

The Advanced Technology Program

Federal Funding for Technological Revolutions: Biotechnology and Healthcare Highlights



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The Advanced Technology Program (ATP) is unique among federal funding programs in that it provides support for U.S. companies engaged in the development of highly innovative and technically challenging platform technologies that would otherwise not be developed, or not be developed in a timely manner. ATP partners with industry to provide funding for early stage technologies that are viewed to be too technically risky or too early for private sector funding sources but nonetheless have the potential to enable broad-based national economic benefits.

In 44 competitions that have been held between 1990 and 2004, 768 projects have been selected for cost-shared funding from over 6,000 proposals. Approximately 30% percent of these awards (224 projects) involve direct developments in biotechnology and/or healthcare related technologies. These awards are consistent with ATP's statute that establishes ATP's role in providing cost sharing support "for the purpose of assisting United States businesses in creating and applying the generic technology and research results necessary to commercialize significant new scientific discoveries and technologies rapidly."

This report highlights several ground breaking innovations in biotechnology and healthcare that ATP awardees are now seeking to commercialize.

ATP Funded Novel Technology Leading to a Potential New Treatment for HIV

Sangamo Biosciences of Richmond, CA is expecting to enter clinical trials for revolutionary AIDS treatment using gene modifying technology.

The ability to regulate gene expression has tremendous therapeutic potential. Sangamo Biosciences has developed engineered Zinc Finger Protein Transcription Factors for Therapeutic Gene Regulation. This therapeutic approach is designed to activate or repress specific genes relevant to human disease including cardiovascular diseases, cancer, and HIV/AIDS.¹

Currently, over 500,000 U.S. citizens have died from AIDS and an additional 400,000 more have AIDS.² More than 225,000 have now been diagnosed as HIV positive.³ Sangamo President Edward Lanphier noted, “without ATP funding, Sangamo Biosciences would not exist, nor would our exciting zinc finger technology have the potential to treat AIDS or HIV.” He further added, “It’s an enormous step forward for HIV therapeutics and circumvents or leapfrogs all the conventional approaches.”⁴

ATP Funded Technology to Provide Treatment for Congestive Heart Failure

Osiris Therapeutics of Baltimore, MD is attempting to regenerate damaged heart muscles by being the first company to take adult stem cell technology into the clinic.

Osiris Therapeutics, Inc. has been working for over a decade to develop and commercialize cellular therapies based on stem cells isolated from readily available adult bone marrow. These stem cells offer the opportunity to provide revolutionary treatments for many disease conditions.

An estimated 4.8 million Americans have congestive heart failure (CHF). Half of the patients diagnosed with CHF will be dead within 5 years.⁵ Coronary heart disease alone is the single largest killer of Americans. There were 494,382 coronary heart disease deaths in 2002.⁶

Our commitment in this new frontier of stem cell therapy is first and foremost patient safety, said C. Randal Mills, Ph.D., President and CEO of Osiris Therapeutics. "This is significant for all of our clinical programs since our stem cells products represent a true platform technology."⁷

¹ http://www.sangamo.com/human/human_thera_overview.html#zfp

² <http://www.cdc.gov/hiv/topics/surveillance/basic.htm>

³ <http://www.statehealthfacts.org/cgi-bin/healthfacts.cgi>

⁴ <http://www.forbes.com/markets/2005/12/17/sangamo-hiv-fda-1217markets01.html>

⁵ <http://library.thinkquest.org/27533/facts.html>

⁶ <http://www.americanheart.org/presenter.jhtml?identifier=3027696>

⁷ <http://www.osiristx.com/news2.php?id=31>

ATP Funded New Technology to Treat Cancer

Receptor Logic of Amarillo, TX is developing a new platform which will lead to more sensitive and accurate tools for diagnosing and treating breast cancer.⁸

Receptor Logic is developing a new class of antibodies that mimic T cell receptors (TCR mimics) and can identify and attack tumor-specific human leukocyte antigen complexes (HLA). HLA are present on virtually all cells and display cancer targets on tumor cell surfaces.

Breast cancer is the most common form of cancer in women and the second leading cause of cancer death for women in the United States. Approximately 186,000 women will be diagnosed with breast cancer this year. Tragically, every 13 minutes a woman dies of breast cancer. It costs \$10,000 - \$15,000 to treat breast cancer when detected early compared to \$60,000 - \$145,000 when it is detected in more advanced stages. Early detection also saves lives. The survival rate for localized breast cancers is 96%, as compared to 21% when the cancer has spread to other areas and organs.⁹ This ATP funded technology from Receptor Logic has the potential to facilitate earlier breast cancer detection and save lives.

ATP Funded Technology Creates “Golden Bullet” for Cancer Treatment

CytImmune Sciences, Inc., of Rockville, MD, is expecting to enter clinical trials soon for cancer treatment.

The technology funded under the ATP award minimizes drug development risks by targeting smaller doses of drugs that ordinarily can not be used at higher doses for reasons such as high toxicity to the patient. To overcome this problem CytImmune Sciences, Inc. attaches drugs to gold nanoparticles and targets the whole complex to tumors. This type of treatment may cause much less harm to the patient because the drug is delivered directly to the tumor, thereby destroying it. When this technology is proven effective, it may be in the front lines of cancer treatment, making surgery, radiation treatment and chemotherapy with morbidity-causing drugs less necessary.

⁸ http://www.receptorlogic.com/news/pr/pr_20040930.html

⁹ <http://www.advamed.org/VOT/savinglives/breastcancer.shtml>

ATP Funded Technology to “Turn On” Gene Therapy

ATP funded RheoGene, Inc. of Norristown PA has developed the RheoSwitch[®] Therapeutic System (RTS) for cell and gene therapy. RTS delivers therapeutic gene products in the patient to the right place, at the right time, and at the right dose.

The power and potential of gene therapy is enormous and *RheoGene* is dedicated to unlocking the power of cell and gene therapy to provide cures for major unmet medical needs.¹⁰

There are no approved gene therapy products on the market today, in part because of the unmet need for efficacy and safety that can only be realized through the use of a targeted, regulated gene expression system. **RTS** has the potential to be safe and effective for a wide range of clinical indications where the controlled delivery of a therapeutic gene product represents the best opportunity for effective treatment or cure.¹¹

ATP Funded Technology Promises to Significantly Reduce Immunological Effects of Protein Therapeutics

Xencor, Inc. of Monrovia, CA., is creating superior proteins therapeutics through the use of pioneering protein engineering technology and turning natural proteins into powerful therapeutic entities.

Immunogenicity can both reduce the efficacy of a therapeutic and cause severe immune reactions. ImmunoPDA[™] technology is the only comprehensive approach to addressing the immunogenicity problem. ImmunoPDA[™] technology is a fully integrated protein design and immunogenicity reduction tool for the creation of safer biotherapeutics. With ATP funding, Xencor, is redesigning and re-engineering therapeutic proteins to be less immunogenic by using innovative computational methods to identify and replace antigenic protein regions (epitopes) for improved safety and efficacy.¹² This platform may minimize or eliminate the over-reaction of the human immune system to protein-based therapeutics. The market for these protein based products is now greater than \$17 billion.

¹⁰ <http://www.rheogene.com/>

¹¹ http://www.rheogene.com/html/about_rheogene.html

¹² <http://www.xencor.com/rad/deimmunization/index.html>

ATP Funded Technology Enables the Next Generation of Biotherapeutics with Superior Pharmaceutical Attributes using a Proprietary Glycan Optimization Technology

*Glycofi Inc. of Lebanon, NH has developed a proprietary protein expression technology that allows for the optimization of the exact sugar structure of a given therapeutic protein. This development can dramatically improve the pharmaceutical attributes of a drug and enhance its therapeutic profile.*¹³

Glycofi is validating the use of this platform technology to fast track monoclonal antibody production. By controlling the sugar structures on antibodies they have shown that the antibodies ability to kill cancer cells can be significantly improved and that therapeutic proteins can be optimized by controlling their sugar structures.¹⁴

This platform demonstrates that antibodies with human sugar structures (glycosylation) can be produced in glyco-engineered yeast cell lines. It also demonstrates that by controlling the sugar structures of antibodies, their therapeutic potency can be significantly improved.¹⁵

Collaborators, including top pharmaceuticals such as Merck, Lilly, and Medimmune, are validating the technology to determine if the platform can be used to create novel drug candidates with superior performance.¹⁶

¹³ <http://www.glycofi.com/glycoproteins.htm>

¹⁴ <http://www.glycofi.com/012306.htm>

¹⁵ <http://www.glycofi.com/012306.htm>

¹⁶ http://www.glycofi.com/biotech_partnerships.htm