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TARGETED TREATMENT FOR CUTANEOUS LEISHMANIASIS

The Statistician's Role in Drug Development

Explained by Professor Neal Alexander

Development of a new drug goes through three clinical phases: **Phase 1** determines whether and at what dose the new drug is safe to use in human volunteers before going to **Phase 2** where the efficacy / therapeutic effect will be tested on patients. Does the drug have side effects when used by patients? **Phase 3** assesses the efficacy, the effectiveness and the safety in a larger group of patients.

In all of these phases the statistician plays a crucial role.

"**The statistician is your lawyer**," is how Marvin Zelen, a pioneer of quantitative methods in drug development, explained the statistician's role, in a seminar I attended in the London School of Hygiene and Tropical Medicine. His point was that both statisticians and lawyers have a responsibility to ensure compliance with established principles and norms.

A statistician should ensure that the design and analysis of a drug trial will stand up to sustained scrutiny. This includes specifying a number of trial participants sufficient to meet its objectives, and the method for them to be allocated between different drugs, e.g. by randomization.

The statistician is also responsible for specifying, and then carrying out, **data analyses which will yield valid conclusions**. For example, much of my own work has been on parasitic diseases, which tend to cluster heavily in a few people. This means that the data have "long tails", rather than a bell curve, and the statistical methods need to be chosen accordingly.

Overall, "The statistician must be instinctively and primarily **a logician and a scientist** in the broader sense, and only secondarily a user of the specialized statistical techniques", to quote Malcolm Rorty, a former President of the American Statistical Association. It's this logical mindset that the statistician applies to the process of drug development.

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