Learning for Life October 20, 2021

Shirley opened the class by discussing the importance of translational science. Translational science is defined as the process of turning observations in the laboratory, clinic, and community into interventions that improve the health of individuals and the public — from diagnostics and therapeutics to medical procedures and behavioral changes.. An example in education might be the impact on early intervention education on childhood outcomes.

Dean Cornelli presented an overview about the number of inventions and research funding at Northwestern. We are living in a time of great innovation and patent filings are higher than ever. Northwestern is the highest funded university in IL and receives $2.2B in revenue from patents and IP licensing. However, the pathway to getting a drug or therapy approved to patient care takes years and costs millions of dollars. The impressive research funding we currently have is simply not enough to address all the patient facing issues (cancer, neurodegenerative diseases, heart disease, infections etc) that we face as a society.

Dean Cornelli outlined the areas we discussed today and introduced the distinguished speakers: Drs. James Conley and Lisa Dhar

Today we discussed:

a) Typical paths and challenges to translational science
b) Strategies to accelerate innovation
c) Select technology areas to watch

Dr. Conley first discussed the challenges we face. Although the NIH funds many impressive advances, only 14% of NIH funding leads to therapies for patients. One example of a breakthrough is Lyrica. Dr. Conley stated Lyrica is "literally a wonder drug that was developed on Northwestern's campus by Dr. Richard Silverman." He had an NIH grant of $600,000 and generated a molecule called pregabalin in his laboratory in 1987. He thought that his molecule might be able to cross the blood brain barrier and reduce seizures. However, it has been a breakthrough drug for fibromyalgia and other painful conditions. Over 9 million people have been prescribed pregabalin in the US. This has contributed to a ripple effect at Northwestern with royalties supporting research and facilities through the NU endowment. Dr. Conley showed the timeline of Professor Silverman's work. He had the grant in 1987 and a patent was approved in 1990. After multiple preclinical and clinical studies, it was launched by Pfizer as Lyrica in Sept 2005. Obtaining a patent is a very long and complicated process. Northwestern partners with many companies to work on the steps in the process because most of the compounds studied don't make it to market. To make it to actual patient care you first have to have a patent, a corporate partner, preclinical trials and then clinical trials. Then the FDA has to approve its use based on the results of the clinical trials. All of these steps had to occur before Dr. Silverman's research in 1987 could be translated into a drug approved for patient care. The process is complicated but this has a purpose –ensure the safety of new drugs that are used on patients.

Dr. Dhar discussed some of the strategies used to support translational science. One is a partnership between the University and a company who licenses a novel compound. Another option is a start- up company. Sometimes this is a good approach because the technologies developed in the research labs are not a single invention. They are a platform for multiple uses so it is harder to find a company to fully utilize the full capabilities of the platform. Dr. Dhar provided several examples of platform technologies that have specific therapeutic possibilities in conditions such as celiac disease, peanut allergy, and

primary biliary cholangitis. One start-up company, Cour, is launching patient trials now. Exicure is a new company working on types of immune-oncology for example carcinoma. Aprinyx is a company looking at therapies for post-traumatic stress disorder, and neuropathy. It is very exciting to watch these compounds progress through the approval process and to think about how they might help patients and the University in the future. Additional Northwestern start-ups are involved in diagnostic testing. For example, Swiftscale biologics is a start-up designed to develop antibody treatments. During COVID antibody therapy came to the forefront and now multiple other uses are being considered. Sibel is another company using health data monitoring to impact early childhood development.

Dean Cornelli commented on strategies to accelerate innovation to market. Some options include federal programs that provide grants. Other options include accelerators and private capital. A partnership between the scientists making the discoveries and the experts at commercialization is needed in order to maximize the translational potential of research discoveries. Northwestern has developed a center to help with this and to connect scientific discoveries with the proper partners to make the successful jump to the patient care market. To best succeed at translational science, these partnerships are vital (university, government, business). The COVID vaccine required these types of partnerships to get to market so quickly. Northwestern has an active partnership with a biotech company named Deerfield partners. The company has a large fund dedicated to Northwestern scientists. This partnership not only helps the research itself but the partnership works because Deerfield Partners has expertise in drug marketing. This is an example of how creative partnerships allow research breakthroughs can translate into patient outcomes sooner.

Dr. Conley next discussed the human capital needed to perform science and make these breakthroughs for patients. We have students and investigators in medical school programs, engineering programs, and chemistry programs who are leading studies that we can “pitch” to investors and companies who not only support research breakthroughs but also hire our graduates and further impact the process within companies, government regulators, and other steps along the way. The close partnership between Northwestern scientists and the Kellogg School is also integral for this successful pathway. We are also able to recruit even better scientists to Northwestern as we develop and support these innovative partnerships and start-up companies. The “buzz” about Northwestern is that it supports this hive of activity needed to maximize translational research discoveries. This infrastructure and collaboration helps us recruit talented scientists who have an eye toward commercialization.

One observation our panelists mentioned is the development of the COVID -19 MRNA vaccine. Traditional vaccine development takes 5-15 years. However, COVID-19 vaccine development occurred in under 2 years. Multiple steps happened at once instead of in sequence. For example clinical trials overlapped with regulatory approval and manufacturing rather than each step occurring sequentially. This partnership between private and government entities led to an amazing result for patients across the world. However, the quick approval has also generated some public mistrust as was noted by an audience member. This is something that will need to be considered in the future and could be enhanced through effective communication.

Overall this session was a wonderful overview of the steps needed for scientific research to translate into patient care advances. Our panelists highlighted the partnerships between scientists, government and private funders needed to successfully navigate this path to support better health for all.

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