EDITORIAL

Computational and Biological Methods for Gene Therapy

With the rapid development of sequencing technologies, it is more efficient to reveal the variations and expression levels of molecules in the genome, transcriptome, and proteome, which have greatly helped to expose the risk factors of human diseases. To achieve the goal, in addition to the biological research of the wet laboratory, more knowledge needs to be mined from a large amount of sequencing data. To this end, bioinformatics methods have been introduced to help researchers identify more etiological genes, biomarkers associated with clinical molecular subtypes and prognosis. Whereas, most of the methods focus on analyzing single omics data, only providing a partial reference to human diseases. Therefore, the major challenge is how to mine novel causal genes and phenotypes of diseases based on the fusing multi-level omics data using system biology approaches, improving the efficacy of gene therapy. Here, a Special Issue of Current Gene Therapy around the topic Computational and Biological Methods for Gene Therapy is proposed. In total, five outstanding works were presented in this thematic issue.

Yang et al. investigated the effects of HKC on DN in the SD rat model and its molecular mechanism. Their results showed that the rats treated with HKC had an improved general state and reduced creatinine, blood urea nitrogen and 24-hour urinary protein levels. The deterioration of renal function was delayed due to the treatment with HKC. They utilized HE staining to observe that HKC can improve histopathological findings in the kidney tissues of DN rats, including kidney fibrosis. Results of western blot and qRT-PCR showed that HKC can inhibit the expressions of SPARC in the rat model of DN. Therefore, their findings demonstrated that HKC inhibited SPARC level and had significant therapeutic effects on DN [1].

Zhao et al. used genes as the bridge to connect AD and miRNAs to infer the AD-related miRNAs. The semi-clustering method was used to avoid the difficulty of obtaining negative samples. They identified 257 potential AD-related miRNAs with a high AUC. Several case studies proved the effectiveness of their results. The method they proposed would be a useful bioinformatics tool to discover novel knowledge-based on huge data [2].

Liu et al. provided a solid piece of evidence to determine the causal relationship between infant length (IL) and type 2 diabetes (T2D) risk through a two-sample Mendelian randomization (MR) protocol. By screening 29, 150 and 2 genetic variants in Diabetes Genetics Replication and Meta-analysis (DIAGAM), they constructed suitable IVs and confirmed that shorter IL contributes no additional risk to T2DM using the inverse-variance weighted (IVW) method. This study innovatively uses MR for the identification of pathogenic phenotypes, which is fast and accurate, and provides a powerful method for the discovery of early prevention strategies and treatment targets for T2D [3].

Deng et al. proposed PCHS, an effective computational method to predict comorbidity using HeteSim scores. PCHS utilized the HeteSim measure to calculate the relatedness scores of different disease pairs across the global heterogeneous network. The prediction model was built using the support vector machine (SVM) based on the HeteSim scores. The results showed that PCHS performed significantly better than previous state-of-the-art approaches and achieved an AUC score of 0.90 in 10-fold cross-validation. Furthermore, some predictions have been verified in the literature, indicating the effectiveness of the proposed method [4].

Wu et al. combined experimental results on 50 singleton patients with CVM and the published literatures, identified SOX9 mutation (p.M469V) may contribute to CVM without other systematic deformities, which provided important implications and better understanding of phenotypic variability in SOX9-related skeletal deformities [5].

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