# FDA Improving Regulatory Oversight of Stem Cell Therapies and Regenerative Medicine Products

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Regenerative medicine is a burgeoning interdisciplinary research field aiming to offer new therapies that replace or regenerate human cells, tissues, or organs with the goal of restoring or establishing normal function. The field includes treatments using stem cells and tissue engineering, which is the use of biomaterials-based scaffolds, seed cells, and bioactive molecules to build biomimetic tissue-like constructs that can be implanted into the body to repair failing tissues and organs.

While regenerative medicine offers significant medical promise, it lacks a comprehensive or consistent regulatory framework. To address this issue, the Food and Drug Administration ("FDA") has announced that it is working to increase regulatory oversight and enforcement activity over regenerative medicine products to better identify fraud and provide a more efficient approval pathway for responsible product developers.[1]

Specifically, the FDA will "advance a comprehensive policy framework that will more clearly describe the rules of the road for this new field."[2] As part of this framework, the agency proposed adding to the list of products eligible for expedited FDA review under the Regenerative Medicine Advanced Therapy ("RMAT") designation. The agency also promised to give product developers "a very reasonable period of time to interact with the FDA in order to determine if they need to submit an application for marketing authorization and to come into the agency and work on a path toward approval."[3]

At the same time, the FDA is stepping up enforcement against developers and manufacturers subject to and attempting to sidestep more stringent regulations. For example, the FDA recently issued a warning letter to a Florida clinic for marketing stem cell products derived from patients' body fat without FDA approval and for significant deviations from current good manufacturing practice (or CGMP) requirements, including some that could impact the sterility of their products and put patients at risk.[4] The agency rejected the clinic's argument that its products qualified as human cells, tissues, and cellular and tissue-based products (or HCT/Ps) and could be manufactured and sold without pre-market approval. Similarly, the FDA seized vials of a rare smallpox vaccine being used by a California clinic to create unapproved stem cell treatments for cancer patients.[5] The agency has said these actions are just the beginning of its increased focus on entities that pose a danger to patients and/or overstep regulatory lines.

The FDA's announcement may present an opportunity for unprecedented agency-stakeholder collaboration in shaping a new regulatory framework for regenerative medicine products. This may be especially timely given the recent approval of Tisagenlecleucel (marketed by Novartis as Kymriah)—the first ever gene therapy to be approved in the United States. Kymriah genetically modifies patients' own T-cells to treat and potentially cure their cancer.[6] It is approved for children and young adults with relapsed or refractory acute B-cell lymphoblastic leukemia—a leading cause of childhood cancer deaths. The drug's price tag comes in at a whopping $475,000, but the

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manufacturer has reportedly agreed with The Centers for Medicare and Medicaid Services (or CMS) to only accept payment if a patient successfully responds to treatment within the first month of infusion.

Companies conducting research on potentially transformative gene therapies are likely to follow in Novartis's footsteps and seek FDA approval, so an improved regulatory framework, streamlined and cost-effective approval processes, and consistent enforcement activity to weed out bad actors will be more important than ever before.

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[1] Press Release, U.S. Food & Drug Admin., Statement from FDA Commissioner Scott Gottlieb, M.D. on the FDA's new policy steps and enforcement efforts to ensure proper oversight of stem cell therapies and regenerative medicine (Aug. 28, 2017).

[2] *Id.*

[3] *Id.*

[4] Press Release, U.S. Food & Drug Admin., FDA warns US Stem Cell Clinic of significant deviations (Aug. 28, 2017).

[5] Press Release, U.S. Food & Drug Admin., FDA acts to remove unproven, potentially harmful treatment used in 'stem cell' centers targeting vulnerable patients (Aug. 28, 2017).

[6] Press Release, U.S. Food & Drug Admin., FDA approval brings first gene therapy to the United States (Aug. 30, 2017).

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