SCOPE:
The purpose of this policy is to define the process utilized for medical necessity review for use of hematopoietic stem cell transplantation (HSCT) in the treatment of aplastic anemia, sickle cell disease, and Thalassemia Major.

DEFINITIONS AND ACRONYMS:
- **Hematopoietic Stem Cell Transplantation (HSCT)**
- **Multisystem Organ Failure (MSOF)** - of heart, lungs and/or kidneys

POLICY:
HSCT is considered under selective circumstances to be useful in a variety of bone marrow conditions in relation to red blood cell production. Acquired aplastic anemia (without red cell production) caused by drugs, infection, immune deficits and various toxins, is considered a candidate for HSCT. Anemias of qualitatively flawed red cell production such as sickle cell disease and/or thalassemia major are two other common conditions for HSCT.

A host of genetic conditions ranging from immune deficiencies to storage related diseases as well as leukodystrophies are also HSCT possibilities.

**Medical Necessity is Established for the Following 3 Conditions**

A. **Aplastic Anemia**
   - 55 years old or younger; if older than 55, then must have previously been treated with immunosuppressive therapy

B. **Sickle Cell Disease**
   - Less than 16 years old with homozygous sickle cell disease with MSOF complications

C. **Thalassemia Major**
   - 30 years old or less with no liver disease
HEALTH PLAN POLICY

Policy Title: Hematopoietic Stem Cell Transplantation (HSCT) – Aplastic Anemia, Sickle Cell Disease and Thalassemia

Policy Number: MUM43
Revision: B

REFERENCES:

- Bone Marrow Transplant 2008; 41:109-117
- Hematology Oncology Stem Cell Therapy 2017

RELATED DOCUMENTS:
None

Nancy Horstmann
Chief Executive Officer Health Plans

David Engleking, M.D.
Medical Director

REVISION HISTORY:

<table>
<thead>
<tr>
<th>Revision</th>
<th>Date</th>
<th>Description of Change</th>
<th>Committee</th>
</tr>
</thead>
<tbody>
<tr>
<td>New</td>
<td>03/08/2018</td>
<td>Initial release</td>
<td>Executive Leadership</td>
</tr>
<tr>
<td>A</td>
<td>04/24/2019</td>
<td>Annual review. Product lines updated.</td>
<td>Executive Leadership</td>
</tr>
<tr>
<td>B</td>
<td>05/18/2020</td>
<td>Annual review. No change to policy content.</td>
<td>Executive Leadership</td>
</tr>
</tbody>
</table>