

Executive Summary

A declaration of collaborative working between Amicus Therapeutics UK Operations and The National Hospital for Neurology and Neurosurgery

The National Hospital for Neurology and Neurosurgery and Amicus Therapeutics UK Operations are engaged in a collaborative working project called:

The National Hospital for Neurology and Neurosurgery Pompe physiotherapy service improvement

This project will run for 24 months after the commencement date and both parties have contributed resources for this initiative.

Start date – H2 2025 End date – H2 2027

Project aims and objectives:

- The aim of the project is to review the current Inherited Metabolic Physiotherapy provision and develop a new physiotherapy service in the metabolic service to better coordinate care for Pompe patients.
- It will allow all Pompe patients (irrespective of treatment) in the service to access a physiotherapy offer (face-to-face, remote appointments, self-management and monitoring) at all stages throughout their disease course, as needed and when any changes occur.
- To support Priority 3 of the Rare Disease Framework - to improve the coordination of care for patients with rare diseases.

Expected outcomes:

- To improve physiotherapy access to all Pompe patients throughout their disease course to optimise benefits of ERTs and to provide expert timely advice as their condition changes.
- To improve frequency of appropriate physiotherapy outcome measures for objective monitoring to review alongside the measures collected by the Pompe MDT – to better quantify disease progression/decline/stability.
- To improve the patient experience of the Pompe specialist service.
- To demonstrate the value of a targeted physiotherapy service in the specialist centre within the two years, including a digital offer.

Key Deliverables:

- Each patient with LOPD to be reviewed 1-2 times a year.
- To develop a digital platform to support patient self-management and self-monitoring.
- Improve the timeliness of physiotherapy intervention.
- To improve the patient outcomes and quality of life, as a result of improved patient reported outcome measures.
- Development of a digital platform to support a targeted physiotherapy service.
- By the end of the 2-year paid project, to demonstrate the value of targeted physiotherapy in the metabolic unit.
- Partner with neurometabolic physiotherapy services nationally to share progress and improvements and then implement in line with recommendations.