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Lab Tour: Patient-derived Stem Cells in Precision Medicine

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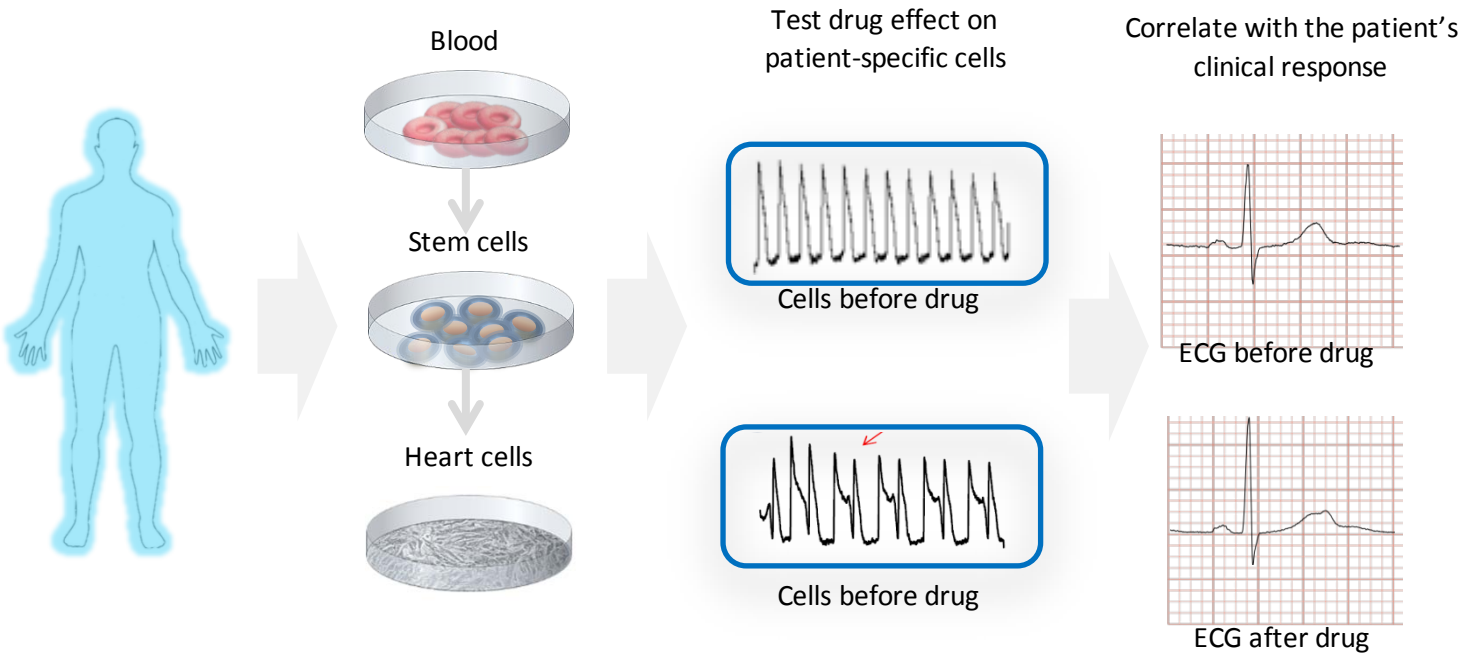

Blood cells are reprogrammed into pluripotent stem cells, which are then differentiated into heart cells. Researchers can then test specific drugs on these heart cells to see whether the cells respond with abnormal electrical activity patterns. We are studying whether this will predict whether the drug will cause abnormal heart rhythms as detected on an ECG.

What's the problem?
Individual patients respond differently to medical therapies. Precision medicine uses a diagnostic test to predict which patients will benefit and which may be harmed by therapies. In August 2015, we published a clinical study[1] to evaluate the ability of novel clinical electrocardiographic ("ECG") device biomarkers to predict the risk of drug-induced abnormal heart rhythms. However, these ECG methods can be used only after patients are exposed to drugs.

What's our approach in ongoing studies?
In our current research, we are evaluating the potential of new laboratory tests that use induced pluripotent stem (iPS) cells to predict how individuals in an FDA-sponsored clinical study respond to drugs that affect the heart. iPS cells can be derived from adult patient blood or skin samples and differentiated into almost any patient-specific cell type (e.g., heart cells, brain cells, and liver cells), carrying genetic and phenotypic characteristics specific to the patient. We are generating multiple patient-specific iPS-heart cells from the 20 participants in our published clinical study.[1] We will use these cells to determine whether the response of an individual patient's stem cells to a specific therapy can predict a personalized response—whether this can show us in advance how the patient will respond to the drug.

What's the potential public health outcome?
This work will advance precision medicine by determining whether a patient's stem cells derived from a small blood sample can predict how the patient will respond to therapies. This may foster invention of new precision medicine diagnostic tests that assess heart toxicity or other risks. In addition, this work will determine if clinical trials performed using stem cells studied in a petri dish work as well as current trials performed in human subjects. This could decrease costs and reduce risks to subjects to bring new therapies to patients faster. Questions? Contact FDA's Office of Media Affairs at 301-796-4540 or fdaoma@fda.hhs.gov.

Reference: [1] *Johannesen et al. Clinical Pharmacology & Therapeutics* 2015 (in press) doi: 10.1002/cpt.205