

Biotechnology Developments and Security

Presentation Summary

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Dr. Dombkowski provides a brief description and analysis of current developments in the area of biotechnology. In the first part of her presentation, she identifies the top three transformational biotechnology breakthroughs that have security and global health applications. She then proceeds to discuss public-private partnerships as an effective model to improve the innovation environment of the biotechnology sector.

The first major biotechnology breakthrough with security applications is the continuous improvements in DNA sequencing technology. In 1990, it cost \$3 billion to fully sequence the DNA of a human being. Today, with more advanced tools, the cost is approaching \$1,000. At this \$1,000-per-sequence price point, the applications of this breakthrough are much more cost effective. Among the latest applications of DNA sequencing is personal profiling: consumer-focused applications are already using cheaper, albeit less precise, techniques that approximate a complete DNA sequence, allowing an individual to pay \$400 to obtain data about their genetic heritage and understand their predisposition to certain diseases, their ability to respond (or not) to certain drugs, and their carrier status as pertains to certain diseases.

Dr. Dombkowski expects that with fast, sub-\$1,000 DNA sequencing technology, we will be able to increasingly understand the molecular basis of disease that in turn will streamline drug development and prescription. In addition, fast, cheap human DNA sequencing will also enable fast, cheap sequencing of pathogens of infectious agents and determine whether they have drug resistant genes. She points out that the security and global health implications of this capability will be profound. For instance, data that advanced technologies provide such as DNA sequencing can be combined with information management tools that can provide increasingly elegant, accurate, timely information about outbreaks and pandemics that might occur in any part of the world.

The next technological frontier covers breakthroughs in “cellular reprogramming” or “manipulating cell fate,” specifically, the invention of techniques to create the so-called “induced Pluripotent Stem Cells” (iPS cells). Of particular military interest in this breakthrough is the possibility that an iPS-cell enabled drug discovery can be applied to prevent/cure devastating neuro-degenerative diseases such as amyotrophic lateral sclerosis (ALS) diagnosed among Gulf War veterans. In the longer term, Dr. Dombkowski states that iPS technologies are expected to lead to ‘true’ regenerative medicine.

The third and final breakthrough is the breadth of progress being made by the private sector on multiple fronts in vaccines, to include: DNA vaccines, vaccine preparations that do not require refrigeration, and micro-needles that enable vaccine delivery. Of particular security and global health significance is the current progress with new manufacturing techniques that could allow for the rapid development of a vaccine against a pathogen of interest at the kind of scale that is needed to address large populations during, say, a pandemic situation.

It is clear that emerging major scientific breakthroughs have security and global health implications. In this context, Dr. Dombkowski points out that there is greater need to sustain biotech innovation, but the financial costs and resource requirements from discovery to development are increasingly prohibitive. It takes at least \$1 billion and 10-15 years to advance a drug successfully from ‘discovery to pharmacy,’ and only a very small percentage of drugs make it all the way. A major way out of this financial challenge is to increase public-private partnerships (PPP) to sustain biotech innovation. She brings up two case studies to illustrate her point. The first is Parmathene, a publicly-traded company that is a so-called “pure play” enterprise in national defense in that its programs are all bioweapon countermeasures e.g., vaccines and prophylactic treatments for Anthrax and nerve agents. The development of these programs is jointly funded by private sources of capital (venture capital, public market, etc.) and by government capital (the U.S. Department of Defense). The second case, Cleveland Bio Labs, is also a publicly-traded company and is similarly funded by a combination of public and private sources. But unlike Parmathene, it combines the development of programs with military applications alongside the development of traditional therapeutic medicines. So while its Protectans programs are being developed to counteract the detrimental effects of radiation therapy in patients undergoing cancer treatment, they are also developing applications in settings where populations might be exposed to radiation from, say, a nuclear weapon or a nuclear disaster. Dr. Dombowski concludes by saying that PPPs should be more fully utilized as institutional models to deliver the medicines of tomorrow. These types of cooperative or collaborative arrangements are a win-win for the stakeholders involved.