STATISTICAL SIGNIFICANCE

Precision medicine aims to maximize medical outcomes by tailoring treatments to the individual characteristics of each patient. In complex, heterogenous diseases, precision medicine strategies that harness and integrate knowledge from individual patients and populations have great potential to improve risk estimation and tailored decision making. Examples of utilizing statistical tools to optimize patient care in a complex rheumatic disease are illustrated below.

Personalized risk estimates in complex diseases

Risk prediction in complex and rare diseases:

It is a major challenge to accurately assess risks of critical events in chronic, multi-organ diseases. For example, systemic inflammatory rheumatic diseases like scleroderma are complex with significant heterogeneity in disease course and the risk of different organ-specific complications. For scleroderma, severe organ involvement can result in early death, and there is a critical unmet need to identify patients at high risk of progression at an early stage of the disease. A major clinical goal is to identify patients who are most likely to progress, as this may provide a window of opportunity to intervene before there is irreversible organ damage.

How do clinicians assess risk for scleroderma patients?

In the clinic, physicians use cognitive skills to integrate information across multiple parameters and organ systems, factoring in a patient's prior trajectory and baseline risk factors, to make estimates about a patient's health state, risk for complications, and need for high-risk therapies. A key component of this mental process is that it is informed by a physician's prior experiences caring for patients with a similar expression of disease,

Precision medicine utilizes data collected from patients and population to produce precise personalized risk estimates and enable tailored treatments. Source: Johns Hopkins inHealth

and therefore is not generalizable across providers - particularly in a rare disease. Also, aggregating each patient's complex, longitudinal data for clinical use requires a tremendous time investment on the part of the treating provider.

Statistics aids medical decision making:

Common questions from the patients to physicians are: what is the current status of my disease; what is my future likely to hold; and how do I compare with other scleroderma patients? Statistics addresses these key questions by (i) aggregating patient-level, multisystem data in a format that is easy for clinicians to access, (ii) harnessing knowledge and known outcomes from other patients who share key clinical characteristics, and (iii) computing and displaying

personalized risk estimates for critical events to improve medical decision making.

Timely risk prediction is essential:

For scleroderma patients, cardiomyopathy, pulmonary hypertension, and interstitial lung disease are events with high morbidity and mortality. Complex statistical models can simultaneously produce individualized risk estimates of these events. To provide the best estimates for a patient, the model utilizes information in that patient's in multiple biomarkers and as well as those of other patients. These tools that generates timely risk predictions are essential because they (1) warn clinicians of higher risk in need of increased monitoring and interventions; (2) reduce concerns in patients at lower risk.