Statistics and Precision Medicine

What is Statistics and What is Precision Medicine (PM)?

- Statistics is the science of learning from data, and accounting for relevant uncertainties. As such, it permeates the physical, natural, and social sciences, as well as public health, medicine, business, and policy.
- PM can be broadly framed as using a patient's specific characteristics in determining the best possible treatment for that individual at a given time under the given circumstances. PM complements traditional evidence-based medicine implemented by physicians by incorporating the increasing body of knowledge—including highly accurate laboratory and imaging diagnostics and epidemiological data sets—about individual forms of disease, individual responses to treatment, and new forms of intervention.

Precision Medicine Challenges of Statistical Nature

- Complex data challenges arise in a broad spectrum of PM activities, ranging from genomic medicine to comparative effectiveness research. As such, many of the statistical challenges of Big Data apply.
- When used for developing treatments and therapies, the analyses of large data sets should recognize that empirical correlations may not reflect cause and effect. The latter is investigated using a combination of substantive knowledge and statistical techniques called "causal inference".
- As each individual is unique, PM is inherently based on knowledge obtained from smaller pools of individuals, making inference much more difficult.
- It is essential to ensure the representative nature of a large cohort of subjects for precision medicine, in order for the results to generalize beyond the initial source of information.

Statistical Objectives in Precision Medicine

- Develop methods to incorporate rigorous statistical analyses of large datasets into decision analyses tailored to the individual patient.
- Develop better methods for validation of risk prediction models, and facilitate sharing of data so that investigators can validate their models.
- Develop solid foundations for drawing rigorous inferences from electronic health records and other large datasets.
- Develop data sharing approaches that facilitate learning from the small and highly dispersed set of cases similar to the patient for whom a decision is to be made.
- Develop methods to efficiently monitor and validate the precision of laboratory diagnostics and diagnostic imaging.
- Develop statistics curricula that teach the modeling and analysis procedures specifically addressing patient care and broader health outcome issues under increased emphasis on individual patient data.

Roles of Statisticians on Precision Medicine Teams

- Statisticians develop approaches to optimally collect and use data to inform which treatment is best for a given individual at a given time under the given circumstances.
- Statisticians translate a medical question into a precise data-based question. This includes carefully describing data structure, the underlying system that generated the data and what the team is trying to assess or predict.
- Statisticians develop data analytics for the health scientists to test and further develop theories in order to develop more informative diagnostics and more effective interventions.
- Statisticians enhance communication across disciplinary boundaries such as the computational, health, and behavioral disciplines.

The American Statistical Association (ASA) is a scientific and educational society of 19,000 members who serve in industry, government and academia in more than 90 countries, advancing research and promoting sound statistical practice to inform public policy and improve human welfare.