

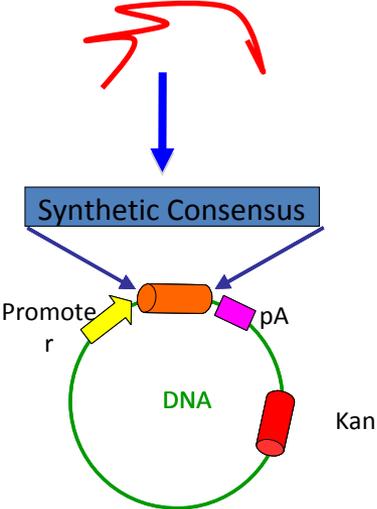


**Presidential Commission
for the
Study of Bioethical Issues
2nd meeting
September 13-14 2010**

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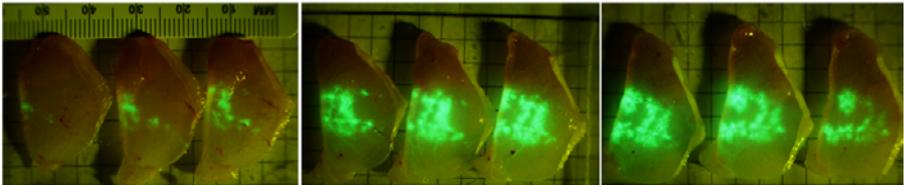
Personal Background in DNA Vaccines (Synthetic Vaccines)

Electroporation Delivery Devices

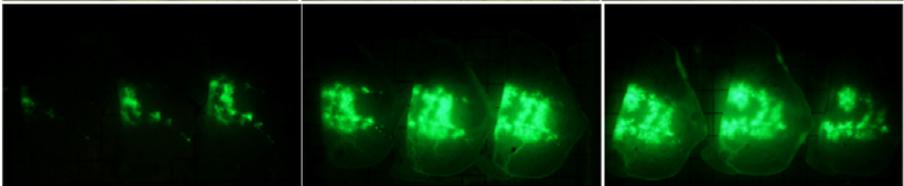


Computer Designed
Synthetic Biology
Produced Vaccine
antigens.
Delivered as DNA
Collections

Muscle+GFP



GFP



Points for Discussion

- System in Place is a highly successful engine for development of new drugs, diagnostics, agricultural and food safety products, jobs and value for the American Public.
- The Human genome was sequenced a decade ago. Human gene sequences encoding mRNA (proteins) were similarly identified almost a decade ago. New human gene sequences are unlikely to be identified with any frequency. Specific disease related gene sequences will be identified only after years or decades of new work.
- New gene sequence patenting is based on specifying an application/method. New invention and awards appear to be limited in scope.
- Synthetic Biology is not new. My lab has used synthetically produced research materials for more than a decade. Synthetic Biology is an extension of research on the frontiers of Molecular Biology/biotechnology such as engineered bacteria, viruses, and animals. Nature is still superior.
- Current drug development costs \$1 Billion USD or more and takes 10-15 or more years to develop. Safe harbor provisions protect developers during the active patent period. Patent effect is limited for new therapeutic development
- If a company cannot be allowed to have patent protection this will not serve the public as drug development will not be supported by industry, products, jobs and all associated benefits will decrease.
- Industry is currently under a great deal of financial pressure (Biotech and Pharma) – it is a time to consider new more industry supportive measures and further enhance incentives.

Synthetic Biology –Unique?



- Synthetic Biology allows rapid construction of nucleic acid sequences in the laboratory that can be used to transform cells and create cell and new organismal phenotypes (Recent C. Venter Work) increases the size of delivered synthetic gene sequences.
- Synthetic Biology is a continuation of the evolution of molecular biology and is part of the field of Mol Biol/Biotechnology – Bacterial transformation, Recombinant viruses, bacteria (vaccines), yeast recombinants...which have been generating new phenotypes for decades. Synthetic gene sequences are in routine use and have been for quite some time.
- Facilitates progress in basic science, Drug development, vaccines, diagnostic advancement, agricultural improvement, improved food science and safety, and biosecurity.
- Nature is still superior - A single cross breeding experiment in cows or dogs can cross thousands of genes and hundreds of traits that breeders can select out.
- Oversight is important and in place in the US for human, animal and laboratory studies.
- We should continue to watch this important area for developments that present unique issues.

Patent & BioTechnology Broad Strokes

- Academics/non for profits have the ability to do basic research in patented areas.
- Human Genome has been sequenced and all open reading frames have been identified. New research may uncover forms that are particularly associated with disease but these may take years or decades of work.
- The average new therapeutic will take more than 10 years to develop (vaccines much longer) years **after** validation of a particular gene target – This is not changed dramatically by synthetic biology
- Companies that embark on product development are protected by Safe Harbor provisions (2005 Merck vs Integra Life Sciences). The safe harbor provisions extend beyond merely research and development of generic drug equivalents to include basic experimental research on new therapeutic drug candidates and other products that require regulatory approval. - there fore original patent positions have only a limited effect on therapeutic development.
- Major Point - Companies cannot justify the hundreds of millions of dollars in investment necessary to undertake new therapy or vaccine development with out being able to establish a patent ownership position with their management.
- Biotechnology is a world wide pursuit. We do not control patenting in other countries. Further curtailing US patents in this area will likely put US at a competitive disadvantage.

System in Place

Over the last 30 years average lifespan in the US has increased **2.2 months per year**

We live longer and more active lives – Many advances- eyesight, hearing, autoimmune, cancer treatments, ED,

Current system Created hundreds of thousands of **Jobs (direct + more indirect)**, new companies

Increased investment by private sector in therapeutic and diagnostic development by hundreds of billions of dollars. Improved safety of food, manufacturing and improved food stocks.

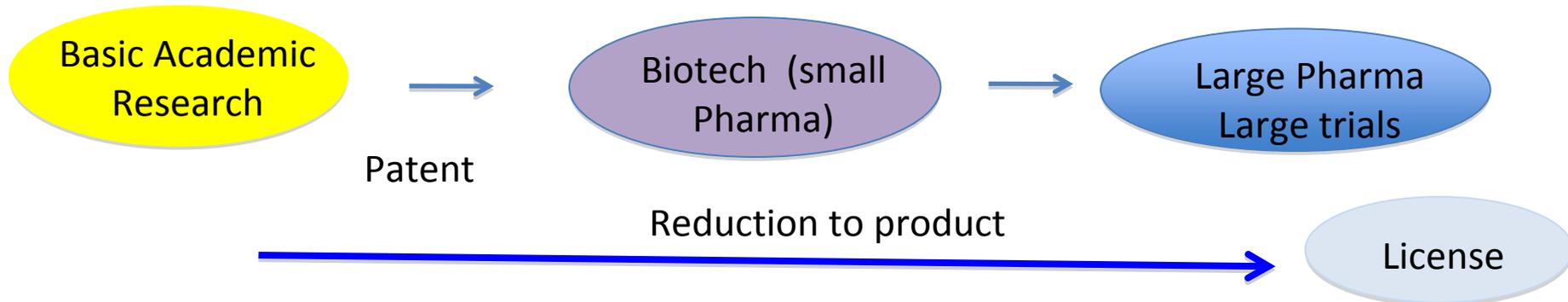
Leveraged public NIH funds 10-50x+ per new drug developed.

Examples many -- HIV was a death sentence in 1990, now in the US its more of a chronic disease, Breast cancer survival, New vaccines, Autoimmune diseases Transplantation....

Impressive Record

- **Today**, there are more than 200 biologic medicines and vaccines that benefit millions of patients worldwide. In addition, there are more than 1,200 biotech diagnostic tests being used in clinics around the world.
- More than 600 new biologic medicines are in development, including treatments for cancer, HIV/AIDS, Alzheimer's disease, and numerous rare conditions.
- It is the most active area in the pharmaceutical industry.

Current Economic Conditions Are Problematic



- Biotechnology industry is under tremendous economic pressure – Raising capital is difficult
- Large Pharma situation is difficult – Consolidation from 17 to just 11 major companies over the last few years – loss of jobs, research outlets, new drug programs.
- US large Vaccine manufacturers situation is even more dramatic – down from 27 vaccine companies in 1980's to just 2 today.
- Important to further incentivize and enhance US competitiveness in this area.

Final Thoughts

- System in place is an example of a highly effective government created incentive program that engages the private sector to leverage public investment, which has resulted in the creation of new therapeutics, diagnostics, agricultural & biosafety products which have positively influenced the quality and quantity of Americans lives. It provided and continues to provide significant job creation & drives billions in private investment into an area of value for US citizens.
- Current systems including patent protection, limited scope of patent allowance, Safe harbor provisions, long drug development time lines provide incentives and stability and allow for continued new therapeutic development and important for US Economy.
- Elimination of patent protection in this area has a high probability to eliminate much industry incentive, lowering US competitiveness and lowering private sector funding thus hurting the American public.
- Due to significant economic pressure in the biotechnology and pharmaceutical industry, additional incentives for therapeutic development and job creation should be an important focus going forward.