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Research Article

Enzyme replacement therapy compared with best supportive care for the treatment of Pompe Disease: a systematic review and network meta-analysis

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Plain language summary

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Late-onset Pompe disease is a rare inherited condition that weakens muscles over time. As the disease progresses, the muscles that help with breathing become weaker, increasing the risk of respiratory failure, a major cause of early death. The main treatment for late-onset Pompe disease is enzyme replacement therapy.

This study examined how effective enzyme replacement therapy is in treating and managing late-onset Pompe disease. Researchers reviewed all available clinical studies comparing enzyme replacement therapy to best supportive care, which includes treatment without enzyme replacement therapy. The analysis included 60 studies: 38 on enzyme replacement therapy and 22 on best supportive care.

Findings suggest that, after 1 year, people who had never received enzyme replacement therapy before showed some improvement in walking distance compared to those receiving best supportive care. However, no significant difference was found in lung function (forced vital capacity % predicted). When comparing different types of enzyme replacement therapy, one drug appeared slightly better for walking distance, but this difference disappeared when adjusting for data variations.

Long-term studies showed that initial benefits of enzyme replacement therapy lasted 1–3 years, but muscle function gradually declined over 10–15 years. However, these results are uncertain due to small study sizes and missing data. Evidence on long-term best supportive care outcomes is also limited, making direct comparisons difficult.

Overall, enzyme replacement therapy provides short-term benefits, but its long-term impact on disease progression and the need for supportive care (such as walking aids or ventilation) remains unclear. Further research is needed to understand the lasting effects of enzyme replacement therapy.