



**Gamida Cell  
Cell Therapy Products**



# Company Profile



- Gamida Cell was established in 1998
- Developing biotechnology products based on stem cells from umbilical cord blood
- Proprietary technologies to expand populations of stem cells
- StemEx<sup>®</sup> in phase III clinical trial. NiCord to start clinical study in 2010
- Broad patent portfolio
- Strategic partnerships with Teva Pharmaceuticals and Amgen
- Strong management, team of 36 people
- \$42 million raised in equity

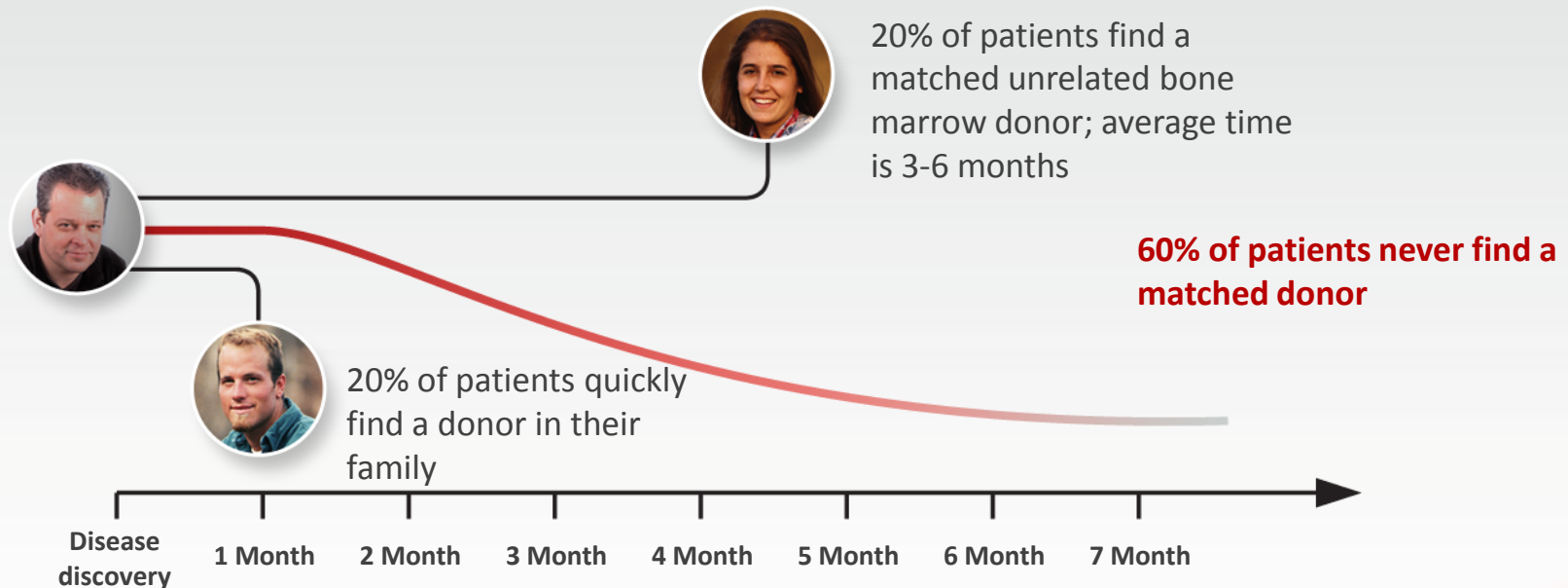
# Target Market in Hematological Malignancies

0600056

- Bone Marrow Transplantation is a potentially life saving treatment for patients with leukemia or lymphoma. In clinical practice for >40 years
- 600,000 patients with leukemia or lymphoma in the developed world
- 160,000 new patients each year
- 50,000 patients each year are indicated for bone marrow transplantation
- Only 20,000 patients each year are getting a transplant
- Annual growth rate in the number of transplantations is 3%

# Finding a Bone Marrow Donor

- Following diagnosis, time to bone marrow transplantation is critical
- Only 20% of patients have a bone marrow donor in their family
- Additional 20% of patients find an unrelated bone marrow donor
- More than half the patients never find a fully matched bone marrow donor; their disease will progress



# Umbilical Cord Blood: Alternative Transplant

>400,000 cord blood units are cryo-preserved in public cord blood banks

## Advantages of Cord Blood Transplantation:

- No need for full tissue matching
- Rapid identification of a cord blood unit for the patient

## Limitation of Umbilical Cord Blood:

- Small number of stem cells

# Innovative Technology to Expand Populations of Stem Cells

Cord Blood Unit



GMP facility  
US, Belgium, Israel



Expansion Cultures



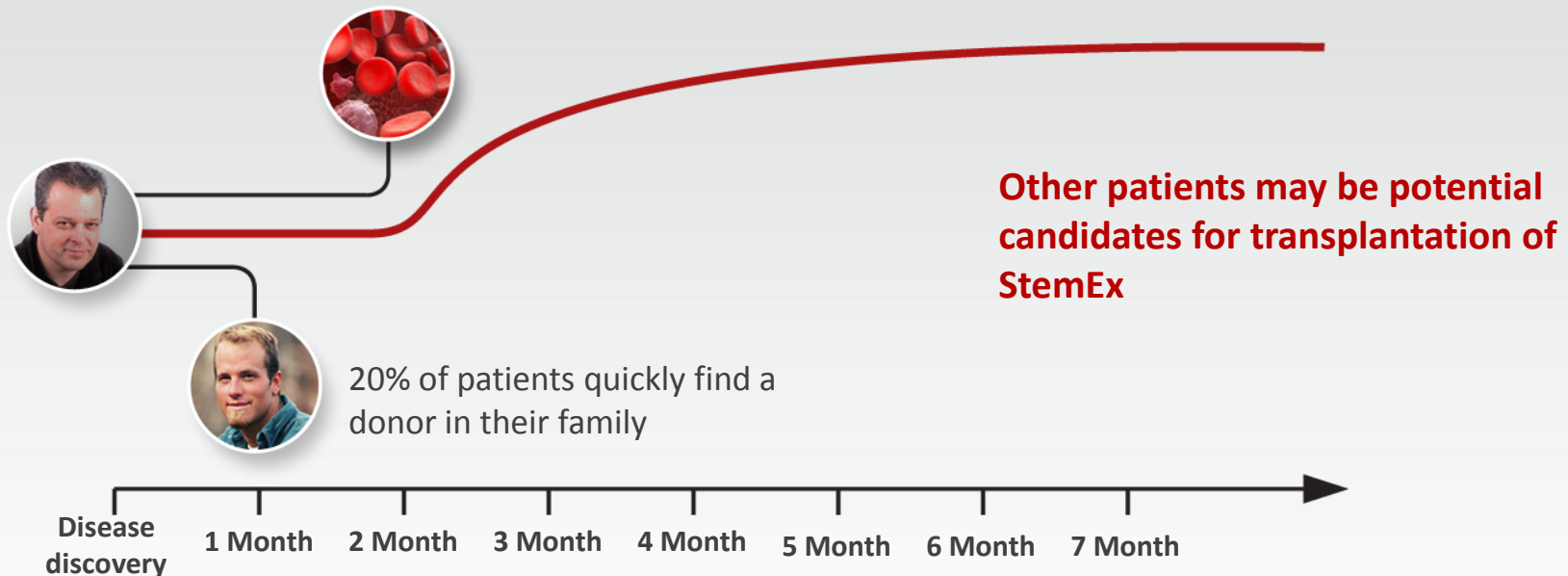
StemEx® for Infusion





# Transplanting StemEx<sup>®</sup>

- StemEx can be rapidly available for transplantation
- 3 week manufacturing process; During this time the patient is treated with high dose chemotherapy and radiation, preparing him for the transplantation



# StemExR<sup>®</sup> Phase I/II Clinical Trial

- StemEx is a single unit of umbilical cord blood, enriched with stem cells
- A phase I/II clinical trial was completed in 2005 at M. D. Anderson Cancer Center, Texas, US
- 10 patients with leukemia or lymphoma were transplanted with StemEx; Follow-up of 180 days
- StemEx demonstrated a good safety profile

| Clinical Endpoint                               | Historical Control Group* | StemEx <sup>®</sup> Phase I/II |
|---|---------------------------|--------------------------------|
| 100 day survival (% patients)                   | 56                        | 90                             |
| Engraftment (% patients)                        | 83                        | 90                             |
| Graft vs Host Disease grade III/IV (% patients) | 21                        | 0                              |
| 180 day survival (% patients)                   | 45                        | 60                             |

(\*)Phase I/II had no control group. This is the historical control cohort for current phase III study, composed of 514 patients transplanted with single un-manipulated unit of cord blood



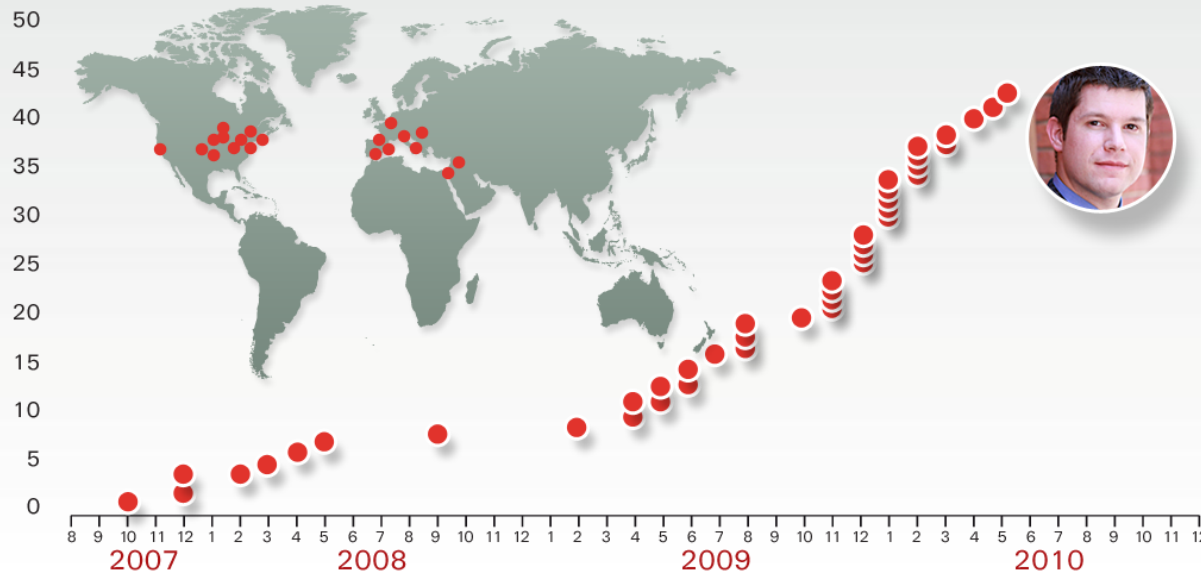
# StemEx<sup>®</sup> Phase III Clinical Trial



- Global pivotal registration phase III study, approved by the FDA under SPA
- Multi-national study to evaluate the safety and efficacy of StemEx
- 100 patients with leukemia or lymphoma will be transplanted with StemEx
- The control group is comprised of 514 similar patients who were transplanted with an un-manipulated unit of cord blood, during the years 1995-2005
- Primary endpoint: 100 days survival (% patients)

# StemEx® - Status of Phase III Clinical Study

- >40 patients already transplanted in 26 clinical sites, in the US, Europe and Israel
- Patients in phase III study were transplanted with, on average, 15 times larger number of stem cells, as compared to the original cord blood unit
- No unusual adverse events reported so far
- DMC (Data Monitoring Committee) recommended the continuation of the recruitment of patients
- Clinical study planned to complete in 2011



# Joint Venture with Teva Pharmaceuticals

- Gamida Cell granted the JV an exclusive license for StemEx® in hematological diseases
- JV is equally owned (50/50) by Gamida Cell and Teva
- Commercialization rights retained by the JV
- Revenues will be split 50:50 between Gamida Cell and Teva
- Neither the JV nor Teva have rights to other products developed by Gamida Cell



# StemEx<sup>®</sup> Business Model

- Significant unmet clinical need for patients indicated for bone marrow transplantation without a bone marrow donor in their family
- Umbilical cord blood is a readily available and safe source for cells
- StemEx may be a solution for patients without a matched donor in their family
- Orphan Drug status in the US and Europe.
- Reimbursement for cord blood transplantation in the US is, on average, \$200,000 (\*)
- Niche product: only 200 transplantation centers in the US. Marketing expenses are expected to be lower

(\*) Boston Healthcare Associates Inc., research performed for the JV in 2009

# StemEx® Business Model (Cont.)



- JV is working to reduce StemEx manufacturing and shipping costs by developing a frozen product that can be manufactured by central manufacturing site(s).
- Expected pricing for StemEx: 20 – 50 thousand dollars (\*)

(\*) Boston Healthcare Associates Inc., research performed for the JV in 2009

Cost of cord blood transplantation is primarily affected by the following parameters (\*):

- Length of hospitalization
- Time to neutrophil engraftment
- Time to platelet engraftment
- Development of Graft-vs-Host disease

The JV is hoping to show improvement in all the above parameters, to justify a higher price for StemEx

(\*) Majhail, N. S., C. G. Brunstein, et al. (2009). "Double umbilical cord blood transplantation." *Curr Opin Immunol* **18**(5): 571-575



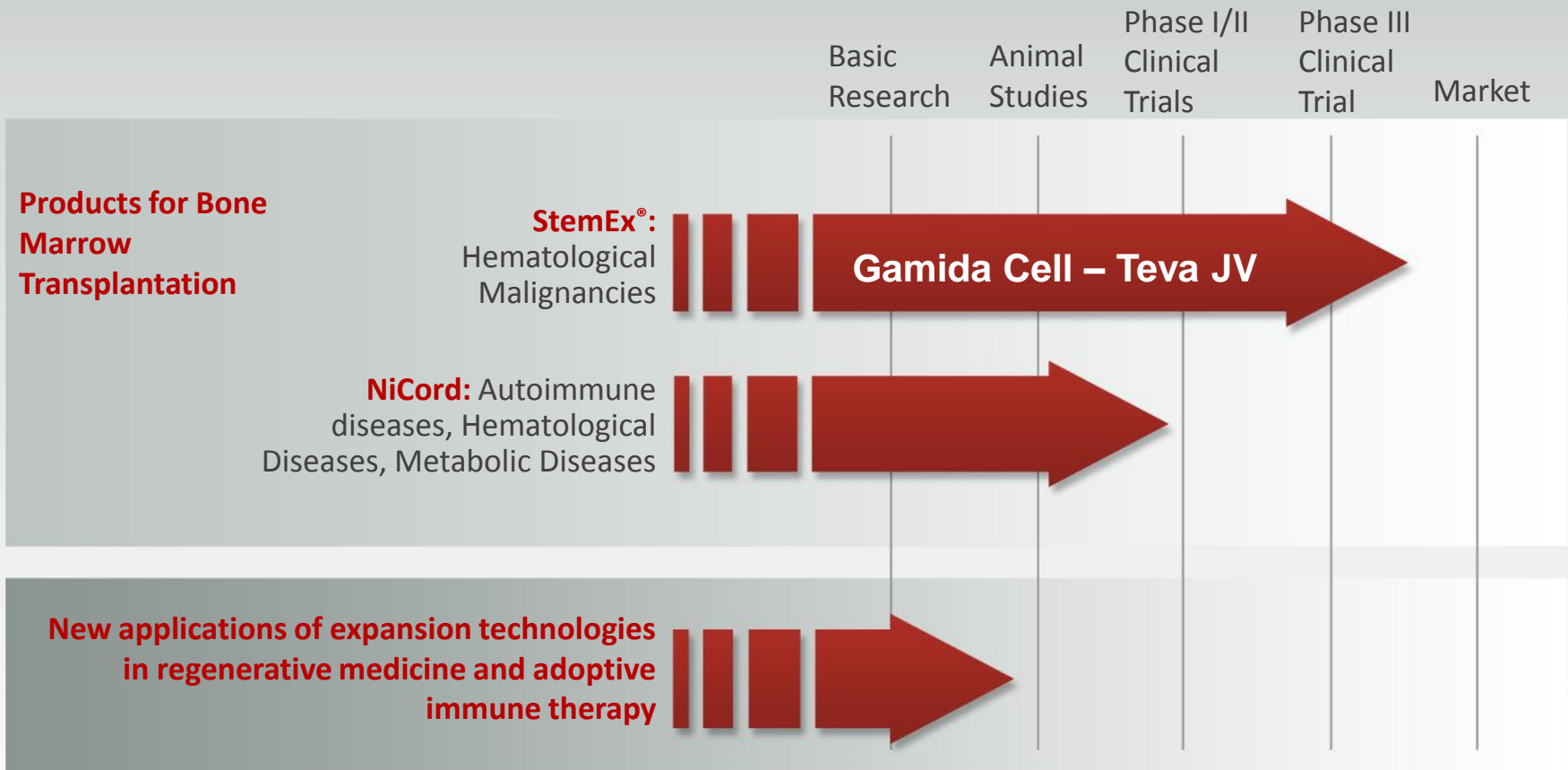
# Competition

- Several companies and research groups are developing technologies and products to improve the clinical outcome of cord blood transplantation: Hutchinson, Mesoblast, Novartis, Cellerant, Fate
- StemEx<sup>®</sup> is in the most advanced stage of development and expected to reach the market first
- Double cord blood transplantation is being tested in the US in a large clinical trial by the NCI. Study planned to complete in few years, outcome not yet available. Small studies with double cord transplantation demonstrate no shortening in time to engraftment

## Indirect Competition

- Drugs to treat various types of leukemia and lymphoma: Vidaza, Revlimid, Thalidomid, Gleevec, Rituxan

# Gamida Cell Pipeline of Products



# NiCord™ Treatment for Severe Autoimmune Diseases

- Autoimmune diseases develop when the patient's immune system attacks his own tissues
- Prevalence: >5% of population
- Main cause for morbidity and mortality in young women
- Autoimmune diseases include: Multiple Sclerosis, Lupus, Rheumatoid Arthritis, Juvenile Diabetes, Crohns' Disease
- Available treatments are symptomatic and include combinations of anti-inflammatory drugs, steroids and suppression of the immune system. In many patients the disease can become resistant to drug treatment, leading to high morbidity and sometimes death
- Preliminary observations demonstrate improvement in the condition of 75% of autoimmune disease patients, following bone marrow transplantation from a donor (\*)

(\*) Allogeneic hematopoietic SCT for patients with autoimmune diseases.  
*Bone Marrow Transplantation* 44, 27-33 (July (1) 2009)

# NiCord™ Treatment for Severe Autoimmune Diseases



- NiCord is based on a unit of umbilical cord blood enriched with stem cells using innovative NAM technology
- Promising animal data
- A pilot clinical trial is planned to begin in the US under IND in 2010
- A phase I/II clinical trial in patients with severe autoimmune diseases is planned to start in 2011
- Gamida Cell owns all rights for NiCord

# Clinical and Scientific Advisory Committee

- Prof. Patrick Stiff, Loyola University, United States
- Prof. Guillermo Sanz, Valencia, Spain
- Prof. Joanne Kurtzberg, Duke University, United States
- Prof. Pablo Rubinstein, NY Blood Center, United States
- Prof. Elizabeth Shpall, M. D. Anderson, United States
- Prof. Mary Laughlin, Case Western University, United States
- Prof. Arnon Nagler, Sheba Medical Center, Israel



**Thank you for your attention**

gamidaCell  
Cell Therapy Technologies