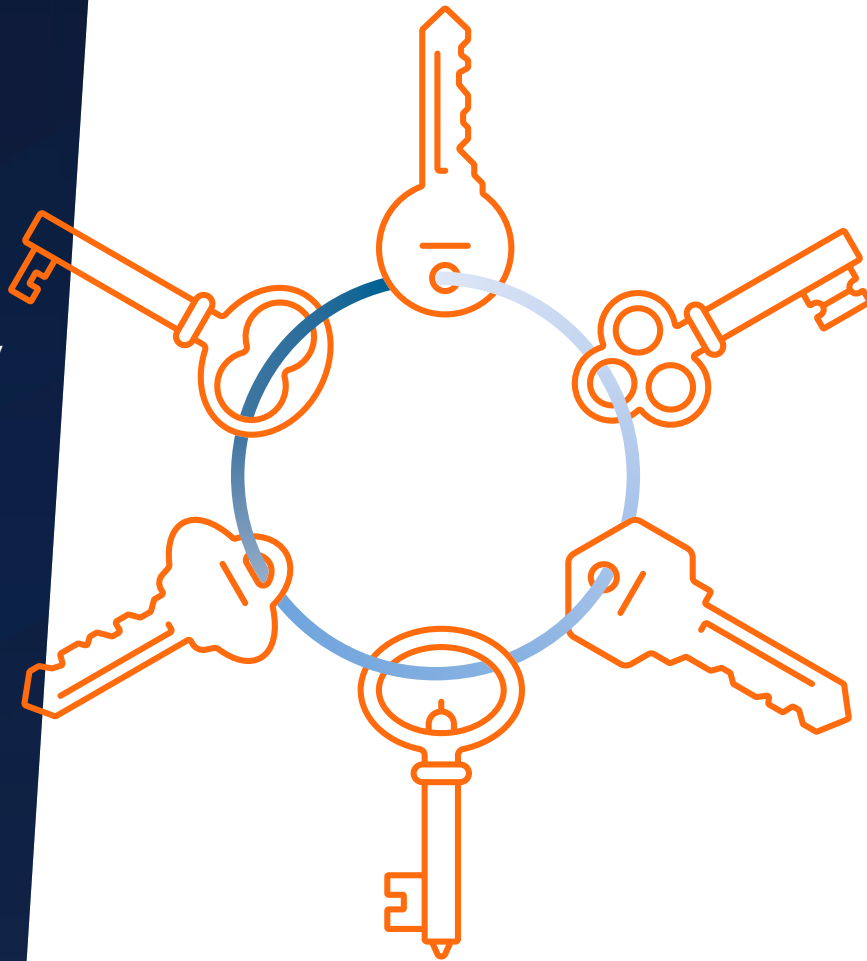


Unlocking the Hidden Links: Similarities Among Rare Diseases?

Rare diseases, by their very nature, are characterized by their uncommonness and diverseness, with each exhibiting a distinct set of symptoms and unique causes. Even among patients with the same disease, presentations can differ. However, while patients' disease experiences may be unique, they collectively share similar narratives when it comes to diagnosis and treatment.



ETIOLOGY

- Gene mutations that manifest during childhood
- Environmental factors considered to play a role



CHALLENGES WITH COORDINATION OF CARE

- Specialized centers are long distances
- Relocate for specialist access
- Necessitates multiple specialists
- Communication challenges



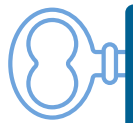
FAMILY & EMOTIONAL IMPACT

- Physical and emotional isolation
- Challenges in managing disease complexities
- Daunting to learn new terminology
- Patients need to be own advocates



FINANCIAL TOXICITY

- Numerous tests and consultations with specialists for diagnosis are expensive
- Ongoing care costs can be substantial at the beginning
- Travel expenses to access specialized care, participate in clinical trials, or seek expert opinions
- Insurance coverage might be limited, leading to high out-of-pocket costs
- Patients or caregivers are often unable to work



LIMITED TREATMENT OPTIONS

- Lack specific targeted therapies
- Less economically viable to invest in treatment research
- Treatments focus only on symptoms or preventing complications; few options treat the source
- Disease-modifying therapies are short-lived
- Curative therapies are rare



DIFFICULT TO DIAGNOSE

- Often mimic other, more common diseases
- Limited HCP awareness
- Lack of diagnostic criteria and resources

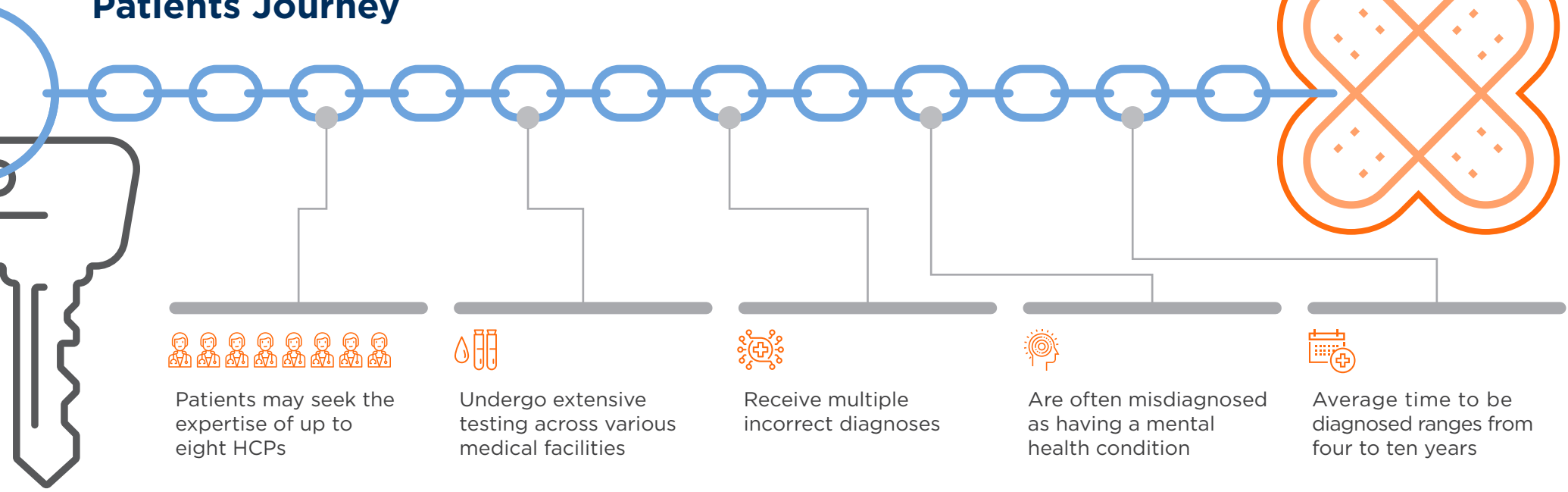


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Patients Journey



▶ UNCOVERING INSIGHTS TO DRIVE ACTION

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