Stem Cell Research Policy: Federal and State

Wise Young, PhD MD

W. M. Keck Center for Collaborative Neuroscience

Rutgers University, Piscataway, New Jersey

Federal Stem Cell Policy

- On August 9, 2001, President Bush recognized the importance of stem cell research and allowed NIH to fund research on embryonic stem cell lines derived before his speech.
- Unintended consequences of this policy:
  - Embryonic stem cell research stalled in the US.
  - Other countries have pulled ahead of the U.S. in all types of stem cell research, including umbilical and adult stem cells.
  - The policy has not saved any embryos.
- Several states have responded by passing laws to support stem cell research.

State-funded Stem Cell Research

- States have begun funding stem cell research
  - California: $300 million/year for 10 year
  - New Jersey: $150 million in 2005 and $30 million/year for 7 years
  - Other states have proposed stem cell research funding:
    - Illinois ($1 billion), Pennsylvania ($1 billion), Washington ($1 billion), Wisconsin ($768 million), Maryland, Minnesota, and several states following suit.
- By 2006, state funding of stem cell research may exceed federal funding by an order of magnitude.
- This is the first time states are funding biomedical research of this magnitude.

Implications of State Funding

- Given NIH funding <15% of research applications in 2005, many scientists may move to states funding research.
- Pharmaceutical and biotechnology companies will concentrate in states funding stem cell research.
- This trend is not good for the country or stem cell research
  - We are not utilizing the talent of all scientists in the country on stem cell research.
  - Scientists in states that do not fund stem cell research will be at a significant disadvantage.
  - Universities and higher education will suffer in states that do not fund stem cell research.

Questionable Assumptions

- Recent scientific advances challenge fundamental assumptions in stem cell debate and policy:
  - Cellular collection vs. production.
  - Cloning vs. immune matching
  - Somatic cell nuclear transfer vs. fusion.
  - Pluripotency vs. tumor risk.
- Stem policy in the United States should take these advances into account.

Conclusions

- The current federal stem cell policy has failed because it has held back stem cell research, America is losing its leadership role in biomedical research, and it has not saved any embryos.
- State funding of stem cell research will concentrate stem cell science in a few states with movement of pharmaceutical and biotechnology industry. This is not good for the nation or stem cell research
- The current stem cell debate ignores scientific advances and is not addressing several serious scientific problems: production of stem cells, immune matching, cell fusion, and tumor risk.
- No current source of cells is sufficient to millions of people. We face a moral catastrophe when the first effective cell-based therapy is reported for a major disease.

Cellular Expansion

- All current sources of stem cells are inadequate:
  - Only 250,000 bone marrow donors
  - Only 150,000 units of umbilical cord blood
  - Fetal and embryonic sources are very limited
- In the near future, clinical trials will show that a cell-based therapy ameliorates or even cures a major disease. If we don't have the cells to treat millions
- The future of stem cell therapies will require growth and production stem cells in large numbers.

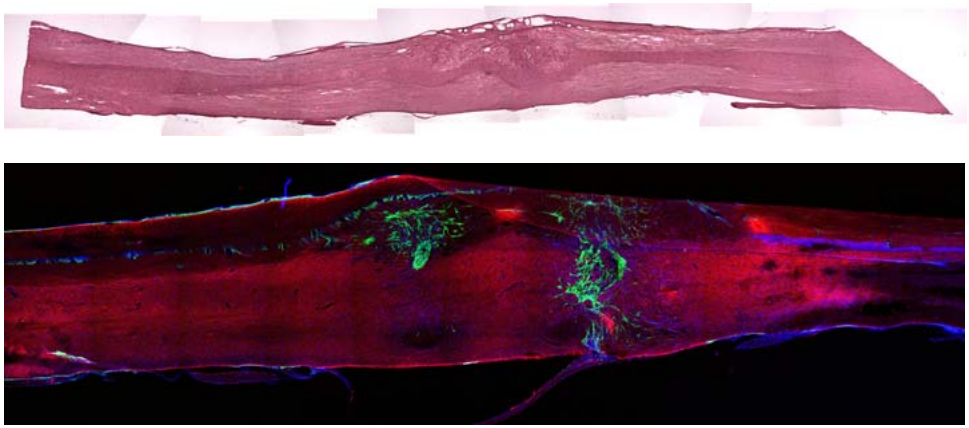
Cloned Stem Cells

- Two false assumptions underlie this discussion.
  - Cloning is necessary to produce genetically compatible cells for transplantation.
    - We have successfully transplanted antigen-matched organs, bone marrow, and umbilical cord blood for decades.
    - Selective immune suppression will soon be available.
  - Somatic cell nuclear transfer is the only way to clone stem cells.
    - Cloning methods now use fusion rather than nuclear transfer.
    - Recent studies suggest that fusion of somatic cells with stem cells can produce stem cells without having to use embryos.

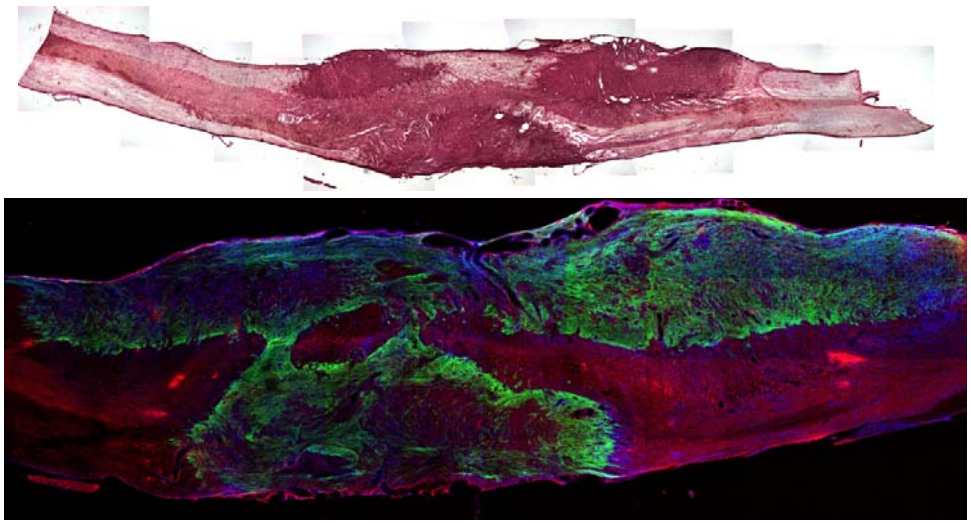
Risks and Expectations

- Any cell cultured for long periods will have a risk of malignant transformation. This is true of embryonic, fetal, umbilical cord blood, and adult stem cells.
- What is a tumor? A tumor is any cell that produces inappropriate types or numbers of cells that do not respect tissue boundaries.
- No cell will behave appropriately in all tissues.
  - How does a cell placed into brain, pancreas, or liver “know” that it should make neurons, insulin-producing cells, or hepatocytes?
  - Researchers are now pre-differentiating cells before transplantation to ensure that they have cells that behave more predictably.
  - Differentiation is turning out to be a good way to screen for malignant transformation.

Neonatal Blood Stem Cell Tumor



2 weeks after transplantation



4 weeks after transplantation

Demand and Supply

- The stem cell debate ignores a very serious problem.
- In the coming years, clinical trials are likely to show that a cell-based therapy can cure or significantly ameliorate a major disease.
- No current source of cells can treat millions of people.
  - Only 250,000 bone marrow donors
  - Only 150,000 units of umbilical cord blood
  - Only 5000 fetal donors per year
  - Only 100 or fewer embryonic stem cell lines
- Demand will outstrip supply. Will we have lotteries or will cell-based treatments be available only to the wealthy and well-connected?