

Vanderbilt University Therapeutics Pipeline and Assets Summary

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Vanderbilt Small Molecule Pipelines

Overview

Vanderbilt University has long been a world leader in pharmacology research, and is ideally positioned to lead drug discovery efforts through expanded partnerships with the pharmaceutical and biotechnology industry. Vanderbilt possesses the major infrastructure needed for drug discovery including high throughput screening (HTS), medicinal chemistry, molecular pharmacology, drug metabolism and pharmacokinetics (DMPK), *in vivo*/behavioral capabilities, and an extensive early through late phase human testing environment.

By making many of the aforementioned assets available to the Vanderbilt faculty, a diverse array of drug discovery projects have been launched. These are led by the Vanderbilt Center for Neuroscience and Drug Discovery (VCNDD). The VCNDD was the first “industry-like” drug-discovery platform program pioneered by Vanderbilt. Formed in 2003 and led by Drs. Jeffrey Conn and Craig Lindsley, who co-led neuroscience drug discovery efforts at Merck, this center now employs more than 100 full-time faculty and staff specialized in a range of bioscience arenas, including medicinal chemistry, molecular pharmacology, DMPK, and *in vivo*/behavioral.

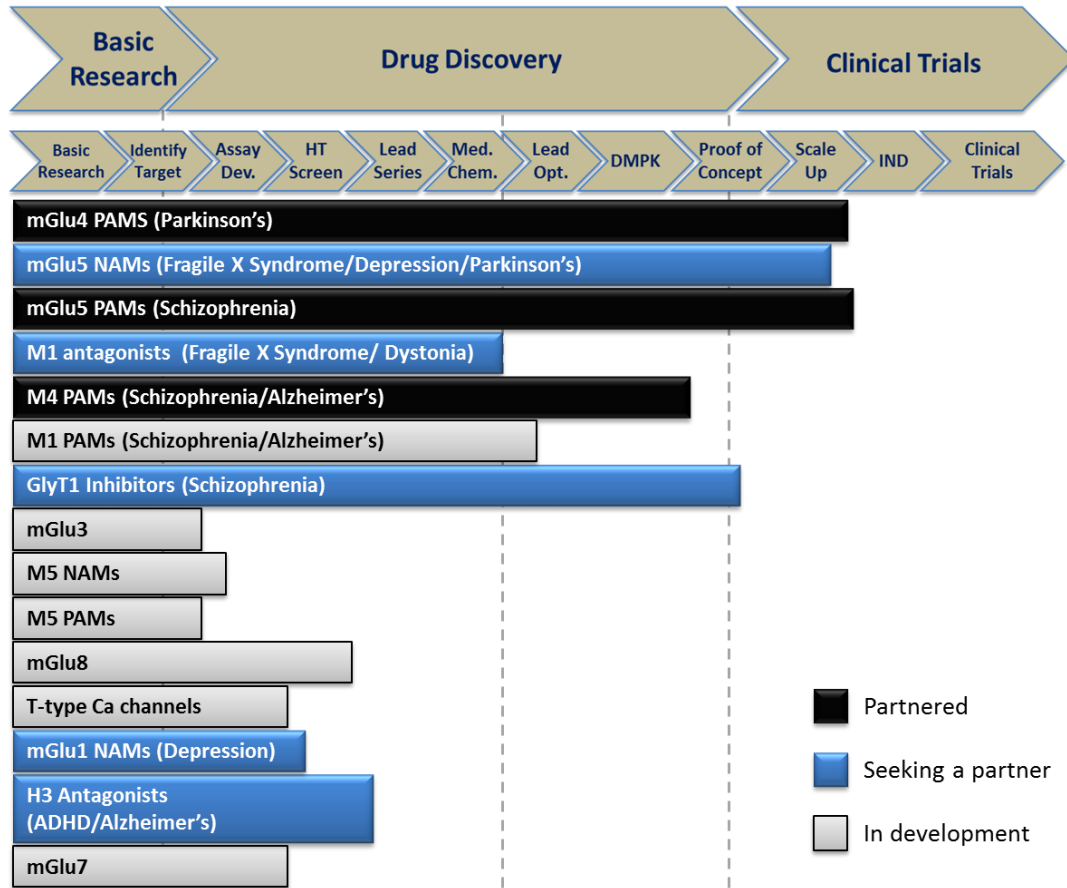
Since its inception, the VCNDD has secured a total of \$85 million in external research funding from industrial, foundation and federal funding sources. This program’s impact is evidenced by its success in partnering with four major pharmaceutical companies and several biopharma companies to develop new drugs for treating schizophrenia, depression, Parkinson’s disease, autism, and Fragile X syndrome. These programs have led to new innovations resulting in six commercial licenses with biotechnology and pharmaceutical companies.

In 2009, Vanderbilt recruited Dr. Stephen Fesik, divisional VP for Cancer Research at Abbott Laboratories, to lead a new cancer drug development effort with goals analogous to those of our already successful program in the neurosciences. In his short time here, Dr. Fesik has grown his research endeavor to more than 28 full time faculty and staff, employing a fragment-based approach to identify novel, potent cancer therapeutics. He has already been awarded a Pioneer Award from the National Institutes of Health for \$2.5 million to support his cancer drug discovery efforts and has been approached by several pharmaceutical companies to begin discussions of partnerships.

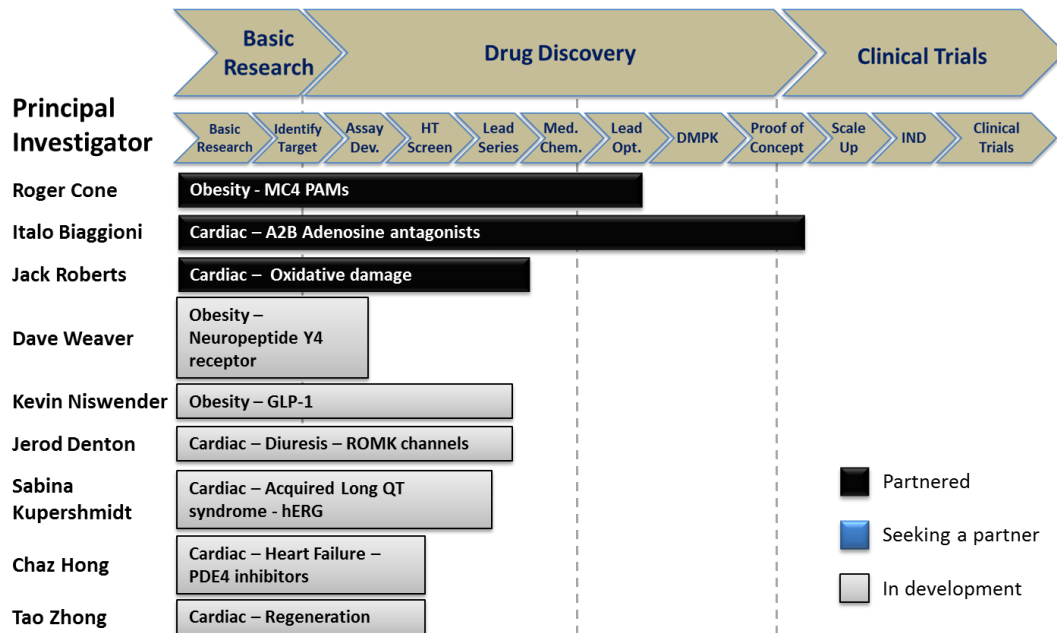
Augmenting these focused drug development programs is an array of core capabilities that Vanderbilt has invested in and developed over the past decade. The Vanderbilt Institute for Chemical Biology (VICB) and Molecular Libraries Probe Production Centers have utilized their HTS and medicinal chemistry capabilities to generate exciting hits for a variety of indications, and boasts a compound library containing more than 400,000 compounds. In addition to providing assay development, medicinal chemistry and HTS screening services to Vanderbilt drug discovery research groups, VICB provides access to an array of industry-trained experts. Dr. Dave Weaver, formerly of Bristol Myers Squibb, has pioneered new fluorescent dyes for use in HT screens, as well as new imaging plate readers and analysis software systems. The core is also currently expanding basic DMPK assays to help advance these projects further.

The following pages provide a brief outline of the Vanderbilt small molecule pipeline.

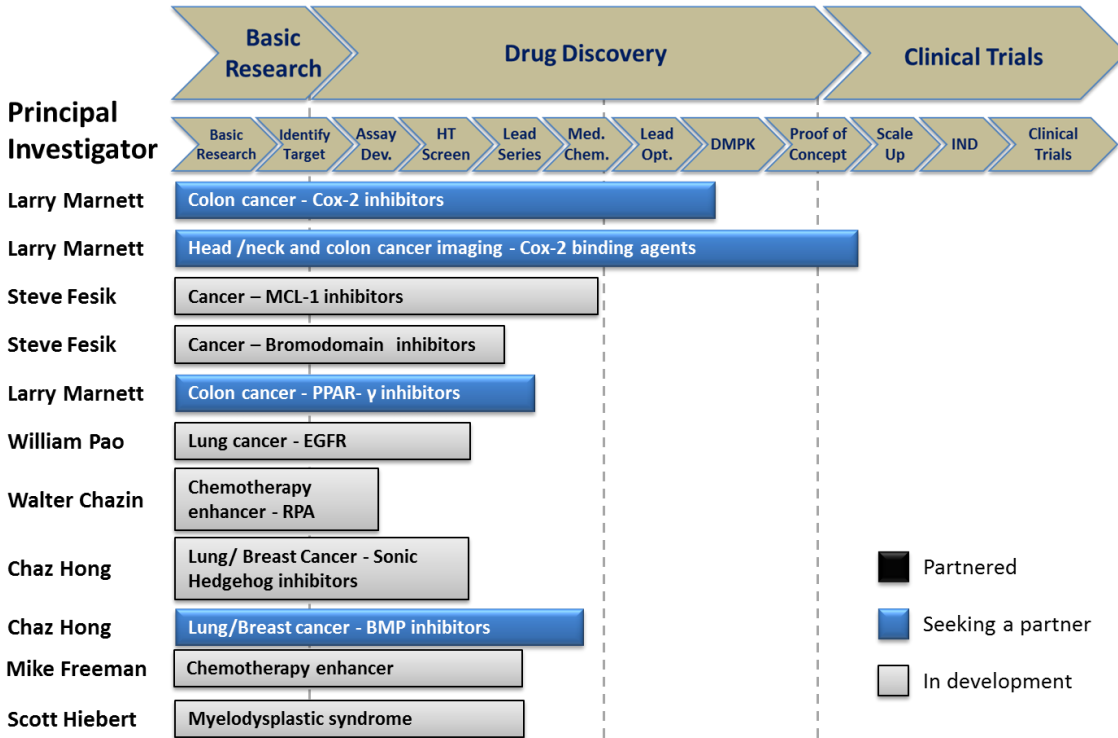
Vanderbilt Center for Neuroscience and Drug Discovery



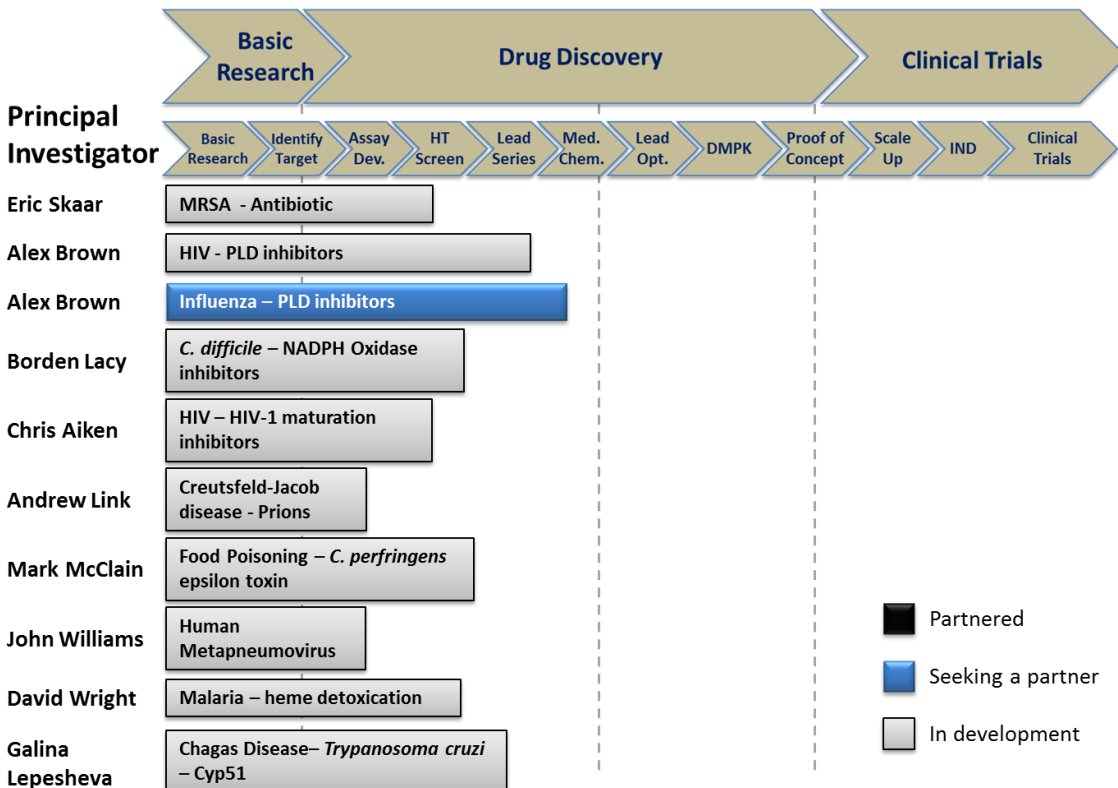
Cardiac and Metabolic



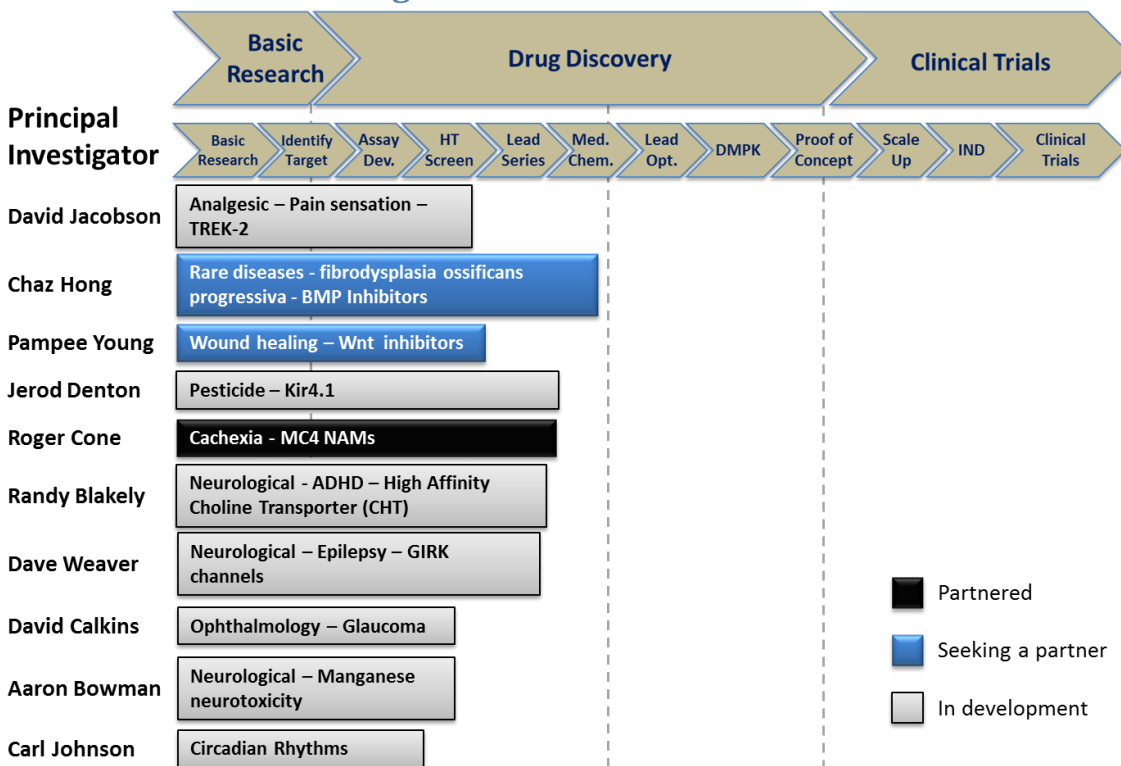
Oncology



Infectious Disease



Other Small Molecule Programs



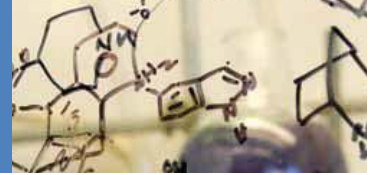
Vanderbilt Biologics pipeline

Overview

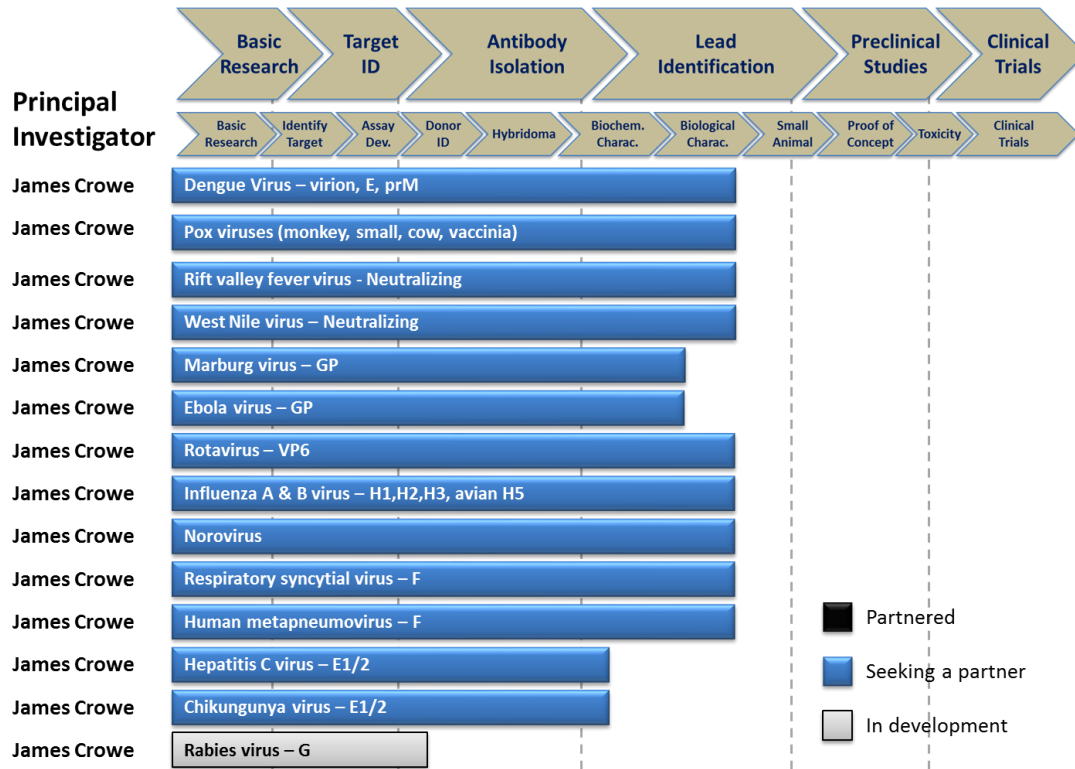
Vanderbilt has many programs in biologic discovery and development. The Vanderbilt Vaccine Center, led by James E. Crowe Jr. MD, has long been at the forefront of discovery and development of human antibodies. The Center has developed unique fusion and single B cell sorting and antibody engineering techniques to isolate and characterize fully human antibodies to many infectious diseases. These techniques are sensitive enough to effectively isolate 1918 Spanish Flu antibodies from survivors, and efficient enough to adapt them to isolate antibodies to emerging viral strains.

There are additional resources to assist any researcher in developing novel monoclonal antibodies. The Vanderbilt Antibody and Protein Resource is expert at creating unique antibodies to any target. Their protein expression systems, coupled with their antibody isolation techniques, have not only provided researchers with unique tools for their research, but they also create potentially therapeutic and diagnostic antibodies. These antibodies, created in small animals, are sequenced and characterized, priming them for humanization or engineering.

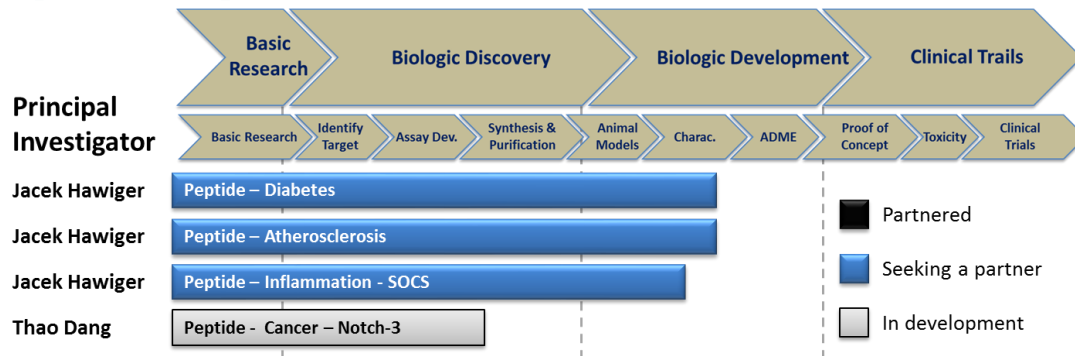
Vanderbilt is also at the forefront of clinical research to test novel Vanderbilt and 3rd party vaccine strategies. The Vanderbilt Vaccine Research Program is one of only eight Vaccine Treatment and Evaluation Units in the U.S. contracted to continue evaluating innovative vaccines for malaria, pandemic flu and other infections over the next seven years. The program is recognized internationally for its work on vaccines against serious childhood infections including respiratory syncytial virus and haemophilus influenzae.



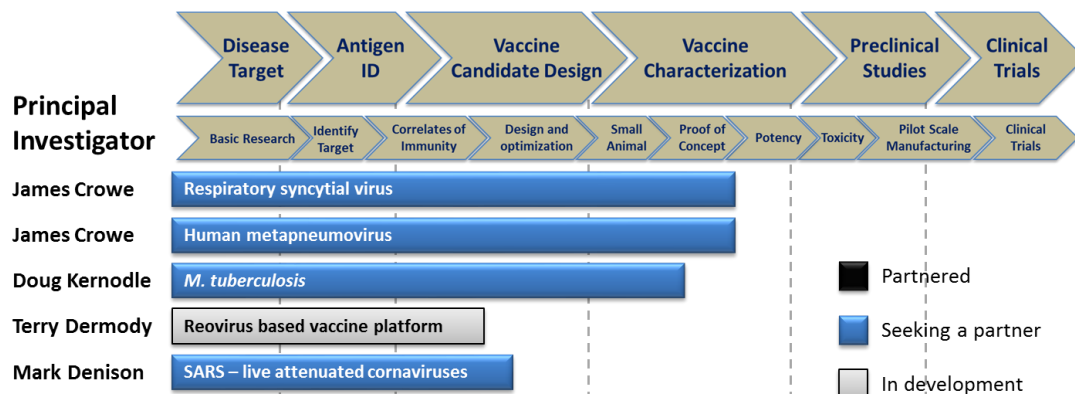
Human Antibody Therapeutics



Peptide Therapeutics

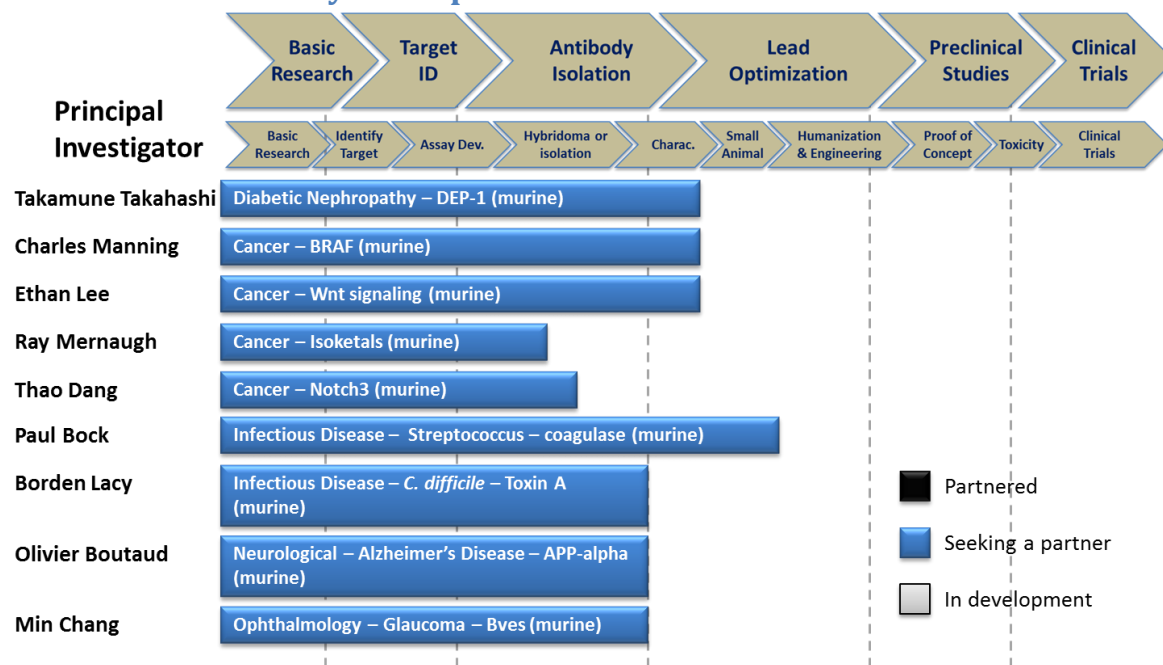


Vaccines





Non-Human Antibody Therapeutics

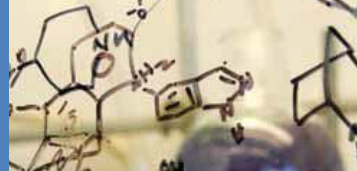


Translational Science and Clinical Trials

As Vanderbilt’s drug discovery scientists create novel compositions with the potential of becoming first in class therapeutics, they can easily partner with disease specialists and world class leading clinical physicians. Such partnerships early in the process can help design more effective and efficient clinical trials for novel therapeutics. These partnerships are further progressed through teaming with the various cores to assist in the development of companion diagnostics and target validation through other institutional initiatives such as personalized medicine.

In 2007 Vanderbilt University received a \$40 million Clinical and Translational Science Award (CTSA) grant, the largest single government research grant in Vanderbilt’s history, to expedite the translation of laboratory discoveries to patients in the community. This NIH grant helps fund the Vanderbilt Institute for Clinical and Translational Research (VICTR), which supports the translation of fundamental scientific discoveries into clinical practice and brings basic and clinical researchers together, and supports the pilot grants, resources and biostatistical and informatics that are essential for translating new findings into health care applications. Based on the success of the Vanderbilt-run CTSA program, the NIH awarded Vanderbilt a renewal of \$46 million in 2011.

Additionally, in 2011 Vanderbilt was awarded a \$20 million grant to be the Coordinating Center for the CTSA consortium, which now includes 60 institutions in 30 states and the District of Columbia. Being awarded as the Coordinating Center further validates the resources and know-how which Vanderbilt have been committed to and invested in for many years around clinical and translation research.



Vanderbilt Cores and Strengths (exemplary list)

BioVU: Vanderbilt's biorepository of DNA extracted from discarded blood collected during routine clinical testing and linked to de-identified medical records in the Synthetic Derivative. BioVU provides enabling resource for exploration of the relationships among genetic variation, disease susceptibility, and variable drug responses, and represents a key first step in moving the emerging sciences of genomics and pharmacogenomics from research tools to clinical practice. A major goal of the resource is to generate datasets that incorporate de-identified information derived from medical records and genotype information to identify factors that affect disease susceptibility, disease progression, and/or drug response.

Select Vanderbilt Core Strengths in Drug Discovery

Vanderbilt Institute of Chemical Biology

VICB provides chemical synthesis, high throughput screening, antibody production, and small molecule NMR services to Vanderbilt and beyond.

Vanderbilt Center for Neuroscience Drug Discovery

In the VCNDD, specialized teams of medicinal chemists, pharmacologists, and pharmacokineticists, and behavioral scientists discover and develop novel compounds for clinical testing.

Vanderbilt Institute for Imaging Science

VUIIS applies in vivo imaging methods for monitoring disease and treatment progression, and employs contrast agents and other image enhancement techniques for improved imaging and analysis.

Mass Spectrometry Research Center

The MSRC is a state-of-the-art mass spectrometry facility focusing on imaging MS for a host of diagnostic and prognostic purposes, and is the home to the National Research Resource for Imaging Mass Spectrometry.

Dept. of Biomedical Informatics

DBMI is the largest academic department of biomedical informatics in the country, with major programs in processing mass spec data, computational structural and chemical biology, and receptor and pathway mapping.

Center for Structural Biology

The CSB applies experimental and computational approaches to calculate the structure of various macromolecules, and identifies novel targets for therapies.

Other Key Cores and Programs at Vanderbilt

- Center for Translational Science Advances
- Natural Products Discovery Program
- Small Molecule NMR Facility Core
- High Throughput Screening Core
- Antibody and Protein Resource Core
- Molecular Libraries Probe Center Network
- Mouse Metabolic Phenotyping Center
- Vanderbilt Brain Institute



Nashville Life Science Real Estate

Nashville is the nationally recognized health care services center, with headquarters for more health care service companies than any city in the US. Nashville has also been recognized nationally as one of the top five cities for entrepreneurship and for starting new companies. And Nashville boasts premium quality life sciences real estate in and around the city, and near Vanderbilt University. Principal among these are:

The Cool Springs Life Sciences Center, where Vanderbilt has located a principal division of its drug research and development laboratories, is located in Franklin, Tennessee, just south of Nashville. Founded by local life sciences entrepreneurs and NobleGene Development, LLC, the Cool Springs Life Sciences Center is a 15 acre campus committed to providing a world class bioscience business environment and infrastructure dedicated to life sciences research, development, and manufacturing. Biomimetic Therapeutics, Inc. is one of the Center's anchor tenants.

oneC1TY, developed by Health Care REIT and partners, is a 19 acre multi-building, mixed-use campus located one mile from Vanderbilt University that caters to the health care, life sciences and technology sectors of the global economy. oneC1TY creates a space for those industries to connect with higher education, research and development, and other related disciplines in a collaborative, interactive campus near the center of Nashville's urban core.