# Great Ormond Street Hospital Pursues Market License for Rare Gene Therapy Independently



Great Ormond Street Hospital (GOSH) is making a groundbreaking move by attempting to obtain the market license for a rare gene therapy independently after the original pharmaceutical company withdrew. This initiative aims to bring life-saving treatments to patients with rare diseases, who often face limited options due to commercial interests of drug companies.

Approximately 3.5 million people in the UK live with a rare disease, with 95% of these conditions lacking effective treatments. GOSH’s move is particularly focused on a therapy for "bubble baby" syndrome, a condition that leaves affected children without an immune system. Securing funding from charities, including LifeArc, the hospital aims to allow these children to lead normal lives.

This marks the first time an NHS trust may gain authorization to market such a drug, potentially setting a precedent for non-profit delivery of crucial medicines. The hospital's endeavor represents an alternative model to traditional pharmaceutical pathways, addressing the gap left by companies prioritizing commercial viability over patient needs.