



CASE STUDY

How OTXL Uses Vibe Bio for Rare Disease Due Diligence

Learn how Orphan Therapeutics Accelerator uses Vibe Bio for rare disease due diligence, pipeline and competitive analysis, and in-licensing strategy.

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OVERVIEW

Accelerating progress in rare disease drug development

[Orphan Therapeutics Accelerator](#) (OTXL) is a nonprofit focused on one mission: overcoming challenges facing ultra-rare disease drug development and commercialization, which have caused many promising investigational treatments to be abandoned or deprioritized. Founding members in OTXL include [Chiesi](#) and [BIAL](#), while [Landmark Bio](#), [DVLP Medicines](#), [Viralgen](#), [Uncommon Cures](#), and Vibe Bio joined as clinical development partners. As the former CEO of [Global Genes](#), OTXL chief executive officer and co-founder [Craig Martin](#) understands both the promise and the pain points in rare disease innovation. Alongside chief operating officer and co-founder [Elizabeth White](#), PhD, a veteran biopharma executive with decades of R&D and corporate strategy experience, the team is building a new kind of accelerator, one that uses technology to drive smarter investment decisions and faster movement for medicines that otherwise might never reach patients.

But finding the right rare disease programs and evaluating them efficiently and rigorously takes time, technical expertise, and access to high-quality data. That's where Vibe Bio comes in. "We knew there was a universe of shelved drugs out there," Martin shared. "We needed a way to evaluate those efficiently, consistently, and with enough depth that our scientific leadership is able to make confident, strategic decisions."

THE CHALLENGE

Due diligence at scale for a new model of drug development

As a nonprofit, the Orphan Therapeutics model depends on making the right calls with limited resources. Yet traditional methods of search & eval—manually researching asset history, mapping competitive landscapes, and estimating development timelines—required hours of effort for every drug program. Another significant challenge is that the focus on rare disease means OTXL is looking at different diseases all the time. There are over 10,000 rare diseases out there, hundreds of which have abandoned or de-prioritized programs. No single institution can be an expert in all of them.

In early diligence phases, OTXL often had to rely on public information. Once OTXL gained access to confidential data rooms, the team was able to go deeper but needed better tools to evaluate those programs with speed and consistency in early and later stages of diligence.

“The work can be incredibly time-intensive. You need to do due diligence right, but you also want to move efficiently, especially when resources are tight.”

Craig Martin, CEO and co-founder of OTXL



THE SOLUTION

Vibe Bio's AI-powered analysis platform

OTXL began working with Vibe Bio to address three recurring challenges:



Search & eval of shelved or in-licensing-ready assets



Landscape analysis to understand competition & differentiation



Pipeline analysis to assess timelines, risk, and readiness



CASE 1

Gene therapy for a rare neurologic disease

In 2024, OTXL engaged Vibe Bio to support further diligence on a gene therapy for an ultra-rare pediatric neurodegenerative program developed at a major academic institution. The program had already reached Phase 2 with human data and was under consideration for in-licensing.

The asset addressed a severe pediatric neurodegenerative disease with urgent unmet need. Vibe Bio assessed the drug's characteristics, developability, and future trial requirements. The analysis, conducted as Vibe Bio developed more comprehensive automation capabilities in its platform, still provided the rigor and depth needed to support prioritization. "The work gave us confidence," Martin noted. "It served as both a prioritization tool and a fundraising tool."

CASE 2

Differentiation in a crowded genetic therapy landscape

In 2025, OTXL partnered with Vibe Bio to conduct a detailed competitive analysis of a gene therapy candidate for an inborn error of metabolism disorder. The investigational gene replacement program is in the clinic.

Using its AI-powered platform, VibeOne, the Vibe Bio team surfaced all known clinical and preclinical programs for the disease, including AAV, mRNA, and gene-editing competitors. The analysis highlighted the strengths of the program of interest, including:

- Differentiated specificity for the target cell type
- Robust preclinical data
- Strong alignment with pediatric treatment gaps

The VibeOne analysis also generated key development risks, such as:

- Durability challenges from pediatric liver growth
- Suitability of the immunosuppressive approach
- Trial operational complexity

The competitive landscape analysis gave OTXL a detailed understanding of how the asset stands apart clinically and strategically, not just by target or modality, but also by potential clinical value for pediatric patients. The OTXL team shared that this work was instrumental in building internal confidence and maintaining asset momentum in their pipeline evaluation process.

"It wasn't just about who else was in the space. **VibeOne helped us understand where this program stood apart**—and why that mattered for patients."

Craig Martin, CEO and co-founder of OTXL



THE EXPERIENCE

Accelerating progress in rare disease drug development

From the beginning, OTXL valued Vibe's transparency, especially about the evolving state of the VibeOne AI platform. "They were always clear about what was manual and what was automated," shared White. "But what mattered most was that when we asked for something, they delivered. And the analysis kept improving."

As a [Founding Clinical Development Network](#) (ClinDevNet) Partner in the Orphan Therapeutics Accelerator, the Vibe Bio team has consistently treated OTXL requests with priority. Follow-up questions were

answered quickly and thoroughly, and the output helped support OTXL's internal decision-making processes.

OTXL plans to use the Vibe platform to get templated, consistent due diligence across all of the drug programs it is evaluating. Features like executive summaries, comparative scoring, and structured recommendations will help OTXL's executive team and investment advisory board assess opportunities more clearly, especially across programs that span vastly different rare disease areas.

"Vibe Bio has been a true partner in every sense of the word. **We need them to get it right**, and everything we've seen shows they're committed to doing exactly that."

Beth White, COO and co-founder of OTXL



THE FUTURE

Vibe Bio as a core component of OTXL's model

As OTXL builds toward a future marketplace, which is intended as a platform to match rare disease assets with mission-aligned funders and partners, VibeOne is emerging as a foundational element.

OTXL hopes to offer a clearinghouse of rare disease programs, each with its own risk profile, predictive analysis, and development roadmap, to accelerate movement beyond the accelerator itself. VibeOne will help power that marketplace by enabling OTXL to pre-screen and package assets for potential funders and collaborators.

A new standard for therapeutics due diligence

Vibe Bio's AI-powered platform, VibeOne, gives biopharma and nonprofit partners like OTXL the ability to conduct deep due diligence, smart pipeline analysis, fast competitive analysis, and streamlined search & eval in a single solution.

By providing structured, data-backed insights with speed and consistency, Vibe Bio empowers partners to de-risk decisions and accelerate the development of high-potential assets. For Orphan Therapeutics Accelerator, that means building confidence, raising capital, and helping programs move forward faster.

To see how Vibe Bio's AI-powered platform can help your team accelerate rare disease search & eval, streamline due diligence, and identify high-priority assets, [get a complimentary demo of VibeOne.](#)