

Concise review: modeling central nervous system diseases using induced pluripotent stem cells.

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Authors: Xianmin Zeng, Joshua G Hunsberger, Anton Simeonov, Nasir Malik, Ying Pei, Mahendra Rao

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Public Summary:

Induced pluripotent stem cells (iPSCs) offer an opportunity to delve into the mechanisms underlying development while also affording the potential to take advantage of a number of naturally occurring mutations that contribute to either disease susceptibility or resistance. Just as with any new field, several models of screening are being explored, and innovators are working on the most efficient methods to overcome the inherent limitations of primary cell screens using iPSCs. In the present review, we provide a background regarding why iPSCs represent a paradigm shift for central nervous system (CNS) disease modeling. We describe the efforts in the field to develop more biologically relevant CNS disease models, which should provide screening assays useful for the pharmaceutical industry. We also provide some examples of successful uses for iPSC-based screens and suggest that additional development could revolutionize the field of drug discovery. The development and implementation of these advanced iPSC-based screens will create a more efficient disease-specific process underpinned by the biological mechanism in a patient- and disease-specific manner rather than by trial-and-error. Moreover, with careful and strategic planning, shared resources can be developed that will enable exponential advances in the field. This will undoubtedly lead to more sensitive and accurate screens for early diagnosis and allow the identification of patient-specific therapies, thus, paving the way to personalized medicine.

Scientific Abstract:

Induced pluripotent stem cells (iPSCs) offer an opportunity to delve into the mechanisms underlying development while also affording the potential to take advantage of a number of naturally occurring mutations that contribute to either disease susceptibility or resistance. Just as with any new field, several models of screening are being explored, and innovators are working on the most efficient methods to overcome the inherent limitations of primary cell screens using iPSCs. In the present review, we provide a background regarding why iPSCs represent a paradigm shift for central nervous system (CNS) disease modeling. We describe the efforts in the field to develop more biologically relevant CNS disease models, which should provide screening assays useful for the pharmaceutical industry. We also provide some examples of successful uses for iPSC-based screens and suggest that additional development could revolutionize the field of drug discovery. The development and implementation of these advanced iPSC-based screens will create a more efficient disease-specific process underpinned by the biological mechanism in a patient- and disease-specific manner rather than by trial-and-error. Moreover, with careful and strategic planning, shared resources can be developed that will enable exponential advances in the field. This will undoubtedly lead to more sensitive and accurate screens for early diagnosis and allow the identification of patient-specific therapies, thus, paving the way to personalized medicine.

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