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Inventing at a Rapid Pace

By Frances Dumenci

Innovation is a major theme in university's strategic plan



With the assistance and support of Virginia Commonwealth University Tech Transfer, investigators are discovering new technologies, including drug compounds, which will benefit human health and our quality of life. And they are doing so at an increasingly rapid pace.

Each year, researchers at VCU create more than 100 inventions. In 2012, a total of 132 patents were filed with 15 patents issued to researchers at VCU. Collaborations between researchers as well as the partnerships between the university and the business community, including venture capitalists, angel investors and start-up companies, have grown impressively and continue to do so.

This acceleration of translational research at VCU underscores the paradigm shift of how drugs and other technologies are discovered and the important role in which universities play within this new world.

At VCU, a strong emphasis has been placed on translational research in its strategic plan, Quest for Distinction, and its vision to be the nation's top public, urban research university.



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"Innovation is a major theme in Quest for Distinction, and our increased emphasis on research and commercialization fits ideally with the highest priorities of any vigorous urban research university," said VCU President Michael Rao, Ph.D. "A critical component of that research mission is to see that our innovative basic research results get translated into products and services that benefit society."

What is translational research?

In academic medical research, the term "translational" has become a new buzzword. The goal of translational research is to improve human health by translating scientific discoveries into practical applications as quickly and efficiently as possible.

Such discoveries typically begin at "the bench" with basic research in which scientists study disease at a molecular or cellular level, then progress to the clinical level, or the patient's "bedside."

It is important to note that not all translational research conducted at universities is drugbased. It is also practiced in the natural, biological, behavioral and social sciences and results in new medical devices, health education interventions and other technologies.

"Translational research seeks to look at and answer questions in new, innovative ways. Seeking these answers requires a cross-disciplinary approach and collaboration from investigators throughout the university," said John Clore, M.D., director of the VCU Center for Clinical and Translational Research. "Today's medical research involves a wide array of disciplines, including but not limited to the life, physical, computer and social sciences, as well as engineering, economics and business."

Funded through a \$20 million Clinical and Translational Science Award by the National Institutes of Health, VCU is part of a nationwide consortium of academic medical centers. The Center for Clinical and Translational Research is the home for this award and works with investigators within VCU as well as researchers across the country to advance science and foster partnerships to accelerate laboratory discoveries into treatments for patients.

A patent example

More than 13 million individuals worldwide are afflicted with sickle cell disease. A hereditary blood disorder, sickle cell disease occurs when the red blood cells form an abnormal crescent, or sickle, shape that block capillaries and other small blood vessels, leading to anemia, stroke and cumulative damage to tissues and organs.

VCU patented the compound 5-HMF, which has been relabeled as Aes-103, for the treatment of sickle cell disease. This new compound was developed by a team from the VCU Institute for Structural Biology and Drug Discovery that included Martin Safo, Ph,D, associate professor of Medicinal Chemistry, Richmond Danso-Danquah, Ph.D., assistant professor of Medicinal Chemistry, and Donald Abraham, Ph.D., the Alfred and Frances P. Burger Emeritus Professor of Medicinal Chemistry and Biological Chemistry and Emeritus Director of the institute.

"We have discovered a new, safer and more effective anti-sickling agent," said Safo. "This compound has a potent anti-sickling effect, both in vitro and in vivo studies show that 5-HMF inhibits the formation of sickled cells in the blood while carrying a minimal risk."

The compound was licensed to AesRx, a start-up company in Newton, Mass., for pre-clinical development and initial clinical trials. The VCU team has also developed second and third generation anti-sickling compounds that are also licensed to AesRx.

"We have developed an excellent relationship with our licensee, a start-up company with highly experienced management that was able to attract national thought leaders to its board and to develop a creative commercialization strategy," said Ivelina Metcheva, Ph.D., executive director of VCU Tech Transfer.

Aes-103 is one of the first molecules to enter the National Institutes of Health Therapeutics for Rare and Neglected Diseases program. The NIH, through a \$5+ million grant, is supporting the manufacture, pre-clinical studies and the first three human trials of Aes-103. The compound is current undergoing phase I clinical trials at the NIH.

"Francis Collins, M.D., Ph.D., the director of the National Institutes of Health, personally supports this project and has held it up as a prime example of translational medicine with academic medical centers working with start-up companies the research and development of life-changing drug therapies," said Francis Macrina, Ph.D., VCU vice present for research.

Image courtesy of Allen Jones, University Relations.

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