SAFEGUARDING BLOOD SAFETY FOR MULTI-TRANSFUSED PATIENTS

Friday, 26 May 2017 | 18:00-19:30
Palais des Nations Building (Room IX)
Achieving Blood Safety and Hematopoietic Stem cell Transplantation Excellence access with Universal Access on a global level

Prof. Dietger Niederwieser / Dr. Mickey Koh
Worldwide Network for Blood and Stem Cell Transplantation (WBMT)

The Worldwide Network for Blood and Marrow Transplantation (WBMT) was formally created in 2007 by leaders from major hematopoietic stem cell transplantation (HSCT) societies and donor registries across the world. With the strong support of the World Health Organization (WHO), the mutual vision of combining efforts towards improving access to and standardization in HSCT, cellular therapy and related fields was achieved. The WBMT is today a global network of 24 international societies representing more than 1500 transplant centers in 78 countries and an NGO in working relations with the WHO. WBMT and WHO would like to further improve HSCT worldwide by supporting every aspect of human HSCT including stem cell donation, advanced cellular therapies and helping to support/provide know-how on all continents.

HSCT is a complex therapy successfully used in increasing numbers for patients with life-threatening disorders. After decades of experience (since 1957), discoveries, and international cooperation through registries and regional societies, HSCT is now accepted as the only curative treatment for many malignant and non-malignant diseases (e.g. Thalassemia). Data provided by WBMT suggest that the 1 millionth HSCT occurred in 2012 and there are now >70,000 procedures annually, with no evidence of a plateau in growth. However, use of HSCT varies considerably among world regions and the types of diseases transplanted. In Thalassemia for example, there are still countries without any activity in the field, other countries with only low transplant activity despite the cost effectiveness of the procedure. The same applies to sickle cell disease.

Experienced, multidisciplinary teams, adequate infrastructure for supportive care, including safety of blood supply and anti-infectives, as well as the ability to collect and share data are required for successful outcomes to ensure access and safety of this life saving modality. WBMT offers HSCT as a paradigm for how an essential therapeutic modality can be successfully deployed on a global scale with benefits for people and nations with diverse economic resources. Biennial global surveys, workshops in low activity countries, consultations with politicians and health authorities are showing first results. WBMT is instrumental in helping to establish safety of blood components as an essential prerequisite for HSCT by educating and twining with evolving countries, by standardization and accreditation of programs around the world.

Through all these activities, WBMT identifies great needs worldwide. The WBMT message to this respectful forum is that we need to encourage governments to include this curative treatment in their national health programs to ensure equity in access and safety around the world. This can be obtained by adapting national legislation, fostering donor registries, linking/establishing outcome registries worldwide, by increased twining with established centers and by developing specialized programs to cure congenital hematopoietic diseases like thalassemia worldwide. WBMT is also looking into the complex issue of advanced cell therapy and the increasing use of stem cells in the treatment of a wide variety of diseases. Some of this treatment remains unproven and should be conducted in formal clinical trials with the appropriate regulatory frameworks.
Achieving Blood Safety and HSC - Transplantation Excellence with universal access on a global level

Dietger Niederwieser
Mickey Koh
Geneva 2017
Networking – WBMT Federation

Worldwide Network for Blood and Marrow Transplantation
NGO in official relations with World Health Organization
WBMT: number of reporting teams per country

The Americas
1. East Coast
2. Central America
3. South America
4. North America
5. Mexico
6. Central America
7. South America
8. North America
9. Cuba
10. Panama

Eastern Mediterranean/Africa
1. North Africa
2. Middle East
3. East Africa
4. Central Africa
5. South Africa
6. Southern Africa
7. East Africa
8. West Africa
9. Central Africa
10. South Africa

Europe
1. Western Europe
2. Eastern Europe
3. Baltic States
4. Russia
5. Turkey
6. Mediterranean Region
7. Southern Europe
8. northern Europe
9. Eastern Europe
10. Central Europe

South East Asia/Western Pacific
1. Southeast Asia
2. Western Pacific
3. South Pacific
4. South Asia
5. Central Asia
6. East Asia
7. North Asia
8. Oceania
9. Central Asia
10. South Asia

Worldwide Network for Blood and Marrow Transplantation
NGO in official relations with World Health Organization
HB: Feb 2016
Transplant rates per 10 million population

- 0 or no report
- 0.1 - 20
- 21 - 100
- 101 - 500
- > 500

WBMT Global Survey: Transplant Rates/ Country

Hong Kong
Singapore

Worldwide Network for Blood and Marrow Transplantation
NGO in official relations with World Health Organization

D. Niederwieser et al, BMT 2016

WBMT Global Survey: Transplant Rates/ Country

<table>
<thead>
<tr>
<th>Category</th>
<th>2006</th>
<th>2012</th>
<th>2013</th>
<th>Δ% (2012-13)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Allogeneic</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Acute Leuk/other leuk/MDS/MPS</td>
<td>12,502</td>
<td>21,362</td>
<td>22,215</td>
<td>+4</td>
</tr>
<tr>
<td>Chronic Leuk</td>
<td>1,890</td>
<td>1,874</td>
<td>1,815</td>
<td>-3</td>
</tr>
<tr>
<td>Lymphoproliferative disorders</td>
<td>3,219</td>
<td>4,322</td>
<td>4,531</td>
<td>+5</td>
</tr>
<tr>
<td>Solid Tumors</td>
<td>150</td>
<td>130</td>
<td>130</td>
<td></td>
</tr>
<tr>
<td>Non Malignant disorders</td>
<td>2,360</td>
<td>4,068</td>
<td>4,364</td>
<td>+7</td>
</tr>
<tr>
<td>BMF</td>
<td>1,292</td>
<td>1,979</td>
<td>2,071</td>
<td>+5</td>
</tr>
<tr>
<td>Others</td>
<td>212</td>
<td>170</td>
<td>144</td>
<td>-15</td>
</tr>
<tr>
<td>Total</td>
<td>20,333</td>
<td>31,926</td>
<td>33,441</td>
<td>+5</td>
</tr>
<tr>
<td><strong>Autologous</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Leukemias</td>
<td>1,726</td>
<td>1,044</td>
<td>832</td>
<td>-20</td>
</tr>
<tr>
<td>PCD</td>
<td>10,675</td>
<td>17,590</td>
<td>18,512</td>
<td>+5</td>
</tr>
<tr>
<td>Lymphomas</td>
<td>10,980</td>
<td>14,331</td>
<td>14,461</td>
<td>+1</td>
</tr>
<tr>
<td>Solid Tumors</td>
<td>2,560</td>
<td>2,884</td>
<td>2,769</td>
<td>-4</td>
</tr>
<tr>
<td>Non Malignant disorders</td>
<td>193</td>
<td>330</td>
<td>275</td>
<td>-17</td>
</tr>
<tr>
<td>Others</td>
<td>96</td>
<td>41</td>
<td>38</td>
<td>-7</td>
</tr>
<tr>
<td>Total</td>
<td>26,230</td>
<td>36,220</td>
<td>36,998</td>
<td>+2</td>
</tr>
<tr>
<td><strong>Total</strong></td>
<td>46,563</td>
<td>68,146</td>
<td>70,439</td>
<td>+3</td>
</tr>
</tbody>
</table>
WBMT Global Survey 2006-2013

numbers of overall HSCT

![Graph showing the numbers of overall HSCT for different years and categories.](image)

numbers of HSCT for Hemoglobinopathies

![Graph showing the numbers of HSCT for Hemoglobinopathies for different regions and years.](image)

<table>
<thead>
<tr>
<th>Category</th>
<th>Americas</th>
<th>Asias</th>
<th>Europe</th>
<th>EMRO/Africa</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>Leukemias</td>
<td>73,4</td>
<td>74,7</td>
<td>72,3</td>
<td>53,2</td>
<td>72,5</td>
</tr>
<tr>
<td>Lymphoproliferative disorders</td>
<td>14,7</td>
<td>10,5</td>
<td>15,9</td>
<td>4,4</td>
<td>13,7</td>
</tr>
<tr>
<td>Solid Tumors</td>
<td>0,1</td>
<td>0,6</td>
<td>0,4</td>
<td>0,0</td>
<td>0,4</td>
</tr>
<tr>
<td>Non Malignant disorders</td>
<td>11,6</td>
<td>13,9</td>
<td>10,7</td>
<td>42,0</td>
<td>13,0</td>
</tr>
<tr>
<td>Others</td>
<td>0,1</td>
<td>0,3</td>
<td>0,7</td>
<td>0,4</td>
<td>0,4</td>
</tr>
<tr>
<td>Total</td>
<td>100%</td>
<td>100%</td>
<td>100%</td>
<td>100%</td>
<td>100%</td>
</tr>
</tbody>
</table>
International context

• “there are now around 33 stem cell products being transported every day across the world to facilitate transplants in another country”

• 12,000+ HSCT products exchanged across borders every year

Increasing **access** to the appropriate therapy
Global Governance

✓ Increasing **access** to the appropriate therapy
Increasing access to the appropriate therapy

Bologna Initiative for Global Vigilance and Surveillance

BIG V&S
Objective: communicating vigilance information as widely as possible in the interests of improving the safety of MPHO worldwide

# More than 900 documented case types can be searched in a structured way by organ, tissue or cell type and/or incident type
   # associated bibliographic references and expert analyses.

# Reactions
   # Infectious transmissions of all types, malignancy transmissions, and genetic transmissions to transplant recipients and to the offspring of children born from donated gametes.

# Events
   # caused by process failures and reactions in living donors

# Site and search tools publicly available (without username and password)

# Language specific interface in Regions.
Countries with HSCT centres and/or cord-blood banks accredited by one or more of JACIE, FACT, AABB

complex process...

- Standards & Quality management help:
  - Reduce risk
  - More consistent care
  - Improve communications
  - Continuous improvement
  - Benchmarking

BEETTER RESULT FOR PATIENT + DONOR
Countries with HSCT centres and/or cord-blood banks accredited by one or more of JACIE, FACT, AABB

- Standards & Quality management help:
  - Reduce risk
  - More consistent care
  - Improve communications
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  - Benchmarking

Better Outcome After Hematopoietic Stem-Cell Transplantation

Use of the quality management system “JACIE” and hematopoietic stem cell transplantation

European Group for Blood and Marrow Transplantation
Centers with FACT-JACIE Accreditation Have Significantly Better Compliance with Related Donor Care Standards

Chloe Anthias 1,*, Paul V. O’Donnell 2, Deidre M. Kiefer 2, Jean Yared 3, Maxim Norkin 3, Paolo Andricioli 4, Bipin N. Savani 5, Miguel A. Diaz 6, Mensah B. Bitan 7, Joerg P. Halter 7, Brent R. Logan 3, 6, 7, Galen E. Switzer 3, 6, Michael A. Pulsipher 3, 6, Dennis L. Confer 3, 6, 7, Bennis E. Sbarbati 7,
Bone Marrow Transplantation is an especially complex process involving therapeutic application of cells for donor selection and screening, infectious disease testing, cell recovery processing, and temporary/long-term storage.

Standards & Quality Management help:
- Reduce risk
- More consistent care
- Improve communications
- Continuous improvement
- Benchmarking

BETTER RESULT FOR PATIENT & DONOR

Includes commercial and non-commercial HSCT and CBB organisations.

Countries with HSCT centres and/or cord-blood banks accredited by one or more of JACIE, FACT, AABB.

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*Correspondence: lacelee@mskcc.org
Conclusions

• Safe blood supply is critical to any HSCT program
• Adapting national legislation
• Fostering donor registries
• Linking and establishing outcome registries worldwide
• Increase twinning with established centers
• Programs for congenital haematopoietic disorders
• Advanced cell therapies should be conducted in clinical trials