Promise for Healthcare:
New Directions in Pharmaceutical Research

Faraz Ali
Vice President, Global Commercial Development and External Affairs
January 10, 2015
These slides and the accompanying oral presentation contain forward-looking statements and information. The use of words such as “may,” “might,” “will,” “should,” “expect,” “plan,” “anticipate,” “believe,” “estimate,” “project,” “intend,” “future,” “potential,” or “continue,” and other similar expressions are intended to identify forward looking statements. For example, all statements we make regarding the initiation, timing, progress and results of our preclinical and clinical studies and our research and development programs, our ability to advance product candidates into, and successfully complete, clinical studies, and the timing or likelihood of regulatory filings and approvals are forward looking.

All forward-looking statements are based on estimates and assumptions by our management that, although we believe to be reasonable, are inherently uncertain. All forward-looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those that we expected. These statements are also subject to a number of material risks and uncertainties that are described in our most recent quarterly report on Form 10-Q, as well as our subsequent filings with the Securities and Exchange Commission. Any forward-looking statement speaks only as of the date on which it was made. We undertake no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.
Agenda

1. Regenerative Medicine Overview
2. bluebird bio Overview
3. Potential Implications for Biopharmaceutical Industry
4. Questions and Discussion
The Coming Wave of Regenerative Medicine
What is “Regenerative Medicine”?

Using cells therapeutically to repair function to bodily tissue or organs.

Inserting or editing genes in patients cells or tissues to treat diseases that are linked to defective or mutated genes needing either correction or improved regulation.

Creating new organs, and tissues to replace or repair existing organ function.

Source: ARM 2013 Annual Report
Regenerative Medicine Industry Overview

The Regenerative Medicine (RM) industry comprises service and manufacturing companies, tools and non-therapeutic products, cell, gene and tissue based therapies, regenerative compounds, devices and biopharmaceuticals.

Regenerative Medicine Industry Sectors

- Cell therapy companies 60%
- Tissue engineering companies 27%
- Gene therapy companies 8%
- Small mol/biologics companies 5%

Source: ARM 2013 Annual Report
Most early commercial experience is with cell therapies and tissue engineering products

Over 40 Cell Therapy Products Commercially Available

- Non-healing wounds: 35%
- Musculoskeletal: 35%
- Skin: 11%
- Cancer: 10%
- Ocular: 7%
- Cardiovascular: 2%

Source: ARM 2013 Annual Report
Cell Therapies and Tissue Engineering Drug Development

Healthy pipeline of cell therapy and tissue engineering drug products

Ongoing Industry-Sponsored Cell Therapy Trials by Phase

<table>
<thead>
<tr>
<th>Phase</th>
<th>Count</th>
<th>Companies</th>
</tr>
</thead>
<tbody>
<tr>
<td>Late-Stage</td>
<td>54</td>
<td>43</td>
</tr>
<tr>
<td>Mid-Stage</td>
<td>209</td>
<td>114</td>
</tr>
<tr>
<td>Early-Stage</td>
<td>63</td>
<td>49</td>
</tr>
</tbody>
</table>

Currently in Late-Stage Industry-Sponsored Cell Therapy Trials (Phase 2/3, 3, pivotal)

- Cancer: 32%
- Musculoskeletal: 28%
- Non-healing wounds: 15%
- Cardiovascular: 11%
- Autoimmune: 4%
- Ocular: 4%
- Stroke: 2%
- Skin: 2%
- Other: 2%

Source: ARM 2013 Annual Report
Growth of “curative” hematopoietic stem cell transplantation (HSCT) procedures

• Recent estimates that 1MM patients have been treated globally (recent volume ~50K/year)

• 70 indications, but volume primarily driven by oncology
Global adoption of “curative” hematopoietic stem cell transplantation (HSCT) procedures

- 177 centers total in US
- Surprisingly high adoption developing worldwide, despite resource limitations
- Growing emphasis on enabling more, e.g. via establishment of national and international marrow and cord blood registries
Gene Therapy Drug Development

2000+ gene therapy clinical studies conducted to date

Indications Addressed by Gene Therapy Clinical Trials

- Cancer diseases 64.6% (n=1107)
- Cardiovascular diseases 8.5% (n=146)
- Monogenic diseases 8.3% (n=143)
- Infectious diseases 8.1% (n=138)
- Neurological diseases 2% (n=35)
- Ocular diseases 1.3% (n=23)
- Inflammatory diseases 0.8% (n=13)
- Other diseases 1.1% (n=19)
- Gene marking 2.9% (n=50)
- Healthy volunteers 2.3% (n=40)

The Journal of Gene Medicine, © 2011 John Wiley and Sons Ltd
The Promise of Regenerative Medicine

Results illustrate this is no longer the stuff science fiction

Clinical Data Maturing*

Lenti

ALD
N=5

B-Thal
N=7

AAV
Hem B
N=14

MLD
N=9

WAS
N=12

ALL&CLL
N=>100

* Patient numbers reflect aggregate patients treated by indication as of ESGCT 2013
The Promise of Regenerative Medicine

Results attracting attention from media

Gene replacement could replace need for life-long blood transfusions

Can You Really Reverse Hearing Loss? Drugmakers Try Gene Therapy
The Promise of Regenerative Medicine

Results attracting attention from policy makers

Energy and Commerce Cures

A path to 21st century cures

S. 2126

To launch a national strategy to support regenerative medicine through the establishment of a Regenerative Medicine Coordinating Council, and for other purposes.

IN THE SENATE OF THE UNITED STATES
MARCH 13, 2014

Mrs. Boxer (for herself and Mr. Kaine) introduced the following bill, which was read twice and referred to the Committee on Health, Education, Labor, and Pensions

A BILL

To launch a national strategy to support regenerative medicine through the establishment of a Regenerative Medicine Coordinating Council, and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the “Regenerative Medicine Promotion Act of 2014”.

SEC. 2. FINDINGS.

Congress finds the following:

(1) Regenerative medicine has the potential to treat many chronic diseases, promote economic
The Promise of Regenerative Medicine

Results attracting investor attention
Introduction to bluebird bio
Why We Do What We Do

Our Vision – Make Hope a Reality

Seeking to transform the lives of patients with severe genetic and orphan diseases through the development of innovative gene therapy products.

Ethan
Aidan
Cameron
Leading Gene Therapy For The Long-Term

Integrated Product Platforms with Broad Therapeutic Potential

Gene Therapy

CAR T

Gene Editing
Lentiviral Stem Cell Platform

- Hematopoietic Stem Cells
  - Marrow
  - Mature blood cells
    - Erythrocyte (Heme, Thal, SCD)
    - Granulocyte
    - Platelets
    - Macrophage (CNS, ALD)
    - B cell
    - T cell (Cancer)

Stem cells

Progenitors

Mature blood cells
How Our Gene Therapy Approach Works

1. Produce Virus With Therapeutic Payload
   - Plasmids
   - Transfect 293T Cell
   - Lentivirus

2. Isolate Target Cells From Patient
   - Bone marrow harvest (SCD)
   - Apheresis
   - Blood stem cells (CD34+)
   - 2 Weeks

3. Transduce Target Cells ex vivo
   - Transduction (~48 hrs)
   - Gene Modified Cells
   - <1 Week

4. Test & Re-infuse Gene Modified Cells
   - Engraftment of modified cells
   - 4-6 Weeks (when ready)

Investments in Product Enhancements
- Improved process
- Selective vector changes

Results
- 25-30-fold reduction in non-infectious viral particles
- 3x vector copy number increase
# bluebird bio Pipeline

<table>
<thead>
<tr>
<th>Products</th>
<th>Program Area</th>
<th>Preclinical</th>
<th>Phase 1/2</th>
<th>Phase 2/3</th>
<th>Rights</th>
<th>Milestones</th>
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<tbody>
<tr>
<td>Lenti-D</td>
<td>CNS Diseases</td>
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<td></td>
<td>Worldwide</td>
<td>Complete enrollment</td>
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<tr>
<td></td>
<td>Childhood Cerebral ALD – Starbeam Study*</td>
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<tr>
<td>LentiGlobin™</td>
<td>Hematologic Diseases</td>
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<td></td>
<td></td>
<td>Worldwide</td>
<td>Complete enrollment &amp; present data</td>
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<td>β-thalassemia/SCD (France) – HGB-205 Study**</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Complete enrollment, present data &amp; define regulatory path</td>
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<tr>
<td></td>
<td>β-thalassemia (U.S./Thailand/Australia) – Northstar Study**</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Enroll patients &amp; present data</td>
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<tr>
<td></td>
<td>Sickle Cell Disease (U.S.) – HGB-206 Study</td>
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<tr>
<td>CAR T Cells</td>
<td>Oncology</td>
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<td>Global Celgene Collaboration</td>
<td>Initiate clinical study in early 2016</td>
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<tr>
<td></td>
<td>Hematologic/Solid Tumors</td>
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<tr>
<td>Early Pipeline</td>
<td>Research</td>
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<td></td>
<td>Worldwide</td>
<td>Advance preclinical pipeline</td>
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<tr>
<td></td>
<td>Undisclosed + Gene Editing</td>
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</tbody>
</table>

* The Phase 2/3 Starbeam Study is our first clinical study of our current Lenti-D viral vector and product candidate
** The Phase 1/2 HGB-205 and Northstar Studies are our first clinical studies of our current LentiGlobin viral vector and product candidate
### β-thalassemia Major: Disease Overview

**Disease**
- β-thalassemia major (e.g. transfusion-dependent)
- Monogenic, severe anemia
- Loss of or reduced β-globin production
- Poor quality of life and shortened lifespan

**Current Treatments**
- Frequent, chronic transfusions leading to iron overload and organ failure
- Ongoing iron chelation, frequently suboptimal
- Allogeneic transplant can be curative (rarely used)
  - Finding a suitable match
  - Morbidity/mortality with graft rejection, graft versus host disease and immunosuppression

**Epidemiology**
- Global prevalence ~288K; incidence ~60K
- US/EU prevalence (treated) ~15K; incidence ~1.5K
  - 60-80% severe/major
- Affects people of Mediterranean, Middle Eastern, South Asian and SE Asian descent
β-thalassemia: Clinical Trial Summary
Second Generation BB305 Vector

(HGB-204)
Phase 1/2, multi-center, global study

- N=15 patients
- Centralized transduction for drug product manufacturing
- Positive data presented at ASH 2014 annual meeting
- Enrollment completion expected in 2015

HGB-205
Phase 1/2, single-center, French study

- N=7 patients
- Positive data presented at ASH 2014 annual meeting
- First SCD patient ever treated with gene therapy in 2014
- Enrollment completion expected in 2015
Consistently Robust $\beta$A-T87Q-globin Levels

Subject

1102

11.0 11.1 10.3 10.1 10.2 9.7 11.0

1106

9.8 8.4 9.6

1201

12.8 11.6 11.0 12.1 13.4

1202

10.2 10.1 8.6 8.6 3.8

Hb A
Hb A2
Hb F
Hb E
Hb g/dL

Months post-drug product infusion

HbAT87Q
All Subjects with at Least 3 Months of Follow-up are Transfusion-Free, Regardless of Genotype

Days Transfusion-Free
as of December 1, 2014

Subjects:
- 1201 β0/βE: 359 days
- 1202 β0/βE: 260 days
- 1102 β0/βE: 154 days
- 1106 β0/β0: 94 days

Months post-drug product infusion
Childhood Cerebral Adrenoleukodystrophy (CCALD): Disease Overview

**Disease**
- Ultra-orphan, X-linked, monogenic, neurological disorder
- Mutated ABCD1 peroxisomal transporter results in toxic buildup of very long chain fatty acids (VLCFA)
- Leads to cerebral inflammation & demyelination

**Current Treatments**
- Untreated cerebral ALD leads to dismal outcomes (vegetative state and death)
- Allogeneic stem cell transplant standard for CCALD (if possible)

**Epidemiology**
- CCALD most severe form of ALD
- ALD incidence: 1 in 20,000 (live births)
- Cerebral disease
  - CCALD accounts for 30-40% of ALD
  - AMN accounts for 40-45% of ALD with 40% cerebral
  - ACALD accounts for 5% of ALD
Promising Clinical Data – CCALD (TG04.06.01) Study

**Natural Course of Disease**

- **NFS** / Loes stable in all subjects, as of last follow up
- **Gad** resolved in 3 out of 4 subjects
- Efficacy results comparable to allogeneic transplant
- No gene therapy-related adverse events

* NFS – Neurological Function Score
** Gad – gadolinium enhancement
Chimeric antigen receptor (CAR) T Cell Overview

- For years, the standard of care for treating cancer has been surgery, chemotherapy and radiation

- CAR T cell therapy represents a promising emerging approach to treating a variety of cancers
  - Clear clinical POC with CD19 antigen

- Uses patient’s own genetically modified T cells to selectively target and destroy cancer cells

- Provides potentially curative option after other treatment approaches have failed
bluebird’s CAR T Program

Collaboration Highlights

- Signed in 2013 with goal of entering clinic in 3 years
- $75M upfront payment; 3 years (extensions possible for up to an additional 3 years)
- bluebird right to 50/50 co-develop, co-promote and profit share in the US
- bluebird is responsible through Phase 1
  - Anticipate entering clinic by early 2016
Our Strategic Intent

**Severe Genetic Diseases**
Hematopoietic Stem Cells (HSCs)

- **Lentiviral Gene Delivery** – High Quality and Large Scale
  - **Cellular Transduction Capabilities** – Global
  - **Gene Editing** – MegaTALs/Homing Endonucleases

**Immunotherapy**
T Cells

- **Engage** with regulatory authorities and payers to chart the course for one-time transformative therapies

- **Leverage, integrate and grow core technologies to build a pipeline and collaborations for the long-term**

- **Advance SGD and immunotherapy products through late stage clinical trials**

- **Deliver transformative data in the clinic on a global scale**
bluebird bio 2020: The Gene Therapy Products Company

2015 - 2020

- Fully Integrated Product Company
- Global Commercial Capabilities & Collaborations
- Tech & Global Collaborations and New Products

Gene Therapy Products Company
- Pipeline of internal programs
- Collaborations
- Approved therapies

2010 - 2014

- Core Program Clinical Data
- Infrastructure & Capabilities
- Early POC Clinical Data

Broad Gene Therapy Infrastructure
- Translational Development
- Manufacturing
- Clinical
- Regulatory
Implications
"Curative" Therapies Represent a Potentially Dramatic Paradigm Shift

Typical Chronic Therapies

- Address disease symptoms
- One size fits all
- Daily/Weekly/bi-weekly/monthly tx
  - Adherence issues
  - Quality of life impact
- Significant additional costs to healthcare system over patient lifetime
- Adjustments can be made over time to optimize tx (e.g. dose, formulation)
- Possible to discontinue or switch to alternative if failure to respond

Gene Therapy

- Potentially "curative"
- Patient’s own cells → ultimate "personalized medicine"
- One-time tx with durable response
  - Adherence irrelevant
  - Patient convenience
- Potential savings to healthcare system over patient lifetime
- Irreversible treatment that cannot be adjusted or removed once done
“The Western world’s first drug to fix faulty genes promises to transform the lives of patients with an ultra-rare disease that clogs their blood with fat. The only snag is the price.”

“It’s unsustainable.”

“There are signs payers are pushing back.”

“Scrupiny of the sky-high prices charged for this wave of new drugs is growing.”

“In the case of gene therapy, extreme pricing may be unavoidable, since a single dose could last a lifetime, giving any drug manufacturer just one shot at recouping its investment.”
Media Response to the Promise of Curative Therapies

The Wall Street Journal

Senate Committee Is Investigating Pricing of Hepatitis C Drug
Gilead Charges $84,000 for a Standard 12-Week Regimen of Sovaldi

The Washington Post

The drug that’s forcing America’s most important – and uncomfortable – health-care debate

Forbes

At $1,000 A Pill, Hepatitis C Drug Sovaldi Rattles Medicaid Programs
The potential savings from regenerative medicine treatments for the United States in terms of reducing the direct costs associated with chronic diseases have been estimated at approximately $250 billion per year.

**Cardiovascular Disease**
- 100 million U.S. people afflicted
- $316 billion – U.S. aggregate direct costs

**Diabetes**
- 25 million U.S. people afflicted
- $175 billion – U.S. aggregate direct costs

**Stroke**
- 795 thousand U.S. people afflicted each year
- $73 billion – U.S. aggregate direct costs

**Alzheimer’s Disease**
- 35.6 million U.S. people living with the disease
- $200 billion – U.S. aggregate direct costs

**Age-related Macular Degeneration**
- 1.8 million U.S. people afflicted
- $255 billion – global direct costs

**Parkinson’s Disease**
- 1 million + U.S. people afflicted
- $23 billion – U.S. aggregate direct costs

**Spinal Cord Injury**
- 275 thousand U.S. people afflicted
- $40.5 billion – U.S. aggregate direct costs

**Peripheral Arterial Disease (PAD)**
- 10 million U.S. people afflicted
- $4.4 billion – U.S. aggregate direct costs

Source: ARM Annual Report
Need to Better Communicate VALUE

APPROACHES THAT SET PRICES BASED ON THE PROMISE OF OUTCOMES REMOVE THE COSTS OF R&D AND MANUFACTURING FROM THE EQUATION, AND FOCUS ALL STAKEHOLDERS ON VALUE.
Regenerative Medicine Illustrates How Biopharma is Prepared to Disrupt Itself

“The success of protein therapy, why would gene therapy be needed?”

In the United States and other developed countries, annual costs for a single adult patient of clotting factors for hemophilia are approximately $150,000 for on-demand therapy and $300,000 for prophylaxis, which could incur a lifetime cost of over $20MM.

This technology may soon translate into applications for other disorders.”

Source: Ponder, NEJM, 2011
Sickle Cell Disease (SCD): Gene Therapy Value Proposition vs Standard of Care

- SCD lifetime cost of care estimated by one KOL to be $8.75MM
- Annual costs for care escalate dramatically as patient ages and disease progresses
- Research with physicians and payers illustrates dissatisfaction with current treatment options

“Unless a cure becomes available this disease will continue to be expensive and chronic.”

**TABLE III. Fees for Life Care Plan for a Patient with Sickle Cell Anemia Based on an Assumption of 50 Year Life Expectancy**

<table>
<thead>
<tr>
<th>Period</th>
<th>Annual Fees</th>
<th>Total Fees/Period</th>
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</thead>
<tbody>
<tr>
<td>1. Age 0 – 5 years</td>
<td>$35,488</td>
<td>$177,439.00</td>
</tr>
<tr>
<td>2. Age 6 – 10 years</td>
<td>$56,576</td>
<td>$282,679.00</td>
</tr>
<tr>
<td>3. Age 11 – 18 years</td>
<td>$111,749</td>
<td>$893,990.00</td>
</tr>
<tr>
<td>4. Age 19 – 50 years</td>
<td>$231,050</td>
<td>$7,393,600.00</td>
</tr>
<tr>
<td>Total for life</td>
<td>$8,747,908.00</td>
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</tr>
</tbody>
</table>

Source: Ballas, AJH, 2009

**Satisfaction With Current Treatments**

<table>
<thead>
<tr>
<th>Treatments</th>
<th>Low</th>
<th>Payer Ratings</th>
<th>High</th>
<th>Comments</th>
</tr>
</thead>
<tbody>
<tr>
<td>Bone marrow transplant (allogeneic)</td>
<td></td>
<td></td>
<td></td>
<td>• Good efficacy but high safety risks and high cost</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td></td>
<td>• Only available as treatment option for limited patients</td>
</tr>
<tr>
<td>Hydroxyurea</td>
<td></td>
<td></td>
<td></td>
<td>• Unsatisfied with efficacy &amp; safety</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td></td>
<td>• Low cost perceived as strength</td>
</tr>
</tbody>
</table>

Source: bluebird bio research on file
β-Thalassemia Major: 
Gene Therapy Value Proposition vs Standard of Care

### Clinical Benefits
- Transfusion independence
- Effects on iron overload, chelation and phlebotomy use
- Safety

### Health-Related Quality of Life Benefits
- Patient-reported outcomes: sense of physical / mental well-being, pain, independent functioning, etc.

### Healthcare System Costs / Resources Benefits
- Averted future costs associated with currently available treatment options (esp lifelong treatment with red blood cell transfusions and iron chelation therapy)

### Societal Benefits
- Improvements in work productivity, school attendance, etc. e.g. due to reduced absenteeism

---

**Payers willing to reward innovation, but expect industry to do a better job quantifying and communicating different sources of value generated**
Potential Implications for Industry

1. Pricing and Reimbursement
2. Deployment and Distribution
3. Global Opportunities
4. Your Organizations?
Potential Implications for Pricing and Reimbursement

Establishing new payment provisions for the high cost of curing disease

By Scott Gottlieb, MD, and Tanisha Carino

July 2014

The special case of gene therapy pricing

Troyen A Brennan & James M Wilson

Gene therapy companies that pursue high, one-time payments for their products risk a backlash from payors. A better solution may lie in a pay-for-performance model.
## Potential Implications for Pricing and Reimbursement

### From risk-sharing to reward-sharing:
How gene therapy can revolutionize medicine and payer-manufacturer relations

<table>
<thead>
<tr>
<th>Year</th>
<th>Description</th>
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<tbody>
<tr>
<td>1</td>
<td>Duration of clinical trials for gene therapy</td>
</tr>
<tr>
<td>2</td>
<td>Cost offsets of a curative therapy over time can be tremendously high!</td>
</tr>
<tr>
<td>1</td>
<td>Actual duration of benefit</td>
</tr>
<tr>
<td>2</td>
<td>Potential unrealized benefit</td>
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<tr>
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<td>5</td>
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<td>6...</td>
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</table>

### Potential innovative reward-sharing scheme for gene therapy:
Annuity-based outcomes-driven contract model

- Manufacturer receives an initial payment based on known therapeutic value...
- ...if after that time no loss of efficacy is seen, manufacturer receives an additional payment
- ...this scheme could continue indefinitely as long as efficacy (per previously agreed upon outcomes) is maintained
Potential Implications for Pricing and Reimbursement

16% of payers currently have outcomes-based contracting arrangements with pharmaceutical companies and one-third expect to support them within three years.

Source: PriceWaterhouseCoopers, 2012
Potential Implications for Pricing and Reimbursement

Source: ISPOR 2012
The Foundation for Accreditation of Cellular Therapy (FACT) establishes standards for the collection, transport, and use of cells. Accredited centers adhere to requirements for staffing, record-keeping, quality management, and procedures for storing and shipping cell products.

| Current Procedures and Precedents | Transplant centers  
|                                  | - Harvest cells for sibling donors and local adult donors  
|                                  | - Existing SOPs conform to FACT requirements  
| Hospitals                        | - Ship cells from unrelated donors  
|                                  | - Submit cord blood to banks  
| Cord blood banks                 | - Ship frozen cells worldwide  
| FDA guidance                     | - CMC for gene therapies  
|                                   | - Validated cell manipulation facilities for HCT  
| Academic hospitals               | - Must use GTP and/or GMP procedures  
| FDA guidance                     | - Potency testing for cell and gene therapies  
|                                   | - Validation of rapid sterility testing  
| Cord blood banks                 | - Cell release criteria under FDA guidance  
| Transplant centers receive frozen cord blood and fresh BM | - Bedside viability test and gram stain prior to HCT  
|                                   | - Cells are handled under GCP / GTP  

The diagram illustrates the steps from Cell Collection to Delivery & Infusion, highlighting the processes involved in the distribution of cell products.
Potential Implications for Distribution

- Global Launch w/ Regional Transduction Centers
- Centralized Transduction Facility in the US
- Patient’s Gene modified cells
- Patient’s CD34+ Cells
- Centralized Transduction

Local Clinical Treatment for:
- CD34+ cell harvest
- Myeloablation
- Transplant
- Follow-up
Developing World Interest in Curative Therapies

- Thailand has one of the largest β-Thalassemia populations
  - ~100K prevalent cases and ~4K incident cases per year
  - Standard of care is lifelong blood transfusion + iron chelation

- 70% patients interested in curative allo HSCT
  - But only ~30% find a match (< 20% find a sibling match)
  - Historically not reimbursed by Thai MOH

- Detailed health economic assessment commissioned by Thai govt in 2010
  - Provided compelling evidence for cost effectiveness of allo HSCT over SOC
  - National coverage for allo HSCT will be initiated as a direct result of analysis
  - Thai govt and KOLs expressing interest in expanding access to curative potential of gene therapy

Source: Leelahavarong, BMC Health Services Research, 2010
Potential Implications for Your Organization?
Conclusions

• Regenerative medicine represents a wave of innovation with incredible clinical promise and unique value proposition.

• Regenerative medicine therapies may introduce paradigm shifts in multiple dimensions that are exciting and challenging.

• There is a lot of value to be created and captured in the process, and the biopharmaceutical industry needs to do a better job of quantifying and communicating this value.

• Biopharmaceutical industry needs to think proactively about these changes and need to engage early with stakeholders, including policy makers, payers, media, etc.
Questions and Discussion