Institute for Health and Society, Medical College of Wisconsin

MEDICAL COLLEGE OF WISCONSIN

Datum

NEWSLETTER
Division of Biostatistics

CTSI
ADVANCING HEALTH THROUGH RESEARCH AND DISCOVERY

Personalized Medicine with Medical Big Data

Ying Liu, PhD, Division of Biostatistics, MCW

Personalized medicine is the practice of tailoring medical decisions and interventions to the individual patient based on his or her predicted response or risk of disease. Personalized medicine has the goal of delivering the right treatment to the right patient at the right time. There are two aspects of developing a personalized treatment plan: the first aspect is to tailor the treatment according to patient characteristics, and the second aspect is to adapt the treatment to changing conditions over time.

Firstly, from the personalized medicine perspective, clinicians can tailor the treatment plan based on information from various sources such as patient demographics, health history, previous treatments, symptoms, etc. With the advance of technology, additional types of medical data are now available such as brain images, genetic information, and activity tracking data. This large collection of patient information, medical big data, can be used to make a more specific patient diagnosis and create a customized treatment plan.

For example, psychiatry disorders are traditionally classified by symptoms, but the classification is not exclusive. Anxiety and depression have some similar symptoms (nervousness, irritability, and problems sleeping and concentrating) and there is evidence that many people suffers from both disorders and have different degrees of anxiety and depression at the same time. Furthermore, the treatments for depression and anxiety are similar, and in many cases, the therapy can be tailored to an individual so that it works to reduce the symptoms of both disorders. Medications to treat the symptoms of both disorders include selective serotonin reuptake inhibitors (SSRIs) and serotonin norepinephrine reuptake inhibitors (SNRIs). Behavioral interventions are also similar for these two disorders. The symptoms and treatment are similar and the causes of the diseases are also correlated. The traditional classification of these two disorders may not be the most informative way to label patients and classify them to the optimal treatment. The emergence of brain imaging data and analysis approaches has opened doors to finding new characteristics based on brain functioning to better classify patients into more specific sub-categories and treat them accordingly. The treatment suggestion then can be based on these patient characteristics regardless of the classical disease model. Researchers are currently trying to identify biomarkers in brain imaging that will moderate the treatment effect; clinicians would use this information to select the best treatment for a patient with certain biomarkers. An example is EMBARC study (http://embarc.utsouthwestern.edu/), which focused on mapping depression medications to patients based on characteristics including EEG, fMRI, MRI, genetics, and clinical measurements.

The second aspect of personalized medicine is to modify the treatment over time. Medical treatment does not only require a decision at the time of diagnosis, but is rather a continuous decision making process. There are multiple critical decision points when the treatment needs to be re-evaluated, such as disease relapses, development of comorbidities, deterioration or alleviation of the disease symptoms, and the disease progression and staging. At each critical decision point, the clinician needs to make a timely decision to adjust the treatment plan according to time varying patient features. We define this sequence of decision making process as a dynamic treatment regime. The Sequential Multiple Assignment Randomized Trial (SMART) design, which requires randomization of patients at each critical decision point, was developed specifically to make inferences about dynamic treatment regimes. A SMART study can be designed to compare different decision paths and find the optimal decision path for each patient according to important biomarkers.

The emergence of medical big data gives rise to both opportunities and challenges for medical researchers. Medical big data allows clinical investigators from all medical disciplines to raise new research questions. Some examples of novel questions are: predicting the symptom and recovery of stroke patients using MRI images, finding biomarkers to decide the best dosage of chemotherapy for each patient, and personalizing the diagnosis for early stage detection of Alzheimer disease using a sequence of biomarkers. Big data is also challenging to work with from a data management and data analysis perspective. Statisticians face the central problem of finding the moderator of treatment in high dimensional features. Identifying biomarkers which are reproducible and intuitively acceptable to medical experts is an even more challenging problem. Modern statistical learning and machine learning methods such as penalized regression and classification were developed to capture the true signal in high dimensional features. Advances in algorithms and distributed computing enable us to analyze high dimensional and large scale data more efficiently. The collaboration between clinicians and statisticians is essential in fulfilling the promise of personalized medicine to improve patient care.