

Project title	The Royal Free Pompe physiotherapy hub and spoke treatment service improvement A collaborative working project between Amicus Therapeutics UK Operations and The Royal Free London NHSFT (The Royal Free)
Project rationale	Project Rationale: • Late onset Pompe disease (LOPD) is a rare, progressive disorder affecting muscle and respiratory function due to acid α-glucosidase (GAA) deficiency. • Holistic and consistent monitoring is crucial in a progressive disease such as LOPD to enable better data collection and inform treatment decisions. • Two new ERT medicines are now available, requiring the service to adapt. • The Royal Free has no dedicated physiotherapy service within the inherited metabolic service • As lysosomal storage disorders (LSD) are rare, patients travel from large distances to see the expert consultant team • The hub and spoke physiotherapy advice service will help local teams treat these patients
Project objectives	Project 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. Potential patient Benefits • Improved patient experience of a specialist Royal Free physiotherapy service. • Improved ability to access the Royal Free Physiotherapy Service. • Optimising patients' physical mobility, limiting or mitigating the physical impact of Pompe disease and deterioration, maintaining static success or improving physical function. • Improvement in patients' activity levels and or targeted exercise training programs and materials available. • Improvement in patients' quality of life and wellbeing. Potential NHS Benefits • Time to set up an effective and sustainable specialist hub and spoke Royal Free Pompe physiotherapy service. • Moving the provision of care from a reactive to a proactive service. • Support the National Rare Disease Framework Priority 3: Better Coordination of Care. • Increased number of data points for more informed clinical decision making. Potential Amicus Benefits • Partnership working with the Royal Free. • Identify challenges and unmet needs in the management of Pompe disease. • Improved understanding of the benefits (or otherwise) of a hub and spoke physiotherapy service and its role in the management in Pompe patients in a large, specialist centre with a diversity of population. • As Amicus produces medicines within the Pompe disease area, if overall patient care is optimised there may be an increase in the usage of these products in line with national and local guidelines.
Project period	Q2 2025 – Q2 2027
Financial arrangements	The project will be supported by a pooling of skills and resources from both Amicus and The Royal Free with a direct financial contribution provided by Amicus of £115,000
Plan for publication	Submit preliminary findings to <i>WORLD Symposium 2026</i> Submission of overall project to <i>WORLD Symposium 2027</i>
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Summary of the roles and responsibilities of each party	Roles and responsibilities are agreed and reviewed at monthly steering group meetings