Bioeconomy White Paper

Georgia Institute of Technology
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Introduction

Georgia Tech is recognized as one of the top research universities in the world as evidenced by its recent top ten listing by Thomas Reuters.[i] Its six exceptional colleges include the largest engineering college in the United States. Research is conducted in each college and Tech's applied research arm, the Georgia Tech Research Institute. Economic development activities reside in the Georgia Tech Enterprise Innovation Institute including one of the top ten incubators in the country and state-wide outreach effort supported by the State of Georgia and federal programs such as the NIST Manufacturing Extension Partnership (MEP).

Georgia Tech recently defined an industry facing research strategy focused both on leading-edge research and economic development. Most universities pursue a linear, sequential flow of discovery-based research to occasional declaration of intellectual property followed by licensing or company formation/spin-out. In contrast, Georgia Tech pursues a concurrent strategy focused on strategic theme areas spanning biotechnology, energy, nanotechnology, innovative materials, future media, and policy. By concurrence, Tech defines and pursues grand challenge problems and provides thought leadership to the overall research community; seeks to create and sustain collaborative partnerships across the research community; and accelerates the maturation and transition of research outcomes into societal use. A few recent examples include the creation of a national roadmap for robotics announced by President Obama in June 2011, a partnership with GE Energy to focus educational programs in engineering and business on innovative energy solutions (resulting in 21 patent applications in two years), and a streamlined licensing program.

In the area of biotechnology, Georgia Tech leverages its partnership with Emory University and the Morehouse School of Medicine as well as an embedded biotech incubator and a bio-device pilot plant, to concurrently conduct discovery-based and translational research with clinicians, trials, and commercialization. The research-to-commercialization process is as innovative as the research itself. It is in the context of the collective experience of over 7,500 researchers and leadership at the national level that Georgia Tech submits this white paper.

Grand Challenges

Simply stated, we must be bolder. The most useful outcome OSTP can take in response to the Bioeconomy RFI call is to facilitate a return to boldness, an attribute that used to distinguish all aspects of *the American way*. Federally sponsored research programs and accompanying transition efforts sponsored by government and the private sector have become too incremental and risk adverse. What is needed are bold, even audacious, statements of grand challenges to ignite passion and excitement and to push researchers and technologists to do more than they think possible. Too often, current federal programs, notably the National Institutes of Health (NIH), require proof of concept with supporting data prior to proposal submission. This has prompted the joke in the research community that the research needs to be done prior to the application for research support. But "the joke" has produced a *laissez-faire culture* that is symptomatic of life in America today. Our country's willingness to boldly state the equivalent of the "JFK moon shot" or the quest in the 1950s to eradicate the world of polio and TB is sorely missing today.

Grand challenges related to the bioeconomy can take two forms. First, bold statements of research outcomes that transform patient care and health (e.g., a pediatric cardiac value implant that "grows" with the child; patient specific, custom cancer therapies based on genome mapping). Second, bold statements of process improvement that will incentivize and accelerate the transition of research results to use (e.g., process redesign of clinical trials and related policies to yield a 5x reduction in time for approval).

Research and Development

While R&D investments in platform technologies can support advances in health, energy, agriculture and the environment, that alone is insufficient to generate the impact required to meet real world needs. NIH, NSF and other key federal agencies together with certain foundations and non-profits do a good job in supporting the development of basic scientific advances. However, as a country, we do not do enough to help translate those basic science discoveries further down the path toward identifiable product opportunities and hence toward meeting real world needs.

Greater funding emphasis is needed in the area of translational research to move discoveries from the lab bench closer toward a product which serves the patient. Translational activities can be as scholarly as basic fundamental research; the unifying feature is the impact of the discovery on the patient and society. Indeed, the more advanced the discovery, the more creativity is needed in developing innovative animal models applicable to human disease in which to test the safety and efficacy of the new device or drug.

Besides federal support for translational research, dissemination of best practices for translations is also needed. For example, the processes advocated by the Coulter Foundation should be adopted throughout the community.

To be truly impactful, translational activities need to occur on three levels: 1) translational research to apply basic scientific knowledge to real world needs as targeted product opportunities, 2) translational teaching to develop educational methods applied to real world needs for both students and faculty, and 3) translational services – engaging in activities that are responsive to real world needs. This has a direct impact and influence on what skills are needed in the workforce.

Translational research by definition means fast, seamless transfer of discoveries from bench to bedside of high impact problems, i.e., problems which truly address unmet medical needs regardless of the commercial potential of such discoveries. High impact problems are beneficially disruptive technology discoveries. In essence such disruptive technologies are often "platform technologies", underpinned by seminal paper(s), strong international patent positions (i.e., own an area based on strategic considerations of the institution), and have the concept proven in multiple, diverse applicable animal models.

In biomedical research, strong emphasis must be placed on translation of the discovery concept into multiple proof of concept studies in various, robust, reproducible animal model systems which are closely representative of human disease. These animal models must also be acceptable to industry and private investors as a solid proof of concept, actually mirroring the intended use. This may require the development of novel and larger animal models and comparative studies. Comparative studies should directly compare the use of current therapies and devices to the new discovery concept.

Further, we should place emphasis on more early product-oriented development work (e.g., advanced working prototypes actually tested in representative animal systems, therapeutics that are formulated and basically characterized as to pharmacokinetic, pharmacodynamics and pharmacological properties, and consideration of other common product developmental hurdles such as sterilization, clinical trial supplies,

safety and tolerability, as applicable). This level of translational research will help assure the project is attractive to a potential industrial partner or to a venture investor.

Translational teaching aims to educate well-rounded future innovators. Students too often may not pursue careers as pure academics and are increasingly seeking practical training for their careers. To meet these needs, academic program faculty, together with other experts in the institution, should provide integrated training in areas such as market research (lecture and lab), MBA essential skills (management, communication, and finance), intellectual property and legal essentials, drug and device product development and regulatory approval processes, and commercialization. The newly proposed Biomedical Engineering Master of Science Degree Program Proposal for Biomedical Innovation and Development (BiolD) at Georgia Tech is aimed at addressing some of these needs. Programs such as Georgia Tech's Technological Innovation: Generating Economic Results (TI:GER), which serve to integrate technology and business approaches under the guidance of an industry-experienced mentor, could be expanded.

As important as our students are, it is equally important to have programs for mentoring faculty in product development processes, regulatory, commercialization and to recognize translational research as a scholarly activity.

Translational service refers to the engagement of faculty and students in activities responsive to real world needs, specifically, to apply their innovative discoveries to address unmet medical needs and societal needs.

Moving life science breakthroughs from lab to market

Product development skills are often limited in academic institutions and need to be acquired and rewarded. Academic institutions can learn from industry: different skills are required to create a new scientific discovery and to then translate and develop that discovery into a product. Hence, the nature and type of people and skill sets required to effectively carry out translational research activities are often different. This is particularly salient in the context of how academic institutions evaluate faculty members.

Translational research is a discipline within its own right and it is the scientific quality by which it should be judged. Similarly the translational scientist, to do it right, must be able to bridge/design the preclinical work with a forward look toward the clinical, regulatory, and commercial paradigms. All of this translates into opportunities for publication in peer reviewed journals, grant funding, and industry funding. Translation, to be successful, requires other factors such as IP protection, understanding of product development, industrial involvement, and licensing. These could serve as other factors meaningfully considered and appropriately weighted in faculty promotional assessment.

Funding for translational research/ early-stage product development is limited and should be enhanced. The federal government could undertake more extensive funding as part of a grander Translational Research Initiative. The funding could be differentially applied. For products with reasonable commercial potential, federal funding could extend through the following phases: development and robust animal testing of operational prototypes. The prototypes would then be licensed to industry to finish product development and gain regulatory approval for marketing. For products with little commercial potential but high unmet medical need, the governmental funding would be extended through to the development of fully operational devices, not just prototypes, for use under humanitarian device or drug exemptions in a single institution. Such products could then be made available to other institutions on a needs basis. The "finished" product might eventually be attractive to industry as significant product development risk would have been eliminated. In either of these applications, it is important to involve experts with industry R&D, product development, and business experience to guide development of new devices and drugs.

Recommendations: Imbed distinct translational research and product development experts within academic departments, as applicable. Coordinate, through collaborations, basic academic research and translational group endeavors recognizing the differences between discovery and development in order to most expeditiously advance projects from discovery through early product development.

a. Develop a strategic approach to which areas of scientific research and discovery an institution is an expert in and align translational activities with the institution's discovery strategy.
b. Consider adding a new group of academic researchers who are experts in the various downstream activities associated with translating discoveries into products – i.e., small through large animal research with an emphasis on model development and characterization of its fit to human diseases and subsequent characterization of new product concepts in these models; medical device design, prototype, scale up and associated activities; pharmaceutical development and pK and pD characterization for potential therapeutics. Ideally, these researchers should have strong industry experience in product development.
c. Establish formal educational programs for faculty, staff, and students in product development of medical devices and therapeutics. This would include a basic understanding of the development processes and supporting activities including manufacturing, regulatory, quality, and safety both domestically and internationally. Such activities are unique to the medical industries.
d. Utilize existing relationships with clinical colleagues at clinical research institutions and in the medical community to add continued strong clinical input to project development and assessment.

Academic institutions tend to loosely coordinate discovery and development activities. The process of translating discovery breakthroughs into product realities is extremely complex. Industry has effectively dealt with these complexities by establishing project teams which bring to bear the required functional expertise in a unified project team; academic institutions could apply and benefit from these principles.

Recommendations:

a. Coordinate translational research and discovery endeavors. Establish a function focused on establishing the coordination of activities from discovery through early development and which brings product development and commercial understanding in advancing strategically important projects. The skills to drive this require industry trained experts who understand the discovery, development, and commercialization processes for both devices and therapeutics and can bring these understandings to bear in an academic environment. The ideal individuals should also have the scientific credentials to engender respect within the academic environment. This coordinated effort should function as a project team to assure a smooth transition of discoveries into products and help speed project throughput.
b. Establish shared objectives for translational research programs. Align all functions supporting technology development and its subsequent commercialization with a common set of shared objectives and goals. By supporting functions this could include licensing/tech transfer, patenting, industrial sponsor/support programs, or bioscience commercialization support functions. An institution can therefore present a common, unified front to commercial partners.
c. Manage conflict of interest considerations**.** Recognize that an institution's strategic emphasis on commercialization and faculty (and supporting staff) involvement in commercialization may present challenges to emerging standards of conflict of interest. Consider establishing a working group aimed at melding emerging expectations regarding conflict of interest with the strategic goals of an institution toward commercialization and does so in a fluid and flexible manner. This may require a cultural shift toward accepting greater, though still maintaining prudent, risk-taking.

d. Foster greater industry involvement. Consider establishing a formal program/process to encourage the establishment of embedded biomedical industry laboratories on campus and which are responsive to both industry needs and academic needs. This would include, amongst other items, office and lab space, protection and sharing of IP, collaborative involvement of industry scientists with corresponding faculty and/or students on campus, streamlined business and technology licensing practices.
e. Establish mechanisms to access 3rd party translational funding from government, industry, foundations, alumni, interested community business people and private investors.
f. Leverage existing programs supported by the federal government and the states. For example, the NSF Innovation Corps model is an excellent initiative designed to accelerate the commercialization of NSF-funded research. Furthermore, efforts such as the Manufacturing Extensions Partnership could be leveraged to support more rapid technology transfer and adoption.

Workforce Development

As previously noted, students are increasingly seeking practical training for their careers and, as such, students provide the marketing "pull" to adapt educational programs. It is equally important to have programs for mentoring of interested faculty in bioscience product development processes, regulatory, commercialization and the evolving health care environment.

Recommendations:

a. **Establish formal faculty/student educational programs in the following areas:**
    1. **Product development run by industry experts and tailored to faculty/project stages and interests**. This should include industry consideration of what constitutes sufficient proof of concept, regulatory, quality, manufacturing and clinical activities. The expertise to organize and manage such a program exists within an institution; resources could be reallocated and/or supplemented with external experts
    2. **Patenting and management of the process from discovery through patent issuance**. The considerations for bioscience projects tend to be rather distinct from other areas.
    3. **Business development related to commercialization of inventions including aspects of project commercial evaluation, competitive assessment, and company formation/management, funding, and partnering strategies.** Much of this might be done in conjunction with the College of Management and existing ATDC commercialization catalysts and expansion of TI:GER to include faculty.
b. Establish a commercial mentorship program for each faculty project.

Reducing regulatory barriers to the bioeconomy

Patent Budgets: A significant constraint for many public and private institutions is reflected in their limited budgets to globally protect intellectual property funded by federal research dollars. This constraint forces institutions to make a choice: not file for patent protection or limit the regions of the world in which they file. Given the early stage nature of most discoveries, so initial research is conducted without the benefit of knowing the commercial scope or potential of these discoveries. Most biomedical discoveries will require an industrial partner who can provide the enormous investments in product development required to gain approval to be marketed. As most drug and device companies now operate globally, when academic institutions do not file for patent protection or do so only in the US, the likelihood of a commercial partner being interested in licensing that discovery is significantly diminished. An industrial

partner will not invest, even when there is large commercial potential, if there is no patent protection to protect their investment in product development and help assure that they can recover their investment. Further, if there are two competing technologies, industry is likely to choose the technology which has global IP protection over a technology, even if better, which only has IP protection in a more limited geography.

The technology development model needs to shift from cost-recovery toward investment. Most research institutions tend to view their investments in technology transfer, licensing, and intellectual property protection from a cost recovery rather than an investment perspective. A new federal funding vehicle could be established to provide resources to U.S. academic institutions to help jump start efforts to globally patent appropriate discoveries and to focus on the translation of discoveries into products. To truly jump start translational research efforts in institutions, specifically those without existing medical schools, requires investment in research facilities to include labs, offices, vivariums, equipment; acquisition of new product development-oriented faculty; and establishment of translational research training programs. Funds to jump start such endeavors could be handled as center or block grants to integrate translational research and technology development into the mainstream of our research institutions which do not currently have medical schools. Since translational activity to realize medical device and/or drug prototypes takes time, the grants should have a funding life of at least five to eight years. To encourage investment in IP protection and in more effective technology development and licensing, those institutions which realize success from enhanced licensing endeavors could receive incentive matching funding from the Federal government to further expand their efforts. Success could be defined as: 1) licensing the technology to a commercial partner for further development, 2) realizing an FDA approved device under a humanitarian exemption for patient use within affiliated institutions, or 3) establishing an embedded industrial laboratory within an academic institution where the laboratory creates a collaborative working relationship between the academic discovery oriented investigators and the product development oriented industrial scientists.

Unreasonable Conflict of Interest Management. More reasonable and pragmatic standards in conflict of interest (CoI) management need to be established. These standards would provide ‘safe harbors’ for academic investigators and affiliated support personnel working with industry that encourage, rather than discourage, interactions and collaborations. In addition, they would, foster establishment of product licenses and start-up enterprises and allow the effective use of unique academic facilities and equipment to benefit the new enterprises. Such standards should reflect the low probability of realizing a product and should be based on realities. Appropriate quality assurance oversight could be established to audit and assure data integrity in those instances where there are concerns. Federal agencies struggle with these very issues. The heads of DHHS/FDA have indicated intent to modify their CoI policies as current policies have limited their ability to fill vacancies on key advisory panels.

Public-private partnerships

Embedded industry-academic laboratories have proven to be successful when appropriately managed.

Tax-incentives could be provided to industry for sponsored research, embedded laboratories and collaborations with academic institutions. Existing tax incentives, generally limited to unrestricted donations, could be expanded to include collaborative targeted research and product development initiatives.

Partnerships with industry in generalized funding of product and technology development can benefit from a combination of tax incentives and sharing of future economic returns. The funding could come from private industry, and private investors, other accredited investors and even the public at large. This

funding could function loosely as an internal venture fund focused within an academic institution on furthering the economic development of its technology. The Georgia Research Alliance, in part, serves as an example, but its reliance on State funding requires supplementation to be more effective.

Conclusion

In order for the U.S. to truly harness biological research innovations in an effort to meet grand challenges, we must make bold, game-changing steps. By focusing on translational research and processes, mitigating barriers, and providing robust programs for faculty and students, we can move beyond incremental changes and, instead, create transformational impact in the fields of patient care, health, and the transition of research results to use.

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[i] http://www.timeshighereducation.co.uk/world-university-rankings/2010-2011/engineering-and-IT.html