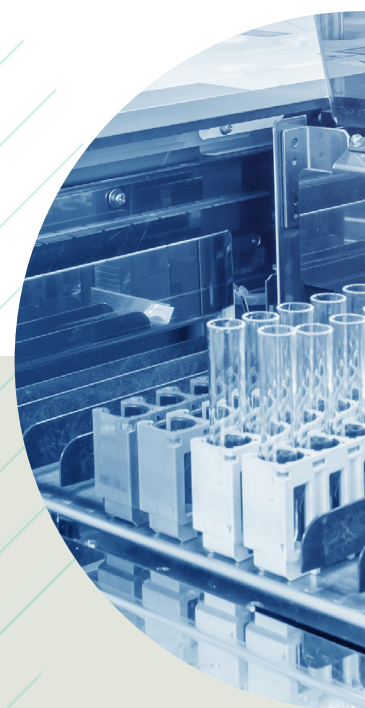


A Krystal Clear Vision:

Develop Genetic Medicines for Patients with Rare Diseases



Krystal Biotech — Delivering New Hope to Underserved Patients

Rare diseases, while often overlooked by the general population, can feel overwhelming to patients, especially when limited treatment options are available. Krystal Biotech, a leader in redosable gene therapy based in Pittsburgh, Pennsylvania, is on a mission to create change. The company is developing genetic medicines for patients with rare diseases, a historically underserved community. The company's gene-therapy platform is based on a non-integrating, replication-defective, type-1 herpes simplex virus (HSV-1) vector. Its flagship product, beremagene geperpavec (VYJUVEK™), is a redosable gene therapy that treats dystrophic epidermolysis bullosa (DEB), an incurable, potentially fatal, skin-blistering condition. VYJUVEK™ has progressed through clinical trials and, as of May 19, 2023, has been approved for the treatment of DEB in patients six months or older by the FDA.

The Impact of Fragmented Data Processes on Collaborative Innovation

Developing novel treatments for rare disease demands both scientific innovation and operational excellence. Krystal Biotech uses its proprietary, redosable HSV-1 gene delivery platform to develop treatments for rare diseases, and they have also brought all stages of viral manufacturing in-house.

With many R&D efforts at play, the company knew that optimizing processes would best position its scientists to innovate, especially as its R&D teams grew. Key areas of focus were both data and process standardization, as well as communication across R&D groups. In particular, capturing and leveraging experimental data was becoming increasingly tedious. Time that could be spent in the lab was instead spent recording and tracking down data across various software programs and systems.

Chelsea Geruschat, Senior Research Associate in Downstream Process Development at Krystal Biotech, explained, "There was a lot of flexibility in our previous system, and while some flexibility in data management is essential, we worried too much could impact our ability to record and organize our experiments and results. Additionally, when data was siloed in large Excel files, efficient retroactive data analysis was becoming a challenge."

Krystal Biotech's leadership knew that their scientists' time was best spent innovating, not wrangling data. It was time to find a solution that would help their R&D teams best optimize their data and communication processes. The team began their search for a solution by first looking for a flexible electronic laboratory notebook (ELN) that would help to standardize data configuration for more streamlined results in the short term. But, knowing the company also needed to maintain flexibility for future R&D experimentation and corporate growth, the team wanted to ensure any new ELN they adopted also tied into a more robust R&D platform that could seamlessly support future needs.



Choosing an R&D Data Platform for Growth

Krystal Biotech sought three key features in a new ELN and scalable R&D platform:

1. An organized system that would allow scientists to analyze data, both in the present and in the future.
2. Data storage that is secure and can grow as needed.
3. The ability to secure data by utilizing multi-factor identification to protect valuable information.

Dotmatics offered each of those features and more.

Erik Schneider, Senior Manager of Downstream Process Development and Validation at Krystal Biotech, explained why his team chose Dotmatics, saying, "In the end, the Dotmatics Platform felt like it would be easy to immediately pick up, while also offering more advanced capabilities. We liked that we could first adopt the basic ELN tool and expand from there. While we wanted to get up and running quickly, it was still important that we have the option to add new tools and functionality down the line, when we get to the point when we need them."

Ultimately, the Krystal Biotech team chose Dotmatics because its platform breadth and flexibility would not only meet its immediate needs, but would also support the company's growth and evolving demands.

| Challenges | Dotmatics Solutions |
|--|--|
| Segmented Experimental Data Data was stored in multiple formats across different R&D teams. | Organized Electronic Lab Notebook Advanced standardized processes for clean data capture in a user-friendly and secure system. |
| Inefficient Data Mining Scientists spent valuable time mining for important experimental process data and results. | Easily Searchable Data Powerful scientific storage and search, including relationship tracking between different experiments, freeing scientist's time by making it easy to extract and analyze data. |
| Time Consuming Manual Data Sharing Storing data in multiple formats meant that cross-functional collaboration required time consuming manual data sharing. | Smooth Cross-Functional Collaboration Research and development scientists united on one system, letting them quickly gather findings and create relationships between experiments, and share work in a permission-controlled environment. |
| Other Systems Have Limited Technology Capabilities Other technologies are unable to support a growing organization and their developing needs. | Continual Product Advancements The Dotmatics Platform offers a wide variety of functionality that can be added on as needed, including specialty biology and chemistry R&D tools, lab systems to manage work requests, inventory, assay data and entity registration, and data visualization and analysis tools to drive decision making |



Freeing Time for Research Exploration with Better Data Processes

With the Dotmatics Platform currently being used by 18 scientists across five of Krystal Biotech's R&D groups, cross-functional communication is better than ever. The standardization of documentation empowers scientists to communicate more effectively and efficiently. Schneider has also noticed an improvement in data integrity, saying, "The act of implementing this system, which has more formal procedures, ensures that data is correct and cross-checked by another scientist, which saves time and prevents mistakes."

Scientists are now able to save time by more easily creating relationships between different experiments, which makes it easy to collaborate, find and extract data, and act on related experimental findings. Implementation of the Dotmatics platform has also given teams an opportunity to evaluate how they think about their data.

"With other ELNs I have used in the past, it always felt like we hit a wall. We would think, 'This is all the ELN can do for us.' With the Dotmatics Platform, we definitely haven't hit a wall, and I don't foresee that happening any time soon."

Chelsea Geruschat,

*Senior Research Associate in
Downstream Process Development*

Looking Ahead: Adopting New Dotmatics Platform Functionality

As Krystal Biotech expands its programs to develop additional novel treatments for the rare-disease community, it will likewise expand its onboarding of scientists onto the Dotmatics ELN and Platform. The company also plans to adopt additional Dotmatics apps and tools and is excited about the potential that the partnership with Dotmatics holds.

Geruschat concluded by saying, "With other ELNs I have used in the past, it always felt like we hit a wall. We would think, 'This is all the ELN can do for us.' With the Dotmatics Platform, we definitely haven't hit a wall, and I don't foresee that happening any time soon. I'm looking forward to the workflows being developed by Dotmatics, and I anticipate they will be useful to our team in the future. It's exciting that Dotmatics hasn't given up on development, because as Dotmatics is developing, we do, too."

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a demo with
Dotmatics and
learn how it can
support your
organization

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