

# Minimum Recommended Schedule of Assessments for MPSI Patients

The Recommended Schedule of Assessments represents the core MPS I disease-related assessments that allow evaluations of a patient's disease progression over time. Physicians will determine the actual frequency of necessary assessments according to a patient's individualized need for medical care and routine follow-up.\*

	Initial Assessments	Every 6 Months	Every 12 Months	Every Other Year
<b>General</b>				
Demographics	■			
Patient Diagnosis <sup>1</sup>	■			
Medical History	■	■		
Physical Examination	■	■		
General Appearance	■	■		
<b>MPS I Disease Clinical Assessments</b>				
<b>Neurologic/CNS</b>				
MRI of Brain	■			■
MRI of Spine	■			■
Median Nerve Conduction Velocity	■			■
Cognitive Testing (DQ/IQ)	■		■	
<b>Ophthalmologic</b>				
Visual Acuity	■		■	
Retinal Examination	■		■	
Corneal Examination	■		■	
<b>Auditory</b>				
Audiometry	■		■	
<b>Cardiac</b>				
Echocardiogram	■			■
ECG	■			■
<b>Respiratory<sup>2</sup></b>				
FVC/FEV1	■	■		
Sleep Study	■		■	
<b>Gastrointestinal</b>				
Spleen Volume <sup>3</sup>	■			■
Liver Volume <sup>3</sup>	■			■
<b>Musculoskeletal</b>				
Skeletal Survey by X-ray <sup>4</sup>	■			■
<b>Vitals and Laboratory Tests</b>				
Height/Weight	■	■		
Head Circumference <sup>4</sup>	■	■		
Blood Pressure	■	■		
Enzyme Activity Level	■			
Urinary GAG Level	■	■		
Urinalysis	■	■		
<b>Functional Outcome Measurements</b>				
MPS Health Assessment Questionnaire, or other tools exploring functional ability and quality of life	■	■		

\*G.M. Pastores et al., The MPS I registry: Design, methodology, and early findings of a global disease registry for monitoring patients with Mucopolysaccharidosis Type I, Mol. Genet. Metab. (2007), doi:10.1016/j.ymgme.2007.01.011

All tests requiring sedation are recommended only if sedation is considered to be safe for the patient.

<sup>1</sup> Diagnosis would include genotype, if available.

<sup>2</sup> May not be possible in non-cooperative patients or patients younger than 5 to 6 years of age.

<sup>3</sup> The recommended method for obtaining organ volumes is MRI or computed tomography to enable quantitative analysis. However, if in the opinion of the clinician it is unsafe to sedate the patient, then ultrasound may be substituted. <sup>4</sup> Measured in pediatric patients only, unless determined otherwise by treating physician.

These recommendations were developed by the MPS I Board of Advisors, a group of physicians who have experience in managing patients with MPS I. The MPS I Registry is sponsored and administered by Sanofi.

