sapio sciences

Case Study

A Unified Informatics Platform Bridging Small and Large Molecule Discovery

With Sapio's unified, science-aware™ LIMS/ELN, PTC Therapeutics integrated its approach to research with a single enterprise platform and consolidated vendor solutions

PTC THERAPEUTICS™

About the Project

Founded in
1998

Location
South Plainfield, NJ

Industry
Healthcare

Website
https://www.ptcbio.com/

Background

PTC Therapeutics is a global biopharmaceutical company focused on the discovery, development, and commercialization of clinically differentiated medicines that provide benefits to patients living with rare disorders. The company adopted Sapio's all-in-one laboratory information management system (LIMS), electronic lab notebook (ELN), and scientific data management platform. The unified platform has enabled PTC to:

- Streamline workflows for compound discovery and rapidly adapt them to the evolving research needs of scientists.
- Unify data capture, access, and analysis to foster better collaboration and decision-making among PTC's team of more than 200 R&D scientists.
- Consolidate disparate informatics vendors onto a single, no-code configurable platform to simplify the user experience and reduce cost.
- End-to-end management of materials, from initial request to storage, locations, movement, inventory, and replenishment.

The Customer

PTC Therapeutics was founded in 1998, focusing on leveraging expertise in RNA biology to discover and develop treatments for patients living with rare diseases. The company has deployed three biotechnology platforms:

- Bio-e, which targets oxidative stress and inflammation pathways to treat cancer and diseases of the central nervous system
- Nonsense suppression to bypass mutations that generate incomplete or nonfunctional proteins, which account for 10-15% of all inherited diseases
- Splicing modulation to reduce the mutations in protein production that cause more than 200 human diseases.

In its first 25 years, PTC has a commercial portfolio that includes six medicines treating Duchene muscular dystrophy, spinal muscular atrophy, and several other rare genetic diseases.

The Challenge

Like many established pharmaceutical and biotechnology companies, PTC had accumulated an array of software to support its researchers, including sample management and compound registration systems, lab automation systems, and data visualization and reporting tools. However, even those systems - well-liked by a team - were problematic from a research informatics perspective: One-off solutions might handle a specific workflow well, but data from those solutions often couldn't be accessed easily by other scientists using their preferred solutions. The resulting data siloes complicated workflows and slowed decision-making.

Additionally, some scientists relied on cobbled-together, home-grown solutions, such as Excel spreadsheets, to manage data. For instance, many individual teams maintained separate spreadsheets to track materials being used in experiments. Plus, some vital information on experiments run at PTC wasn't being digitized; most scientists at PTC still documented their work in paper lab notebooks.

The Solution

PTC reviewed an extensive list of potential vendors and spoke to other pharmaceutical and biotechnology companies about their preferred vendor solutions. PTC ultimately selected the Sapio Sciences unified lab informatics platform, which offers a genuinely configurable, no-code/low-code, all-in-one platform essential to PTC's research informatics team requirements.

- A complete solution that would provide researchers with easy access to the research data needed to support their daily tasks and effectively collaborate with their peers.
- A genuine platform delivering templated workflows and processes out of the box, with the ability to add functionality without complex coding.
- The ability to configure the platform to individual user roles – such as turning functionality on and off or setting permissions to access data.
- The capability to take full control over the solution configuration, rather than having to rely on vendor professional services.

> **Our aim with the Sapio platform is to foster a collaborative workspace where chemists and biologists can share data easily and focus on their scientific work,** rather than being held back learning software and figuring out how to get data.
>
> **- Joseph Tivade**
> Senior Analyst at PTC Therapeutics

> Having supported piecemeal solutions for years, our research informatics team sought a platform that would **capture data produced by all our researchers and provide easily configurable ways to expose data to the right scientists at the right time and in the right way.**
>
> **- Joseph Tivade**
> Senior Analyst at PTC Therapeutics

Results/Benefits

PTC has opted for a phased implementation of the Sapio platform, focusing initially on rolling out the ELN functionality for managing materials and inventory. "With materials management we can realize immediate cost savings with the Sapio platform because it feeds into many downstream processes. For example, by simply helping scientists know where materials currently reside, they can avoid purchasing the same items over and over," said Joseph Tivade, Senior Analyst at PTC Therpaeutics.

As hoped, the PTC research informatics team has complete control over the solution, though they have utilized some professional services to get things up and running. The team is also beginning to focus on leveraging the platform to handle PTC's small molecule processes from assay development to high-throughput screening, structure-activity relationship (SAR) analysis, secondary screening, pharmacology, and analytical studies.

"Through a combination of Sapio's out-of-the-box workflows, no-code configurability, and some custom services, we've built a solution that can support our research data processes now and grow with us as we consider how to, for instance, feed research data into machine learning," Tivade said. The team also looks forward to integrating the Sapio solution with a purchase order system and other related software to support scientists in their work.