Abstract

This thesis work focuses on the development of statistical methods which permit to make use of available evidence to support decision-making. In particular, it deepens three research areas: the incorporation of historical data in early phase clinical trials, a novel method to perform adaptive screening in a certain sub-population and the comparison of different estimation methods in adaptive designs with time-to-event endpoints. Four methodologies are presented. The first one regards the incorporation of healthy volunteer data on receptor occupancy in a phase II proof of concept trial. The second one regards an analysis on the incorporation of preclinical animal toxicological data in a phase I trial. The third one, motivated by a case-study on a COVID-19 screening in a university community, regards a novel methodology to test adaptively whether a certain subpopulation proportion follows the same time evolution as the general population proportion. The last one is a comparison of different estimation methods to account for selection bias in adaptive enrichment designs with time-to-event endpoints.

These methodologies are valuable quantitative tools to include available evidence to support decision-making. They have strong theoretical foundations and have been tested in real life case studies. Moreover, they can potentially be applied to a variety of other problems and provide useful tools that can help to make more accurate and informed decisions.