

Abstract Title: Challenges in managing LSDs within a cost constrained system: A perspective from UK

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Abstract: The National health service in the United Kingdom dates back to the postwar period. The service was founded with the virtue of being free at the point of delivery and funded from general taxation. This legacy gives it the dual handicap of lack of personal accountability allied to a third part payer. It is the central challenge which no politician has hitherto had the courage to confront. The only truly fundamental reform, dating back to the Thatcher years of the 1990s, was the creation of the internal market which separated Purchasers who commission specified services from Providers who provide them; but this remains an internal and virtual market which is cost constrained by the central exchequer.

The current total budget is about £135 billion per annum, approximately 8% of GDP, but the service is plagued by strike action (doctors, nurses, ambulance, drivers), exorbitant waiting lists for elective services, and alarming inconsistencies across the country despite central directives. Repeated promises of fundamental reform by all political parties ahead of an anticipated general election in 2024 are unlikely to be delivered, as voters have an almost religious regard for this free model, a theoretical paradise but an unachievable practical nightmare.

Amidst this turmoil, however, the UK can boast of a first-rate service for patients with rare diseases. Potential treatments are expertly assessed with diligence, rigour and transparency. Only cost-effective interventions are approved, and these can only be delivered within a regulated specialist platform. Rare diseases affect fewer than one in 2000 individuals, and more than 80% are inherited; although individually rare, collectively nearly one in 15 people will be affected at some point in their lifetime. Services for patients with 'ultra-rare' lysosomal storage disorders are provided by specialist centres in London, Birmingham, Manchester and Cambridge. All centres provide multidisciplinary and coordinated services conforming to a centrally commissioned specification comparable to the highest international standards.

What is the explanation for this central paradox? It is the loud and influential patient voice. It is heard at every level, constantly demanding equitable access to top class services for LSD patients. It is almost as though they are invited to the backseat of an Ambassador on a bustling highway full of auto- and bicycle rickshaws.

Area of expertise: Haematology; Stem Cell transplantation; Lysosomal Storage Disorders; Gene therapy