Precision medicine: Statistical model can calculate potential success of new drugs

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The aim of "precision medicine" is to devise individualised treatment strategies and therapies. Statistical methods play an important role in predicting the efficacy of drugs from clinical study data, based on patient characteristics. A research team from MedUni Vienna's Institute of Medical Statistics has now presented new mathematical methods that

efficiently identify the characteristics that are relevant for such predictions. These methods can also be used to calculate the range of statistical variation of these predictions.

We know that drugs do not have the same effect on everyone. Researchers in precision medicine are therefore conducting clinical studies to identify subgroups of patients who respond particularly well to the active agent and do not have an elevated risk of side-effects. This process is based on modern diagnostic techniques such as genome sequencing and molecular imaging.

The data derived from these studies will be used for statistical analyses to more accurately predict drug efficacy. This will involve statistical techniques to filter out relevant biomarkers from the plethora of data. These biomarkers are, for example, certain genetic mutations or laboratory values plus other patient characteristics such as age, gender or disease stage.

The biomarkers identified in this way can then be used to develop models to predict the subgroups of patients for whom treatment with a newly developed drug will be more effective than the standard treatment. For example, in cancer studies it is possible to predict in which patients a new drug might have a life-extending effect. So-called regression models and variable selection methods are used to do this. However, statistical predictions are always subject to a certain range of variation. The fewer data that are available from patients participating in studies, the less accurate the prediction will be. An aim in medical statistics is therefore to minimise the range of variation to allow the most accurate possible prediction of the efficacy of a particular treatment.

The recently published research paper describes the design of new statistical prediction methods to be used in the development of new drugs. Based on clinical studies, it is possible to use these algorithms to

identify relevant biomarkers and to assess the statistical reliability of predictions. This means that, when developing new drugs, it is possible to predict more accurately the patient subgroups for whom treatment will be effective and safe. This is an important step towards improving the reliability of predictive models in precision medicine and assisting the development of individualised treatments.

More information: Nicolás M. Ballarini et al. Subgroup identification in clinical trials via the predicted individual treatment effect, *PLOS ONE* (2018). DOI: 10.1371/journal.pone.0205971

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