

House Judiciary Subcommittee
Intellectual Property, Competition and the Internet

Hearing

“Driving American Innovation: Creating Jobs and Boosting Our Economy”

Regenerative Medicine

Strategic Innovation for Patient Health, Economic Benefit, and Job Creation

The U.S. Department of Health and Human Services (HHS), calls regenerative medicine the “next evolution of medical treatments.” Its report, “2020: A New Vision – A Future for Regenerative Medicine,” says the field not only “holds the realistic promise of regenerating damaged tissues and organs in the living body,” but “empowers scientists to grow tissues and organs in the laboratory and safely implant them.” Indeed, regenerative medicine is not a pipe dream, but is already making its mark on health care. Skin and cartilage substitutes are available through regenerative medicine techniques and laboratory-grown bladders, tracheas, blood vessels and other tissues have been implanted in patients.

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Thank you, Chairman Goodlatte, Ranking Member Watt, Vice-Chairman Coble and members of the committee, for inviting me to testify today. It is an honor to be here. Following is my testimony regarding the field of regenerative medicine.

Background

Regenerative Medicine is an interdisciplinary field with scientists in molecular biology, genetics, cell biology, physiology, pharmacology, biomaterials and nanotechnology working collaboratively to deliver therapies that repair, replace or regenerate organs and tissues. The field is composed of the sub-disciplines of tissue engineering, cell therapies, and a new area often called healing therapies.

- Tissue Engineering is the science of growing replacement tissue in the lab to replace damaged or diseased tissue and organs. The process usually starts with a three-dimensional structure called a scaffold that is used to support cells as they grow and develop. Skin, blood vessels, bladders, trachea, esophagus, muscle and other types of tissue have been successfully engineered; and some of these tissues have already been used in treating human disease.
- Cell therapies apply living cells to an organ or tissue to promote healing and regeneration from within. Cell therapies are being delivered today for cartilage reconstruction, bone reconstruction, and in inflammatory and immune response problems. In the future, cell therapies hold promise for treating liver disease, diabetes, neural disorders, renal failure and other chronic conditions. Cell therapies are a promising area of research since it is simpler to heal existing tissues and organs than to replace them.
- Healing therapies are similar to cell therapies in that the goal is to restore the function of a tissue or organ. However, rather than using cells alone, various strategies are currently being studied with promising results, including using biomaterials to aid in cell recruitment for regeneration, and using small molecules to trigger a regenerative effect.

The goal of many regenerative therapies is to use a patient's own cells. These cells can include adult stem cells (found in many organs and tissues, including brain, bone marrow, and the blood) and progenitor cells (an immature type of cell found in almost every organ in the body). In cases where a patient's own stem cells cannot be obtained, there are several sources of stem cells. For example, scientists at the Wake Forest Institute for Regenerative Medicine discovered a type of versatile stem cell in amniotic fluid and placenta (afterbirth). And, using a new technique, stem cells can be created from skin cells.

The Wake Forest Institute for Regenerative Medicine (WFIRM) is recognized as a leader in the translation of scientific discoveries into therapies to benefit patients. Almost 300 scientists and staff collaborate on regenerative medicine initiatives at the institute, which is located in Piedmont Triad Research Park in Winston-Salem North Carolina. Its physicians and scientists were the first in the world to engineer laboratory-grown organs that were successfully implanted into humans. The Wake Forest Institute for Regenerative Medicine is involved in the full spectrum of activities required to move technologies from basic research to commercialization and the clinic. Examples include the following:

- The team is working to engineer replacement tissues and organs and develop healing cell and healing therapies for more than 30 different areas of the body. Several regenerative medicine therapies are already in patients, and others are in the pipeline, ready to begin testing in patients within the next few years. Projects range from treatments designed to cover burn wounds with the patient's own skin cells to the engineering of several tissue structures.
- The institute has seen rapid growth in intellectual property activity since its launch in North Carolina in 2004. The institute has filed a total of 85 invention disclosures and 175 patent applications adding immediate job growth among scientists and offering the

- potential for increased job growth as these technologies mature. The number of personnel has increased over 1000%.
- The Wake Forest Institute for Regenerative Medicine facility includes a Good Manufacturing Practices (cGMP) production facility that allows for the preparation of tissues and cell therapies under U.S. Food and Drug Administration guidelines. This facility helps accelerate clinical translation and commercialization.
 - The Wake Forest institute is involved as a member of the Armed Forces Institute of Regenerative Medicine (AFIRM), an \$85 million federally funded effort to apply regenerative medicine to battlefield injuries. The partnership involves support from all branches of the military, the Veterans Administration, and the National Institutes of Health. The program is administered through the US Army Medical Research & Materiel Command. AFIRM involves more than 30 institutions across the country working on regenerative medicine therapies. This project has brought significant funding to scientists to rapidly develop new treatments to benefit wounded warriors as well as civilians. Several clinical trials are currently active under this program.

The Potential

Regenerative Medicine, being an innovative new technology, has the opportunity to offer unique health benefits, benefits in effectiveness and cost reduction, and the potential to be the foundation of an exciting new job creating industry.

1. Patient Benefits: Unlike many other technologies, the science of regenerative medicine has the potential to not only manage disease, but also to provide a cure. The health benefit potential to patients receiving regenerative medicine technologies has already been shown with several technologies.

2. Health Care Cost Benefits: Because of its potential to cure – rather than merely treat disease –regenerative medicine offers the opportunity to combat rising health care costs. The opportunity for health care savings in the future is significant. In addition to the obvious benefits of reducing human suffering from disease, regenerative medicine has the potential to positively impact health care costs and workforce productivity and longevity.

Early estimates project that regenerative medicine therapies will result in direct health care cost savings in the United States of \$250 billion per year for the chronic diseases of renal failure, heart failure, stroke, diabetes, burn and spinal cord injuries. These savings are expected to grow as the technologies mature.

For example, according to “Economic Impact of RM, Cell Therapy and Tissue Engineering,” introducing a cure for juvenile diabetes five years earlier – such as in 2030 instead of 2035 – would generated an estimated \$12.5 billion in additional productivity.

3. Economic Benefits: Intellectual property protection in the field of regenerative medicine, as a result of increased funding and innovation, would enable the development and manufacturing of these technologies in the US, rather than abroad. According to Life Science Intelligence, the largely untapped global market potential for tissue engineering and regenerative medicine products will exceed \$118 billion by 2013. When new regenerative therapies are commercialized, there is the potential for new companies, manufacturing centers, medical device companies and added jobs.

Regenerative medicine represents the potential for economic benefit through the growth of companies and research institutions dedicated to its technologies. Some indicators of the economic potential of the regenerative medicine industry include:

- According to a 2001 U.S. National Academy of Sciences report, the potential number of patients who could benefit from regenerative medicine include: cardiovascular disease (58 million); autoimmune disease (30 million); diabetes (16 million).
- As an example, a large health care company entered the regenerative medicine market in 2002 with a bone morphogenic protein used in spine fusion. This became the first blockbuster regenerative medicine product with sales of more than \$600 million four years later.
- In 2008, there were over 500 companies involved in cell therapy.
- According to Global Industry Analysts Inc., “the active support of governments around the world and technological breakthroughs are expected to result in high growth for the industry in the near future.”

4. Job Creation: Employment in regenerative medicine is expected to increase as the research infrastructure grows. This will include both the number of working scientists and the potential for new companies based on the technologies they develop. Every new job in this sector could have a multiplier effect of up to 5.7 additional jobs in other employment sectors throughout the community, according to published findings by BIO, the nation’s leading biotechnology organization.

- Success in regenerative medicine will assist the United States maintaining its leadership in the health care field. The *US Department of Health and Human Services Report on this field states “...regenerative medicine will be the standard of care for replacing tissue/organ systems in the human body.”* Because regenerative medicine has the potential to dramatically alter the health care landscape, it will likely displace traditional therapeutic creating significant opportunities for job producing young companies.

Improving Innovation and Technology Delivery in Regenerative Medicine

There are many things that could be done to increase the pace of innovation and commercialization in the regenerative medicine field. The HHS report recommends a government/academic model for regenerative medicine, citing that a similar model helped grow the nation’s semiconductor industry from \$8 billion to \$170 billion in a 10-year period. Funding can help accelerate the translation of scientific discoveries through pre-clinical and clinical trials, manufacturing and commercialization strategies.

Intellectual property protection (patents in the case of most bioscience) helps ensure that new regenerative medicine therapies developed in the United States are commercialized. Since it takes a long time to perform clinical trials and clear the regulatory pathway the current patent process is essential to preserve the competitive position of the invention during this development period. In addition a patent once granted can aid in funding of the technology in a startup or add value to a larger company that licenses the technology for commercialization.

Funding of the technology and the new ventures that use them is essential to the competitiveness of the United States. The risk inherent in new technology ventures makes it very difficult to obtain startup capital for these startups. It is so difficult that this problem has been termed by the entrepreneurial community “the valley of death”. A mitigating factor and another area of very important funding are the SBIR (Small Business Innovation Research) and STTR (Small Business Technology Transfer) programs. These programs reserve an allocation of funding to

federal research groups for young companies that are the ones that are actually producing jobs and doing the important work of commercialization to bring technologies to patients. Significantly expanding the allocation of these agencies (such as NIH) so that more money is available for commercialization could be one of the most important things to do to speed innovation.

Streamlining the approval by the FDA of early stage clinical trials is another way that innovation could be improved in regenerative medicine. In recent years, the development of new therapies in the field of regenerative medicine that often involve new science that do not necessarily fit under the currently established regulatory guidelines, has made the regulatory pathway for these technologies difficult. Establishment of new regulatory guidelines that keep pace with the new scientific discoveries in this field, and targeted and collaborative efforts to reduce the cost and streamline the FDA regulatory process, would pay great dividends. This is an imperative for the United States since these regulatory retractions are significantly less in European countries and Asia.

In summary, the support of regenerative medicine, assuring intellectual property protection, expanding commercialization strategies such as SBIR/STTR programs, and streamlining approval of early stage clinical trials will help the United States maintain its leadership position in this sector by accelerating the clinical translation of scientific discoveries, and increase its economic base through manufacturing and job creation.

Appendix: About the Wake Forest Institute for Regenerative Medicine

The Wake Forest Institute for Regenerative Medicine (www.wfirm.org) is an international leader in translating scientific discovery into clinical therapies. Its physicians and scientists were the first in the world to engineer laboratory-grown organs that were successfully implanted into humans. Today, this team is working to engineer replacement tissues and organs and develop healing cell therapies for more than 30 different areas of the body.

Approximately 300 scientists collaborate on regenerative medicine research at the institute, which is part of Wake Forest University and is located in Piedmont Triad Research Park. When complete, the park will be the largest urban research park in the nation. As a premier tenant in the park, the institute is seen as an integral factor in drawing private sector business to the region.

A large number of scientific presentations and publications from the institute's work were highlighted over the last year in media outlets around the world, including 60 Minutes, the CBS Evening News, ABC World News, CNN International, *Smithsonian* magazine, *Newsweek*, and National Geographic.

Achievements of Institute Scientists Include:

- First to demonstrate that complex layered tissue structures can be engineered using cells. (1994)
- First in the world to use biomaterials alone, without the addition of cells, in patients for the regeneration of tissues. (1996)
- First team in the world to create a laboratory-grown organ -- engineered bladder tissue that was successfully implanted in patients. (1998)
- First team in the world to engineer functional blood vessels that were implanted pre-clinically and survived long term. (2001)
- First team in the world to create functional solid organ constructs experimentally, a miniature kidney that secretes urine. (2003)
- Identified and characterized a new class of non-controversial stem cells derived from amniotic fluid and placenta, which show promise for the treatment of many diseases. These amnion stem cells have been proven to differentiate into many tissue types, including blood vessel, bone, liver and muscle. (2007)
- Selected to co-lead the Armed Forces Institute of Regenerative Medicine, an \$85 million, federally funded project to apply the science of regenerative medicine to battlefield injuries. (2008)
- First team in the world to engineer solid organ constructs (miniature human liver tissue and erectile tissue) using a strategy of recycling donor organs, with potential applications to other organs, including the kidney and pancreas. (2010)