

Ongoing multisystemic assessments in patients with Morquio A²

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;