RAREBASE

How the Rarebase Function product engine maximizes efficiency in rare disease drug discovery

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Individuals and families affected by rare diseases have no time to waste searching for effective therapies. The current drug discovery pipeline has

expensive, and can overlook rare diseases altogether. It's time to reimagine rare disease drug discovery by thinking practically and on a larger scale to meet the needs of the rare disease patient community.

Read on to discover how the Rarebase strategy maximizes efficiency in rare disease research by providing faster, more cost-effective solutions.

Reshaping the approach to rare disease drug discovery

Traditional approaches typically investigate one drug candidate and disease at a time, dragging out development times and ballooning production costs. As many rare diseases are linked to a single root cause gene, that genetic mutation can become a primary target of drug discovery research. However, "targeting each disease-causing gene one by one hinders the speed of drug discovery," explains Rarebase Chief Scientific Officer Chris Moxham, PhD, "We need to look at the bigger picture and combine our research efforts to reach more rare disease patients."

> **Function combines science and technology to "identify drug candidates based on**

> **scale using innovative technologies," says Dr. Moxham.**

That's where the Rarebase drug discovery product engine — Function™ — comes into play. Function combines science and technology to "identify drug candidates based on each root cause genetic mutation, but are applied at scale using innovative technologies," says Dr. Moxham.

Function scales drug discovery by simultaneously examining thousands of novel and FDA-approved small molecule drug candidates through high-throughput screening. We treat control cells with a battery of compounds and evaluate them against the expression of thousands of gene targets. If a drug can change the expression of a root cause gene in a manner that can rebalance the underlying biology of a disease, it becomes a candidate for further testing in cell models of the disease. In this way, Function accelerates drug discovery by identifying potential drug candidates across a large swath of multiple rare conditions related to a single cell type.

Maximizing resources to build a path forward

starting with a reliable treatment modality. The most practical option is small molecules, which are more extensively studied with regard to drug development and reduce financial barriers to treatment access. By starting with well-characterized small-molecule drugs, the Rarebase strategy provides an accelerated launching point for rare disease drug discovery.

> **“Our strategy prioritizes practicality and scale to provide faster and more cost-effective solutions for many rare genetic diseases at once.”**

The combined small-molecule approach with high-throughput screening maximizes efficiency for rare neurological diseases. “Our strategy prioritizes practicality and scale to provide faster and more cost-effective solutions for many rare genetic diseases at once,” says Dr. Moxham.

To date, Rarebase has generated a premiere drug screening dataset that works to provide a more comprehensive solution for rare diseases. Creating a practical path forward for the rare disease community remains central to this mission.

Contact us to learn more.