

DISCOVERING THE EFFICACY OF A NEW DRUG

FOR PHARMACIA & UPJOHN

After investing heavily to investigate a new potential drug, the compound was not passing the statistical tests required by the FDA. Pharmacia & Upjohn invited Elder Research to examine the data and determine the drug's viability. The research discovered a real effect, and these decisive results were communicated to decision makers using a novel visualization technique.



INDUSTRY

- » Pharmaceutical

BUSINESS NEED

- » Determine if a new drug was viable before investing \$1 billion to complete the FDA application process

SOLUTION

- » Used rigorous data science and a novel visualization technique to determine that the drug showed a marked positive effect compared to a placebo

BENEFIT

- » Determined the drug had a very positive effect
- » The drug became a huge business success and benefitted millions of patients

THE CHALLENGE

Pharmacia & Upjohn was at a crossroads. The company had invested hundreds of millions of dollars investigating a new potential drug to treat a mental ailment, and they had zeroed in on a compound that showed promise. To be approved, the compound had to be significantly more effective than a placebo — where patients react physically to a treatment with no compound. (In fact, the “placebo effect” is so powerful that some Pharmacia & Upjohn scientists believed it accounted for 70% of the efficacy of their existing drugs.) Crushingingly, after months and \$10 million invested in a double-blind trial with 1,000 patients, the compound was not passing the statistical tests the FDA would use. To go forward with the multi-year FDA application process would require another billion dollars of investment — an amount so large, they'd have to team with a larger rival company to proceed. The meeting with the potential partner to make the investment decision was looming in several weeks, and the mood was grim.

The research manager at Pharmacia & Upjohn respected Elder Research's skill in data analysis and urgently invited us to examine the data from a fresh perspective to determine the compound's viability. Perhaps astonishingly to those who prioritize domain experience over data science skill, the data had a dramatic story to tell.

THE SOLUTION

Pharmacia & Upjohn were evaluating the treatment's effect by three competing tests — all accepted in the academic literature. Yet all three were failing. To examine these results, Elder Research took three steps. First, we determined there was information in each test; they were correlated but sufficiently different to provide more information together than alone. Second, the time-critical business need was to make a rational investment decision on whether or not to go forward, not to pass the specific FDA tests. So, we focused on visualizing the effects of treatment, rather than on statistical significances. (While necessary for FDA approval, the statistical significance would arise naturally from the extensive drug trials that would follow the investment, if the compound had merit. Our job was to determine if it had valid potential.) Third, previous analyses had measured patient outcome on an absolute scale. However, distributions seemed to vary significantly by several factors, especially the country of the patient and doctor, so we focused instead on relative improvement (or worsening) after treatment.

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In Figure 1, the 500 patients who received the placebo are summarized in a three-dimensional density plot we custom-designed for this project. The 500 points do not appear as dots, as they would in a 3D scatterplot, but are instead summarized by density manifolds; that is, by “shrink wrapped” shapes that depict the 3D data quartiles. The outer red shape (sliced through so we can see inside it) is the smallest shape that contains three-quarters of the data. Inside it, the green shape (also sliced) is the smallest shape that contains half the data. Lastly, the two blue shapes contain the densest quarter of the data. All the points started at the origin in the center of the cube. Those that moved toward the upper-right corner got better (that’s the peak of all three tests), and those that moved toward the lower-left got worse (the nadir of all three tests). Examining the density, a small group of patients moved up very noticeably, but an apparently larger group got somewhat worse.

By way of contrast for the drug, as shown in Figure 2, the movement toward getting better (upper-right) is very strong and not countered in the data depicted. Clearly, the compound is having a very positive effect.

RESULTS

When shown the plots and the research behind them, Pharmacia & Upjohn moved forward confidently and partnered with Pfizer (who later acquired them) to make the \$1 billion investment. Years of further work proved the drug’s efficacy to the regulatory agency; it was approved, adopted, and a blockbuster success. Such an outcome is so rare that it was one of only three drugs the firm created in that decade.

The data science triumphs were discovering a real effect where experts in the domain had been stymied, and communicating decisive results to decision makers with a novel visualization. Ultimately, the client experienced a huge business success and millions of their patients benefited from improved treatment. A great success for all!

CUSTOMER INFORMATION

The Upjohn Company, a pharmaceutical manufacturing firm founded in 1886, and Pharmacia, a pharmaceutical and biotechnological company in Sweden, merged in 1995. In 2003, Pfizer acquired Pharmacia, making Pfizer the world’s leading research-based pharmaceutical company.

ABOUT ELDER RESEARCH

Elder Research is a recognized leader in the science, practice, and technology of advanced analytics. We have helped government agencies and Fortune Global 500® companies solve real-world problems across diverse industries. Our areas of expertise include data science, text mining, data visualization, scientific software engineering,

and technical teaching. With experience in diverse projects and algorithms, advanced validation techniques, and innovative model combination methods (ensembles), Elder Research can maximize project success to ensure a continued return on analytics investment.

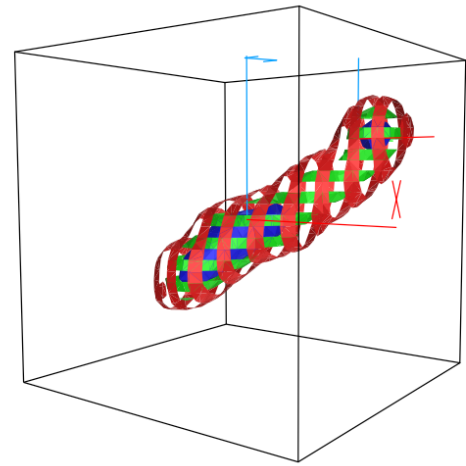


Figure 1. The Non-Effect of the Placebo: Balanced masses of results moved from the origin (cube center) to the best response corner (upper-right) and the worst response corner (lower-left).

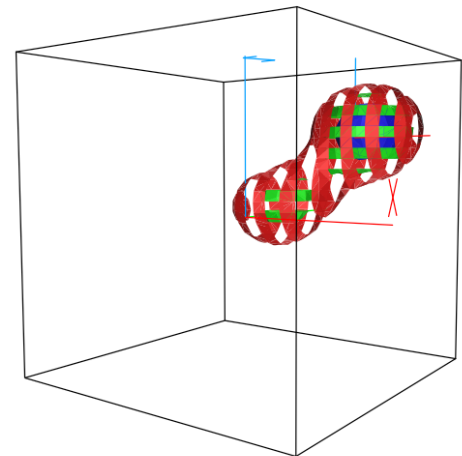


Figure 2. The Strong Effect of the Drug: All the patients depicted (the densest 75% of those taking the compound) got mildly to very much better, as they moved from the origin toward the best corner (upper-right).

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