



Advancing a New Pathway for Regenerative Cell Therapy in the United States

Background

- Cell therapy—which involves the use of human cells to restore healthy function in the human body—represents one of the most promising areas for the next generation of groundbreaking treatments.
- Cell therapies show promise in the areas of cardiology, neurology, oncology, and ophthalmology. Thousands of clinical trials are now underway that are focused on cancer, heart disease, kidney disease, urologic diseases, trauma-related burns and wounds, and diabetes. Others are underway to address diseases for which there is no cure, such as Alzheimer's, multiple sclerosis, and Parkinson's.

The Challenge

- Despite the benefits of these treatments, cell therapies are generally not accessible to patients in the United States due to the Food and Drug Administration's (FDA) regulatory approach that has not kept up with scientific advancements.
- Increasingly, patients are forced to go to other countries to access treatments. U.S. companies are also increasingly moving to and making investments in other parts of the world, including Europe and Japan, who have modernized their regulatory frameworks to bring safe and effective treatments to patients.
- In December 2015, the Bipartisan Policy Center released the report, [*Advancing Regenerative Cellular Therapy: Medical Innovation for Healthier Americans*](#), which contains recommendations for advancing cell therapy in the U.S.

Today's Regulatory Approach for Cell Therapy

- Currently, there are only two pathways to bring cell therapies to patients in the U.S.
 - Under current law, a very narrowly defined set of treatments under the category of "human cells, tissues, and cellular and tissue-based products

(HCT/Ps)—regulated under section 361 of the Public Health Service Act—can be offered to patients with no pre-market review by clinics that follow requirements of 1271.

- All other HCT/Ps—those regulated under section 351 of the Public Health Service Act—are treated like drugs—even if they involve the use of a *patient's own cells*. They require a full Biologics Licensing Application, which may be appropriate for drugs and biologics, but not for human cells. For example, cells that are extracted in the doctor's office or from a cord blood bank for use in the same patient.
- As a result, since 2001 when the regulatory structure for HCT/Ps was created:
 - Only a handful of cell therapies have been approved by the FDA, using an approach traditionally used for chemical drugs, which may cost \$1 billion or more and take a decade or longer to come to market despite their effectiveness shown across numerous clinical trials.
 - There is a proliferation of more than 500 clinics offering cell therapies, as practice of medicine, some of which may require greater regulatory review and approval.
- The regulatory system for cell therapies is clearly broken and change is needed.

The Solution

- Informed by research, guidance from an expert panel, and discussions with multiple public and private sector organizations across the health care field, BPC [recommends](#) that a “middle ground” pathway be created for cell therapies.
- To create such a middle ground pathway for cell therapy, the FDA should be given the flexibility to use “expedited programs” that already exist under current law, for cell therapies. The FDA uses its discretion when deciding whether to apply expedited programs to drugs going through the approval process. In fact, the majority of drugs approved in 2014, 2015, and 2016 to-date were approved under these expedited programs.
- The FDA's Guidance, [Expedited Programs for Serious Conditions – Drugs and Biologics](#), provides a summary of its expedited programs, which are intended to facilitate and expedite the review of new drugs to address unmet medical needs in the treatment of serious conditions.

- Again, it is important to note that the use of expedited programs is at the sole discretion of the FDA. Giving the FDA the flexibility to use expedited programs for cell therapies does not force the FDA to do so. Also, under the law, expedited programs do not dictate the number of phases of clinical trials that a drug must go through—again, this is at the sole discretion of the FDA.
 - Regarding the definition of a serious condition, the guidance states the following, “whether a disease or condition is serious is a matter of clinical judgment, based on its impact on such factors as survival, day-to-day functioning, or the likelihood that if left untreated, will progress from a less severe condition to a more serious one.”
 - Because many regenerative cell therapy treatments hold the promise of reducing the need for artificial knees or hips by restoring cartilage, etc., which could be defined as “available therapies” to address a medical need, clarifications may be needed to address the unique aspects of cell therapies.
- There has been considerable policy interest in advancing regenerative cell therapies.
 - In March 2016, the bipartisan, bicameral [Reliable and Effective Growth for Regenerative Health Options \(REGROW\) Act](#) was introduced, essentially creating a middle ground pathway in the form of a “conditional approval program,” similar to laws recently passed in Japan and Europe.
 - In April 2016, the Senate Health, Education, Labor, and Pensions (HELP) Committee unanimously approved bipartisan legislation ([S. 2700](#)) that included provisions to lay the groundwork for a modernized regulatory framework for cell therapy, by calling for the establishment of standards to support development, evaluation, and review, and provide greater regulatory clarity and predictability.
- Providing the FDA with the authority to utilize an existing FDA expedited program (verse creating a new conditional approval program) to support a middle ground pathway, informed by such standards, provides a reasonable, stepwise approach toward improving the regulatory framework for cell and other advanced therapies.

Next Steps

- There is growing support among policymakers to advance a middle ground pathway, informed by new regenerative medicine standards, that gives the FDA flexibility and the authority to apply—at the FDA’s sole discretion—existing expedited programs, such as the accelerated approval program, for cell therapies.
- This incremental step represents a compromise approach that preserves the gold standard for safety and efficacy and will enable the U.S. to regain its leadership position on the innovative therapies that show great promise for people suffering with Alzheimer’s, cancer, heart disease, stroke, trauma-related wounds and burns, and many other debilitating diseases for which there is no cure.