

# **The Science of Cord Blood Stem Cells: An Introduction**

January 2009

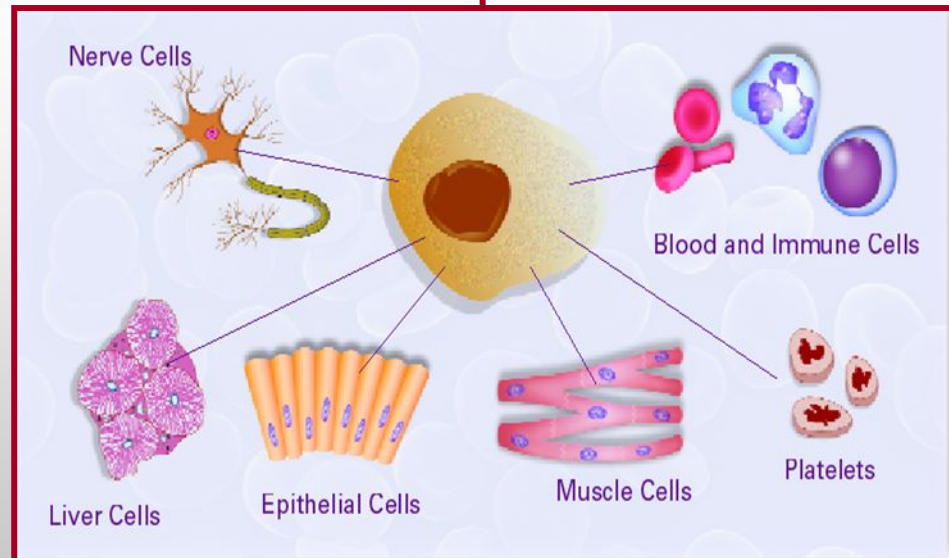
# Stem Cells are a Valuable, Limited Resource

## Stem Cells are “Master” Cells

- Building blocks of organs, tissues, blood, and the immune system

## Types of Stem Cells

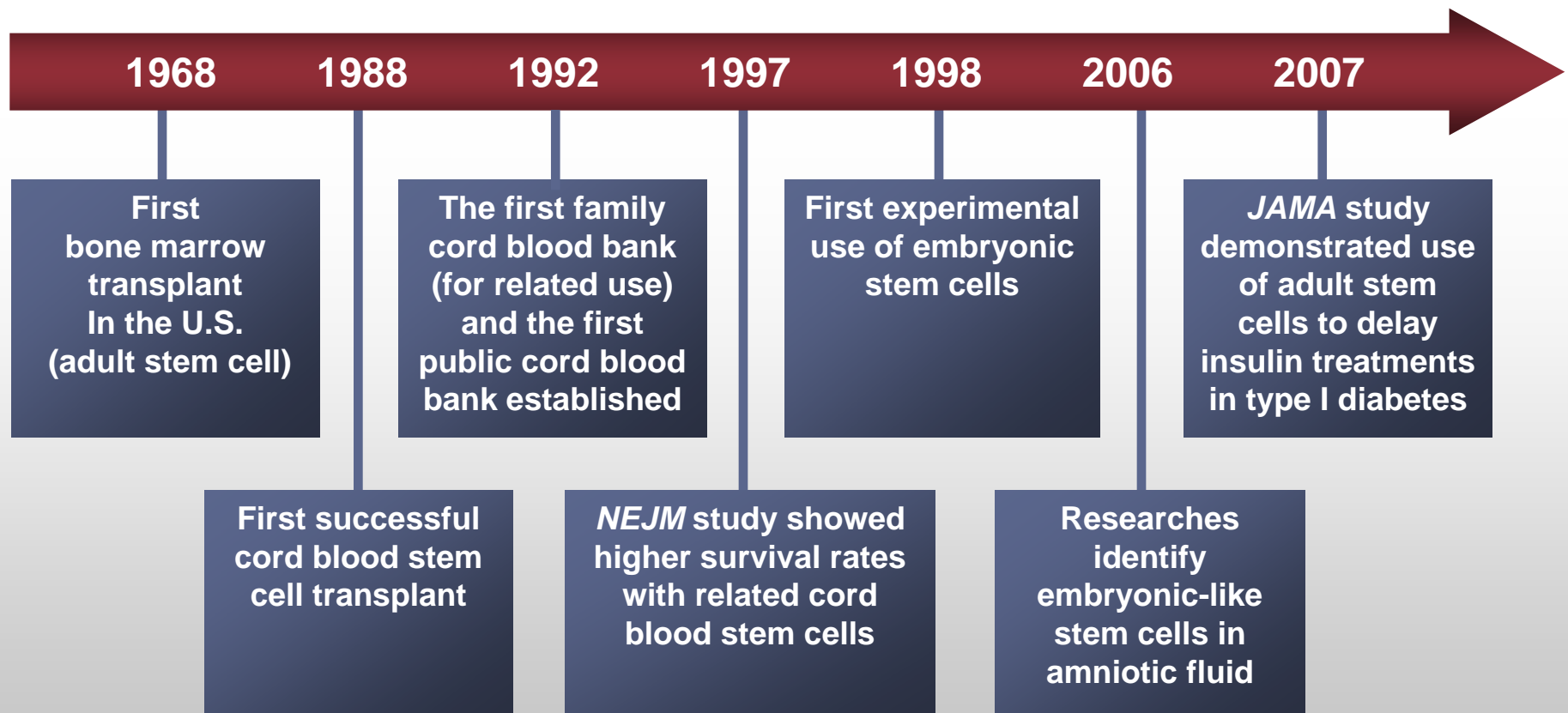
- Umbilical Cord
- Bone Marrow
- Peripheral Blood
- Amniotic Fluid
- Adipose Tissue
- Embryonic



# Three Distinct Categories: Not all stem cells are the same

	<b>Advantages</b>	<b>Limitations</b>
<b>Embryonic Stem Cells</b>	<ul style="list-style-type: none"><li>▪ High developmental potential</li><li>▪ High proliferative capacity</li><li>▪ Little risk of viral contamination</li></ul>	<ul style="list-style-type: none"><li>▪ Limited supply</li><li>▪ Ethical issues</li><li>▪ No clinical data</li><li>▪ Biological constraints</li></ul>
<b>Newborn Stem Cells</b> <ul style="list-style-type: none"><li>▪ <b>Cord Blood</b></li><li>▪ <b>Amniotic Fluid</b></li></ul>	<ul style="list-style-type: none"><li>▪ Younger, excellent proliferation and differentiation abilities</li><li>▪ Immediately available</li><li>▪ Less stringent HLA requirements</li><li>▪ Lower risk of GvHD, infections</li><li>▪ Autologous transplants possible</li></ul>	<ul style="list-style-type: none"><li>▪ Delayed short-term engraftment</li><li>▪ One-time supply</li></ul>
<b>Adult Stem Cells</b> <ul style="list-style-type: none"><li>▪ <b>Bone Marrow</b></li><li>▪ <b>Peripheral Blood</b></li></ul>	<ul style="list-style-type: none"><li>▪ Ability to differentiate</li><li>▪ Rich concentration of stem cells</li><li>▪ Greatest amount of historical data</li><li>▪ Rapid engraftment</li><li>▪ Good historical data</li><li>▪ Less invasive than bone marrow</li><li>▪ Facilitates easier autologous transplants</li></ul>	<ul style="list-style-type: none"><li>▪ Complex harvest</li><li>▪ Minimal residual disease (MRD)</li><li>▪ Increased potential for GvHD</li></ul>

# History of Stem Cell Research and Development

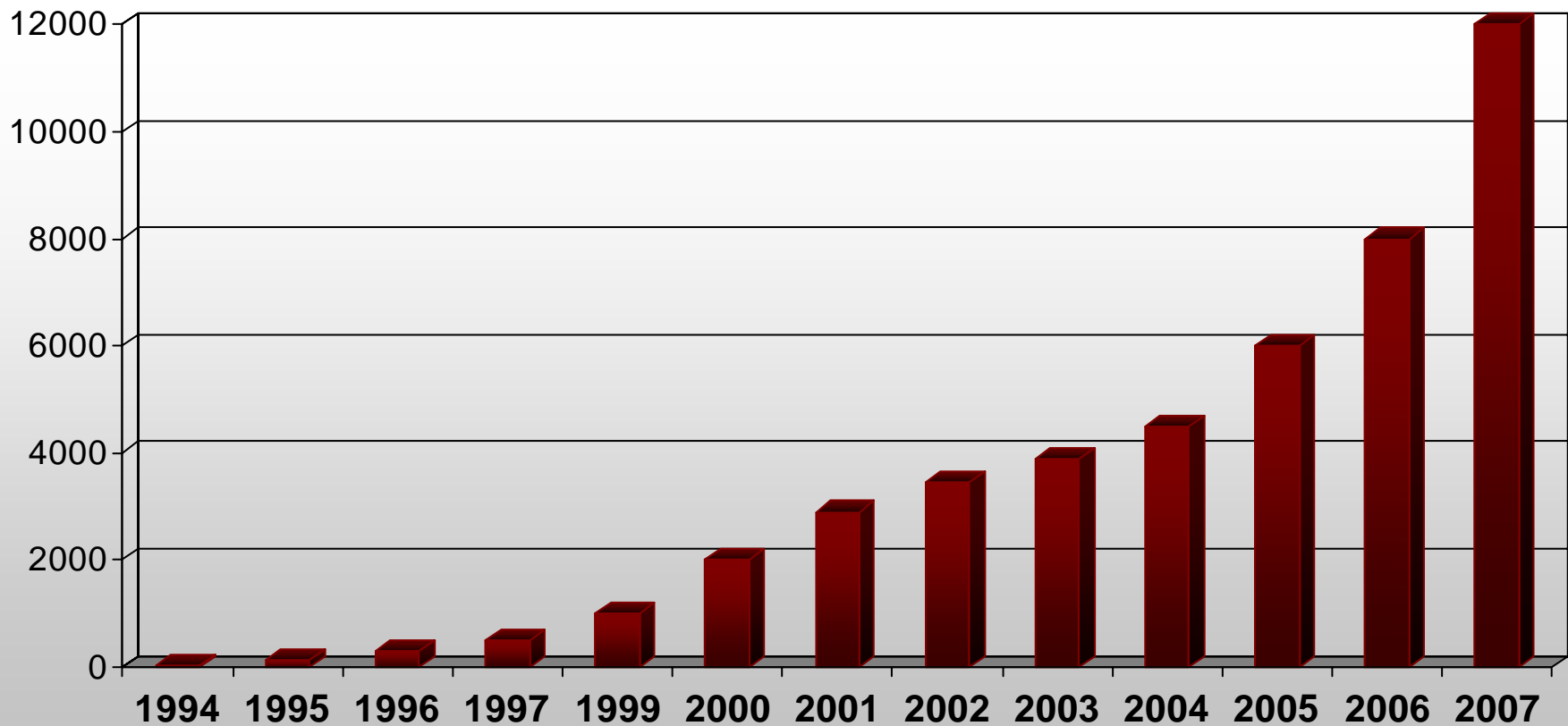


# Cord Blood Stem Cells have untapped potential

- Cord blood stem cells can be easily collected and stored
- Long-term cryopreservation has no adverse effect
- Clinical studies over the past 20 years demonstrate the efficacy and safety of cord blood stem cells
- Currently, cord blood-derived stem cells have clinically utility in more than 70 diseases
  - Two diseases ten years ago
- Banking cells for future therapies has been validated by science
- Cord blood stem cells possess significant future potential through expansion, gene therapy, and cell-specific therapies

**Increasingly being used to save lives**

**Cord Blood Stem Cell (CBSC) Transplants**



# Currently used to treat more than 70 chronic or life-threatening diseases

## Malignancies

- Leukemia
- Lymphoma
- Multiple Myeloma
- Hodgkin's Disease
- Retinoblastoma
- Histiocytosis
- Solid Tumors

## Blood Disorders

- Sickle Cell Anemia
- Thalassemia
- Aplastic Anemia
- Thrombocytopenia
- Diamond-Blackfan
- Amegakaryocytosis

## Immunodeficiencies

- SCID
- Ataxia Telangiectasia
- Wiskott Aldrich
- DiGeorge
- Kostmann
- Omenn

## Autoimmune Diseases

- Multiple Sclerosis
- Systemic Lupus
- Rheumatoid Arthritis
- Evan Syndrome
- Crohn's Disease

## Metabolic Disorders

- Leukodystrophy
- Gaucher Disease
- Krabbe Disease
- Hunter Syndrome
- Hurler Syndrome
- Niemann-Pick

## Other Inherited Disorders

- Osteopetrosis
- Osteogenesis Imperfecta
- Lesch-Nyhan Syndrome
- Tay-Sachs

# Pluripotency + Controlled Differentiation: Critical components for regenerative therapy

- In regenerative therapies, stem cells must be:
  - Pluripotent: Have the ability to differentiate into a specific cell type
  - Controlled: Must be able to safely and effectively work with existing cells and tissues to improve and repair function
- Embryonic stem cells are able to differentiate into many different cell types, but once placed within a study animal may give rise to tumor formation
- Cord blood stem cells appear to be primitive enough to differentiate into multiple cell types, yet mature enough to work within the body's existing framework

# Growing scientific evidence supports future potential



## Regenerative Medicine: Focus on autologous use of stem cells

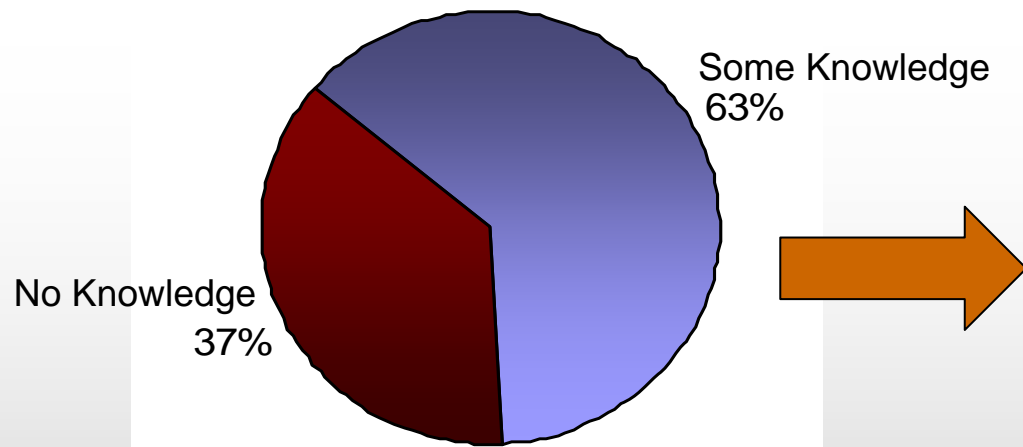
- Heart disease/angiogenesis
- Diabetes
- Stroke recovery
- Parkinson's disease
- Brain/spinal cord injury
- Amyotrophic lateral sclerosis
- Muscular dystrophy
- Liver disease

# Brain Injuries: A Significant Health Issue

- More than 1.4 million sustain brain injuries from accidents yearly in U.S.
  - 235,000 are hospitalized
  - 1.1 million are treated and released from an emergency department
- The leading causes of TBI are:
  - Falls (28%)
  - Motor vehicle accidents (20%)
- Highest risk groups
  - 0- to 4-year-olds and
  - 15- to 19-year-olds
- Direct medical costs and lost time:
  - ~ \$60 billion in the US in 1995
  - ~\$2M/patient lifetime cost
- CDC estimates that more than 5.3 million Americans currently have life-long need for help to perform activities of daily living

# Patient Education Isn't Happening

## Patients' Knowledge of Umbilical Cord Blood Banking



- 74% describe themselves as minimally informed
- 2.6% describe themselves as extremely knowledgeable

### In addition:

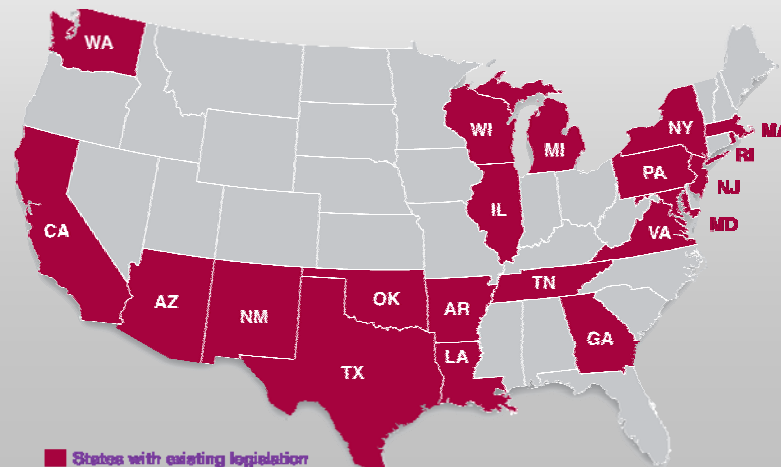
- 45% of patients were unaware of the potential for cord blood use by a sibling
- 84% percent of patients expect their healthcare provider to be able to answer their cord blood banking questions
- 14% of patients are educated by their healthcare provider on cord blood banking

Source: Perlow J.H. Patient's knowledge and understanding of umbilical cord blood banking. *Journal of Reproductive Medicine*. 2006;51(8):642-8.

# But Becoming Public Health Policy

- Every pregnant woman should be educated about the benefits of cord blood stem cells and informed of options for banking prior to her third term:
  - Institutes of Medicine Study in 2004
  - Federal Stem Cell Act of 2005
  - Legislation in 20 states (2006-2008)

More than two-thirds of US pregnancies occur in states that have enacted legislation on cord blood education



**The greatest threat to progress  
is not ignorance . . .  
but rather the illusion  
of knowledge.**