



## Integrating treatment advances for alpha-mannosidosis into effective MDT care

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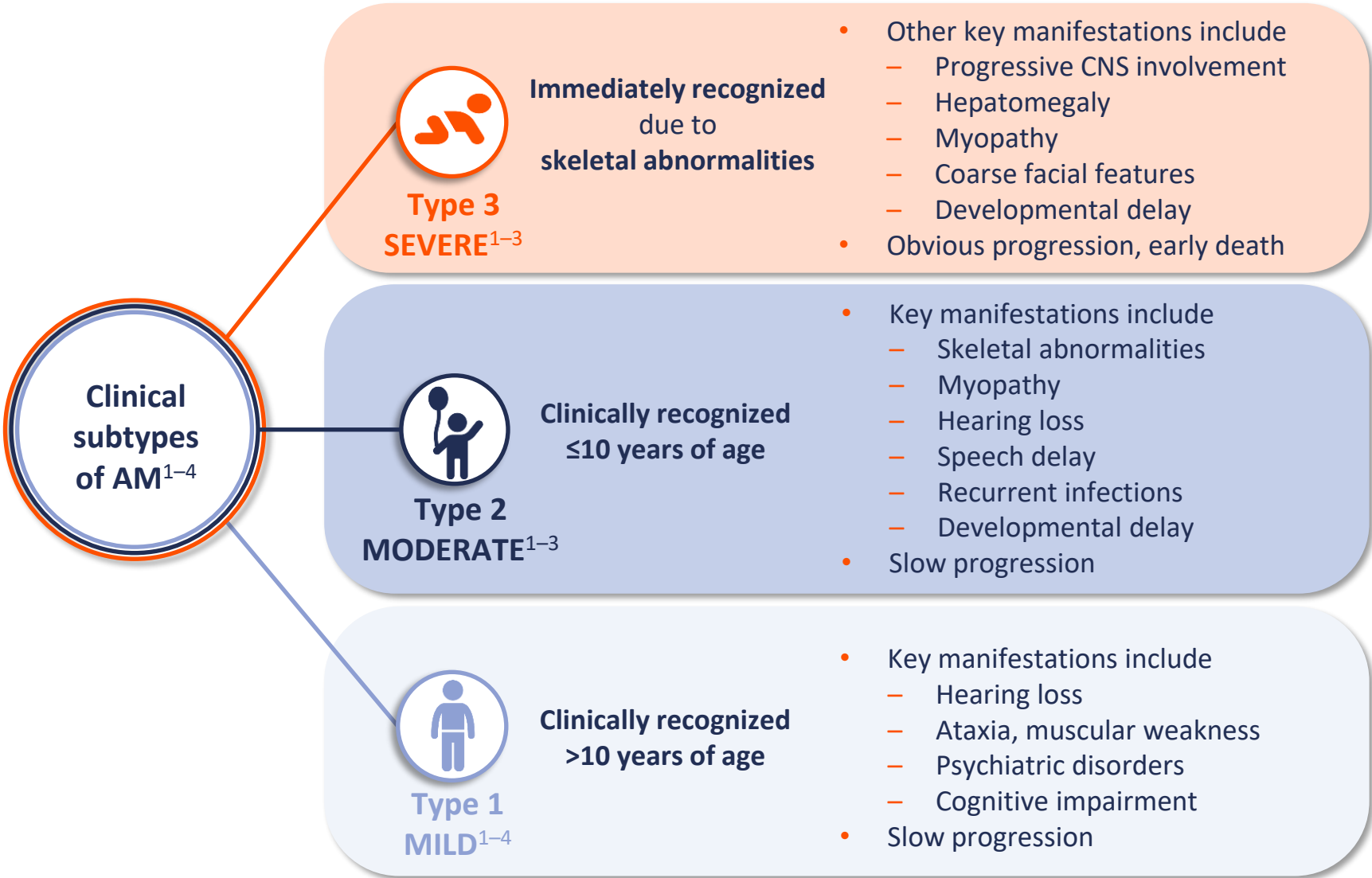
Practice aid for the management of people living with alpha-mannosidosis

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Treatment needs may vary by clinical subtype and evolve along the lifespan of people living with AM



Initial signs and symptoms are not specific to the disease, leading to diagnostic delays (mean delay ~5 years)<sup>1</sup>



## Consensus recommendations on coordinated care

### 2024 DELPHI consensus study on monitoring and integrated care<sup>5</sup>

Assessments in newly diagnosed patients	<ul style="list-style-type: none"><li>Genetic testing</li></ul>	<ul style="list-style-type: none"><li>Baseline assessments</li></ul>
Routine follow-up and care	<ul style="list-style-type: none"><li>Behavioural/psychiatric</li><li>Biochemical assays</li><li>Cardiac function</li><li>CNS manifestation</li><li>Cognitive impairment</li><li>Hearing assessments</li></ul>	<ul style="list-style-type: none"><li>Immune function</li><li>Muscular/motor function</li><li>Ophthalmologic pathologies</li><li>Patient-reported outcomes</li><li>Skeletal abnormalities</li><li>Other manifestations</li></ul>
Treatment-related follow-up and care	<ul style="list-style-type: none"><li>ERT-related monitoring</li><li>Post-HSCT monitoring</li></ul>	<ul style="list-style-type: none"><li>Supportive care monitoring</li><li>Integrated care coordination</li></ul>



### Consensus statement on MDT care<sup>5</sup>

*"Patients with AM should have a long-term multidisciplinary care team that includes an AM specialist to coordinate the team, as well as a group of specialists with expertise in each patient's specific disease manifestations"*

## Available disease-modifying therapies



### Infusion of exogenous functional enzyme that does not cross the blood–brain barrier

#### Velmanase alfa

**EU indication:** Treatment of non-neurological manifestations in patients with mild-to-moderate AM<sup>8</sup>

**US indication:** Treatment of non-CNS manifestations of AM in adult and paediatric patients<sup>9</sup>



#### Benefits

Phase III data show improvements in biochemical and functional parameters<sup>10</sup>



#### Safety considerations

Administration may result in IRRs, including anaphylactoid reaction<sup>8,9</sup>

IRRs may be mitigated by pre-treating with antihistamines, antipyretics and/or corticosteroids<sup>8,9</sup>



### Transplant functional enzyme-producing cells, with healthy donor cell CNS engraftment



#### Benefits

Data are limited, but studies show HSCT attenuates CNS disease, and can alleviate neuropathology<sup>11</sup>



#### Safety considerations

Reports of GvHD, and cases of re-transplantation due to graft failure<sup>12</sup>

Recipients are at higher risk for autoimmune haemolytic anaemia and pulmonary complications<sup>8</sup>

## Abbreviations and references

### Abbreviations

AM, alpha-mannosidosis; CNS, central nervous system; ERT, enzyme replacement therapy; GvHD, graft versus host disease; HSCT, haematopoietic stem cell transplant; IRR, infusion-related reaction; MDT, multidisciplinary team.

### References

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The guidance provided by this practice aid is not intended to directly influence patient care. Clinicians should always evaluate their patients' conditions and potential contraindications and review any relevant manufacturer product information or recommendations of other authorities prior to consideration of procedures, medications, or other courses of diagnosis or therapy included here.

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