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# How third-generation drug discovery is transforming rare disease treatment development

With its Healnet platform, Healx pairs cutting-edge AI with industry-leading drug discovery and rare disease expertise to identify new uses for known compounds. The company now has over 20 programs in development.

Drug discovery is changing. The phenotypic and target-based approaches that have been used over the past 100 years are giving way to a new artificial intelligence (AI)-based, hypothesis-free and highly scalable model that stands to accelerate innovation in the field. Healx has established itself at the leading edge of this third generation of drug discovery by creating a suite of AI-driven methods that promise to increase the speed, scale and chance of success of treatment development.

The need for a new approach to the discovery of medicines, and in particular to the discovery of treatments for rare diseases, is clear. Despite rare conditions affecting more than 400 million people across the globe, roughly 95% of rare diseases lack approved treatments today. This huge unmet need reflects the shortcomings of established approaches to drug discovery.

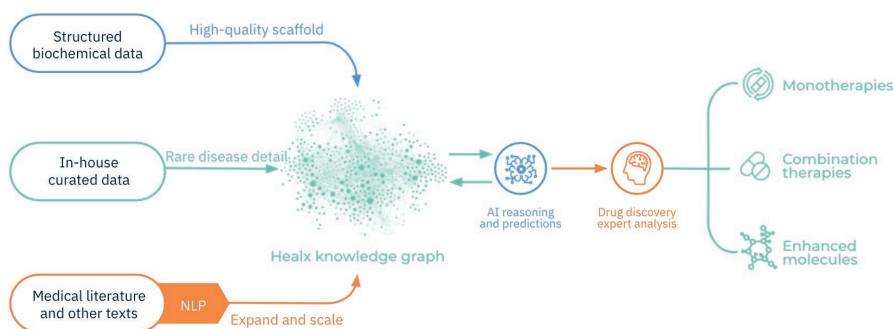
By using the traditional target-based approach that has dominated drug discovery since the 1990s, companies have typically spent 12–14 years developing each new therapeutic and seen 90% of candidates that enter human testing fail<sup>12</sup>. While the target-based model, like its phenotypic predecessor, has uncovered many important drugs, its high costs and risks have deterred investment in diseases that affect small patient populations as companies seek to ensure that their spending is recouped.

But, there is a better way to discover drugs. In this new generation of drug discovery, researchers are moving away from single targets and are deploying sophisticated technologies to understand the complex biological mechanisms that drive disease. These advances are cutting the impact of human bias and enabling the identification of novel treatment opportunities at striking speed and scale.

## Building a third-generation approach

Healx is demonstrating the power of this new wave with its Healnet platform. Healnet integrates data from biomedical research, scientific literature, patient insights and Healx's own curated sources to form a rare disease knowledge graph (Fig. 1).

By applying state-of-the-art AI models to the graph, Healnet rapidly identifies novel disease-compound relationships with the highest chances of success. These AI algorithms combine the best of 100 years of drug discovery, drawing on both traditional phenotypic and target-based



**Fig. 1 | The Healx Healnet platform.** AI, artificial intelligence; NLP, natural language processing.

approaches as well as cutting-edge omics and expression-based practices.

Healx pairs the platform with human drug discovery expertise to find new uses for known drugs, opportunities to combine compounds and ways to enhance molecules. The input of this team of experts is critical to the effective use of the AI. It is humans that select the diseases to work on and analyze the AI predictions to ensure that the best opportunities are pursued through to clinical trials.

**Partnerships are a key part of Healx's pipeline expansion strategy, and the company is actively looking for rare disease assets that are in or near human clinical trials**

Healx's novel approach has already shown its value. The company has over 20 programs in its pipeline, and its lead, for fragile X syndrome, went from the in silico discovery phase to the identification of active preclinical combinations in under 24 months. The truncated timeline was enabled by an AI-powered approach that could cut the total time needed to get a drug to market to 6–8 years.

Healx's fragile X program is just the start. By 2025, the biotech plans to launch around 50 programs, setting it up to bring treatments to millions of underserved rare disease patients. Healx is able to forecast such a rapid expansion of its pipeline because of its AI-powered drug discovery methods, focus on derisked drug candidates and the application of financial risk modelling to its portfolio.

## Partnering to leverage Healnet

Partnerships are a key part of Healx's pipeline expansion strategy, and the company is actively looking for rare disease assets that are in or near human clinical trials. The plan is to leverage the clinical, regulatory and commercial infrastructure built by Healx to advance in-licensed molecules in orphan indications.

Healx is also looking to use its capabilities to support biopharma partners. Healx could use Healnet to find new indications for a partner's existing assets—in a similar way to the work it is doing with Ono Pharmaceutical—or to look for drug candidates with promise against particular target diseases. Healnet can also identify companion drugs that could boost efficacy, mitigate toxicities and otherwise improve the risk-benefit profile of assets.

While Healx is applying Healnet internally to rare diseases, the platform could be applied to a broader range of conditions. Biopharma companies that partner with Healx to access the fruits of the platform stand to position themselves at the vanguard of a change that is transforming the pace, scale and success rate of drug discovery.

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