

Executive Summary

A declaration of collaborative working between Amicus Therapeutics UK Operations and The Royal Free London NHS Foundation Trust

The Royal Free London NHS Foundation Trust and Amicus Therapeutics UK Operations are engaged in a collaborative working project called:

The Royal Free Pompe physiotherapy hub and spoke treatment 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:

- To support priority three of the Framework – Improving the coordination for care for patients living with rare diseases.
- To build a specialist physiotherapy service providing expert assessment and treatment to Pompe patients at all stages of their disease progression with the intention of providing more equitable and consistent care and access to care for all patients.
- To improve patient outcome measures and to distinguish disease progression as well as improve overall patient experience.
- To demonstrate the value of an enhanced physiotherapy service dedicated to Pompe disease patients and that takes into account the clinical care provided by the treating physician and other healthcare professionals within the Hospital's centre of excellence for Pompe treatment.
- To develop the hub physiotherapy monitoring service using 'the North Star' approach and to follow current physiotherapy expert consensus guidelines for Pompe.
- To identify the local spoke physiotherapy treatment service and to provide ongoing support with education on Pompe specific physiotherapy treatment.

Expected outcomes:

- Evidence to support the development of a business case for an integrated, effective and sustainable physiotherapy model to all adult LOPD patients in the specialist centre within the two years.
- 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 deliver expert physiotherapy advice and treatment for patients with Pompe throughout their disease progression.
- To improve the patient experience of the Pompe specialist service.

Key Deliverables:

- Evidence that supports the benefit and value of a "hub and spoke" physiotherapy service available to Pompe patients.
- Increase in number of contacts with a physiotherapist for each Pompe patient for the purpose of monitoring, review and mitigating the risks of deterioration in muscle function.

- To increase the quality and quantity of patient reported outcome data used by the multidisciplinary team for patient monitoring. E.g. Change in function, assessment of patients who are wheelchair bound, reduction in pain etc.
- Subject to the data collected, evidence to support the development of a business case demonstrating the value of an enhanced physiotherapist service being available to Pompe patients (and the healthcare professionals responsible for their care) and validation of the hub and spoke physiotherapy model.
- Share interim findings with the neurometabolic physiotherapy services nationally to share progress and improvements and then implement in line with recommendations.