FDA Announces Regulatory and Enforcement Policy Shift for Regenerative Medicine

On August 28, 2017, FDA Commissioner Scott Gottlieb announced a significant shift in the way the Agency intends to regulate stem cell therapies and other types of regenerative medicine. The detailed announcement is focused on two parallel priorities: the creation of a new regulatory framework to ensure that promising regenerative therapies make it to patients quickly, and increased enforcement against those who “make hollow claims and market unsafe science” to promote illegal products.

Stem cell therapies and other types of regenerative medicine currently fall within one of two distinct regulatory frameworks. Some products are regulated as human cells, tissues, or cellular and tissue-based products (HCT/Ps), which FDA regulates only under rules designed to minimize the transmission of communicable diseases.1 These rules, which have been in place for more than 15 years, apply to products that meet certain criteria, including that they are not more than “minimally manipulated” between the time they are retrieved from a donor and implanted into a recipient, and marketed for a use in the recipient that is “homologous” to the tissue’s function in the donor.

Any product that does not fit within that framework is subject to full FDA regulation as a drug, biological product, or medical device, and the product’s manufacturer must obtain an appropriate marketing authorization from FDA prior to marketing—a process that can take years and cost millions of dollars.

This “all-or-nothing” approach to the regulation of regenerative therapies has taken on new importance in recent years following the release of several draft guidance documents that have attempted to narrow the scope of products that are regulated solely as HCT/Ps and to require premarket review for additional categories of regenerative products and therapies.2 Those draft guidances have been controversial and were the subject of a two-day public hearing in September 2016.3

FDA’s announcement could, then, bring welcome clarity to manufacturers, clinicians, and other developers of regenerative therapies. The announcement suggests that the Agency will create a new framework through

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1 See 21 C.F.R. Part 1271.


3 FDA, Public Hearing; Request for Comments – Draft Guidances Relating to the Regulation of HCT/Ps (Docket No. FDA-20150D-3719).
a “comprehensive policy” that “will establish clearer lines around when these regenerative medicine products have sufficient complexity to fall under the Agency’s current authority, and then define an efficient process for how these products should be evaluated for safety and effectiveness.” FDA plans to roll out the framework via a series of new guidance documents in fall 2017.

An additional key aspect of the announcement for current manufacturers of HCT/Ps is that FDA plans to establish a compliance period and to “give current product developers a very reasonable period of time to interact with” the Agency while working toward approval. This marks an important shift in compliance policy, as the Agency has previously issued warning and untitled letters demanding the immediate withdrawal of HCT/Ps that the Agency believes should be subject to premarket review.

At the same time, the FDA announcement makes clear that the Agency intends to increase enforcement against manufacturers and product developers that pose risks to the public health. As examples, FDA announced two enforcement actions against developers of stem cell therapies: a warning letter issued to one developer alleging significant violations of FDA’s current good manufacturing practice (GMP) regulations, resulting in products that are unlikely to be sterile, and a seizure action against another involving vials of a rare smallpox vaccine being used to create stem cell treatments for cancer patients.

Overall, the FDA announcement is cause for restrained celebration by developers of stem cell therapies, who may now have a more streamlined and predictable path to market in the coming months, and by current HCT/P manufacturers who have faced significant regulatory uncertainty and uneven enforcement with respect to currently marketed products. All stakeholders will now keenly await the announced guidance documents to understand how FDA will implement this new regulatory framework.

If you have any questions regarding this Sidley Update, please contact the Sidley lawyer with whom you usually work, or

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