



OFFICE OF TECHNOLOGY LICENSING

INTELLECTUAL PROPERTY NEWSLETTER

2018 Issue

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TECHNOLOGY TRANSFER PROFESSIONALS DAY

On Tuesday, December 12, St. Jude celebrated the Association of University Technology Managers (AUTM) Technology Transfer Professionals Day by the windows in front of the Kay Kafe. This was the 37th anniversary of the Bayh-Dole Act which dramatically expanded the profession by engaging academic institutions in the technology transfer process. Guests spoke with our technology transfer professionals about disclosing inventions, the licensing process, and ways to partner, participate and earn money. The OTL also distributed exclusive stainless steel coffee mugs to inventors of recently awarded patents, and gave out small “bright idea” light bulbs filled with lemon candies to those who successfully completed a quiz about our processes.



Chad Riggs explains the long process to take Anaplastic Lymphoma Kinase (ALK) drugs from the benchtop to the clinic to Rebecca Bramlett from surgical services, while Esther Allay talks to Aman Seth from Pathology about Material Transfer Agreements.



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SERUM INSTITUTE OF INDIA PICKS UP THE BALL TO COMMERCIALIZE RESPIRATORY SYNCYTIAL VIRUS (RSV) VACCINE

In April St. Jude Children’s Research Hospital exclusively licensed a patented RSV vaccine candidate, known as SeVRSV, to the Serum Institute of India. RSV is seasonal, like the flu, and transmissible by adults who may be unaware they have it; yet RSV is the most common cause of serious lower respiratory infections in infants. Current treatment options are limited and there are no approved vaccines. Worldwide as many as 34 million children younger than 5 years old experience an acute lower respiratory infection caused by RSV each year, with 10% requiring hospitalization.

This SeVRSV vaccine is the result of a multi-year effort at St. Jude to find a way to protect newborns from RSV, which led to the production of clinical grade vaccine by our GMP facility subsidized by a former corporate sponsor. After speaking with several interested companies, the decision was made to partner with Serum Institute of India. Under our agreement, Serum Institute will undertake clinical trials of SeVRSV outside the U.S. and complete development of the vaccine, initially for use in South America, Africa and much of Asia. At the same time, St. Jude is working with other academic organizations to support initiation of a clinical trial here in the U.S.

The World Health Organization (WHO) estimated that the SeVRSV vaccine is one of dozens of candidate RSV vaccines in development worldwide. SeVRSV is designed to be administered to infants via nasal droplets and uses a mouse parainfluenza virus modified to carry an RSV gene. To the immune system, it looks similar to the human parainfluenza virus, which is the most common cause of croup in children.

According to Julia Hurwitz, St. Jude researcher and co-developer of the vaccine, the SeVRSV vaccine is expected to be available for use in the next 5-10 years at a very low cost for the developing world. Hurwitz added, “I would like to see the vaccine used globally in every newborn to prevent RSV in the future.”

About Serum Institute of India

Serum Institute of India is the world’s largest vaccine manufacturer by number of doses, selling more than 1.3 billion doses globally to treat diphtheria, tetanus, pertussis, Hib, BCG, r-Hepatitis B, polio, measles, mumps and rubella. Their vaccines are accredited by WHO and are used in the national immunization programs of about 140 countries across the globe, saving millions of lives throughout the world. An estimated 65% of the world’s children receive at least one vaccine manufactured by the company.

PATIENTS AROUND THE WORLD CONTINUE TO BENEFIT FROM ST. JUDE RESEARCH THROUGH OTL LICENSING EFFORTS (NUMBERS ARE LIKELY MUCH GREATER, THIS IS JUST WHAT WE CAN CONFIRM)

Indication	Treatment Tech.	Humans Treated
Influenza	Vaccine Production (Plasmid rescue)	Over 100 Million
ALK Positive Cancer	Drug (Xalkori, Zykaia, Alunbrig	3,000+/yr
Cancer (ALL, CLL, NHL)	CAR Cell and Gene Therapy w/41BBzeta	1,000+
Hemophilia	Gene therapy (Factor VIII, IX)	30+
X SCID	Gene Therapy	14
Diagnostics	Thiopurine Tolerance (TMPT, CD-19 MAb, ALK+)	500,000+./yr.

Licensees and others now using St. Jude patents include Bristol Myers Squibb, Juno, Novartis, Celgene, uniQure, AstraZeneca, Boehringer Ingelheim, Pfizer, Becton Dickinson, Insight Genetics, Quest Diagnostics, Prometheus Diagnostics, Pfizer, Teva, Ariad, Xcovery, Abbvie, Cancer Genetics, Cell Marque, Cell Signaling, Cytocell, Dako, HTG Molecular, ThermoFisher, Leica Biosystems, Pharmingen, Response Genetics, Santa Cruz Biotech, Ventana.

INVENTORS WORK WITH START-UP TO DEVELOP TREATMENTS FOR NEUROLOGIC AND METABOLIC DISEASES

Back row left to right:

Mi-Kyung Yun,
Anne Edwards,
Rajendra Tangallapally,
Chitra Subramanian,
Julie Maier

Front row left to right:

Richard Lee, Stephen White,
Suzanne Jackowski,
Charles Rock



The molecular model in the foreground represents one of the lead compound candidates that is under evaluation for the first-in-human trial. In collaboration with the team at CoA Therapeutics, 120 new molecules were designed, synthesized and tested for efficacy in cells. Synthetic scale-up and testing in mice for selected compounds was followed by pharmacokinetic analyses.

Researchers at St. Jude discovered a novel set of pantothenate kinase (PanK) modulators from compounds in the St. Jude chemical library and are working with a small company, CoA Therapeutics, Inc. (CoA-Tx), to further refine the compounds for the treatment of a neurodegenerative disease, Pantothenate Kinase-Associated Neurodegeneration (PKAN), and the metabolic diseases propionic acidemia and type II diabetes. These compounds are also useful as molecular probes for elucidating the cellular role of coenzyme A (CoA) in neurological disorders, metabolic disorders, and cancer. Two classes of compounds have emerged, activators and inhibitors, which can increase or decrease PanK activity in cells in a concentration-dependent manner by a unique mechanism of action. The activators may improve neural transmission to alleviate movement disorders caused by a CoA deficit, and improve the metabolic imbalance associated with inherited disorders of fatty acid metabolism. The inhibitors may improve abnormally high CoA levels associated with fatty liver and metabolic syndrome or type II diabetes.

BridgeBio contacted Dr. Suzanne Jackowski in 2016 while performing due diligence, seeking her expert opinion about a new potential therapeutic for PKAN. BridgeBio is a venture capital firm with a business plan for starting new companies based on a book written by Andrew W. Lo entitled Adaptive Markets: Financial Evolution at the Speed of Thought which describes risk reduction for investment in early discovery endeavors. Under confidentiality, Dr. Jackowski decided to disclose St. Jude's new potential therapeutic approach developed by the PanK project team. The team included Drs. Lalit Sharma, Richard Lee, Steve White and Chitra Subramanian, together with Dr. Chuck Rock and Jackowski, who worked for more than 2 years to identify and optimize drug-like compounds that activated PanK to substantially raise cellular CoA levels, crossed the blood-brain barrier, and were orally bioavailable.

More about the Diseases

PKAN, formerly called Hallervorden-Spatz syndrome, is an ultra-rare inherited disease caused by mutations in the PANK2 gene and associated with progressive movement dysfunction, brain iron accumulation and neurodegeneration. Molecular genetic sequencing of the PANK2 gene confirms approximately 95% of those affected have two identifiable mutations in this gene and the remaining 15% only have one identifiable mutation.

HMG-CoA lyase deficiency presents in the first year of life. The amino acid leucine cannot be processed, and ketones cannot be made; causing vomiting, diarrhea, dehydration, lethargy and weak muscle tone that leads to breathing problems, convulsions, coma and death.

Hereditary CoA sequestration diseases include over half of the recommended newborn screenings by the American College of Medical Genetics. Most of these diseases are chronic but many are associated with morbidity due to hypoglycemia, hyperammonemia, coma and fatty liver. Propionic and methylmalonic acidemia are among the most severe. Dietary constraints are imposed on patients to avoid metabolic imbalance, transplant trials and gene therapy have had limited, non-sustainable success, and trials for systemic iron-removal have recently been completed with the results being evaluated, but there remains no cure.

Ultimately, St. Jude licensed its intellectual property rights to BridgeBio, who formed CoA-Tx in March 2017, devoted solely to the clinical development of the PanK modulators. CoA-Tx experts in pre-clinical and clinical development are working in conjunction with the scientific team at St. Jude under a research agreement that is focused on optimization of the chemistry for the activator series, on evaluation of efficacy for PKAN or CoA-deficiency in the CNS, and for propionic acidemia and related genetic diseases that arise from defects in fatty acid and CoA metabolism. CoA-Tx is now assembling the infrastructure to prepare for an investigational new drug application to the FDA and human trials.

INVENTORS RECEIVE MORE THAN \$1.7MILLION FROM FY2017 LICENSE REVENUE

Licensing income is shared with inventors and creators of licensed materials, who receive between 30 to 50 percent of net income produced by their inventions according to a formula set out in the faculty handbook. Total allocations for FY 2017 exceeded \$1.7 million distributed to 115 current and former St. Jude inventors, with 9 inventions generating net income over \$50,000.

Each invention is treated as a separate financial unit so that income and expenses associated with each invention can be tracked and invention specific allocations can be made. In FY2017 financial activity was recorded for 154 inventions with 99 generating a net income for St. Jude.

All St. Jude employees have a risk-free opportunity to have their inventions or reagents considered for patenting and/or licensing by submitting invention disclosure forms to the Office of Technology Licensing. Employees can fill out and submit the simple disclosure form available at <https://home.stjude.org/technology-licensing/Pages/forms.aspx> or they can contact the Office of Technology Licensing for a Microsoft word version of the form.

Keep in mind that patience is a virtue when it comes to generating revenue from inventions. The vast majority of allocations this year were based on invention disclosures submitted more than 10 years ago. If you are interested in learning about some of the past inventions that have been developed into products and contributed to our licensing success, you can click the “Success Stories” link on our internet site <http://www.stjude.org/technology-licensing>.



Scott Elmer congratulates Dr. Brenda Schulman and Dr. Danny Scott on their shiny new inventor mugs given to them on Technology Transfer Professionals Day as a reward for receiving a granted patent application. They await their licensing revenue allocations if and when the patent is licensed and commercialized.

If your patent issued last year and you missed getting your mug, stop by our office and see us, we want you to have it!



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