Personalized Medicine: A New Era in Endocrinology

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As no two people are alike, conventional systems of medicine, which are based-on disease-specific pathways regardless of the patient's genetic make-up, are not effective strategies. A large body of evidence demonstrates that symptomatic disease management and pharmacotherapy needs to be revolutionized to reduce the probable risk of drug’s adverse reactions, toxicity, and lack of therapeutic efficacy. In this respect, the US Food and Drug Administration (FDA) have recommended the use of approximately 100 drugs only after genetic testing. Moreover, 90% of drugs did not successfully pass through phases II clinical trials or FDA submission from 2007 to 2010 (1,2). Today, it is assumed that the huge variation in response to treatment comes from the differences in genetic context along with biological and environmental factors (3). Among this factors, the patient’s genetic make-up plays a pivotal role and is responsible for approximately 20% to 95% of different clinical outcomes which may lead to increased adverse events and health costs (2). Dramatic improvements in biotechnology over the past years has led to major accomplishments in uncovering the molecular basis of a variety of diseases. The human genome project was a turning point in clarification of the complexity of these biological pathways which allowed scientists to predict the susceptibility of the patients to specific diseases and optimize their response to pharmacotherapy (4).

The application of genomic analysis tools such as whole-genome single nucleotide polymorphism (SNP) chips, non-coding RNA, high-throughput screening, microarrays and next-generation sequencing (NGS) with lower cost has been increasingly embraced by the scientists. The emergence of personalized medicine (PM) is attributed to these achievements which promise the potential of new personalized methods to the accurate and cost effective treatment for each patient according to his unique molecular information and biomarkers (5-7). PM as a young but an increasingly growing and valuable area of individualized medicine is an integrated and coordinated treatment approach based on unique genomic profile, specific biomarkers and environmental information of each patient (4). Because of extraordinary significance of PM in understanding the mechanism of diseases to find the most effective health care strategies, its role has been highlighted to reshape traditional medicine.

In this issue, we hope to explore the great importance of PM as a predictive strategy in order to improve patient-specific diagnosis. We will give the readers a comprehensive overview of some aspects of PM including pharmacogenetics and ethical consideration of PM, as well as personalized cancer therapy, individualized regenerative medicine, endocrinology, etc. Pharmacogenetics as the study of genetic variations is responsible for different drug responses, helps to stratify patients based-on their unique genetic context and predicts how each person will respond to a particular medication. Therefore, pharmacogenetics as an evidence-based approach contributes to lower the risk of drug adverse reactions and promoted effectiveness and cost-effectiveness of medical interventions, leading to improved and optimized treatment strategy for a particular disease in accordance with individual’s specific genomic profile (1). PM that emphasizes on targeted therapy could be used in a variety of incurable disease such as cancers, Alzheimer's disease, cardiovascular diseases, endocrine disorders, etc. In terms of endocrinology, the molecular pathways, genetic and molecular analysis based on extracted data from different "omics" technologies such as genome-wide association studies, molecular biomarkers, and clinical phenotypes are the key components of multidisciplinary setting for the management of the endocrine diseases in the near future (7,8). It is time to apply valuable data acquired by genomics, transcriptomics and metabolomics methods to discover the fundamental principles underlying the mechanisms of complex diseases. All these novel genome-wide approaches are capable of providing comprehensive data which can reveal the unique genetic profile of each individual (7).

It could be concluded that the implementation of
PM in medicine, would result in targeted diagnostics and therapeutics based on unique genetic profile, specific biomarkers, molecular pathways, as well as environmental risk factors for each individual which could lead to more efficient and accurate tailored treatment with reduced adverse reactions. Furthermore, due to the potential of PM as a predictive tool to anticipate individual’s susceptibility to disease and their specific drug response, PM has been considered as an emerging evidence-based and cost-effective model for health care providers in order to prescribe the right treatment, to the right patient, at the right time.

References