Partner Rare

CRACKING THE EMA CODE

Article 7

KEITH WILLIAMS

# Hope for Rare Diseases: Drug Repurposing for Better Patient Care in Europe

Partner Rare

For patients with rare genetic diseases, the struggle extends far beyond the challenges of the disease itself. Multiple co-morbidities often accompany these conditions leading to complex polypharmacy treatment. The reliance on multiple medications exposes patients to a higher risk of adverse effects from inactive ingredients, or excipients in these drugs.

The situation becomes even more concerning when considering paediatric patients. Many existing medications are not suitable for children due to limited data on their safety and efficacy in younger age groups. Additionally, these older drugs often come in forms, like tablets, that are difficult for infants and neonates to take. Furthermore, many licensed drugs contain potentially harmful excipients, such as alcohol and propylene glycol, which immature kidneys in young patients struggle to eliminate.

This creates a significant unmet need for patients with rare diseases, particularly children. Traditional drug development is a lengthy and expensive process, making it less attractive for niche conditions affecting relatively small patient populations.

# Drug Repurposing: A Promising Approach

Drug repurposing, also known as repositioning, offers a glimmer of hope for these patients. It involves identifying existing, licensed drugs with established safety profiles and investigating their potential effectiveness for treating new conditions, including rare diseases.

This approach offers several advantages:

## Reduced Time and Cost

Since the safety of the drug is already established, repurposing can significantly shorten the development timeline and reduce the overall cost compared to developing entirely new medications.

## Improved Patient Outcomes

Repurposed drugs can offer better-tolerated formulations, like liquids or injections, that are easier for paediatric patients to take. Additionally, by eliminating harmful excipients, repurposed drugs can minimize the risk of adverse effects.

## Patient-Centric Development

Repurposing efforts should be driven by the specific needs of patients. Clinicians and patient organizations can provide valuable input regarding the limitations of current treatment options and the desired characteristics of repurposed medications.

# Case Study: Levocarnitine Solution for Children

A prime example of successful drug repurposing is the development of a sugar- and alcohol-free Levocarnitine paediatric solution by Cenote Pharma. This solution addressed the specific needs of children with rare genetic diseases by providing a safer and more easily administered form of medication compared to existing options. This patient-centric approach resulted in the solution becoming the preferred choice throughout the UK due to its beneficial profile.

# Challenges and Considerations

While repurposing offers significant advantages, there are challenges to overcome.

## Niche Market Considerations

Identifying repurposing opportunities requires specific knowledge of both the disease and the existing medications. Collaboration with clinicians and patient organizations is crucial for success.

## Pricing and Market Access

Pricing strategies must ensure that repurposed drugs are accessible to patients without attracting parallel imports, where cheaper versions are imported from other countries.

## Clinical Differentiation

When existing standard treatments are available, repurposed drugs need to demonstrate clear advantages, such as improved patient tolerability or a more convenient delivery form.

# Partnering for Success

## Partner Rare's Expertise in Drug Repurposing for Rare Diseases

Drug repurposing for rare diseases in Europe requires specialized knowledge and expertise to navigate the complex regulatory landscape and ensure successful market access.

Our team has successfully managed the entire repurposing lifecycle, from identifying promising candidates and securing intellectual property rights, to designing and executing clinical trials, obtaining regulatory approvals, and achieving profitable market entry across Europe.

Our team of Rare Disease industry experts possess a deep understanding of the European market and the unique challenges associated with drug development for niche populations

Partner Rare

## Our Services to Assist you Include:

### Identifying Repurposing Opportunities

Partner Rare leverages their network of clinicians and patient advocacy groups to pinpoint unmet medical needs and identify existing drugs with potential for repurposing in the rare disease space.

### Clinical Our Design and Execution

Their team can guide companies through the design and execution of clinical trials specifically tailored for rare disease populations, ensuring efficient data collection and robust results.

### Regulatory Expertise

Partner Rare navigates the intricate regulatory hurdles associated with drug repurposing in Europe, ensuring compliance and facilitating a smooth path to market approval. Their in-house legal experts possess a deep understanding of the legal landscape surrounding drug repurposing in Europe.

Partner Rare

## Market Access and Pricing Strategies

Partner Rare's Expertise in Market Access for Rare Disease Therapies

Successfully launching a repurposed drug for a rare disease requires a nuanced understanding of market access and pricing strategies. It's a balancing act: ensuring affordability for patients while securing the commercial viability of the treatment. This is where Partner Rare excels.

Our team's unparalleled experience spans the entire drug repurposing life cycle, from target identification and clinical development to market authorization and successful commercialization, culminating in profitable exits. We've repeatedly demonstrated our ability to identify promising repurposing candidates, navigate the complexities of European regulatory frameworks and health technology assessments, and optimize pricing strategies to achieve sustainable market access.

If your company or institution is developing treatments for rare diseases in Europe, Partner Rare is your trusted partner in navigating the complexities of drug repurposing.

Contact us today to discover how our tailored solutions and proven track record can unlock the potential of your therapy and make a real difference for patients.

# About the Author

Keith Williams is a seasoned rare disease expert with over two decades of experience in commercializing orphan drugs and licensed generic rare disease products across the UK, Ireland, Nordics, and the EU. He has extensive experience in drug repurposing for rare and orphan diseases, with a proven track record of successful strategy management and buyouts. Keith's specialist understanding of patient and payor requirements allows him to successfully redevelop existing medications to meet the changing needs of healthcare bodies across the UK, Nordics, and Europe. With extensive contacts among key decision makers and senior HCPs at the forefront of drug development guidelines and principles, Keith brings a comprehensive perspective from his senior roles at GSK, MSD, LEO, and Lediant Biosciences.

At Partner Rare, he leverages his expertise to guide clients toward successful market entry and launch, with a focus on innovative solutions and patient well-being.

To explore how Keith, and Partner Rare can help you navigate drug repurposing rare diseases therapies in Europe, contact us for a complimentary consultation.


Partner
Rare