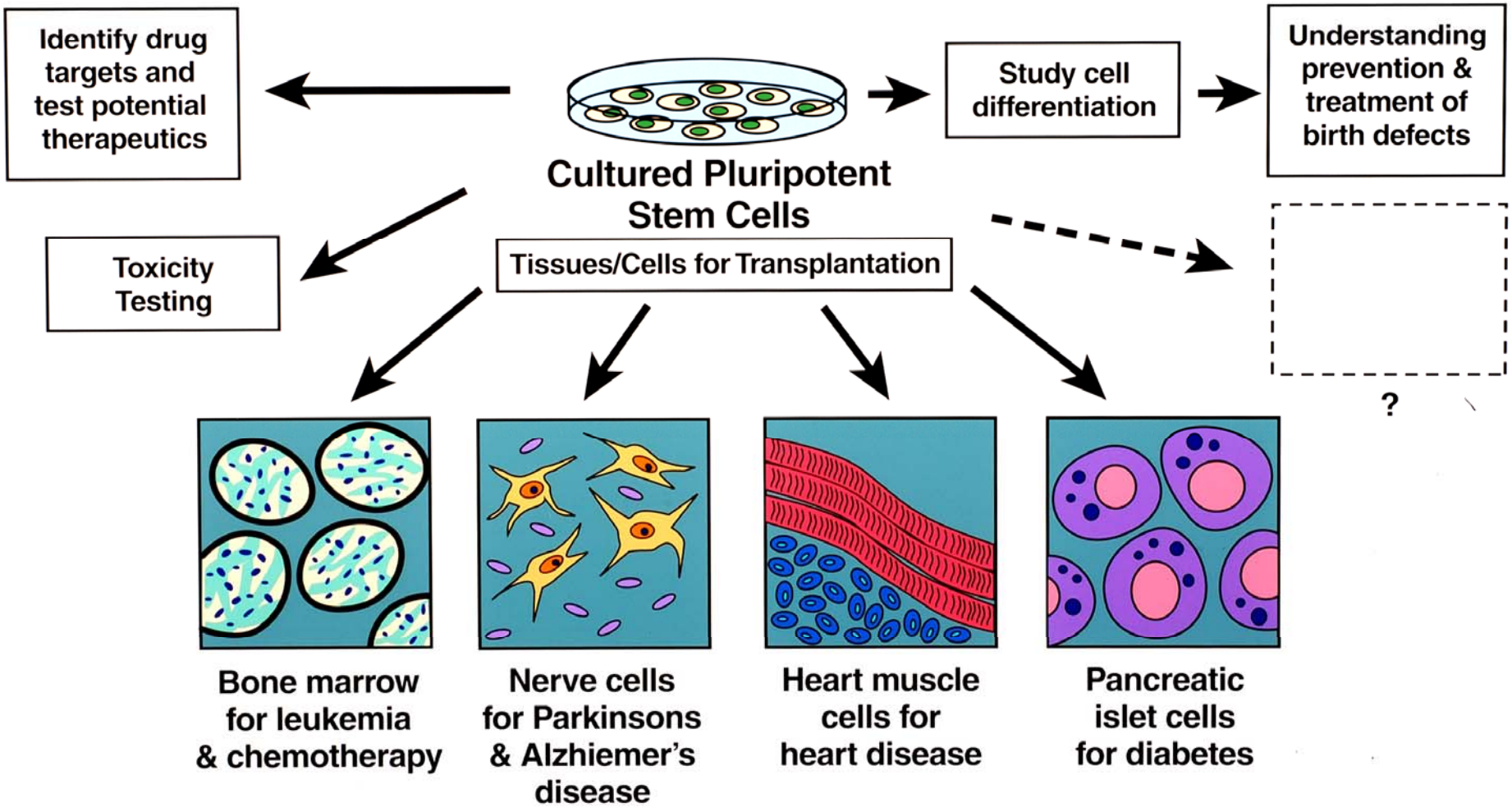
The Business of Stem Cells: Re-examining Federal, State, and Private Funding and Regulatory Initiatives

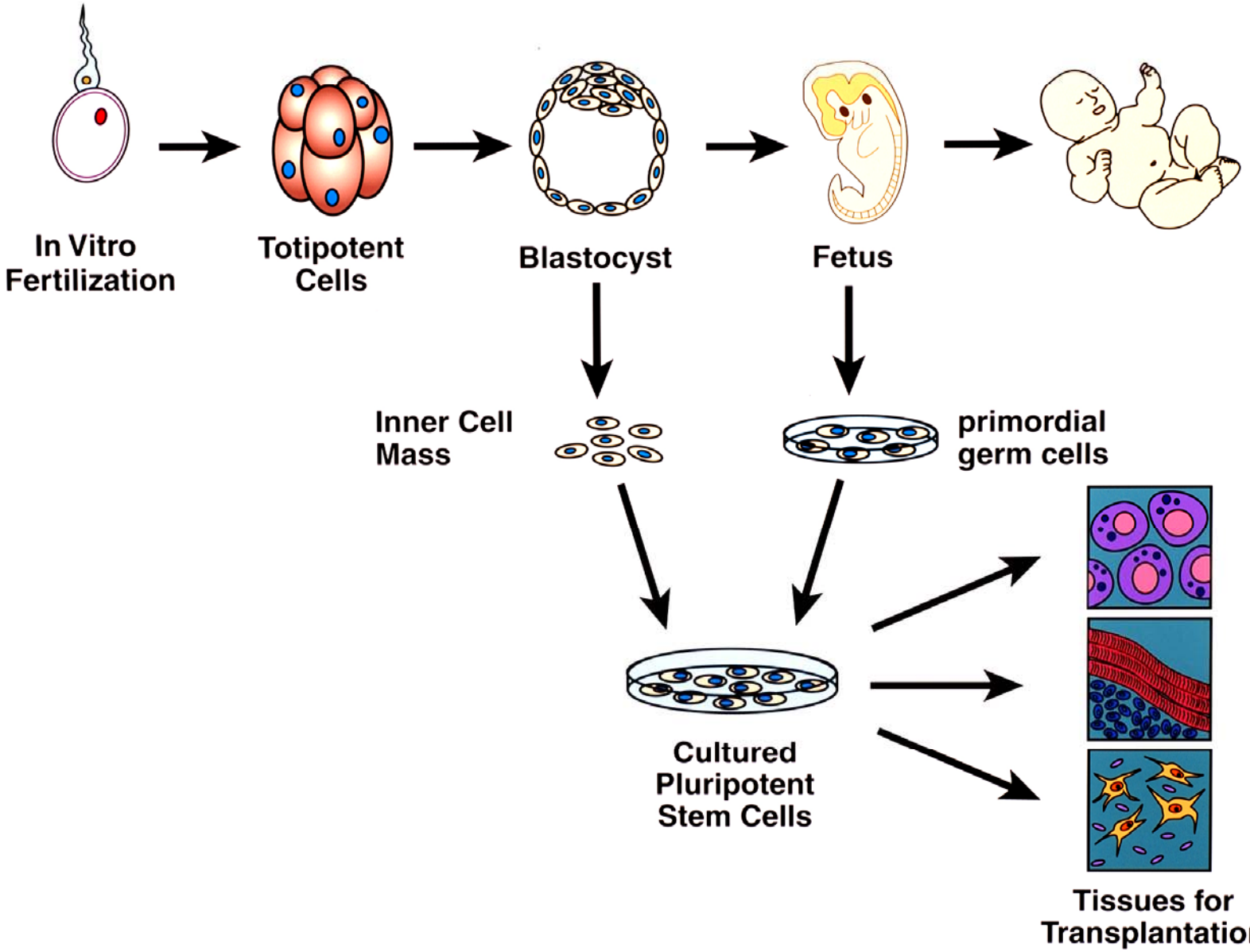
Wohlstetter Conference Center

March 9, 2005

The Promise of Stem Cell Research

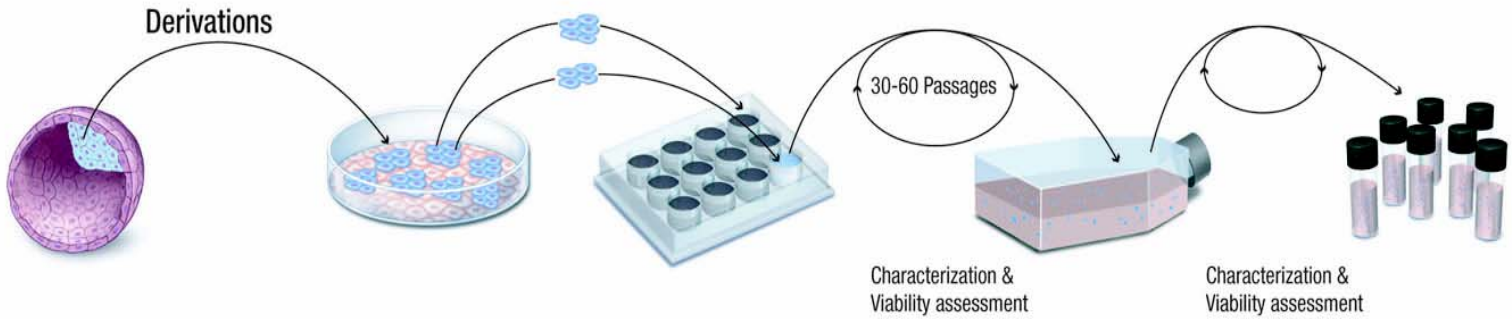


Two Ways to Make Pluripotent Stem Cells



Establishing Human Embryonic Stem Cell Lines

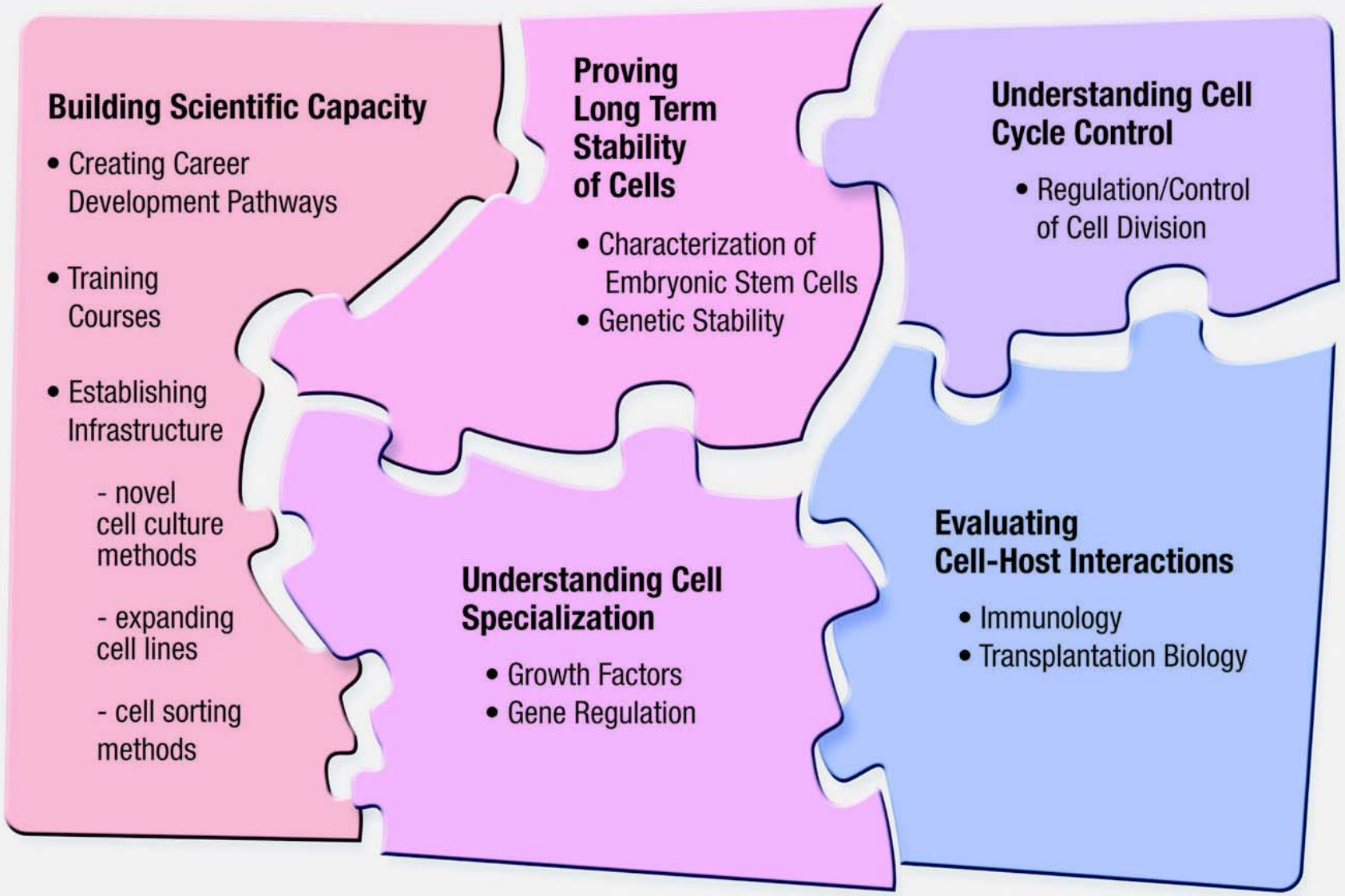
Blastocyst	Primary Colonies	Sub-cultures	Cell Lines Established	Cells for Distribution
Removal of 50-100 cells from inner cell mass	1-1000 Cells	1000 Cells per well 10 % Survival	Expansion of sub-cultures 1 flask grows up to 100,000 cells - 1 bank requires about 2 billion cells	Approximately 2 million cells needed per vial
3-5 days	1-7 days	1-7 days per passage	6-9 months	Ready for distribution



Up to 1 Year

The Scientific Challenges of Human Stem Cells

Basic Research Phase →



The Immediate Challenge for NIH and Human ES Cell Research

- Generate and characterize distribution quality human ES cell lines from the NIH Registry
- Stimulate more research on basic biology
- Train investigators to culture and use stem cells

Eligibility Criteria for Federal Funding

Stem Cell Information

The official **National Institutes of Health** resource for stem cell research

Information on Eligibility Criteria for Federal Funding of Research on Human Embryonic Stem Cells

On August 9, 2001, at 9:00 p.m. EDT, the President announced his decision to allow Federal funds to be used for research on existing human embryonic stem cell lines as long as prior to his announcement (1) the derivation process (which commences with the removal of the inner cell mass from the blastocyst) had already been initiated and (2) the embryo from which the stem cell line was derived no longer had the possibility of development as a human being.

In addition, the President established the following criteria that must be met:

- The stem cells must have been derived from an embryo that was created for reproductive purposes;
- The embryo was no longer needed for these purposes;
- Informed consent must have been obtained for the donation of the embryo;
- No financial inducements were provided for donation of the embryo.

In order to facilitate research using human embryonic stem cells, the NIH created the Human Embryonic Stem Cell Registry, which lists the human embryonic stem cell lines—at varying stages of development—that meet the eligibility criteria. Listed below are entities that have developed stem cell lines that meet the President's criteria and are therefore eligible for federal funding. Please click on the name of the laboratory or company for contact information.

NIH Stem Cell Registry

Name	Derivations	Available Lines
BresaGen, Inc., Athens, Georgia * The cells in line BG04/hESBGN-04 failed to expand into undifferentiated cell cultures.	4	3*
Cell & Gene Therapy Research Institute (Pochon CHA University), Seoul, Korea	2	
Cellartis AB, Göteborg, Sweden * Cell line SA03/Sahlgrenska 3 withdrawn by donor.	3	2*
CyThera, Inc., San Diego, California * The cells failed to expand into undifferentiated cell cultures.	9	0*
ES Cell International, Melbourne, Australia	6	6
Geron Corporation, Menlo Park, California	7	
Göteborg University, Göteborg, Sweden	16	
Karolinska Institute, Stockholm, Sweden * The cells failed to expand into undifferentiated cell cultures.	6	0*
Maria Biotech Co. Ltd. – Maria Infertility Hospital Medical Institute, Seoul, Korea	3	
MizMedi Hospital—Seoul National University, Seoul, Korea	1	1
National Centre for Biological Sciences/Tata Institute of Fundamental Research, Bangalore, India	3	
Reliance Life Sciences, Mumbai, India	7	
Technion-Israel Institute of Technology, Haifa, Israel	4	3
University of California, San Francisco, California	2	2
Wisconsin Alumni Research Foundation, Madison, Wisconsin	5	5

If investigators or institutions have additional human embryonic stem cell lines that they believe are eligible for listing on the Registry, please contact NIH at stemcell@mail.nih.gov.

FY 2003 NIH hESC Awards

- 8 Infrastructure Awards
- 26 Investigator-initiated Awards
- 67 Administrative Supplements
- 3 Pilot & Feasibility Projects (Beta Cell Consortium)
- 2 Postdoctoral Fellowships
- 6 Training Grants (5 short-term cell culture training courses
- 3 Exploratory Center Research Grants

Infrastructure Awards

- Awards to organizations with entries on NIH hESC Registry available for Federal funds to develop into distribution-quality cell lines
- Two year period of support
- Nine awards for total of over $6M
- Led to development of 22 hESC lines ready for shipment with more to come in near future

Stem Cell Training

- Short-term Courses in Human Embryonic Stem Cell Culture Techniques held at
  - The Jackson Laboratory
  - Univ. of Georgia
  - Univ. of Pittsburgh/Magee-Women's Res. Inst.
  - Children's Hospital of Orange County
  - Technion-Israel Institute Of Technology

NIH Intramural Research

- Several laboratories at NIH are currently using hESC in their research
- Expanding interest as cell line availability becomes more straightforward
- Creation of a stem cell characterization unit within Intramural Research Program at NIH

Centers of Excellence for Translational Stem Cell Research

- Multidisciplinary teams of stem cell experts, clinical researchers, and transplant surgeons
- Speed translation of basic knowledge to clinical therapies for human disease
- Test adult and embryonic stem cell therapies for many diseases including diabetes, heart disease, and neurological disorders

National Embryonic Stem Cell Bank

- Ready source of human embryonic stem cells (hESCs)
- Compare and expand hESCs available to NIH-supported scientists
- Ensure consistent hESC quality
- Reduce cost to obtain cells listed on the NIH Registry

NIH Supports Research on Many Types of Stem Cells

- Early phase of research—breakthroughs with clinical relevance might emerge from research on many types of stem cells
- FY2003 investment in human non-embryonic stem cell research--$190.7 million
- FY2003 investment in human embryonic stem cell research--$20.3 million
- Promote growth in both areas in the future

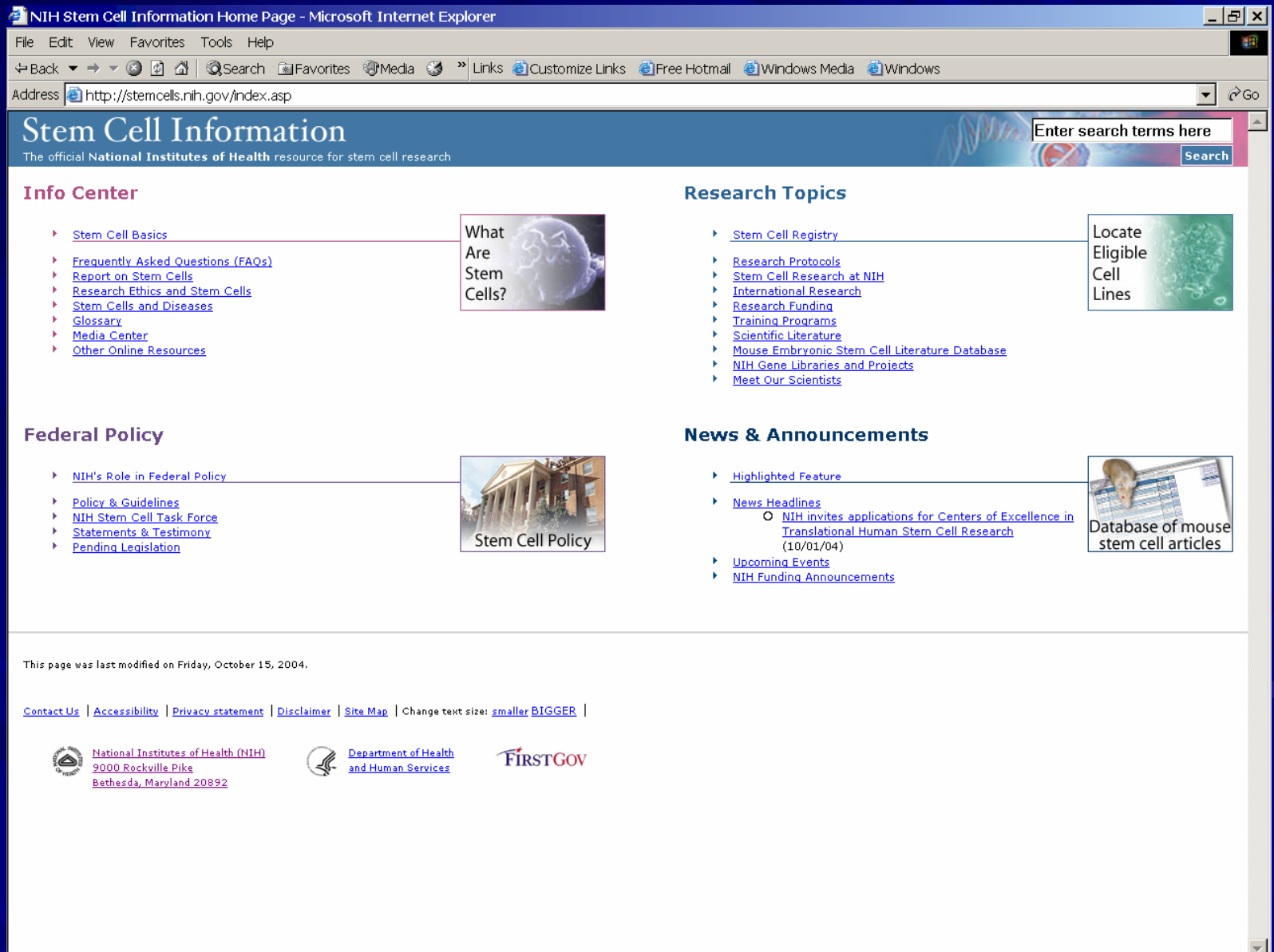
On the Research Horizon

- Definition of standardized human ES cell culture conditions that obviate the need for either mouse or human feeder cells
- Enabling tools and technologies to further characterize stem cells as they become specialized cells (specific antibodies, etc.)
- Define the molecular pathways that specify differentiation into different specialized cells

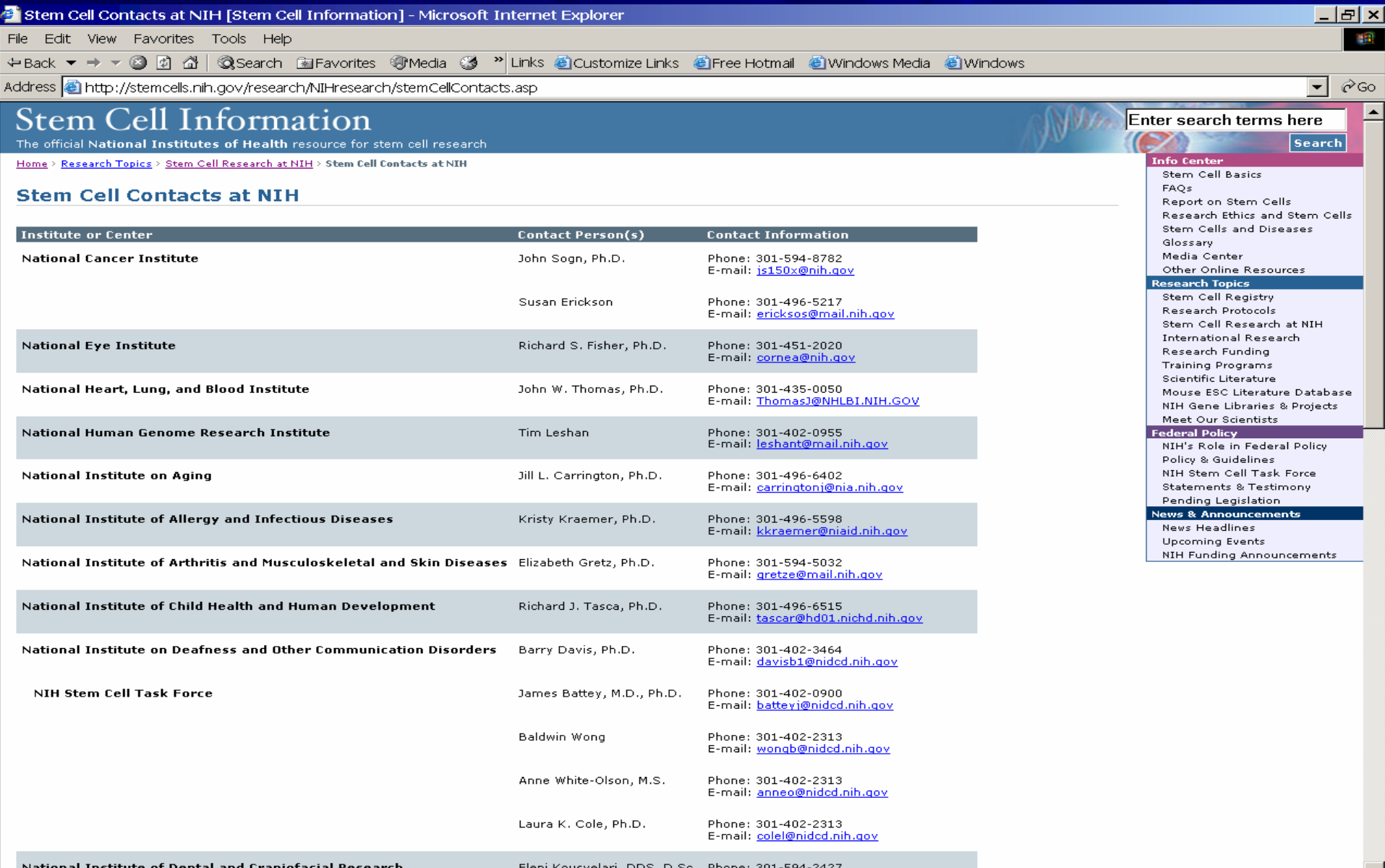
On the Research Horizon (cont.)

- Determine the factors/conditions critical for long-term survival and function of transplanted cells in a host
- Understand control of cell division—essential to expand cells before specialization, but must be tightly regulated after transplantation for therapy

NIH Stem Cell Website



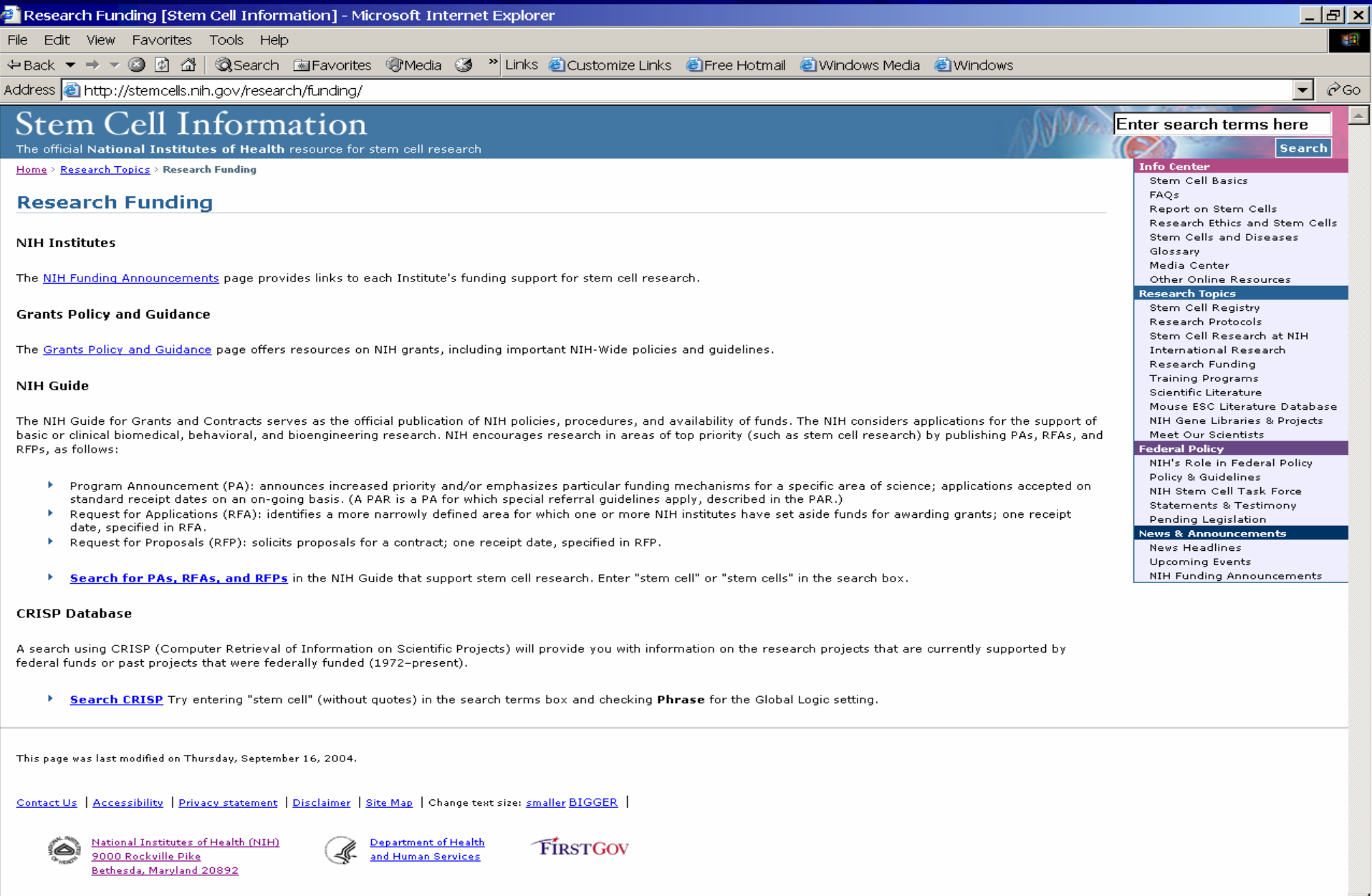
Stem Cell Contacts at NIH



Stem Cell Contacts at NIH

Institute or Center	Contact Person(s)	Contact Information
National Cancer Institute	John Sogn, Ph.D.	Phone: 301-594-8782 E-mail: js150x@nih.gov
	Susan Erickson	Phone: 301-496-5217 E-mail: ericksos@mail.nih.gov
National Eye Institute	Richard S. Fisher, Ph.D.	Phone: 301-451-2020 E-mail: cornea@nih.gov
National Heart, Lung, and Blood Institute	John W. Thomas, Ph.D.	Phone: 301-435-0050 E-mail: ThomasJ@NHLBI.NIH.GOV
National Human Genome Research Institute	Tim Leshan	Phone: 301-402-0955 E-mail: leshant@mail.nih.gov
National Institute on Aging	Jill L. Carrington, Ph.D.	Phone: 301-496-6402 E-mail: carringtonj@nia.nih.gov
National Institute of Allergy and Infectious Diseases	Kristy Kraemer, Ph.D.	Phone: 301-496-5598 E-mail: kkraemer@niaid.nih.gov
National Institute of Arthritis and Musculoskeletal and Skin Diseases	Elizabeth Gretz, Ph.D.	Phone: 301-594-5032 E-mail: gretze@mail.nih.gov
National Institute of Child Health and Human Development	Richard J. Tasca, Ph.D.	Phone: 301-496-6515 E-mail: tascar@hd01.nichd.nih.gov
National Institute on Deafness and Other Communication Disorders	Barry Davis, Ph.D.	Phone: 301-402-3464 E-mail: davisb1@nidcd.nih.gov
NIH Stem Cell Task Force	James Battey, M.D., Ph.D.	Phone: 301-402-0900 E-mail: batteyj@nidcd.nih.gov
	Baldwin Wong	Phone: 301-402-2313 E-mail: wongb@nidcd.nih.gov
	Anne White-Olson, M.S.	Phone: 301-402-2313 E-mail: anneo@nidcd.nih.gov
	Laura K. Cole, Ph.D.	Phone: 301-402-2313 E-mail: colel@nidcd.nih.gov
National Institute of Dental and Craniofacial Research	Eleni Kousvelari, DDS, D.Sc.	Phone: 301-594-2427

NIH Funding Opportunities



NIH Stem Cell Website and Registry

- Stem Cell Information
  - http://stemcells.nih.gov
- Human Embryonic Stem Cell Registry
  - http://escr.nih.gov