

Title of proposed program: Establishing Clinical Utility for Informed Decision Making

Submitting Source: NIH, Strategic Planning Meetings

What is the major obstacle/challenge/opportunity that the Common Fund should address? What would the goals of the program be?

A new era of health technology has emerged. For example, genomic assays and technologies are being developed and targeted for adoption into mainstream healthcare for the prevention and management of multiple diseases and conditions. Social science research has provided information about decision making and has developed effective behavioral interventions for many conditions. The expectation is that information generated from these and other technological innovations will help guide decisions related to treatment options, reproductive strategies, and lifestyle behavior changes, leading to improved outcomes and a more informed and healthy society.

But how do we know if expectations are being met? Although many technologies demonstrate analytical and clinical validity, improved outcomes will be realized only when the clinical utility is demonstrated in a practical setting. Clinical utility refers to the balance of benefits and harms associated with the use of the test/technology in practice, including the improvement in measurable clinical outcomes and usefulness/added value in clinical management and decision making compared with not using the test/technology (EGAPP Working Group Recommendation Statement, 2009). In order to effectively establish clinical utility, there must be sufficient clinical data that can be evaluated using a systematic and rigorous approach.

The goal of this initiative is to accelerate a coordinated national research effort to expand, through support of training, infrastructure, and clinical research, the evidence needed for establishing the clinical utility of new and emerging technologies. This will in turn facilitate and expedite effective transition of these technologies into practice, resulting in improved healthcare at both the individual and population levels.

Why is a trans-NIH strategy needed to achieve these goals? What initiatives might form the strategic plan for this topic?

No single IC has the capability to support and integrate all the approaches needed to achieve the goals of this initiative. A trans-NIH strategy is needed to establish research priorities and to provide infrastructure and training. Potential initiatives include 1) trans-NIH/HHS workshop to discuss topics related to clinical utility, including processes used for assessing clinical utility, design of research studies for generating the evidence for assessing clinical utility, challenges encountered in establishing clinical utility, etc.; 2) expert working panel to prioritize diseases/conditions to be targeted by this initiative, followed by identification of technologies, associated with diseases/conditions designated as high priority, where analytical and clinical validity have been established but there is insufficient evidence to assess clinical utility; and 3) establishment of “clinical utility cores” to provide the resources, infrastructure, and opportunities for performing the clinical research studies needed to generate evidence to assess clinical utility.

If a Common Fund program on this topic achieved its objectives, what would be the impact?

Expedited development and strengthened use of evidence-based knowledge to guide consumers, providers, and other stakeholders in making informed decisions, thereby closing the gap between discovery and practice.

Evaluation of Genomic Applications in Practice and Prevention (EGAPP) Working Group Recommendation Statement. (2009). *Genetics in Medicine*, 11(1), 67-73.