



Progressing Alzheimer's Therapy

Preclinical safety evaluation of a small molecule ligand that promotes neuron survival and protects against neuron degeneration

INTRODUCTION & BACKGROUND

Alzheimer's disease is the most common form of dementia, with an estimated 26 million sufferers worldwide. A number of treatments have been developed to provide some symptomatic benefits, but no therapies that halt the progression of the disease have been developed to this point. More than 500 clinical trials have been conducted on compounds with the goal of identifying a possible cure.

CHALLENGE

With so many failures in finding a drug that shows the ability to slow or halt the disease progression in clinical trials, and increases in prevalence of the disease across a globally aging population, there is great pressure to find effective therapies and correlate market opportunities for successful advances of pharmaceutical or biotech companies.

APPROACH & EXECUTION

Ricerca's objective was to help the Sponsor get the compound to the clinic as quickly as possible. We designed a preclinical safety evaluation that included production of the API, evaluation of off-target activity, a hERG study, oral safety pharmacology studies, PK evaluations, transporter evaluation, comparative in vitro metabolism, CNS penetration, protein binding, p450 inhibition, urinary excretion, a series of single-dose and repeat-dose toxicology studies, and a complete genotoxicity panel of studies.

RESULTS

This effort provided our client with all of the required data to submit for FDA approval of an IND application and initiation of Phase I clinical trials in healthy volunteers.

IMPACT

- Clinical safety studies were able to begin on the shortest possible timeline.
- The ligand had a robust preclinical safety profile supported by extensive data that would benefit the project through all subsequent phases, with the goal of becoming a successful, efficacious commercial therapy.

TESTIMONIAL

"Ricerca proved to be a very knowledgeable, capable partner in the development of our candidate. The scientific teams communicated openly and helped to overcome challenges quickly and get us into the clinic."

– Client Attribution