

Project title	Pompe physiotherapy service improvement A collaborative working project between Amicus Therapeutics UK Operations and Salford Care Organisation (Part of Northern Care Alliance)
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. - Salford, the UK's largest Pompe specialist centre, currently has only one physiotherapist for inherited metabolic services. - More patients need to be seen, and more frequent in-person appointments are required, but current capacity is limited.
Project objectives	Project objectives - To improve patient outcome measures and overall patient experience. - To demonstrate the value of an enhanced physiotherapy service. - To support priority three of the Rare Disease Framework – Improving the coordination for care for patients living with rare diseases Potential patient Benefits - Improved patient experience of the Salford physiotherapy service. - Improved ability to access the Salford physiotherapy service. - At least maintain or potentially improve patients mobility and accessibility. - Improvement in patients training programs and materials available. - Improvement in patients quality of life and wellbeing Potential NHS Benefits - Support the National Rare Disease Framework Priority 3: Better Coordination of Care. - Increased number of data points for more informed clinical decision making regarding starting, switching and stopping of ERT - To increase the capacity of the Salford Pompe physiotherapy service. - Increased scope of what is possible for delivery through service expansion. Potential Amicus Benefits - Partnership working with the Salford Royal NHS Foundation Trust - Identify challenges and unmet needs in the management of Pompe disease - Improved understanding of the physiotherapy service and its role in the management in Pompe patients - 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	Q3 2024 – Q3 2026
Financial arrangements	The project will be supported by a pooling of skills and resources from both Amicus and Salford Care Organisation, with a direct financial contribution provided by Amicus of £130,800
Plan for publication	Submit preliminary findings to <i>WORLDSymposium 2026</i> Submission of overall project to <i>WORLDSymposium 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