



## Vanderbilt University Collaboration Aims to Fight Obesity with Novel Treatment

When Roger Cone, Ph.D., joined Vanderbilt University in 2008 as professor and the chair of the Department of Molecular Physiology and Biophysics, he was eager to continue his promising research on the human melanocortin-4 receptor (MC4R) as a drug candidate for the treatment of severe childhood obesity.

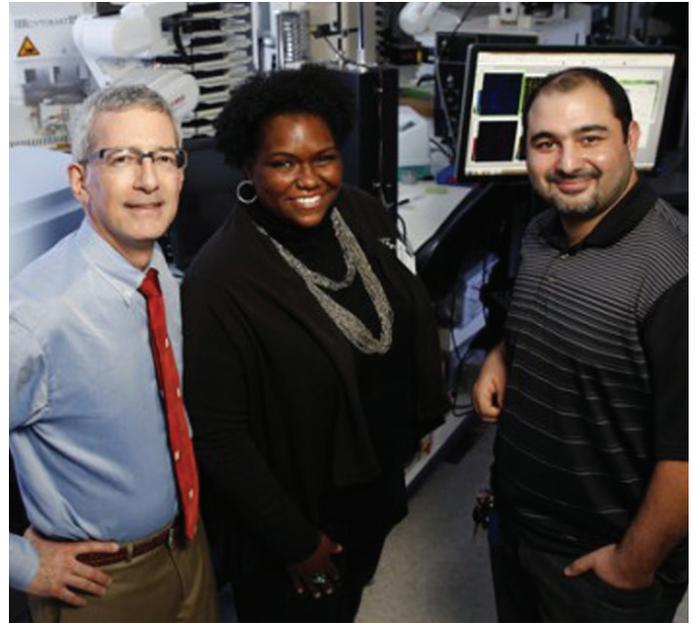
After discovering MC4R with his team in 1993 and demonstrating its role in obesity in 1997, the technology was subsequently licensed and resulted in several pharmaceutical companies initiating clinical trials with MC4R agonists that failed to advance due to target-specific side effects.

However, based on his extensive knowledge of the MC4R, Dr. Cone was convinced there was an alternative approach. Shortly after joining Vanderbilt University, Dr. Cone launched a new research program funded by the National Institutes of Health (NIH) utilizing the high throughput screening capabilities of the Vanderbilt Institute for Chemical Biology to identify a series of drug-like compounds that indirectly increase MC4R activity instead of directly activating the receptor. These “allosteric modulators” are less likely to induce the side effects that were observed during clinical trials with drugs that directly and potentially activated every MC4R throughout the brain.

Dr. Cone and his team were eager to advance these findings, and wanted to try and bring the full medicinal chemistry expertise of a major pharmaceutical company to the problem. Through a colleague, Dr. Cone was put in touch with GlaxoSmithKline (GSK) as a potential pharmaceutical collaborator to advance his research findings to clinical stage.

From there, things moved very quickly, with GSK recognizing the potential of Dr. Cone’s research and its possible impact on treating common obesity.

Once contract negotiations were completed, the GSK and Vanderbilt research teams got started right away, with Vanderbilt conducting the pharmacology and pre-clinical testing, and GSK trying to develop chemically similar compounds with improved activity and efficacy. As part of the agreement, GSK is providing research support to Vanderbilt for three years, and additional payments for meeting project milestones and a share of royalties. The goal is to begin phase I human trials within three years.



**Dr. Roger Cone**  
Vanderbilt University

“The way that this DPAC collaboration was set up is very novel, and the structure of the contract recognizes our contributions through milestone and royalty payments irrespective of who the intellectual property ends up with,” said Dr. Cone. “We are excited to have the opportunity to work with an amazing team at GSK to validate our research and attain our goal of having a compound in clinical stages by 2016.”

Three months into the collaboration, GSK is already very close to replicating Vanderbilt’s screening methodology.

“We have the pharmacological expertise and the screening techniques needed to advance this research, but without GSK’s depth of medicinal chemistry expertise and industrial approach to drug discovery, we wouldn’t be where we are today,” said Dr. Cone. “They recognized the potential of our discovery and worked diligently through the negotiation stage to help take our research to the next level. With GSK as our collaborator, we are confident that we can help develop a novel treatment for people with severe obesity.”

To learn more about GSK’s DPAC program, please visit [dpac.gsk.com](http://dpac.gsk.com)