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.

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