

DRUG REPURPOSING FOR RARE DISEASE TREATMENT LEADS TO REDUCED RISK, COST, AND DEVELOPMENT TIME

CASE STUDY: PARC CIENTÍFIC BARCELONA



"If you don't have Integrity, you may never find the information you need."

Jordi Quintana
Director of Drug Discovery Platform
Parc Científic Barcelona

Parc Científic Barcelona (PCB; Barcelona Science Park), home to three Research Institutes and 75 companies, acts as an incubator for biotechnology companies with a wide range of research support technology. As Director of the Drug Discovery Platform of PCB, Jordi Quintana oversees novel research and offers services to the pharmaceutical industry and academia. In 2009 Jordi and his team secured funding to investigate a rare disease, amyloidosis. In particular, they set out to study a subset of the disease called familial amyloid polyneuropathy (FAP).

FAP is an autosomal-dominant disease, so only one copy of the defective gene, inherited from either parent, will lead to the condition. It is a fatal and incurable disease and the only current treatment is liver transplantation, which is not always successful.

Because of the rare nature of the disease and the limited patient base, traditional drug discovery and development techniques would not be considered in finding a treatment for this condition. As a result, Jordi turned his attention to alternative approaches to seek a novel treatment for FAP.

"For such rare diseases, repurposing is a viable strategy for drug discovery," says Jordi. *"This involves the investigation of pharmaceutical compounds already on the market, or in advanced clinical phases, which were initially developed for different therapeutic applications that might also be applied to the rare disease under study."*

REPURPOSING DRUGS

With the high cost of developing drugs and fewer blockbusters being developed, more researchers are turning to repurposing. One approach to drug repurposing is to mine the vast amount of pharmacological, biological, and chemical data generated by the pharmaceutical industry and academia.

Thomson Reuters IntegritySM can reveal connections between a drug, target, and disease that had not previously been identified. As a user of *Integrity* since its launch, Jordi knew the value that it would bring to his initial research into finding a novel therapy for FAP. *"When Integrity arrived in 2001, it was a disruptive innovation."* Jordi says. *"We were now able to get information from Integrity that we could not get so quickly from any other source."*

IDENTIFYING NOVEL THERAPIES

Using *Integrity*, Jordi and his team compiled a list of compounds already in the market or in advanced clinical phases. They searched the chemical structures of these compounds to study those that could be more active for the target protein related to the rare FAP disease.

With a filtered, prioritized list of compounds in hand, the team is working on testing them on the rare disease target protein, using the synthesis pathways described in *Integrity*. When compounds active against the disease target protein are found, these may move more rapidly into clinical trials. This is because these compounds have already overcome the necessary steps to reach the market or clinical phases for the original therapeutic application. Employing this approach will significantly shorten the development time and reduce both risk and cost.

INTEGRITY PROVES CRITICAL

Jordi finds *Integrity* invaluable in his approach. *"Some databases don't have drug names, and others only have CAS[®] Registry Numbers, but Integrity will find these and more,"* he says. *"With other search protocols, so many results may be returned that it would be impossible to read them all to find that little piece of information you want."*

Ease of data export is also important to Jordi and his team. With *Integrity* they can do searches and export chemical structures and SAR tables, which allows them to perform additional analysis offline. Although they still use other data sources in their research, *Integrity* is the key to their current research methodology at PCB. *"If you don't have Integrity, you may never find the information you need,"* says Jordi. *"It's that critical."*

Using their repurposing approach and *Integrity* as a tool, the team at the Drug Discovery Platform of PCB hope to identify a pre-existing drug that will be effective in treating this fatal disease.

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