocular pressure | <ul style="list-style-type: none"> • At diagnosis, as clinically indicated |
| Audiological Multimodal hearing assessment | <ul style="list-style-type: none"> • At diagnosis, annually |
| Abdominal Assessments of gastrointestinal problems | <ul style="list-style-type: none"> • As clinically indicated |
| Dental Evaluation of oral health | <ul style="list-style-type: none"> • At diagnosis, annually |
| Endurance 6MWT | <ul style="list-style-type: none"> • At diagnosis, annually, before and regularly after initiation of ERT |
| QoL Reproducible, age-appropriate QoL questionnaires (e.g. EQ-SD-SL) | <ul style="list-style-type: none"> • At diagnosis, annually |

Adapted from Hendriksz, *Am J Med Genet Part A*, 2014.

Abbreviations: 6MWT, 6-minute walk test; CT, computed tomography; ERT, enzyme replacement therapy; FVC, forced vital capacity; MRI, magnetic resonance imaging; MVV, maximum voluntary ventilation; QoL, quality of life.