Introduction

Regenerative medicine has been a rapidly growing market over the past two decades, as doctors and pharmaceutical companies have developed increasingly innovative treatments that rely on human cells and tissues. According to a recent study from the Tufts Center for Study of Drug Development, regenerative medicine is poised to become a $68 billion worldwide market by 2020.

Meanwhile, both Congress and the FDA have signaled their intentions to make regenerative medicine a priority. The 21st Century Cures Act, signed into law in December 2016, laid the groundwork for an expedited review process for certain regenerative medicines. And FDA Commissioner Scott Gottlieb has said he wants to work closely with doctors and companies to move regenerative medicines through the approval process more efficiently. At the same time, Gottlieb has shown a willingness to go after companies that are flouting the FDA's regulations.

As with many emerging technologies, the regulatory landscape is still in a state of flux, with regulators attempting to keep up with marketplace innovations. But the FDA took an important step toward solidifying its regulatory regime in November 2017 when it finalized two important guidance documents that provide interpretations of key provisions of the agency's regenerative medicine rules.

Many regenerative products are being developed by independent doctors or small companies without either the financial resources or regulatory experience of the bigger players in the pharmaceutical industry. This makes it all the more crucial for these doctors and companies to fully understand the FDA's rules, and how to best stay in compliance with what can often look like a moving regulatory target.

The good news for these smaller players is that certain regenerative therapies deemed less risky by the FDA are subject to only minimal regulatory oversight, requiring only a registration process and assurances that the doctor is abiding by certain donor eligibility rules and the FDA's current good tissue practice guidelines. The bad news, however, is that these exempt therapies represent a relatively narrow band of the regenerative medicine world, and drawing the line between those that do and those that do not qualify can be tricky.

This report will walk readers through that line-drawing process, unpacking the FDA's recent guidance and analyzing the agency's thinking on key regulatory definitions. A number of concrete, real-world examples will be used to show how the FDA's thinking has evolved since it first began regulating regenerative medicine products in the late 1990s, and to understand where that thinking is today.

The report also answers a number of frequently asked legal and regulatory questions about bringing regenerative products to market, including the ins and outs of the IND and BLA processes, the advantages and disadvantages of conducting foreign clinical trials, and regulatory regimes outside the U.S. that may provide opportunities to get innovative treatments more quickly to market.

Significant portions of this report are based on recent FDAnews webinars conducted by Andrew Ittleman and Kelly Lightfoot. Ittleman is a founder and partner of Fuerst, Ittleman, David & Joseph, where he represents clients in the regenerative medicine field and other FDA-regulated industries. Lightfoot is a senior associate at Fuerst, Ittleman, David & Joseph, where she represents biologics manufacturers, clinical trial sponsors, medical device manufacturers, cryogenic banking facilities, medical tourism businesses and physicians engaged in the development of autologous stem cell therapies.