Stem Cell Science Leaders Say
Nation Must Pursue all Avenues of Stem Cell Research

Hopes for Medical Research at the Crossroads Again

Continuing support for stem cell research that seeks to understand and treat a myriad of complex diseases and injuries affecting millions of Americans cannot be held hostage to political decisions that are based on unsound or premature interpretations of scientific discoveries. Patients, their relatives, friends and indeed the entire community depend on scientists to use all their experience and innovation to find solutions that will result in a better quality of life and health for all those affected.

Human Embryonic Stem Cell (hESC) Research Must Continue

Recent exciting advances in the reprogramming of cells in adult human tissues, to convert them into cells that resemble embryonic stem (ES) cells (induced pluripotent stem cells, iPS cells), or to redirect cell fate to enhance tissue repair, have captured the imagination of the scientific community worldwide. Many scientists are very optimistic about the future of this new research. Unfortunately, some in political circles have interpreted this enthusiasm as a verdict that research on human ES cells is no longer necessary. This conclusion is premature and is not scientifically justified.

All stem cell types and approaches are critical to developing research strategies that will ultimately provide new means for tissue regeneration and repair. Supporting all forms of stem cell research is in the best long-term interests of a broad spectrum of patients with complex and debilitating diseases and injuries.

It presently is not possible to predict exactly what research strategy will provide the most successful therapeutic intervention for a given disease or condition. While simple therapies may evolve around the use of small molecule drugs or delivery of autologous (patient's own) cells, it is more than likely that complex diseases will require a battery of drugs to control the immune and regenerative processes, and several types of laboratory produced cells to repair and rebuild damaged cells and tissues.

hESCs Remain the Prototype Pluripotent Cell

The basic tools for these discoveries will include the "gold standard" embryonic stem cells that remain the benchmark for assessment of pluripotency, or the ability to develop into all the fully functional cells of the body. The critical role of hESCs has been recognised by all the primary world scientific bodies, including, in the U.S., the National Academies of Sciences (advisors to the nation on science and engineering). This body has recently released the 2008 Amendments to the National Academies' Guidelines for Human Embryonic Stem Cell Research. They conclude "The substantial public support for hES (human embryonic stem) cell research and the growing trend by many non-federal funding agencies and state legislatures to support this field requires a set of guidelines to provide the framework for hES cell research." These guidelines set out the principles for ethics for scientists working out the cause of diseases, the possible repair mechanisms, the strategies for treatments and potential cures, and the hopes and aspirations of many millions of those who are directly affected with the worst of handicaps and degenerative diseases.

Induced Pluripotent Stem (iPS) Cells

Research efforts on ES cells, iPS cells and reprogramming of adult or tissue stem cells are not alternative or mutually exclusive pathways to discovery, but are in fact complementary and synergistic. It is most important to recognize that the recent success in reprogramming adult human cells into cells that closely resemble ES cells would not have occurred without the last decade of human ES cell research. Although ES cells and these newer iPS cells were both first discovered in the mouse, the human versions of these cells require significantly different conditions to sustain their growth. It was only through research on human ES cells that scientists determined the necessary conditions for iPS cells, and had we relied only on information from studies in the mouse, the recent attempts at deriving human iPS cells would have failed. Although the current generation of human iPS cells are unsuitable for human clinical use, we anticipate rapid improvements in the methods to derive these cells. However, it will likely be several years before we know whether the resulting iPS cells differ in clinically significant ways from human ES cells. During these years, the continued research being done on human ES cells is again expected to be directly applicable to human iPS cells. Should human iPS cells turn out to be somehow fatally flawed as a therapeutic platform, vital research on human ES cells must continue to move forward.

Adult or Tissue Stem Cells: A Cautionary Tale

Those rushing to restrict scientific investigation on the basis of early stage research findings that happen to suit a political agenda would do well to remember an important lesson the community has learned over the past five years or so.

As physicians and scientists it is our obligation to understand and to report the basic biomedical principles that underlie the development and function of the body, independent of politics, religion, hope and hype. Just 5-6 years ago a few laboratories claimed to have evidence that any adult tissue stem cell (e.g. blood-forming) could change its fate to produce other tissues (e.g. heart or brain) simply by placement in the other tissue, leading to a process called transdifferentiation. Those opposed to embryonic stem cell research on political, religious, and personal moral grounds cited these early, incomplete studies as established fact, and leapt upon them as a rationale to restrict human ES cell research.

Careful research has shown these claims to be erroneous interpretations of rare phenomena. Adult tissue stem cells are robust precursors of their own tissues, but are generally unable to produce other tissues without significant experimental modification of their genetic makeup. Only true pluripotent stem cells have the potential to develop into all cell types. The focus on transdifferentiation research has distracted resources from research that is more likely to yield new scientific insights and new therapies. Research must continue with all types of stem cells, and efforts to restrict this research based on recent “breakthroughs” must be resisted as such “alternatives” sometimes do not stand the test of time.

The Risk of Making Scientifically Ill-informed Determinations

The entire biomedical research enterprise is endangered when ideology or politics replace dispassionate investigation. Such ill-considered interventions have led to very serious threats to the entire U.S. government-funded research enterprise, replacing science with ideology, and replacing rational regulation with restrictions based on ideology that delay the search for truth and for new therapies. Banning research because it doesn’t fit the ideology of political

leadership has happened in the past in other countries, with the inevitable outcome of the withdrawal of those countries and their scientists from participation in the biomedical discoveries that dispassionate, regulated, but ideologically free research continually provides. And in those countries, the biomedical research and development communities could not engage in discoveries that lead to medical benefits, and they could not follow-up with the commercialization required to deliver the treatments to patients.

Discovery, Regulation and Medical Application

Governments must not limit free expression of thoughts and ideas, or freedom of inquiry. Scientists must perform research ethically, in accordance with regulatory oversight from legislation, local and national institutional review boards, the FDA, and the National Institutes of Health. The U.S. has been in past decades the world's center of biomedical innovation and the locus of the birth and development of the entire biotech industry. Literally millions of patients have seen their lives improved worldwide through therapies that resulted from adherence to these principles of freedom to pursue ideas and research directions without ideological bans. This has clearly been the American way.

David Baltimore, Ph.D.
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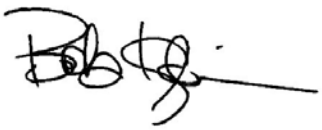

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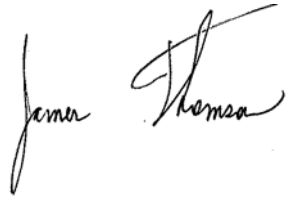

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