Refer patients to a geneticist for testing as soon as Morquio A is suspected[2]

Earlier diagnosis of Morquio A may improve long-term patient outcomes[2]

- Common paediatric conditions may signal Morquio A[6]
- Some ENT manifestations are common in the very early stages of Morquio A[6]
- A geneticist can perform an enzyme activity analysis to rule out or confirm a diagnosis of Morquio A if these signs are present and persistent in patients[2]

Rule out or confirm Morquio A with an enzyme activity analysis[2]

References: 1. Bhattacharya K, Balasubramaniam S, Choy YS, et al. Overcoming the barriers to diagnosis of Morquio A syndrome. *Orphanet J Rare Dis.* 2014;9:192. **2.** Hendriksz CJ, Berger KI, Giugliani R, et al. International guidelines for the management and treatment of Morquio A syndrome. *Am J Med Genet A.* 2015;167A:11-25. **3.** Montaño AM, Tomatsu S, Gottesman GS, Smith M, Orii T. International Morquio A registry: clinical manifestation and natural course of Morquio A disease. *J Inherit Metab Dis.* 2007;30:165-174. **4.** Hendriksz CJ, Harmatz P, Beck M, et al. Review of clinical presentation and diagnosis of mucopolysaccharidosis IVA. *Mol Genet Metab.* 2013;110:54-64. **5.** Tomatsu S, Montaño AM, Oikawa H, et al. Mucopolysaccharidosis type IVA (Morquio A disease): clinical review and current treatment. *Curr Pharm Biotechnol.* 2011;12:931-945. **6.** Mesolella M, Cimmino M, Cantone E, et al. Management of otolaryngological manifestations in mucopolysaccharidoses: our experience. *Acta Otorhinolaryngol Ital.* 2013;33:267-272. **7.** Hendriksz CJ, Al-Jawad M, Berger KI, et al. Clinical overview and treatment options for non-skeletal manifestations of mucopolysaccharidosis type IVA. *J Inherit Metab Dis.* 2013;36:309-322. **8.** Wood TC, Harvey K, Beck M, et al. Diagnosing mucopolysaccharidosis IVA. *J Inherit Metab Dis.* 2013;36:293-307. **9.** Tomatsu S, Alméciga-Díaz CJ, Barbosa H, et al. Therapies of mucopolysaccharidosis IVA (Morquio A syndrome). *Expert Opin Orphan Drugs.* 2013;1:805-818.

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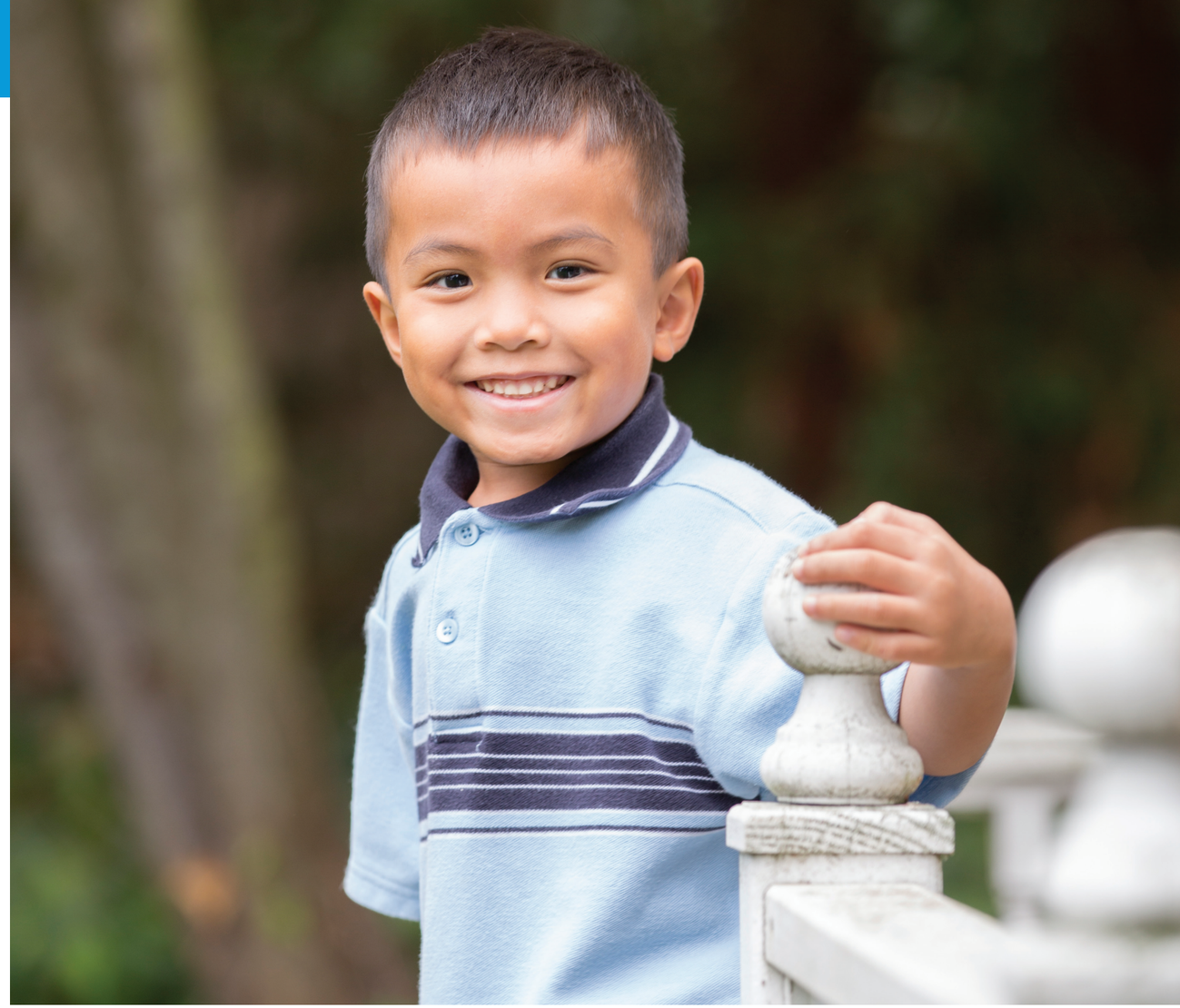

WOULD YOU SUSPECT MORQUIO A?

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Morquio A can progress to serious complications regardless of phenotype[1]

Morquio A is a multisystemic genetic disorder[1]

- Deficient enzyme activity leads to an accumulation of glycosaminoglycans (GAGs), which places patients at risk for progressive organ damage
- High degree of genetic heterogeneity may be responsible for the wide spectrum of phenotypic presentations

Diverse presentations of Morquio A can complicate patient identification[2]

- Patients with a nonclassical phenotype can be easily missed, even among specialists[1]
- Diagnosis of Morquio A is often delayed over 2 years due to misdiagnosis[1,3]

Nonclassical musculoskeletal presentation can include the following[1,2,4]:

- Potential height of > 140 cm
- Hip stiffness and pain
- Lack of overt skeletal manifestations

Classical musculoskeletal presentation can include the following[2,5]:

- Height of < 120 cm
- Overt spinal and skeletal abnormalities
- Chest abnormalities
- Genu valgum
- Abnormal gait
- Joint laxity

In an international Morquio A registry, 25% of Morquio A patients have a nonclassical phenotype[3]

Common paediatric conditions may signal Morquio A[6]

Ear, Nose, and Throat (ENT) manifestations are common in the very early stages of Morquio A, and patients may visit their paediatrician or paediatric ENT before being diagnosed[6,7]

Consider Morquio A in patients who have an occurrence of any of the following signs or symptoms:

- AIRWAY OBSTRUCTION[6]
- UNEXPLAINED CARDIOVASCULAR INVOLVEMENT, SUCH AS HEART MURMUR, IN PAEDIATRIC PATIENTS[2,7]
- REDUCED EXERCISE CAPACITY[8]
- LOSS OF MILESTONES[2,3,9]
- VALGUS KNEES EARLIER THAN 2-3 YEARS OF AGE[3,9]

Consider Morquio A in patients who have frequent recurrence of or persistence with any of the following signs or symptoms:

- CONDUCTIVE HEARING LOSS[6,7]
- SENSORINEURAL HEARING LOSS[6,7]
- OTITIS MEDIA[6,7]
- ENLARGED TONGUE[6]
- UPPER RESPIRATORY TRACT INFECTIONS[6,7]
- BEGIN TO FALL OFF THE GROWTH CHART[4]