2016 Million Dollar Bike Ride
Pilot Grant Program

**Application Title:** Rational Design of Effective Therapeutics for Treatment of the 1282X CFTR Disorder

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**Institution:** University of Pennsylvania

This grant addresses a specific Cystic Fibrosis, CF-W1282X, an hereditary disease that results in the formation of an abnormal CFTR protein needed for normal cellular function. Patients can be treated with therapeutic agents, called nonsense suppressors (NonSups), which repair the defective CFTR protein. However, the utility of currently available NonSups is limited by either low potency or toxic side effects. A really effective therapy for CF-W1282X will require the development of a new NonSup that overcomes these limitations. Such development is, at present, hindered by a lack of understanding of how the current best NonSups actually work. The goal of our research is to obtain such understanding, providing the basis for the design of more effective NonSups for treating W1282X and other CFTR disorders.