Todd and Karen Wanek Family Program for Hypoplastic Left Heart Syndrome

Regenerative Medicine: Idealism into practice

Program Director: Tim Nelson, MD, PhD
Program Manager: Mindy Rice, MBA, CSSBB
HLHS: The Patient’s Unmet Need

Survival stats:
1st: 95%
2nd/3rd: 80%
Age 5: 50%

1982 Norwood Procedure
2007 Survival stats:
1st: 95%
2nd/3rd: 80%

1500 born/year
15,000 living w/ HLHS

The Cure!
Cost/benefit of cell based therapy highlights the need for disruptive innovation
- Scalable to other congenital heart diseases

<table>
<thead>
<tr>
<th></th>
<th>Fontan</th>
<th>Transplant</th>
<th>Fontan + Cell</th>
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<tbody>
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<td>Surgery/post-surgery care</td>
<td>$450-700K</td>
<td>$1M</td>
<td>$450-700K</td>
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<td>First three years</td>
<td>$500-750K</td>
<td>$1.3-1.5M</td>
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<tr>
<td>Annual cost post surgery(ies)</td>
<td>$3-5K</td>
<td>$35-50K</td>
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<td>Additional transplant*</td>
<td>Cardiac: $1M</td>
<td>Cardiac: $1M Other: $1M+</td>
<td>n/a</td>
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<td>Stem cell therapy</td>
<td>n/a</td>
<td>n/a</td>
<td>$30-50K</td>
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* there are occurrences where transplant is not preventable; regardless of the treatment path (Fontan/transplant), additional transplants could be necessary
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**5-Year Goal:**
Implement a multi-center trial for cell-based therapy
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5-Year Goal: Implement a multi-center trial for cell-based therapy
Strategy: A Process Perspective

**Suppliers**
- HLHS Patients
- Physicians
- Scientists

**Inputs**
- Disease Traits
- Clinical Experience
- Current Knowledge

**Process**
- Define the Scope
- Plan the Work
- Execute the Science
- Apply the Knowledge

**Outputs**
- Gap in Knowledge
- Goals/Milestones
- Protocols
- IRB Approvals
- Projects
- Papers, Grants, Prototypes, Intellectual Property
- New Cell-Based Therapy/Treatment

**Customers**
- Investigative Teams
- Dedicated Workforce
- Investigative Teams
- HLHS Patients

**Customer Inputs**
- Investigative Teams
- HLHS Patients
- Physicians
- Scientists

**Supplier Outputs**
- Scientific Information
- FDA-Approved Prototypes

**Supplier Inputs**
- Subjects, Labs
- Tissues, Samples
- Images, Data

**Customer Outputs**
- Investigative Teams
- Companies
- Companies

**Customer Inputs**
- Investigative Teams
- HLHS Patients
Strategy: Knowledge to Delivery Roadmap
Budget/Financing

- Biorepository
- Regenerative Strategies
- Genetics
- Imaging

New Knowledge
Patient Needs
Clinical Applications
IP and Commercial Application

Regenerative Strategies
“This gift is made in honor of our daughter and all children afflicted with this condition.”
--Todd and Karen Wanek
Thank you!