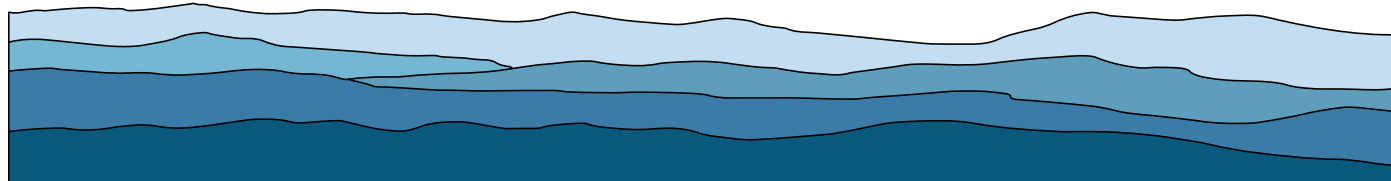


HIGHLANDS IN CHEMISTRY SEMINAR SERIES



PAUL HELQUIST

UNIVERSITY OF NOTRE DAME

“Drug Development in a Highly Interdisciplinary and Multi-institutional Setting”

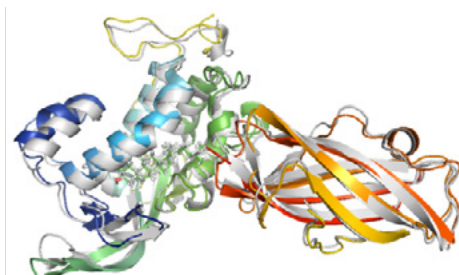
The discovery and development of new therapeutic agents requires expertise in a very broad range of disciplines, including chemistry, biochemistry, biology, medicine, business, and government affairs. Few academic programs cover all of the required fields to pursue drug development beyond initial, basic discovery research. With a mission-oriented goal of pursuing treatments for some very rare, lethal, genetic disorders, laboratories in chemistry, biochemistry, and biology at Notre Dame have formed a consortium with biomedical researchers and business specialists at several other universities, the National Institutes of Health, start-up companies, and private foundations. The overall team has provided the wherewithal to go from basic synthetic chemistry and computational modeling studies all the way through biological and pre-clinical investigations and on to human clinical trials approved by the FDA. This lecture will trace the 15-year history of development of therapies for Niemann-Pick type C disease, an exceedingly rare lysosomal storage disorder, which is most commonly diagnosed in young children and which progresses over a period of ten years, leading to death of the children, usually as teenagers. The need for efficient syntheses of potential drugs led to the discovery of many broadly useful synthetic methods, which will be highlighted in this lecture along with describing the rest of the drug development pathway.

MARCH 20, 2020

2:30PM

HAHN HALL NORTH 140

FACULTY HOST:
PAUL CARLIER



Left: Computational model of the exchange of lysosomal cholesterol between the NPC1 and NPC2 proteins, depicting the origin of lipid trafficking disruption in Niemann-Pick type C disease and the basis of drug development efforts for this lethal lysosomal storage disorder.