

**CONCEPT CLEARANCE RECORD  
FY 2016 RESEARCH INITIATIVE – NCATS**

TITLE: SaME Therapeutics: Targeting Shared Molecular Etiologies Underlying Multiple Diseases to Accelerate Translation

OBJECTIVE(S): To develop a matrix of diseases and molecular etiologies to identify shared molecular etiologies (SMEs) underlying multiple diseases, and to stimulate novel clinical trials of SME-targeted drugs based on grouping patients by SME rather than clinical phenotype.

DESCRIPTION: In contrast to the current approach to disease based on clinical presentation, the concept of SaME therapeutics is to focus on shared molecular etiologies underlying multiple diseases using systems biology as a framework for drug development and clinical trials. An important part of this initiative will be a bioinformatics component, including crowdsourcing approaches, to create a checkerboard-like matrix of diseases and molecular etiologies to catalyze translation.

IMPORTANCE: While the number of disorders with a known molecular basis continues to increase rapidly, the number with an effective treatment continues to lag far behind. What is needed to overcome this translational roadblock is a fundamental change in the current symptom-based, one-disease-at-a-time approach to drug development and clinical trials. An explicit focus on identifying SMEs for translation represents such a fundamental change, which ultimately will bring more rationally designed treatments to more patients more quickly.

HISTORY: This concept was submitted by NCATS as an NIH Common Fund proposal in 2014 and approved by the NIH Council of Councils.

PROJECT/PROGRAM OFFICERS:

**Philip J. Brooks, Ph.D.**

Program Director

Division of Clinical Innovation

Phone: 301-443-0513

E-mail: [pjbrooks@mail.nih.gov](mailto:pjbrooks@mail.nih.gov)

**Danilo A. Tagle, Ph.D., M.S.**

Associate Director for Special Initiatives

Office of the Director

Phone: 301-594-8064

E-mail: [tagled@mail.nih.gov](mailto:tagled@mail.nih.gov)