



Timely ReAuthorization of Necessary Stem-cell Programs Lends Access to Needed Therapies (TRANSPLANT) Act of 2021

Advisory Council on Blood Stem Cell Transplantation

September 28, 2023



Topics

- **Timely ReAuthorization of Necessary Stem-cell Programs Lends Access to Needed Therapies (TRANSPLANT) Act of 2021**
- **Review and Findings**
- **Discussion**
- **Next Steps**

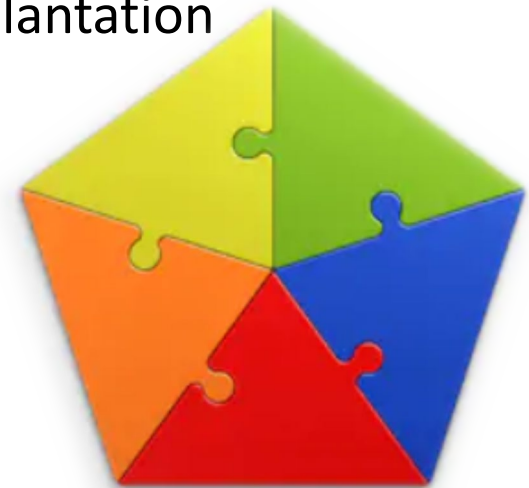


TRANSPLANT Act of 2021

- **Timely ReAuthorization of Necessary Stem-cell Programs Lends Access to Needed Therapies Act of 2021** (Public Law 117-15)
- Reauthorized HRSA's **C.W. Bill Young Cell Transplantation Program (CWBYCTP)**
 - The Program facilitates stem cell transplants from an unrelated marrow donor, peripheral blood stem cell donor, or cord blood unit
- Requires periodic review of state of science and recommendations on the **appropriateness of the inclusion of adult stem cells and birthing tissues as new types of therapies** in the CWBYCTP
 - Review at least every 2 years, with the first due by **May 2023**
 - Recommendations report to Congress due by **June 30, 2025**

C.W. Bill Young Cell Transplantation Program

- Purpose is to increase the number of bone marrow and cord blood transplants for recipients suitably matched to biologically unrelated donors
- Provides a structure to facilitate blood stem cell transplantation with blood-forming cells from unrelated donors for individuals with leukemia and other life-threatening blood disorders
- Five key functions/components
 - Facilitate bone marrow and cord blood transplantation
 - National Cord Blood Inventory
 - Office of Patient Advocacy
 - Single Point of Access
 - Stem Cell Therapeutic Outcomes Database



Criteria for Inclusion of New Cellular Therapies in the CWBYCTP

- To be considered for inclusion in the CWBYCTP for new uses outside of hematologic or immunologic reconstitution, adult stem cells or birthing tissues should meet three criteria:
 1. They are utilized as treatments for serious or life-threatening conditions,
 2. They require donor matching if appropriate, and,
 3. Have been demonstrated to be safe and effective as evidenced by FDA approval, or if FDA approval is not required, through adoption as a standard of care.

REPORT TO CONGRESS

The Appropriateness of the Inclusion of Adult Stem Cells and Birthing Tissues as New Types of Therapies in the C.W. Bill Young Cell Transplantation Program

Required by Section 2(c) of the Stem Cell Therapeutic and Research Reauthorization Act of 2015 (Public Law 114-104)

Department of Health and Human Services

August 2019



2023 Review of the Science

- Using the criteria, the FDA conducted an updated review of the state of the science.
- Despite scientific and technologic progress in the field, the present situation with stem cell and birthing tissue products available for clinical use is largely unchanged from that at the time of the 2019 report.
- Review of progress in the field will continue to be conducted at regular intervals.



Discussion

- Inclusion Criteria
- Review of the Science



Next Steps

- Continue to review of the state of science
- Continue to consult with Advisory Council on Blood Stem Cell Transplantation (ACBSCT)
- Update recommendations for 2025 Report to Congress