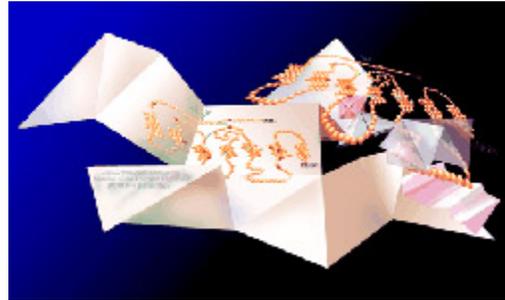


P. Michael Conn

In work conducted over the last 15 years, Conn and his team have identified an underlying biological principle that has dramatically changed scientists' understanding of cellular mutations that result in human disease. He has demonstrated that it is possible to manipulate and redirect the routing of non-functional receptors, ion channels and enzymes thereby curing disease:

<http://en.wikipedia.org/wiki/pharmacoperone>



It is becoming well-recognized that mutations of receptors, enzymes and ion channels frequently result in protein misfolding and subsequent retention by the cell's quality control system. Misfolding can result in protein molecules that retain intrinsic function yet become misrouted within the cell and, for reasons of mis-location only, cease to function normally and result in disease. This contrasts with the prior presumption that mutational inactivation always reflects loss of intrinsic function (i.e., a receptor that either fails to recognize ligand or does not couple productively to its effector).

Recognition of this alternate concept immediately presents the therapeutic opportunity to correct misrouting and rescue mutants, thereby restoring function and, potentially, curing disease.

Pharmacoperones are small molecules that enter cells, bind specifically to misfolded mutant proteins, correct their folding, and allow them to escape retention by the cellular quality control system. They then route to the plasma membrane (or other site) where they can function normally. The biochemical mechanism of action has been identified and an in vivo proof-of-principle has been accomplished.

In principle, the pharmacoperone rescue approach might apply to a range of human diseases that result from misfolding - among these: cystic fibrosis, hypogonadotropic hypogonadism, nephrogenic diabetes insipidus, retinitis pigmentosa, hypercholesterolemia, cataracts, and neurodegenerative diseases (Huntington's, Alzheimer's, Parkinson's). In the case of particular proteins, this approach has succeeded with a striking number of different mutants, supporting our view that pharmacoperones will become powerful ammunition in our therapeutic arsenal.

This lab has provided an in vivo proof of principle and developed high throughput screening assays for pharmacoperone drugs. For recent publications:

PUBMED Link:

[http://www.ncbi.nlm.nih.gov/sites/entrez?cmd=search&db=pubmed&term=conn%20pm\[Author%20Name\]](http://www.ncbi.nlm.nih.gov/sites/entrez?cmd=search&db=pubmed&term=conn%20pm[Author%20Name])

BIOGRAPHY

P. Michael Conn is the Senior Vice President for Research and Associate Provost, Texas Tech Health Sciences Center. He is The Robert C. Kimbrough, Professor of Internal Medicine and Cell Biology/Biochemistry. He was previously Director of Research Advocacy and Professor of Physiology and Pharmacology, Cell Biology and Development and Obstetrics and Gynecology at Oregon Health and Science University and Senior Scientist of the Oregon National Primate Research Center (ONPRC). He served for twelve years as Special Assistant to the President and Associate Director of the ONPRC. After receiving a B.S. degree and teaching certification from the University of Michigan (1971), a M.S. from North Carolina State University (1973), and a Ph.D. degree from Baylor College of Medicine (1976), Conn did a fellowship at the NIH, then joined the faculty in the Department of Pharmacology, Duke University Medical Center where he was promoted to Associate Professor in 1982. In 1984, he became Professor and Head of Pharmacology at the University of Iowa College of Medicine, a position he held for eleven years.

Dr. Conn is known for his research in the area of the cellular and molecular basis of action of gonadotropin releasing hormone action in the pituitary and therapeutic approaches that restore misfolded proteins to function. His work has led to drugs that have benefitted humans and animals. He has authored or co-authored over 350 publications in this area and written or edited over 200 books, including texts in neurosciences, molecular biology and endocrinology. Conn has served as the editor of many professional journals and book series (*Endocrinology*, *Journal of Clinical Endocrinology and Metabolism*, *Endocrine, Methods*, *Progress in Molecular Biology and Translational Science* and *Contemporary Endocrinology*). Conn served on the National Board of Medical Examiners, including two years as chairman of the reproduction and endocrinology committee. The work of his laboratory has been recognized with a MERIT award from the NIH, the J.J. Abel Award of the American Society for Pharmacology and Experimental Therapeutics, the Weitzman, Oppenheimer and Ingbar Awards of the Endocrine Society, the National Science Medal of Mexico (the Miguel Aleman Prize) and the Stevenson Award of Canada. He is the recipient of the Oregon State Award for Discovery, the Media Award of the American College of Neuropsychopharmacology and he was named a distinguished Alumnus of Baylor College of Medicine in 2012. Conn is a previous member of Council for the American Society for Cell Biology and the Endocrine Society and is a prior President of the Endocrine Society, during which time he founded the Hormone Foundation and worked with political leadership to heighten the public's awareness of diabetes. Conn's students and fellows have gone on to become leaders in industry and academia. He is an elected member of the Mexican Institute of Medicine and a fellow of the American Association for the Advancement of Science. He is the co-author of *The Animal Research War* (2008) and many articles for the public and academic community on the value of animal research and the dangers posed by animal extremism. His op/eds have appeared in *The Washington Post*, *The LA Times*, *The Wall Street Journal*, the Des Moines Register, and elsewhere.