Vanderbilt-developed treatments for Alzheimer’s, Parkinson’s, schizophrenia, Charcot-Marie-Tooth disease, epilepsy

At Vanderbilt, researchers are developing the next wave of state-of-the-art therapeutics to address major diseases—all due to the initial investment in basic research from the National Institutes of Health. Those investments have led to partnerships with leading pharmaceutical companies who are working to commercialize the therapeutics. Diseases these researchers are developing treatments for include:

- **Alzheimer’s Disease**—Vanderbilt University’s Warren Center for Neuroscience Drug Discovery has an exclusive worldwide licensing and collaboration agreement with ACADIA Pharmaceuticals Inc. to develop and commercialize treatments for central nervous system disorders, such as Alzheimer’s disease and schizophrenia. One of the compounds included in this agreement is a lead compound in the treatment of Alzheimer’s disease and schizophrenia, VU319, which was discovered by Vanderbilt researchers at WCNDD, with support from NIH, and may slow memory loss accompanying serious brain disorders. The first human trial of VU319 was unique in that it was the first time Vanderbilt University had initiated a drug-discovery effort based on early basic science and advanced it into clinical trials in humans without partnering with a pharmaceutical company.

- **Parkinson’s Disease**—WCNDD has developed a Parkinson’s disease drug that has been selected for Phase 1 clinical trials by Appello Pharmaceuticals Inc., a clinical-stage therapeutics company focused on finding innovative early-stage drug candidates for patients with nervous system disorders. The drug is intended to work with an existing Parkinson’s disease treatment, levodopa, to lengthen the time that people gain relief from their Parkinson’s symptoms without experiencing debilitating side effects such as dyskinesias (uncontrollable involuntary movements).

- **Schizophrenia**—Researchers in the School of Medicine Basic Sciences have identified a protein in the central nervous system, known as mGlu1, as a potential target for novel treatments of schizophrenia and may provide relief for patients, allow them to reintegrate into and contribute to society, and diminish the burden on our health care systems. The drug reverses working memory deficits, a hallmark of schizophrenia for which there is currently no treatment.

- **Charcot-Marie-Tooth Disease**—Building on funding from NIH, Vanderbilt has partnered with Ancora Innovation LLC on an early-stage drug discovery project targeting Charcot-Marie-Tooth disease, an inherited disorder involving progressive damage to peripheral nerves that affects one in 2,500 people. Vanderbilt School of Medicine Basic Sciences researchers used NIH funds to illuminate the cause of Charcot-Marie-Tooth disease.

- **Epilepsy**—The Ancora-Vanderbilt collaboration is developing therapeutics for a rare form of epilepsy. The program is aimed to address an infant-onset seizure disorder caused by mutations in a gene that affects neurotransmission in the brain and can result in abnormal electrical activity, such as seizures. These conditions are resistant to most current epilepsy treatments. Thus, new therapeutic approaches are desperately needed.

**NIH training grants**

At Vanderbilt, a significant NIH funding mechanism consists of National Research Service Awards, which include institutional training grants and F (fellowship) awards that support training doctoral students in the biomedical sciences. The Vanderbilt Medical Scientist Training Program, which has been supported by NIH training grant (T) awards since 1976, prepares students for faculty and research positions with an integrated curriculum that features a strong core education in medicine and intensive training in scientific inquiry. Successful completion of the program leads to both M.D. and Ph.D. degrees, preparing the next generation of physician scientists. The Initiative for Maximizing Student Development at Vanderbilt University, which has been supported by NIH awards since 2000, aims to increase the number of Ph.D.’s. awarded to graduate students in biomedical research who are underrepresented in science.

Currently, Vanderbilt has 88 individual F awards and 22 institutional training awards, which support more than 250 students and provide more than $12 million in annual funding. Students supported by NIH NRSA fellowships have contributed to an understanding of fundamental biological principles and have discovered therapeutic strategies for treating diseases like Charcot-Marie-Tooth and COVID-19.

**Three Vanderbilt scientists awarded NIH grants for high-risk, high-reward research**

Three Vanderbilt scientists, one in Peabody College of education and human development and two in the School of Medicine Basic Sciences, received grants from the NIH High-Risk, High-reward Research Program for their unconventional, bold approaches to research that advances knowledge and enhances health. The program supports highly innovative biomedical or behavioral research proposed by extraordinarily creative scientists that will have a significant impact. The grants will be used to develop a noninvasive brain stimulation protocol to treat low reading comprehension in adults; to use structural biology and enzymology to understand how mitochondrial proteins assemble in cells to produce energy and maintain human health; and to determine the mechanisms that drive viral infection by characterizing these molecular processes in cellular environments.
Vanderbilt Institute for Surgery and Engineering

The Vanderbilt Institute for Surgery and Engineering, supported in part by $30 million in active NIH grants, many of which are R01s, is an interdisciplinary, trans-institutional institute that supports interactions between the university’s Schools of Engineering and Medicine in order to develop methods, devices, algorithms and systems to improve patient care. The program adapts to contemporary treatment paradigms and strives to develop new understandings of disease and dysfunction by studying the procedure—from brain tumor surgery, neuromodulation implants and robotic surgeries in the lung, prostate and kidney, to arterial and ablation therapies in the liver, radio-oncological procedures in the breast and eye, and more.

Seventy graduate students have earned their doctorates since the program’s start, and there are 124 current students engaged in research at ViSE. In addition, 42 patents and 21 licenses have come out of this collaborative work. ViSE is also engaged in research at VISE. In addition, 42 patents and 21 licenses have come out of this collaborative work. ViSE is also supporting an NIH-funded program for engineering Ph.D. students by providing immersive levels of clinical contact.

NIH SBIR/STTR funding at Vanderbilt

Six companies with ties to Vanderbilt received highly competitive Small Business Innovation Research/Small Business Technology Transfer grants from NIH. Those included:

- **EndoTheia Inc.** – a medical device company that develops next-generation devices for flexible endoscopy.
- **iDBiologics Inc.** – a fully human monoclonal antibody platform that enables rapid discovery and optimization of broad and potent therapeutic molecules.
- **Virtuoso Surgical Inc.** – brings benefits of robotic technology to endoscopy for surgeons and patients worldwide.
- **Yaya Scientific LLC** – innovative, diagnostic, therapeutic and integrated hardware solutions for biomedical problems.
- **Very Real Help Inc.** – a groundbreaking technology, like virtual reality, to transport you to entire worlds built for wellness.
- **VoluMetrix LLC** – Creates noninvasive technologies for vital monitoring to enhance well-being.

The SBIR/STTR program encourages domestic small businesses to engage in federal research and development with the potential for commercialization and enables small businesses to explore their technological potential and provides the incentive to profit from its commercialization. This program is crucial to the innovation ecosystem at Vanderbilt, especially as it relates to getting research and development off the ground in the early stages of these companies. In 2021, nine startups with roots at Vanderbilt were awarded more than $4.5 million in SBIR/STTR grants and matching funds from Launch Tennessee.

The Wond’ry, Vanderbilt’s Innovation Center, and the Center for Technology Transfer and Commercialization at Vanderbilt University contributed to the startups’ growth in various capacities, from assisting with ideation and R&D to facilitating invention disclosures and commercialization services. Founders of these startups span Vanderbilt faculty, alumni, staff and students.

According to Launch Tennessee, companies awarded their SBIR/STTR matching funds have created more than 505 direct jobs and had an economic impact of $146,125,214 in Tennessee—a return of $11.24 for every public dollar invested into the program.

Vanderbilt Center for Addiction Research

Founded in 2016 within the School of Medicine Basic Sciences, the Vanderbilt Center for Addiction Research, with nearly $69 million in funding from NIH since its founding, is a multidisciplinary center that bridges scientific discovery, policy work and scientific communication to lessen the impact of addiction on society. VCAR takes advantage of the unique environment at Vanderbilt University and brings together researchers with expertise to focus on the causes and consequences of addiction.

Collaboration between human and basic science researchers in this space allows for truly translational work that combines technological innovation in the neuroscience field with work that focuses on the factors that are affecting individuals suffering from addiction. Members of the center work together with the common goal of understanding the factors that give rise to addiction to develop better pharmacotherapies to treat this devastating disorder. Beyond NIH-funded foundational scientific discoveries, VCAR researchers also do a range of work in the public policy space that is focused on high-risk groups. Finally, VCAR is strongly committed to the dissemination and communication of scientific findings with the scientific community and the public. This is achieved through courses that allow for greater access to cutting-edge neuroscience approaches; research opportunities in VCAR labs for students; programming aimed at educating local and national communities; and finally, exhibits and content focused on destigmatizing addiction as a disease.

Vanderbilt researchers work to understand and treat cancer

Researchers in the School of Engineering and College of Arts and Science are focused on understanding cancer and developing new treatments and therapeutics. With support from NIH, these researchers are:

- Enabling the development of new therapies that make aggressive cancer cells easier to kill before they spread to other parts of the body.
- Working to understand tumor cells that circulate in the blood with the hope of finding ways to interrupt that process.
- Using machine learning to predict the effects of tumor variants on sensitivity to chemotherapy.
- Using nanoparticles to increase antitumor immunity in patients with ovarian cancer—one of the most lethal malignancies.
- Developing technology that seeks to boost a person’s immune system to better fight cancer.
- Using nanoparticles to stimulate immunity pathways in children with neuroblastoma—one of the most common forms of childhood cancer—with the aim of making immunotherapy treatments for neuroblastoma more effective.
- Developing new technology to facilitate understanding of the response of cancer cells to fluid flow, and hopefully ways to interrupt the metastasis process.

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Understanding how the brain works and develops

Researchers in the School of Engineering, Peabody College, and College of Arts and Science are focused on understanding how the brain works and develops. With support from NIH, these researchers are:

- Investigating brain-body connections to advance our understanding of aging in normal and pathological brains and developing machine-learning methods that can automatically reconstruct physiological signals from functional magnetic resonance imaging data.
- Developing a prototype headband to measure brain activity that could have widespread application in studying and ultimately treating ADHD and other neurological disorders. The device is lightweight, portable, and inexpensive to construct and can be worn at home.
- Studying parent-child proximity and emerging psychopathology to improve assessments of the early caregiving environment and develop effective preventative interventions.
- Measuring how the brain records and manages what we learn from different experiences over time, and adapting an existing computational model of memory for the primate brain to offer an important step for future interventions.

$2.3M NIH grant for substance use disorder prediction technology

A biomedical informatics researcher in the School of Nursing received a $2.3 million, five-year grant from NIH to focus on developing technology that can identify through the electronic health record people who are likely to have substance use disorder, with the specific application of supporting genetic research across multiple organizations. Opioid and substance use disorders are not well documented, which gives geneticists a lot of trouble conducting research at the desired scale. The researchers’ informatics and clinical backgrounds will help create a framework that allows health care providers in any organization to develop their own definition of substance use disorder—and then leverage data collected by multiple organizations to compare them equally across the different organizations.

Improving treatments for mental health

Researchers in the School of Medicine Basic Sciences and College of Arts and Science are focused on improving treatments for mental health. With support from NIH, these researchers are:

- Studying ketamine’s fast-acting antidepressant effects, especially for patients who have treatment-resistant depression, and exploring whether a new class of therapeutics can provide rapid antidepressant treatment, filling a major gap in care for depression.
- Looking at how next-gen schizophrenia drugs affect brain networks, cognition, motivation and mood through the use of augmented reality.
- Studying how mindfulness strategies may also moderately improve cognitive function, including attention, perception, declarative memory, language, construction, reasoning and executive function—the skills we use in daily life.

Addressing inequities in biomedical training, education

Researchers in the School of Medicine Basic Sciences, School of Nursing, and the College of Arts and Science are focused on addressing inequities in biomedical training and education. With support from NIH, these researchers are:

- Collaborating with other researchers who have graduated from, attended, taught at, or been heavily mentored by faculty at historically Black colleges and universities to review existing strengths and opportunities that will enable more Black graduates from predominantly white institutions to enter STEM fields at higher rates than today.
- Participating in a collaborative research project to reduce health disparities associated with chronic health conditions such as obesity, hypertension, diabetes, cardiovascular disease and cancer by supporting the delivery of evidence-based health promotion programs in churches serving African American communities.
- Creating a program aiming to provide underrepresented minority early-career and mid-career faculty with tools and resources needed to be successful biomedical scientific leaders by centering wellness and resiliency.

Ending ongoing pandemics

Researchers in the School of Engineering and College of Arts and Science are focused on ending ongoing pandemics. With support from NIH, these researchers are:

- Gaining a more complete understanding of the critically important life stage that is the target for the majority of mosquito control programs.
- Working to understand how Plasmodium falciparum—the pathogen that causes malaria in humans—affects the mosquitoes that spread the disease and how it actually may promote reproduction and disease transmission.
- Developing an artificial intelligence algorithm that can precisely track and count mpox (formerly known as monkeypox) lesions as well as develop lesion classification guidelines, which will be used when evaluating the potential uses of therapy.

EndoTheia Inc. has created a flexible endoscopic device to augment performance and improve the quality of care with minimal workflow disruption. (EndoTheia Inc.)

For more information, please contact the Vanderbilt Office of Federal Relations:
Christina West 202-216-4370 | Heather Bloemhard 202-216-4368 | federalrelations@vanderbilt.edu
FY 2022