

Cell Therapy Data Collection Work Group

Advisory Council on Blood Stem Cell Transplantation
29-May-2014

Conference call participants

- ▶ Edgar Milford (Chair, ACBSCT)
 - ▶ Claudio Anasetti (WG Chair)
 - ▶ Joanne Kurtzberg (Member, ACBSCT)
 - ▶ Doug Rizzo & Marcelo Pasquini (Contractor for Stem Cell Therapeutics Outcomes Database)
 - ▶ Patricia Stroup (Executive Secretary, ACBSTC)
 - ▶ Shelley Grant, Nawraz Shawir, Passy Tongele, and Robert Walsh (HRSA, Division of Transplantation)
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Background

- ▶ The CIBMTR has provided updates to the ACBSCT regarding its efforts with collecting outcomes data from entities involving infusion of blood cells from a donor that are outside of the traditional scope of cell transplantation for hematopoietic reconstitution.
- ▶ In May 2012, the CIBMTR asked the ACBSCT members to provide assistance in reviewing the scope of Public Law 109–192, as authorized in 2005, and as amended through reauthorization in 2010, as Public Law 111–264.
- ▶ Dr. Milford established a Workgroup of Cellular Therapies during the ACBSCT meeting in May 2012, to assess the work scope of the Stem Cell Therapeutics Outcomes Database, as required by the HRSA contract with the CIBMTR.

Fact finding – CIBMTR

- ▶ Drs. Rizzo and Pasquini provided an update of the CIBMTR efforts, since approximately 2010, to capture and report on data from emerging cellular therapies for alternative applications involving a donor (e.g., related transplants, the fields of cardiac, neurologic, and regenerative medicine).
- ▶ Explored options with the California Institute for Regenerative Medicine for ways and timeframes to collect outcomes data.
- ▶ CIBMTR established a sub-work group to identify best practices for capturing data from these procedures that are not aimed at hematopoietic reconstitution, created forms, established data collection time frames and information technology systems that would enable capturing information on a voluntary basis from organizations outside of its traditional scope.

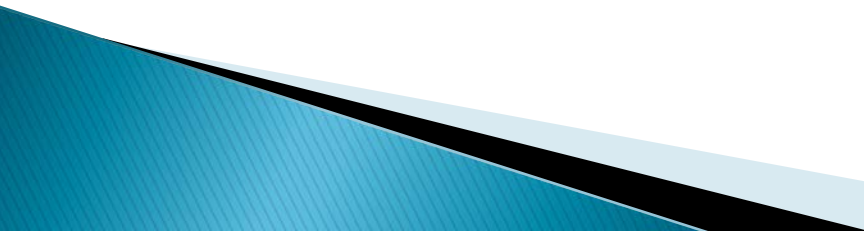
Challenges – CIBMTR

- ▶ Most cellular therapy activities are performed under the cover of intellectual property or commercial sponsors and therefore investigators are not willing or able to share data.
- ▶ Despite comprehensive efforts, the response to data collection has been low. For example, the current survey collection rates have been approximately 20% and most of the data have come from traditional hematopoietic cell transplant centers that have been conducting traditional and non-traditional cell therapy activities.
- ▶ To date outcomes data have been collected from approximately 400 cases, mainly from 6 different transplant centers, 94% of the data have been from one center. Therefore, the present data registry may not be representative of the broader cellular therapy field.

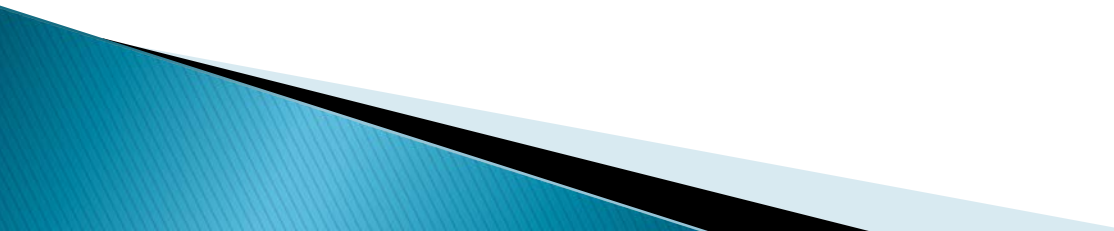
Opportunities – CIBMTR

- ▶ A grant received by the CIBMTR – UR 124 – from the National Institutes of Health is to explore cellular therapy data collection.

Fact finding – HRSA

- ▶ HRSA asked its Office of General Counsel to review the legislation as specified in Public Law 109–129, and, as amended in 2010, as Public Law, 111–264.
 - ▶ HRSA’s Office of General Counsel specified that the HRSA has the latitude to define by the Secretary what is in the best interest of patients regarding the capturing of data for the SCTOD contractor.
 - ▶ HRSA has limited availability of resources for broad data collection.
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HRSA's position

- ▶ It would be in the best interest of the legislation and the patients that it serves to continue capturing data on outcomes of blood stem cell transplantation and other cellular therapy activities that involve a donor (i.e., volunteer adult donors and umbilical cord blood units that are facilities through its C.W. Bill Young Cell Transplantation Program and its National Cord Blood Inventory Contractors).
 - ▶ This scope would include cellular therapy beyond the traditional indication of cellular therapy for the purpose of hematopoietic reconstitution.
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Summary considerations

- ▶ The ACBSTC may determine that data from cellular therapies from sources outside the C.W. Bill Young Transplantation Program and the national Cord Blood Inventory Contractors for purposes other than hematopoietic reconstitution can be captured by the CIBMTR on a voluntary basis.
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