



BIPARTISAN POLICY CENTER

## Advancing a New Regulatory Approach for Regenerative Cell Therapy

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 treatments in the areas of cardiology, neurology, oncology, ophthalmology, and orthopedics. There have been promising studies for those with Alzheimer’s disease, Parkinson’s disease, heart disease, diabetes, and cancer.

Despite the benefits of these treatments, cell therapies are generally not accessible to patients in the United States. This is due to an outdated regulatory framework that treats the regulation of cells – even from one’s own body – as drugs, requiring on average more than \$1 billion and 10 to 12 years before a therapy can get to market.

In December 2015, with guidance from a panel of scientific and academic experts, the Bipartisan Policy Center released recommendations to address this problem. BPC’s report, [\*Advancing Regenerative Cellular Therapy: Medical Innovation for Healthier Americans\*](#), calls for the advancement of a new regulatory pathway that will accelerate the approval of cell therapies shown to be both safe and effective.

Below are two approaches for advancing a new regulatory pathway:

1. Through a **conditional approval pathway**, which would provide the Food and Drug Administration (FDA) a flexible tool by which it could—at its discretion—conditionally approve certain cell therapies that have a lower risk profile—for a limited period of time—following submission and FDA approval of safety and efficacy studies (clinical trials). Detailed FDA reporting would be required during the conditional approval period and a full Biologics License Application (BLA) would be required to be submitted as part of the full approval process.
2. Through extending the existing FDA **accelerated pathway program** to include regenerative therapeutic products (which could include a broader range of therapies, including cell therapy, gene therapy, gene-modified cell therapy, therapeutic tissue engineering product, human cell or tissue product, or combination product using any such therapy or product), while assuring that:
  - a. The unique and distinct attributes of human cells, tissues, or cellular or tissue-based products are considered;
  - b. A written description of the rationale for FDA decision-making is provided;

- c. Decisions could be based on safety data for particular cells or tissues for similar uses;
- d. Approvals would not be unreasonably withheld; and
- e. The treatment of a serious condition to which accelerated approval would apply could include a chronic, persistent, or recurring condition that affects day-to-day functioning without taking into account the availability or lack of alternative treatments.

Both approaches are expected to achieve the primary goal of BPC's recently released report, which is to accelerate the availability of safe and effective cell therapy treatments to patients in the U.S. by modernizing the current regulatory approach. The second approach – extension of the accelerated pathway program – builds upon an existing FDA program and therefore can be implemented more quickly. Like the conditional approval approach, the accelerated pathway program provides FDA full discretion in providing approval for only those therapies and products it deems safe and effective. The accelerated pathway program also enables reimbursement of therapies and products upon approval.

BPC recognizes the importance of compromise. BPC's mission is to drive principled solutions not only through rigorous analysis, but also reasoned negotiation taking into consideration the full range of perspectives.

Advancing regenerative cell therapy is one of numerous recommendations included in its report, [\*Advancing Medical Innovation for a Healthier America\*](#), developed with the guidance of BPC Advancing Medical Innovation co-chairs former Senate Majority Leader Bill Frist, MD and former Rep. Bart Gordon.