Enabling consistency in pluripotent stem cell-derived products for research and development and clinical applications through material standards.

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Public Summary:
There is a need for physical standards (reference materials) to ensure both reproducibility and consistency in the production of somatic cell types from human pluripotent stem cell (hPSC) sources. Techniques to identify "signatures" for therapeutically relevant cell types, such as neurons and cardiomyocytes that can be derived from hPSCs, would be of significant utility for clinical purposes.

Scientific Abstract:
There is a need for physical standards (reference materials) to ensure both reproducibility and consistency in the production of somatic cell types from human pluripotent stem cell (hPSC) sources. We have outlined the need for reference materials (RMs) in relation to the unique properties and concerns surrounding hPSC-derived products and suggest in-house approaches to RM generation relevant to basic research, drug screening, and therapeutic applications. hPSCs have an unparalleled potential as a source of somatic cells for drug screening, disease modeling, and therapeutic application. Undefined variation and product variability after differentiation to the lineage or cell type of interest impede efficient translation and can obscure the evaluation of clinical safety and efficacy. Moreover, in the absence of a consistent population, data generated from in vitro studies could be unreliable and irreproducible. Efforts to devise approaches and tools that facilitate improved consistency of hPSC-derived products, both as development tools and therapeutic products, will aid translation. Standards exist in both written and physical form; however, because many unknown factors persist in the field, premature written standards could inhibit rather than promote innovation and translation. We focused on the derivation of physical standard RMs. We outline the need for RMs and assess the approaches to in-house RM generation for hPSC-derived products, a critical tool for the analysis and control of product variation that can be applied by researchers and developers. We then explore potential routes for the generation of RMs, including both cellular and noncellular materials and novel methods that might provide valuable tools to measure and account for variation. Multiparametric techniques to identify "signatures" for therapeutically relevant cell types, such as neurons and cardiomyocytes that can be derived from hPSCs, would be of significant utility, although physical RMs will be required for clinical purposes.

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