Drug repurposing for rare disease treatment leads to reduced risk

Director of the Drug Discovery Platform of Parc Científic Barcelona (PCB), Jordi Quintana and his team secured funding to investigate a rare disease, amyloidosis. They set out to study a subset of the disease called familial amyloid polyneuropathy (FAP). Because of the rare nature of the disease and the limited patient base, traditional drug discovery and development techniques were not an option.

As a result, Quintana turned his attention to repurposing as a viable strategy for drug discovery. “This approach 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,” said Quintana. To accomplish this, PCB required a solution that would unlock hidden insights in the vast amount of pharmacological, biological, and chemical data generated by the pharmaceutical industry and academia.
"We were able to get information from [Cortellis Drug Discovery Intelligence] that we could not get so quickly from any other source. If you don’t have [Cortellis], you may never find the information you need. It’s that critical."

Jordi Quintana
Director of Drug Discovery Platform, Parc Científic Barcelona

Finding the information needed to connect the dots

Quintana and his team looked at a number of solutions but found that Cortellis Drug Discovery Intelligence fit the bill – revealing connections between a drug, target, and disease that were not previously identified.

**Identifying promising compounds**

Using Cortellis Drug Discovery Intelligence, Quintana 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 now can test them on the rare disease target protein, using the synthesis pathways described in Cortellis Drug Discovery Intelligence. Employing this approach will significantly shorten the development time and reduce both risk and cost.

Unlock the hidden insights in data with Cortellis Drug Discovery Intelligence

- **Save time** leveraging the most comprehensive pipeline data
- **Increase confidence** in making R&D and go/no-go decisions
- **Apply integrated** biology, chemistry and pharmacology data
- **Utilize in-depth** scientific expertise – built by scientists, for scientists

For more information on how Cortellis Drug Discovery Intelligence can help you accelerate drug development, visit our website at:

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