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Editorial

Progress and prospects in stem cell therapy and drug discovery

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During the past few years, we witnessed explosive progress in the field of stem cell research, leading to an increasing hope for more effective cell replacement therapies to treat various diseases. The self-renewal capability of stem cells, combined with their ability to differentiate into different cell types under programmed conditions, enables improved models for drug discovery and mechanism-based studies of diseases, hence laying the foundation for their therapeutic applications[1]. In fact, induced pluripotent stem cell (iPSC)-based transplantation therapy has already been performed in rodent models[2].

The examples of major progress are seen in the multiple fronts. Active small molecules have been identified to promote stem cell self-renewal, proliferation, differentiation and somatic cell reprogramming[3]. The effective applications of iPSC technology open a new path for cell replacement therapies and drug screening. Furthermore, patient-specific iPSCs and subsequently differentiated cells manifesting disease phenotypes allow reconstitution of human disease pathology in cell culture, thus offer precious opportunities to investigate human proteins in the native cellular context. The benefits of investigating human proteins provide the opportunities to evaluate both general toxicity for therapeutics and, more excitingly, to test drug effects under specific genetic background. In many ways, these options are more physiologically relevant and cost-effective than animal models.

This special issue includes eleven articles that highlight both new findings and emerging ideas in different aspects of stem cell technologies[4, 5], clinical therapy[6–10] and drug discovery[11–14]. The ensemble of these impressive articles provides an excellent reference map for the experts and a point of entry into this exciting research field for the novice. It is evident that stem cell research has evolved from a subject of basic experimental biology to a highly sophisticated, penetrating scientific establishment that is poised to catalyze translational medicine bringing research findings in laboratories to patient benefits in clinic.

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