



Drug Repositioning in Rare Disease



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Drug development is slow

Repositioning existing medicines
offers a different path.

One that centres people, not
pipelines.

Rare disease cannot wait.



The Challenge

For most rare conditions, there is no licensed treatment. Families face years of uncertainty.

Traditional drug development is too long, too costly and too narrow for small populations.

People feel left behind.



What Drug Repositioning means?

Repositioning uses medicines we already understand. Instead of starting from zero, we explore whether an existing drug can safely help a different condition.

It is practical, evidence-led and often faster for communities who have waited too long.



Why it matters for Rare Disease?

Repositioning can shorten timelines.
It can reduce risk. It can open doors
for conditions that struggle to
attract investment.

**Most importantly, it gives families
a sense of movement and hope.**



Where things get stuck

Evidence is scattered.

Data is hard to access.

Funding is limited.

Regulation is complex.

And lived experience is not always centred in decision making.

These barriers slow progress for the people who need it most.



Centre lived experience

Families often spot patterns long before the evidence catches up. If you are working on repositioning, how are you bringing lived experience into the room. What could we strengthen together.

Repositioning works best when researchers, clinicians, charities and communities learn together.

Thank You

We help health, research and community organisations build real, meaningful relationships with the people they serve.

We strengthen involvement, grow stronger projects and support sustainable funding.

Our expertise in PPIE, Asset Based Community Development, funding strategy and drives lasting impact.

Explore our toolkits: **Beyond Tokenism, Deepening Co-creation and Safeguarding research integrity**

Contact Us

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