STEM CELLS CLAIMS AND FACTS

CLAIM #1: Stem cells for all cell and tissue types have not been found in the adult human.

FACT: No human stem cell—adult or embryonic—has been found for all cell and tissue types. However, researchers have identified a cell in mice, derived from adult bone marrow, which appears capable of becoming virtually any cell in the body. "Our findings are significant," according to the researchers, "because we identified one cell that has the capacity to differentiate into all of these organs." According to the researchers, a "similar cell probably exists in humans, but we haven't tested that yet. … There is no reason to assume that a cell similar to one identified in the current study doesn't exist in humans." (Cell 105, 369- 377; May 4, 2001, "Adult Stem Cell Found That Can Transform into Nearly Any Organ Cell Type," Reuters Health; May 3, 2001).

No embryonic stem cell has shown similar success.

It is misleading to assume that there needs to be a separate adult stem cell for each tissue; there are numerous recent reports of adult stem cells being transformed from one tissue type to another (e.g., bone marrow to liver or nerve; nerve to blood). Thus adult stem cells have the capacity to form many more tissues than the one from which they are derived (e.g., "Multilineage potential of adult human mesenchymal stem cells," Science 284, 143-147, Apr. 2, 1999; "Liver from Bone Marrow in Humans," Hepatology 32, 11-16, July, 2000; "From marrow to brain: expression of neuronal phenotypes in adult mice," Science 290, 1775-1779; Dec. 1 2000; "Turning blood into brain: Cells bearing neuronal antigens generated in vivo from bone marrow," Science 290, 1779-1782; Dec. 1 2000; "Adult Bone Marrow Stromal Cells Differentiate into Neural Cells In Vitro," Experimental Neurology 164, 247-256; Aug. 2000; "Turning Brain into Blood: a hematopoietic fate adopted by adult neural stem cells in vitro," Science 283, 534-537, Jan. 22, 2000).

Adult stem cells are considered by some researchers (including NIH funded researchers) to be pluripotent, similar to embryonic stem cells, with the ability to form any tissue necessary (e.g., "Multi-Organ, Multi-Lineage Engraftment by a Single Bone Marrow-Derived Stem Cell," Cell 105, 369-377; May 4, 2001; "Generalized Potential of Adult Neural Stem Cells," Science 288, 1600-1663; June 2, 2000; Adult Rat and Human Bone Marrow Stromal Cells Differentiate Into Neurons," Journal of Neuroscience Research 61:364-370; Aug. 2000).

CLAIM #2: Embryonic stem cells offer greater potential for clinical use than adult stem cell.

FACT: A single human clinic use for embryonic stem cells has yet to be found. Adult stem cells, on the other hand, have been successfully utilized for a number of conditions and have, in many cases, shown more potential than embryonic stem cells.

Cultured human embryonic stem cells have not, for instance, been made to differentiate into many tissue types, including cardiac or pancreatic stem cells. Adult stem cells for these tissues, however, have been identified in mice, and adult pancreatic stem cells have been used to reverse diabetes in mice ("Reversal of insulin-dependent diabetes using islets generated in vitro from pancreatic stem cells," Nature Medicine 6, 278-282; March, 2000). While the adult stem cells successfully reversed diabetes in the mice, mice receiving embryonic stem cells still died from diabetes. As with most biological discoveries, animal models have paved the way for clinical uses in humans; we should expect that these same adult stem cells to be present in humans.

Moreover, while human embryonic stem cells have yet to be shown to undergo differentiation to insulin-secreting cells, scientists at Harvard Medical School cultured human pancreatic ductal cells under specific conditions, inducing the cells to form islet buds and secrete insulin. They report: "Thus, duct tissue from human pancreas can be expanded in culture and then be directed to differentiate into glucose responsive islet tissue in vitro. This approach may provide a potential new source of pancreatic islet cells for transplantation" ("In vitro cultivation of human islets from expanded ductal tissue," Proc Natl Acad Sci USA 97, 7999-8004; July 5, 2000).

Researchers in France have found further evidence for adult pancreatic stem cells in humans. The pancreatic cells from healthy donors, when placed into culture, proliferated and expressed characteristics critical for production and secretion of insulin ("Adult human cytokeratin 19-positive cells re-express insulin promoter factor 1 in vitro: Further evidence for pluripotent pancreatic stem cells in humans," Diabetes 49, 1671-1680; Oct. 2000.) The results are another step toward treatment of diabetes using adult stem cells.

A recent comprehensive review (1/01) in the British Medical Journal of possible stem cell treatments for diabetes notes: "Human pancreatic duct cells have also been grown successfully in vitro and induced to differentiate," and "Not only does the use of adult donor ductal cells avoid the controversy of using fetal cells but there are fewer biological problems associated with making beta cells from duct cells than from, for example, embryonic stem cells." The authors conclude: "Of the techniques described above, the most promising is generation of beta cells from pancreatic duct cells. It is inherently a shorter biological step to make a beta cell from a duct cell than it is from other possible cells, such as embryonic stem cells and haemopoietic stem cells" (P. Serup et al., "Islet and stem cell transplantation for treating diabetes," British Medical Journal 322, 29-32; 6 Jan 2001).

Regarding cardiac tissue, research using adult stem cells to treat heart disease has been done with mice and in clinical trials in humans. Adult stem cells in mice have been shown not

only to form cardiac tissue, but also to successfully regenerate damaged heart tissue ("Bone marrow cells regenerate infarcted myocardium," Nature 410, 701-705; Apr. 5, 2001; "Marrow stromal cells for cellular cardiomyoplasty: Feasibility and potential clinical advantages," The Journal of Thoracic and Cardiovascular Surgery 120, 999-1006; Nov. 2000).

In human clinical trials, patients have been successfully treated for heart disease using their own muscle stem cells ("Myoblast transplantation for heart failure." Lancet 357, 279-280, Jan 27, 2001; "Doctor Puts Arm Muscle Cells Into Patient's Heart," Associated Press, May 30, 2001; "First Percutaneous Endovascular Case of Heart Muscle Regeneration Completed with Bioheart's MyoCell(TM) Product," PRNewswire, May 30, 2001.) No embryonic stem cells have ever been reported to be used in human clinical trials.

CLAIM #3: Stem cells in adults are often present in only minute quantities, are difficult to isolate and purify, and their numbers may decrease with age.

FACT: Research is showing these claims not to be true. Data from April 2001 shows that only ONE transplanted adult stem cell may be able to regenerate tissue in several parts of the body ("Multi-Organ, Multi-Lineage Engraftment by a Single Bone Marrow-Derived Stem Cell"; Cell 105, 369-377; May 4, 2001).

In fact, research indicates that previously reported human stem cell frequencies and their self-renewal activity have been markedly underestimated and that sufficient numbers of adult stem cells can be easily generated for clinical applications ("High marrow seeding efficiency of human lymphomyeloid repopulating cells in irradiated NOD/SCID mice," Blood 96, 3979-3981; Dec. 1 2000; "Ex vivo expansion of human umbilical cord blood and peripheral blood CD34(+) hematopoietic stem cells"; Experimental Hematology 28, 1297-1305; Nov. 1 2000).

Canadian scientists have identified a way to make adult stem cells grow in the laboratory in much the same way as they do in the developing human embryo. Adult bone marrow stem cells and cord blood cells, when treated with a naturally-occurring protein dubbed "sonic hedgehog" by its discoverer, grow in culture similar to the way that embryonic stem cells grow. The protein stimulates growth of significant quantities of adult stem cells (G. Bhardwaj et al., "Sonic hedgehog induces the proliferation of Immunology 2, 172-180; Feb. 2001

In March 2000, researchers in Philadelphia identified the conditions to allow large-scale expansion of adult stem cells in culture, making these cells an almost unlimited resource. The researchers achieved a billion-fold increase in a few weeks for bone marrow stem cells in culture. ("Rapid expansion of recycling stem cells in cultures of plastic-adherent cells from human bone marrow," Proceedings of the National Academy of Sciences 97, 3213-3218; March 28, 2000).

In August 2000 research funded by the NIH itself and the Christopher Reeve Paralysis Foundation found that adult human bone marrow stem cells can create a "virtually limitless supply" of nerve cells ("Christopher Reeve Paralysis Foundation Funds Breakthrough Research," Press Release of the Christopher Reeve Paralysis Foundation, 8/14/00). According to the

published research results, the adult stem cells "grow rapidly in culture, precluding the need for immortalization, and differentiate into neurons exclusively with use of a simple protocol" ("Adult Rat and Human Bone Marrow Stromal Cells Differentiate Into Neurons," Journal of Neuroscience Research 61, 364-370: Aug. 2000).

CLAIM #4: Brain cells from adults can only been obtained with a complex and invasive procedure that carries the added risk of further neurological damage.

FACT: Human neural stem cells have been isolated from other, more accessible regions of the brain from numerous volunteers ("Isolation and Characterization of Neural Stem Cells from the Adult Human Olfactory Bulb, Stem Cells 18, 295-300; July 2000), and even from cadavers ("Progenitor cells from human brain after death," Nature 411, 42-43; May 3, 2001).

The NIH/Christopher Reeve Paralysis Foundation research demonstrates that adult bone marrow stem cells can form nerve cells, eliminating the need to isolate such cells from the patient's brain: "The marrow cells are readily accessible, overcoming the risks of obtaining neural stem cells from the brain, and provide a renewable population. Autologous transplantation overcomes the ethical and immunological concerns associated with the use of fetal tissue" ("Adult Rat and Human Bone Marrow Stromal Cells Differentiate Into Neurons," Journal of Neuroscience Research 61, 364-370; Aug. 2000).

In addition, studies with mice have shown that, given appropriate signals, neural stem cells do not need to be removed from the brain at all for growth. Rather, they can be stimulated to regrow while still residing within the brain. The re-growth could take place even in regions of the adult mammalian brain that do not normally undergo new cell growth. The researchers report: "Our results indicate that neural replacement therapies for neurodegenerative diseases and CNS injury may be possible through manipulation of endogenous neural precursors in situ" ("Induction of neurogenesis in the neocortex of mice," Nature 405, 951-955, 6/22/00). Again, discoveries in animal models will almost certainly lead to applications in humans.

CLAIM #5: In disorders that are caused by a genetic defect, the genetic error likely would be present in the patient's stem cells, making cells from such a patient inappropriate for transplantation.

FACT: Such transplantation is exactly what was done for three children in France, as reported in April of this year. The infants, who had a genetic defect that caused severe immunodeficiency disease, had some of their own bone marrow cells removed. The cells were cultured, the defective gene causing the immune deficieny replaced, and the children were then treated with their own stem cells. This experiment using adult stem cells appears to be the first successful instance of a cure by human gene therapy ("Gene Therapy of Severe Combined Immunodeficiency (SCID)-X1 Disease," Science 288, 669-672, 4/28/00).

Moreover, correction of the genetic defect may not always be necessary to effect a cure with adult stem cells. The British medical journal Lancet reports researchers treating systemic lupus (an incurable and sometimes fatal autoimmune disease) using the patients' own bone marrow cells. When transplanted back into the patients, the cells appeared to have overcome the defect in all patients and repaired organ damage previously considered permanent. The scientists noted: "It is mysterious that the transplanted cells, which have the same genetic defect that made the patients' immune cells go wrong in the first place, did not grow up to repeat the mistakes of their siblings" ("Treatment of severe systemic lupus erythematosus with high-dose chemotherapy and haemopoietic stem-cell transplantation: a phase I study," Lancet 356, 701-707, August 2000).

CLAIM #6: The embryos from which the stem cells would be derived are spares that are leftover at fertility clinics. They have no use and will be destroyed regardless of whether they are used for research or not.

FACT: No human embryo is "spare" or "leftover." This inaccurate assumption is based on a misreading of the NIH guidelines for embryonic stem cell research. When fertility clinics classify frozen embryos as "in excess of clinical need" (which simply means that they are not needed by the parents NOW to meet their reproductive goals), parents are given several choices: to keep storing the embryos for possible later use (chosen by the vast majority), to donate them to another couple so they can have a child, or to discard them. The NIH guidelines say that the option of having the embryos killed for federally funded stem cell research must be offered AT THE SAME TIME AS ALL THESE OTHER OPTIONS. The government will be giving parents an incentive to destroy their embryos instead of preserving their lives.

Embryo adoption affords every embryo not currently needed hope for life. Based upon a conservative figure of only 150,000 frozen human embryos in in-vitro fertilization clinics, a conservative thaw survival rate of fifty percent, and a pregnancy rate of between sixteen to thirty percent (national average), between 11,000 to 22,000 children could be born and placed for adoption with some of the 2 million infertile married couples in America.

Every human embryo is needed to be given a chance for life by the countless loving couples who cannot conceive children themselves. A decision to authorize the federal funding of human embryo destruction is a decision to deny life to at least 11,000 to 22,000 children.

It is also false to state that all of the embryos being used for research are only fertility clinic "leftovers." Some researchers have created human embryos for the specific purpose of killing them to obtain their stem cells. The Jones Institute for Reproductive Medicine in Virginia, for example, created 110 embryos intended merely for the harvesting of stem cells. Sperm and egg donors were screened to ensure the creation of the healthiest embryos (Los Angeles Times, July 11, 2001). A Massachusetts biotechnology company-- Advanced Cell Technology of Worcester-- is attempting to create cloned human embryos from which embryonic stem cells could be derived for research purposes (Washington Post, July 12, 2001).

CLAIM #7: The prohibition on federal funding will end research on embryonic stem cells.

FACT: Privately funded embryonic stem cell research has occurred for years without federal funds and will be unaffected by the prohibition on federal funding. Researchers have obtained funding from investors and biomedical charities such as the Juvenile Diabetes Research Foundation, and obtained stem cells from suppliers in Israel, Australia and the United States. A team of researchers from Singapore, Australia and Israel have also started a new distributing company, ES Cell Australia Pty. to dispense these cells. The University of Wisconsin, where human embryonic stem cells were first discovered in 1998, has established the WiCell Institute that supplies cells to nearly 30 academic labs and one company. Scientists forecast that more labs will be providing access to cells within several years (The Wall Street Journal, March 21, 2001). In addition to the 30 requests for embryonic stem cells that have been filled by WiCell, another 60 requests are currently pending (New York Times, July 10, 2001).

With a $58.5 million gift from an anonymous donor, the Johns Hopkins University School of Medicine will create an Institute for Cell Engineering that will include stem cell research. (Associated Press, January 31, 2001). The institute, slated to be the first initiative of its kind at an academic medical center, will "advance Hopkins'… program of embryonic stem cell research" according to the University. The center is scheduled to open in 2003 (Johns Hopkins release, January 30, 2001).

Privately funded stem cell research is also being conducted at Harvard University (San Francisco Chronicle, February 25, 2001).

Dr. James A. Thomson, the first person to isolate stem cells from human embryos, admits that even without federal funding "the research will go forward" (New York Times, July 10, 2001).

CLAIM #8: Federal law allows for funding of stem cells derived from embryos.

FACT: Despite the ongoing debate, the prohibition on embryonic stem cell research using federal funds is quite clear: Federal funds can not be used for any studies that involve stem cells derived from human embryos.

Section 510 of the Health and Human Services appropriations bill for Fiscal Year 2001 (Public Law 106-554) states: "None of the funds made available in this Act may be used for (1) the creation of a human embryo or embryos for research purposes; or (2) research in which a human embryo or embryos are destroyed, discarded, or knowingly subjected to risk of injury or death greater than that allowed for research on fetuses."

If the NIH instructs researchers in how to select, obtain and destroy embryos to qualify for federal stem cell research grants, and the federally funded research depends for its existence on such destruction of embryos, it is hard to deny that the NIH is supporting "research in which" embryos are destroyed.

Unquestionable, deriving stem cells from an embryo subjects the embryo to "risk," "injury" and almost universally results in the "death" of the embryo. Any research on such cells, therefore, is prohibited by both the letter and intent of the law.