A practical example of application

This example focuses solely on the statistical aspect of a clinical investigation. The study design intentionally includes certain suboptimal elements to provide valuable insights. The general scientific objective is to measure the effectiveness of a newly developed drug in reducing LDL cholesterol levels in a group of male patients aged 50-55 years. The 20 subjects are randomly divided into two subgroups of 10, labeled "test" (who receive the drug) and "control" (who receive a placebo).

Protocol

Compatibility: E1 protocol ($\alpha_1 = 0.250$, $\alpha_2 = 0.125$, $\alpha_3 = 0.063$, $\alpha_4 = 0.032$, $\alpha_5 = 0.016$.), see Table 1.
Effect size (mg/dL): detrimental [1, + ∞[, null [-3, 0], low [-7, -4], medium [-14, -8], large [-30, -15], hazardous]-∞, -31].

Model specification

Cholesterol levels are measured before treatment and four weeks after initiation (Table 2).

Test group (mg/dL)		**Control group (mg/dL)**	
Before	After	Before	After
196	170	146	141
212	198	156	160
154	150	184	177
212	200	170	172
187	182	220	209
182	174	196	201
126	135	169	171
167	161	220	214
157	159	180	179
159	140	213	210

Table 2. LDL cholesterol values of the test and control groups before and after drug administration (true and placebo, respectively).

A Welch t-test will be applied to the two "after – before" difference datasets of the test and the control groups. The data are highly compatible with the hypotheses of normality and absence of outliers (Figure 2). The initial conditions are similar in light of the research objective: in the "test vs. control" comparison, we have an average value (SD) of 175 (28) mg/dL vs. 185 (26) mg/dL.

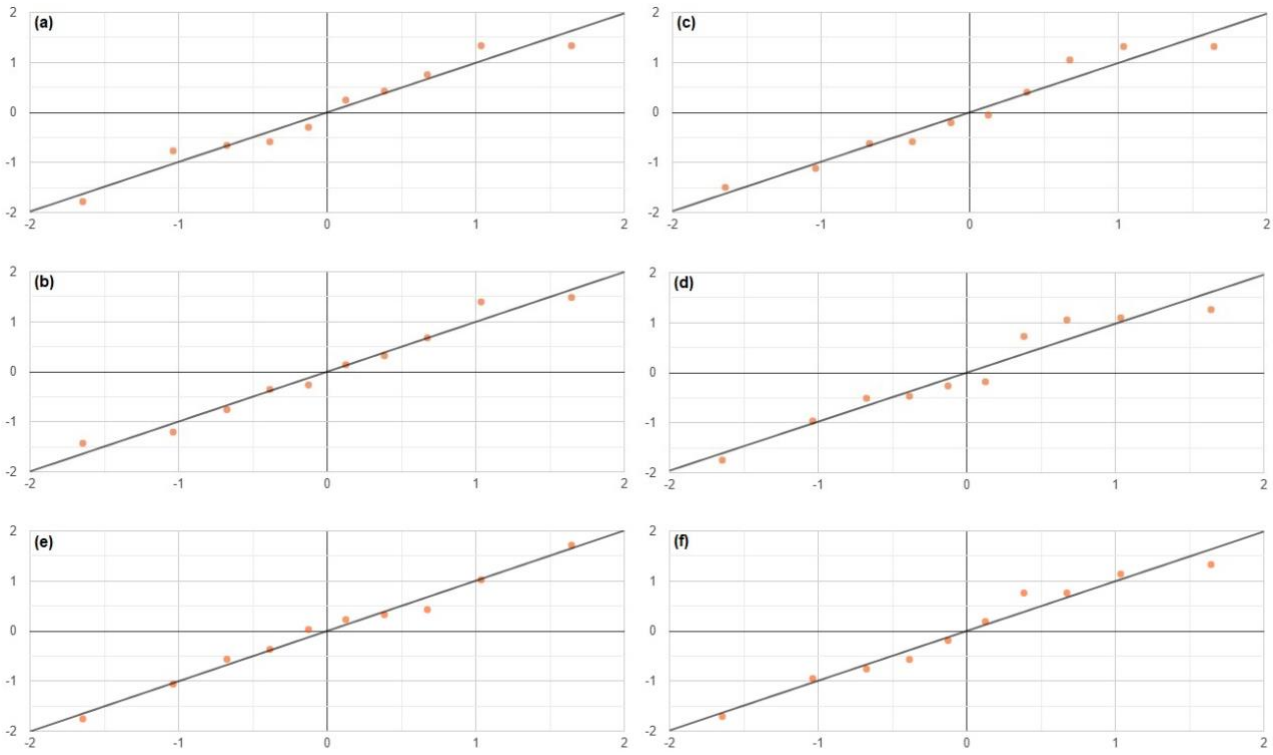

Figure 2. Q-Q plots for test group before (a), test group after (b), control group before (c), control group after (d), test group difference "after – before" (e), and control group difference "after – before" (f). Image generated with Statistics Kingdom (n.d.).

Statistical result

According to the research protocol, the results showed a marked conditional compatibility with the hypothesis of a small/medium effect (experimental mean change = -6.3 mg/dL, Figure 3). As assessed by the Welch's t-test, the P-values associated with point hypotheses ranging from -11 mg/dL to -2 mg/dL were greater than 0.25 (marked compatibility, according to the a priori protocol). Detrimental hypotheses of an increase in cholesterol showed moderate or lower compatibility ($P \leq 0.06$). Therefore, these findings are in line with a non-harmful average effect. However, other scientifically relevant hypotheses concern the small sample size, which is plausibly unable to adequately represent the variety of clinical scenarios within the entire target population.

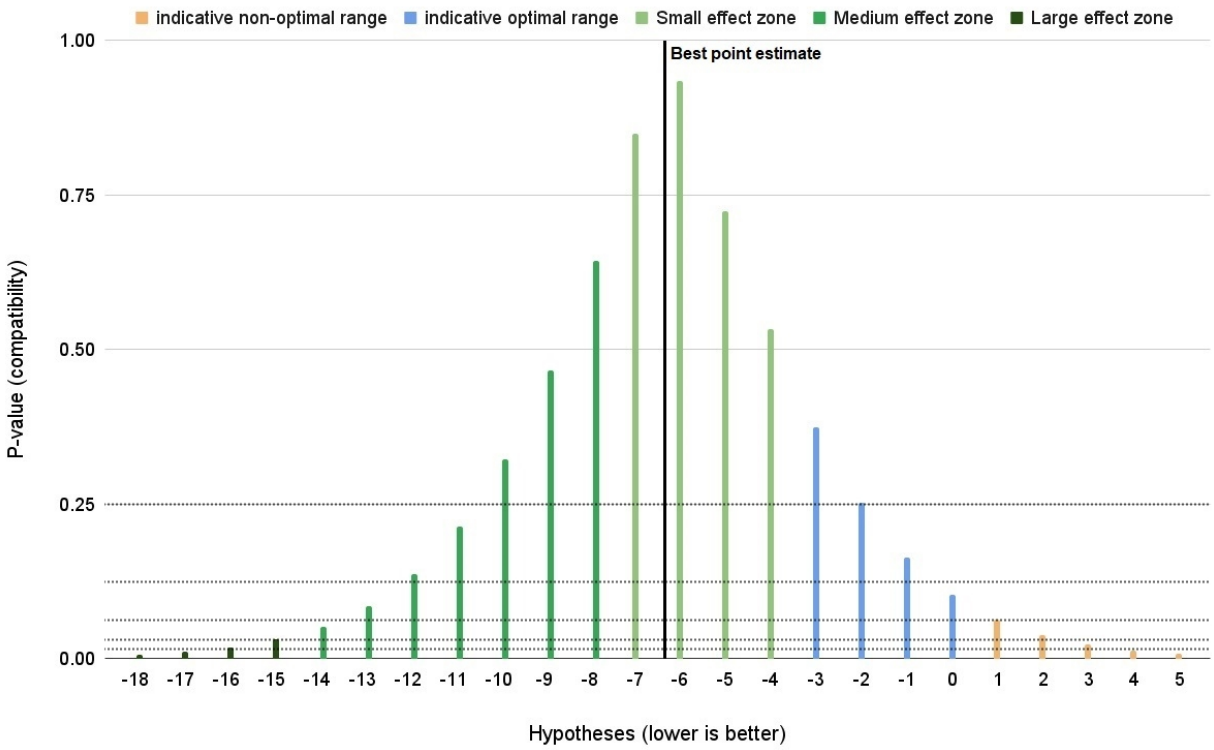

Figure 3. P-value discrete distribution: conditional compatibility between various hypotheses of average reduction in LDL cholesterol levels (mg/dL, horizontal axis) and the experimental result (black vertical line). The horizontal dotted lines represent the compatibility gradation thresholds from $\alpha_1 = 0.250$ to $\alpha_5 = 0.016$ according to the established protocol.

Conclusions

If the follow-up period confirms the absence of serious side effects, these findings could justify further research.