TECHNOLOGY ASSESSMENT

Regenerative Medicine

Therapeutic Applications, Challenges, and Policy Options

Highlights of GAO-23-105430, a report to congressional committees

July 2023

Why GAO did this study

Regenerative medicine represents a paradigm shift in the medical field because it aims to restore or supplement function, rather than just treating symptoms, and opens the door for personalized therapies.

GAO conducted an assessment of current and emerging regenerative medicine technologies and therapeutic applications. This report examines (1) current and emerging regenerative medicine technologies and therapies and their potential benefits, (2) challenges that hinder their development and use, and (3) policy options that could help enhance benefits and mitigate challenges associated with these technologies and therapies.

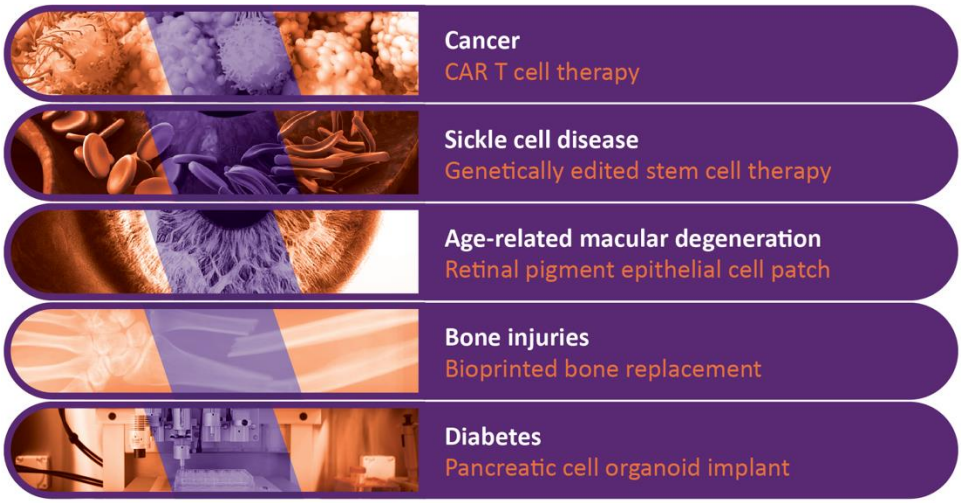
GAO reviewed scientific and policy literature and other key reports; convened a 3-day expert meeting; and interviewed subject matter experts and stakeholder groups including government agencies, such as the Department of Health and Human Services, non-government organizations, industry, academia, end user groups such as patient groups. GAO is identifying policy options in this report.

View GAO-23-105430. For more information, contact Karen L. Howard at (202) 512-6888 or HowardK@gao.gov.

What GAO found

Regenerative medicine offers the hope of being able to restore or replace cell, tissue, and organ functions affected by disease, injury, or aging. This may eventually help manage or cure many conditions that are currently considered chronic, untreatable, or terminal.

Examples of Diseases and Regenerative Medicine Therapies That Might Address Them



Source: GAO (analysis); Designcells/antoniotruzzi/firefightermontreal/sutthab/rfbsip/stock.adobe.com (images). | GAO-23-105430

GAO identified many challenges that may affect the development and use of regenerative medicine technologies and therapies including:

Challenges related to standardization. Standards are rules, conditions, guidelines, or agreed-upon practices that are adopted within an industry to provide developers with a common framework and promote consistency. Developing regenerative medicine standards is challenging because these technologies and therapies are complex and rapidly evolving. In addition, standards require consensus from stakeholders, which may be difficult to obtain.

Challenges related to regulation. The Food and Drug Administration (FDA) ensures the safety, efficacy, and security of human medical products in the U.S. through regulation. Regenerative medicine faces challenges related to regulation, including difficulty navigating a complex regulatory framework, uncertainty over which regulatory pathway is most appropriate for certain emerging technologies and therapies, and staffing shortages at FDA and collaborating agencies.

Challenges related to manufacturing. Manufacturing is the creation of products from starting materials, in a way that is generally consistent and reproducible. It is a key step for many emerging technologies and therapies, but the cells, tissues, and organs used for regenerative medicine are complex and difficult to manufacture at scale. Other challenges related to manufacturing include a lack of infrastructure and difficulty ensuring quality and consistency.

GAO developed 11 policy options that could help address the challenges or enhance the benefits of regenerative medicine. These policy options are provided to inform policymakers of potential actions to address the policy challenges identified in this technology assessment. They identify possible actions by policymakers, which include Congress, federal agencies, state and local governments, academic and research institutions, and industry. Policymakers would need to consider the impacts these new technologies will have on existing federal programs that are already strained. We suggested possible federal components for the policy options. See tables 1-3 for a full list of the policy options, potential implementation approaches, and opportunities and considerations.

Selected Policy Options to Mitigate Challenges Associated with Regenerative Medicine Technologies and Therapies

Selected policy option	Opportunities	Considerations
Invest in standards development. (report p. 25) This policy option could help address the challenge that standards require consensus.	• Could streamline standards development, which may, in turn, accelerate innovation, increase product safety and reliability, accelerate regulatory review, and decrease costs of regenerative medicine therapies.	• Existing organizations may not include all stakeholders, and stakeholders may hesitate to accept standards created without their input. • Industry stakeholders may hesitate to adopt standards if they perceive it will cost them a controlling position in the market. • Standards should be appropriately flexible to allow for innovation, while still being detailed and specific enough to support manufacturing of consistent, quality products.
Provide opportunities for increased interactions between regulatory experts (at FDA or in industry) and smaller companies, especially early in the development process (report p. 31) This policy option could help address the lack of access to regulatory expertise.	• May provide more timely advice and avoid unnecessary delays or uncertainty by pursuing the wrong regulatory pathways or submitting data that do not meet regulatory requirements.	• May require additional resources to bolster the workforce of regulatory scientists at FDA or public-private partnerships. • FDA may be limited in its ability to advise companies early in the process so as not to create a conflict of interest.
Consider whether changes to the framework for evaluating combination products and medical devices to accommodate emerging technologies and therapies may be necessary. (report p. 32) This policy option could help address whether current regulatory pathways are sufficient for emerging technologies and therapies.	• May encourage innovators, researchers, and developers of new products to provide valuable feedback to regulators.	• Coordinating among stakeholders to consider changes to regulatory pathways may be time- and resource-intensive. • If such consideration leads to recommended changes to the framework, statutory and regulatory changes may be necessary.
Provide more oversight and feedback to suppliers to increase consistency in starting materials (report p. 39) This policy option could help address inconsistency in starting materials for manufacturing.	• May accelerate manufacturing by reducing variation in input materials. • May reduce the risk of failure during product development.	• Starting material suppliers may lack incentives to follow standards if they lead to higher costs.

Source: GAO. | GAO-23-105430