Quality Control strategy for Regenerative Medicine in CMC development

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JCR Pharmaceuticals Co., Ltd
Director
Regenerative Medicine, Research Institute, Research Division
Background: Company Profile
About TEMCELL® HS inj.

Case study; TEMCELL® HS inj.
Quality control strategy
Quality management for materials

Summary
Company Profile
Company profile : JCR Pharmaceuticals

Founded : September 13, 1975
Representative : Shin Ashida (Chairman, President & CEO)
Headquarters : Ashiya, Hyogo Prefecture, JAPAN
Employees : 541 (As of Sep. 30, 2017)
Ownership : Public (listed on 1st Sec. Tokyo Stock Exchange, in 2013)
Securities code : 4552
Mission : To discover, develop, manufacture and market biotherapeutic products for human healthcare
R&D Focus : Rare diseases/orphan drugs, Recombinant Protein, Allogeneic Regenerative Medicine
Location and Core Products:

GROWJECT®
(Recombinant human growth hormone)

Epoetin Alfa BS inj. JCR
(Recombinant Erythropoietin)

TEMCELL® HS Inj.

Ashiya: Headquarters
Kobe: Research Institute, 4 Plants

Sapporo
Sendai
Tokyo
Okayama
Nagoya
Fukuoka
Takamatsu
Development History : TEMCELL® HS Inj.

2003  License agreement with Osiris therapeutics(USA)*

2007  Phase I/II clinical study

2011  Phase II/III clinical study

2013  Orphan drug designation

2014  Application for new cell-based medicine

2015  Sep 18, First approval for allogeneic regenerative medicine in Japan

2016  Feb 24, commercial launch

*The licensor has been changed to Mesoblast Group (Australia) following the assignment of hMSCs-related rights from Osiris to Mesoblast in October 2013.
Background: TEMCELL®HS Inj.

Human Bone Marrow-derived Mesenchymal stem cells

<table>
<thead>
<tr>
<th>components</th>
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<tbody>
<tr>
<td>active ingredient</td>
<td>Mesenchymal</td>
<td>72x10^6 cells</td>
</tr>
<tr>
<td>stem cells</td>
<td>DMSO</td>
<td>10%(v/v)</td>
</tr>
<tr>
<td>inactive ingredients</td>
<td>Human derived</td>
<td>from donation</td>
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<tr>
<td>Albumin</td>
<td>Ringer solution</td>
<td></td>
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</tbody>
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Packaging: freezing bag packed in paper box

Storage: Vapored liquid nitrogen (under -130 degrees C.)

Expiration period: 5 years from manufacturing date
**Background : TEMCELL® HS Inj.**

**Indication**

Acute GVHD after hematopoietic stem cell transplantation

**Dosage and administration**

administer 2 million cells / kg body weight via intravenous infusion

*total 8 infusions per 4 week-treatment (2 infusions per week)*

Additionally administer one infusion weekly for 4 weeks depending on the degree of symptoms