



Case study: Preclinical data analysis

Challenge

In a preclinical study on Alzheimer's disease, we sought to evaluate two promising treatments (Mirdametinib and IGF-1¹) to correct the deficit related to the shank3 protein in a specific mouse model (3xTg-AD-Shank3^{Δex4-9}). It was critical to determine their effectiveness before progressing to costly clinical studies. My role was to oversee the entire process: from data collection to interpretation, ensuring the results were reliable and actionable.

Key Objectives

- Evaluate whether Mirdametinib and IGF-1 could effectively increase Shank3 levels in the mouse model.
- Avoid unnecessary investment in ineffective treatments.
- Generate robust, actionable data to guide the design of future clinical studies.

Approach

1. The first step consisted of extracting proteins from brain regions (cortex and hippocampus) to quantify them using western blot and ELISA.
2. The second step involved organizing the raw data in a spreadsheet (Excel) and then transferring them into SAS JMP for rigorous processing. This included data cleaning (e.g., duplicates and entry errors), identifying variables (categorical vs. numerical), and normalization against a reference protein.
3. Data distribution was assessed to determine whether parametric tests (ANOVA, Student's t-test, etc.) or non-parametric tests (Kruskal-Wallis, Mann-Whitney, etc.) should be used. Comparisons were then refined with appropriate post hoc analyses.
4. The results were visualized using GraphPad Prism to provide a clear and immediate view of emerging trends.

Results and Impact

The overall findings revealed that none of the pharmacological treatments tested had a significant effect on brain Shank3 levels. This allowed us to rule out an ineffective avenue and redirect efforts toward alternative therapeutic strategies. By transforming complex data into clear, actionable insights, I contributed to an informed strategic decision and the opening of new therapeutic directions.

¹ insulin growth factor-1