

Validation of Novel Drug Targets for Neurodegeneration



Contact

Matt Keon
Co-founder

Website

<https://genieus.co>

Industry

Biotechnology

Location

Sydney, Australia

Employee count

5-10

Overview

GenieUs Genomics is an Australian biotech company exploring a novel drug discovery approach to neurodegenerative diseases. GenieUs utilises an integrated machine learning approach to analyse patient sequencing data and generate detailed molecular maps which enables the company to identify potential targets unique to a given patient. These targets can then be translated into autologous cell models and ultimately into humans, developing bespoke biological therapies.

GenieUs also envisions usage of its unique technology for additional applications such as biomarker discovery, drug stratification and biobanking analysis.

Challenges & Objectives

GenieUs focuses on an unbiased approach for the discovery of combination therapies. Treating each neurodegenerative disease uniquely, GenieUs is different from the traditional single target for single disease approach prevalent in the existing biotech market.

Generating multiple therapies for each different patient and disease subtype is a challenging undertaking. Sequencing errors appear frequently in their genomics pipeline and therefore highly reliable target validation becomes critical for drug discovery success. One of the key challenges for GenieUs has been to find the right partners to scale its validation pipeline in a timely and efficient manner. Working with academic labs came with longer waiting times, high overhead costs and unpredictable timelines.

Action & Results

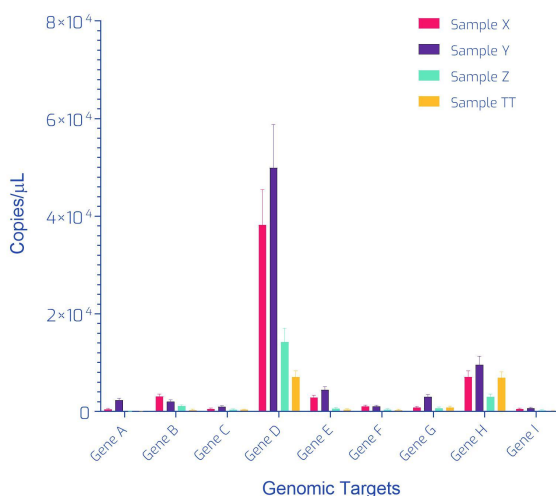
GenieUs looked for two crucial factors while finding the right partner: speed of data generation and critical scientific project oversight. Arctoris' expertise and instant availability of validation assays made it the right collaborator for GenieUs. Working with Arctoris, GenieUs was rapidly able to progress the different mutations from its Structural Variants project into the validation phase - critical for accelerating its drug development pipeline. In a second project, a set of patient samples were analysed using digitalPCR, providing GenieUs with highly sensitive readouts on the gene expression changes.

The Arctoris team worked very closely with GenieUs, providing scientific input during hypothesis generation, experiment planning and execution as well as during the data interpretation phase. GenieUs benefited from the timely generation of data presented in an accessible and informative way, being directly integratable into their internal workflows. Support provided by Arctoris in data interpretation helped GenieUs make critical go or no-go decisions in a timely manner.



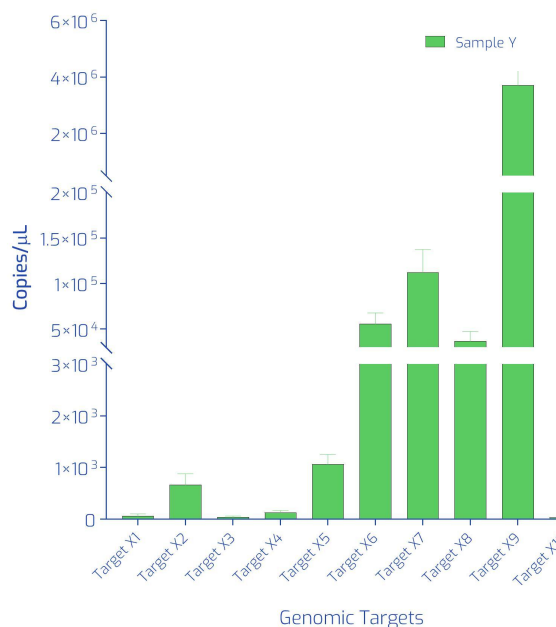
“Time is of the essence for a biotech company like us. Additionally, critical and open thinking is crucial. Working with Arctoris, we found both right from our first project brief on throughout the whole project.”

- Matt Keon, GenieUs, Co-founder



Differential miRNA expression levels.

Absolute copy number of miRNA targets extracted from PBMCs of individuals enrolled in the clinical study was determined using digital PCR. Error bars denote standard deviation.



Differential gene expression using digital PCR.

Figure depicts the absolute copy number of mRNA targets in samples taken from healthy and affected individuals enrolled in the clinical study. Error bars denote standard deviation.

Value

Arctoris' core scientific knowledge, readily available assays and reliable data generation enabled GenieUs to reliably validate key targets from its pipeline and accelerate their development. Even during the COVID-19 pandemic, GenieUs was able to continue their validation work without any disruptions. Looking forward, GenieUs will continue working with Arctoris to advance its genomics-powered therapeutics pipelines.