# Great Ormond Street Hospital to Independently License Gene Therapy for The 'Bubble Baby' Syndrome



Great Ormond Street Hospital (GOSH) in London is set to independently license a gene therapy for ADA-SCID, commonly known as "bubble baby" syndrome, following the withdrawal of a pharmaceutical company that initially planned to market the therapy. The hospital aims to make the treatment more accessible on a non-profit basis without industry involvement, addressing concerns about the affordability of gene therapies.

ADA-SCID is a rare genetic disorder that affects the immune system, making common infections potentially fatal. Approximately one to three babies are born with this condition each year in England. The current standard treatments include weekly enzyme injections or bone marrow transplants, both of which carry risks and require lifelong medication.

The gene therapy developed by GOSH has shown a success rate of over 95% in clinical trials. It involves extracting stem cells from the patient, genetically modifying them, and reintroducing them to generate a healthy immune system. This could potentially be a one-time treatment, offering a lasting solution and improving quality of life significantly.

Professor Claire Booth, a principal investigator from GOSH and University College London, highlights the necessity of this development as commercial interests wane in the field. In collaboration with the medical charity LifeArc and GOSH’s charitable arm, the hospital has received £350,000 to prepare a regulatory application for next year. This initiative could establish a new model for providing access to gene therapies for rare diseases.

Dr. Catriona Crombie from LifeArc emphasized the importance of overcoming the commercial barriers that prevent effective therapies from reaching patients. This licensing attempt by GOSH may serve as a foundational example for other gene therapies facing similar challenges in reaching the market due to financial viability concerns.