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Submitted on-line

RE: Request for Input of Administration on the NIH-Industry Pilot Program Regarding the NIH-Industry Program to Discover New Therapeutic Uses for Existing Molecules, NOT-RM-13-021

The Association of American Medical Colleges (AAMC) is pleased to respond to the recent request for information (RFI), referenced above, on the research community’s experiences with the new drug repurposing pilot program. Understanding that the RFI is seeking primarily information on the experiences of institutions, firms, and investigators who participated in this first year for application, or who may participate in the program in future, the AAMC has relayed the request to the research leadership of our member organizations and has encouraged them to respond. As you know, AAMC’s member organizations include all 141 U.S. allopathic medical schools, nearly 400 teaching hospitals and health systems, and 90 academic societies.

The Association would like to add here (under comment field 9 in the on-line response form) several additional observations and recommendations specifically related to evaluation of the repurposing program and its potential outcomes. Our comments here follow upon the AAMC’s original letter of June 1, 2012, shortly after the repurposing program was established, and reaffirms those recommendations.

In our earlier comments, the AAMC urged NIH and NCATS to “imbed within the pilot program a process and outcome evaluation to help guide later implementation and improvement of the resource-sharing program.” The current RFI clearly reflects NCATS’ commitment to ongoing refinement of the program, and we believe that the center is also developing methods to identify outcomes and to measure or evaluate success of the program overall. We offer several further points for consideration in the program’s evaluation.

The scope and potential impact of this program is broader than new pharmaceutical uses: The AAMC believes that the criteria for success of the pilot program are broader than the number of compounds discovered to be efficacious or the magnitude of their effects. Of course, we share as the primary goal that the repurposing program will identify novel therapeutic or other uses for these compounds, but it is also important to remind the public and the nation’s leadership that, as with all research, important knowledge and benefits accrue in various ways, even by negative results from promising hypotheses. NCATS should consider how effectively findings—
including negative findings—are disseminated and used to further public health, scientific discourse and research training. The AAMC has the utmost confidence in the NIH, the academic research community, and peer review to identify, evaluate, and make use of significant scientific and health findings from this research.

Notably, the collaborations established under the program create excellent opportunities for involvement and training of physician scientists, and may in fact motivate some physicians to engage more closely in research. Given the current attention of a new working group on the physician scientist workforce in the NIH Advisory Committee to the Director, NCATS should endeavor to track the involvement of trainees in these programs, especially physician scientists.

Addressing health disparities: The RFI requests comments on whether there should be extended involvement, for example by firms contributing compounds, in reviewing projects or proposals. The AAMC agrees that diverse perspectives should be included at various stages of program review. Program review should also include broad perspectives on how a repurposing program does or can better address health needs for all Americans, including addressing disparities in treatment. Such review should help:

- Target repurposing opportunities toward diseases with marked inequities in prevalence or outcomes.
- Engage communities to assure that subjects included in safety and later efficacy trials are representative of various demographic groups, especially those groups that suffer disproportionately from morbidity and mortality.
- Strive for (at least) equitable dissemination of results and products/drugs so that the development of novel treatments doesn’t paradoxically increase population disparities via differential dissemination.

The NIH should also commit to effectiveness trials and patient-centered outcomes research to assure that treatments are not only clinically beneficial in real world settings but that they are palatable to patients and communities. The results should be used to spark new repurposing efforts and discovery should treatments prove less effective than efficacious.

Potential for scale up and proliferation: Another dimension of success in this program is the extent to which this pilot program evolves and inspires imitation and variation, both within NIH or beyond. The AAMC fully hopes that the fact that the center has been able to establish this program and catalyze the initial collaborations will encourage other firms to make available "failed" compounds or approaches for further research with academic partners. If so, such collaborations should be attributed as a success for this program as well, even if outside of the NCATS’s purview.

In its June 2012 letter, the AAMC supported the development of template agreements and recommended that the current templates should be modified after involvement of academic partners in the research collaboration (members of the academic community, we understand,
were not involved in developing the initial templates). We have received indications from academic institutions that the template agreements in fact were difficult at times to change through negotiation with industry partners. We are concerned that overly restrictive terms or rigidity in negotiating these terms could be a hindrance to the program’s future success or expansion. Ideally, templates should remove *cause* for protracted negotiation, not remove opportunity for negotiation.

In closing, the AAMC congratulates NCATS for the successful launch of the drug repurposing program, as well as the firms, institutions, and investigators that have established these pioneering collaborations. The Association is proud that seven member institutions are among the academic partners awarded these initial projects. The ability of this new organization to bring about what for so long had been called for by the health and academic communities is a notable achievement.

Please contact Heather Pierce, J.D., M.P.H. at hpierce@aamc.org or Stephen Heinig at sheinig@aamc.org in my office with any questions about these comments.

Sincerely,

Ann C. Bonham, Ph.D.
AAMC Chief Scientific Officer