MELIOR DISCOVERY®

CASE STUDY

Accelerating Gene Therapy Innovation Through Expert Collaboration

Rapid data delivery and customized models that enabled investor confidence, funding success, and advancement to larger studies.

How Melior Discovery is Accelerating Gene Therapy Innovation

As a specialized *in vivo* pharmacology CRO Melior Discovery has distinguished itself through its pioneering achievements in animal model studies, as well as *in vivo* phenotypic screening, and drug repositioning. With decades of experience, Melior empowers clients to rapidly evaluate therapeutic candidates, generating high-quality data that supports confident, informed decision-making.

Replay Bio is a pioneering biotech developing an HSV gene therapy platform for genetic diseases. This case study highlights how Melior enabled Replay Bio to quickly refine its designs by delivering essential data, adaptable models, and a highly collaborative approach.

Project Summary

CLIENT:
Replay Bio

INDUSTRY:
Gene therapy for rare genetic diseases

THE CHALLENGE:
Replay Bio needed quick access to high-quality *in vivo* data to efficiently iterate on its HSV vector design and meet critical development and funding timelines.

MELIOR'S SOLUTION:

- Developed a customized model using Replay Bio's HSV vectors.
- Integrated scientific insights by collaborating closely with Replay Bio and leveraging past research to ensure the model was tailored to their unique requirements.
- Conducted comprehensive validation testing to confirm the model's reliability and its ability to generate accurate data for optimizing therapeutic vectors.

RESULTS & BENEFITS:

- Key data points were delivered within days, accelerating Replay Bio's iterative design process and enabling timely decision-making.
- The data package, including long-term durability results, supported compelling presentations to stakeholders.
- Melior's adaptable approach ensured the project met evolving requirements, which is critical for Replay Bio's development timeline.

> *It definitely feels like they want this to work as much as we do. When we asked them questions on how something was performed, their communication was very clear, and it felt like they were an extension of our team.*

replay

Lachlan MacKinnon
CEO and Co-Founder, Replay Bio

About Lachlan

Lachlan, Co-founder and CEO of Replay, He is also a Director of Depixus and holds an MA in Chemistry from the University of Oxford.

The Challenge: Balancing Speed and Cost with In-Depth Expertise

Replay Bio aims to revolutionize genetic disorder treatments by leveraging the HSV vector's capacity to carry up to 152,000 bases, which far surpasses AAV capabilities. However, designing and testing such a complex vector posed significant hurdles:

- Aligning sequence design, construction, and testing to meet tight deadlines.
- Receiving rapid performance feedback to refine sequences and optimize delivery mechanisms.

To keep pace with these demands, Replay Bio sought a partner who could deliver not just speed but also expertise, adaptability, and high-quality data. Melior could integrate into their workflow, enabling frequent design iterations and providing insights that would accelerate progress toward clinical readiness.

The Solution: Customized Models and Expedited Data Delivery

From the outset, Melior Discovery demonstrated its ability to address Replay Bio's unique challenges by developing a customized model tailored to its HSV vector platform. This model allowed for the application and precise tracking of vector expression over time. Melior's expertise in creating and refining *in vivo* models ensured that the data generated was directly applicable to Replay Bio's therapeutic goals.

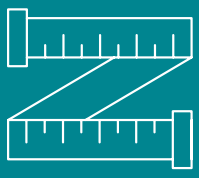
Melior's flexibility and responsiveness stood out as they expedited specific data points, such as the seven-day analysis within a 30-day study, enabling Replay Bio to iterate on vector designs faster. This quick turnaround allowed Replay Bio to refine its therapeutic candidates within a compressed timeline, helping them stay on track for critical milestones.

> *When I first met Vivian, I shared a publication with her, and she was really like a thought partner in designing that experiment. She didn't just do what we told her—she really thought through it with us.*

Renata Martin
Director of Gene Therapy, Replay Bio

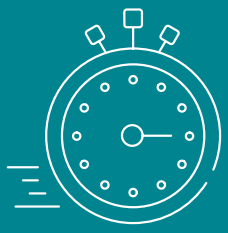
This partnership was further enhanced by Melior's flexible experimental design, allowing Replay Bio to request expedited data at specific points and adjust protocols to meet evolving project demands.

Why Did Replay Bio Choose Melior Discovery?



TAILORED EXPERTISE

Melior's ability to develop a customized model addressed the specific needs of Replay Bio's HSV platform.



SPEED AND FLEXIBILITY

Melior provided rapid data delivery and accommodated changes in study design to align with Replay Bio's goals.

COLLABORATIVE PARTNERSHIP

Melior's proactive communication and investment in understanding the project made them an integral part of Replay Bio's team, enabling confident and efficient decision-making.

The Outcome: Accelerating Innovation with Actionable Data

The results of their collaboration with Melior were transformative for Replay Bio. By receiving fast and relevant data, they could iterate on HSV vector designs more efficiently, reducing the time and resources typically required for such complex gene therapy projects. Melior's high-quality, timely data provided Replay Bio with a compelling case to present to investors, showcasing durability and efficacy data crucial for gaining buy-in and maintaining project momentum.

This collaboration demonstrates the impact of a specialized CRO that adapts to the needs of innovative biotech firms. Melior's commitment to Replay Bio's project, from the customized model to the flexibility in data delivery, enabled Replay Bio to focus on advancing its therapeutic platform. Together, they addressed immediate testing needs and established a foundation for future studies, positioning Melior as a valuable partner in Replay Bio's progress toward clinical readiness.

> *The data package was really crucial for our progress, especially in terms of timing for the end of the year. It allowed us to demonstrate the durability of our gene therapy platform and make informed decisions.*

Renata Martin
Director of Gene Therapy, Replay Bio

Collaborative Innovation as a Catalyst for Gene Therapy

The partnership between Replay Bio and Melior Discovery demonstrates the transformative potential of a CRO that prioritizes adaptability, expertise, and teamwork. Melior's delivery of tailored solutions, expedited data collection, and active collaboration with Replay Bio allowed the team to refine their HSV vector efficiently and maintain momentum toward key milestones.

This case study demonstrates how Replay Bio, with Melior's support, addressed the challenges of developing an HSV platform through rapid data delivery, tailored solutions, and collaborative innovation. It highlights the critical role of the right CRO partnership in advancing gene therapy innovation.

Melior's Thoughts:

We love working on projects like Replay's that let us integrate different elements of our expertise, particularly when we can bring our experience to impact study design. Our client's success is our success when our data is part of the story. We absolutely get the crucial importance in balancing timelines with quality and attention to detail to make those successes possible.

Andrew Reaume, Ph.D.,
President & Co-founder
Melior Discovery

We requested that the seven-day data point samples get expedited for a quick analysis, and Melior was able to deliver exactly what we needed. Their flexibility in experiment design and speed to deliver the data has been really instrumental for us.

Renata Martin
Director of Gene Therapy,
Replay Bio

For more on this success story and to start your own, contact Melior at

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Find out more about Melior at https://www.meliordiscovery.com/