STATISTICS POWERS CLINICAL TRIAL EVALUATION

STATISTICAL SIGNIFICANCE

The complexity of chronic disease lies in two aspects: (i) the multi-state property with different progression; (ii) the non-spontaneous resolvent and rare complete cure. As a result, the treatment strategy is usually state-specific and aims to prevent the progression between states. Statistics evaluates treatment effectiveness at different stages and compare different treatments at the same stage, which significantly aids in developing clinical trials, reducing prevalence of chronic disease, and improving health care's outcomes.

Why are complex chronic diseases challenging?

According to a 2022 report from the Centers for Disease Control and Prevention (CDC), "six out of ten Americans live with at least one chronic disease, such as heart disease and stroke, cancer, or diabetes, which are the major causes of mortality and disability in America." Numerous clinical trials are carried out annually all over the world to identify possible risk factors for the advancement of chronic diseases and to identify novel treatments to halt or delay the progression.

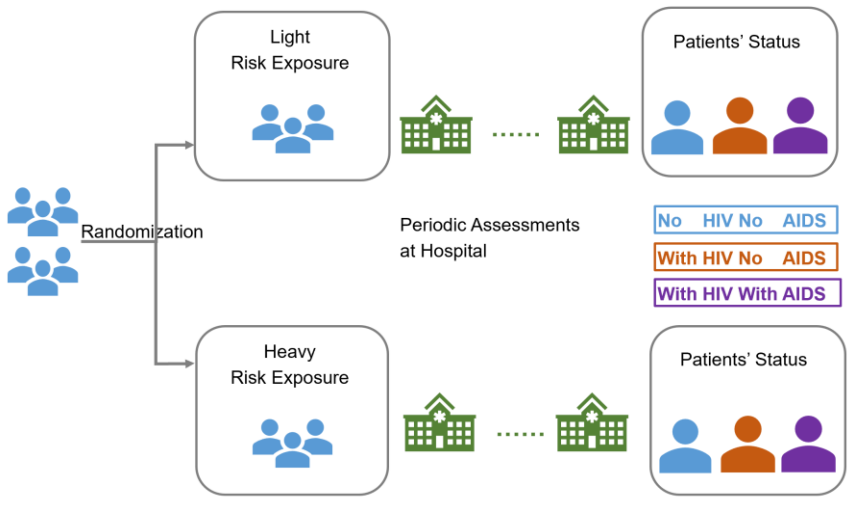
When doing the clinical trial analytics, one of the main challenges is the unclear and complex association between two progression stages. The incorrect specification of such dependence raises the risk of inaccurate analysis results, which may further deceive the public with inaccurate suggestions, unreliable drug development assessments, and improper patient treatment decisions. Another significant problem is that, as a result of patients' routine hospital visits, the disease stage transition is only known to occur between the patients' two assessment time points rather than being precisely observed. This ambiguity makes it more challenging to estimate the likelihood of transition at any time point.

"Chronic disease in America" Source: Centers for Disease Control and Prevention (CDC)

Chronic Disease Treatment Evaluation

How statistics addresses challenges?

To demonstrate the abovementioned challenges, a key statistically significant assessment criterion is based on how the probability curve for moving on to the next stage changes over time. For instance, individuals who had been exposed to the human immunodeficiency virus (HIV) were all at risk of contracting HIV and developing AIDS. Using statistics can create curves to represent the chance of getting HIV after being exposed to a risk factor and the likelihood of surviving from the time AIDS first manifests until death may both be represented. These curves can then be used to demonstrate how exposure risk affects how long it takes for an individual to get AIDS as well as how treatment affects that time frame. Sieve approximation and Monte Carlo calculation algorithms have been devised, along with statistical techniques like joint modelling and survival analysis.



Chronic Disease Example: HIV-AIDS clinical trial. Patients are at different disease progression statges at the end of study. The HIV infection time and AIDS development time are only known between two assessment time points.

Statistics improves chronic disease treatment

In randomized trials with discrete and continuous risk factors/treatments, the proposed method can be used to describe continuous change in the probability of reaching the next progression stage. The statistical tool produces reliable clinical trial analysis results by carefully taking into account potential ancillary data. These results are crucial for the future prevention and control of chronic disease as they provide significance to:

- healthcare providers with better understanding of chronic disease progression
- pharmaceutical companies with guidance for new drug testing development
- clinical researchers to draw reasonable and accurate inferences of clinical trial data for chronic diseases
- the authority, such as CDC and FDA, with support of guidance on chronic disease prevention and control