



Improving Human Life by Advancing
the Field of Transplantation

AST BOARD OF DIRECTORS 2013-2014

PRESIDENT

Daniel R. Salomon, MD
The Scripps Research Institute

PRESIDENT-ELECT

Kenneth A. Newell, MD, PhD
Emory University

IMMEDIATE PAST-PRESIDENT

Roslyn B. Mannon, MD
University of Alabama at Birmingham

TREASURER

Kimberly Ann Brown, MD
Henry Ford Hospital

SECRETARY

Anil Chandraker, MD, FRCP
Brigham and Women's Hospital

COUNCILORS-AT-LARGE

James S. Allan, MD, MBA
Massachusetts General Hospital

Rita R. Alloway, PharmD
University of Cincinnati

Ronald G. Gill, PhD
University of Colorado, Denver

Michael G. Ison, MD, MS
Northwestern University

Paul Martin, MD
University of Miami

Dianne B. McKay, MD
University of California, San Diego

Larry B. Melton, MD, PhD
Baylor Medical Center, Dallas

David P. Nelson, MD
Integris Baptist Medical Center of Oklahoma

Thomas Pearson, MD, DPhil
Emory University

EXECUTIVE VICE PRESIDENT
Susan J. Nelson, CAE

EXECUTIVE DIRECTOR
Libby McDannell, CAE

June 2, 2014

The Honorable Fred Upton
Chairman
Energy & Commerce Committee
U.S. House of Representatives
2125 Rayburn House Office Building
Washington, DC 20515

The Honorable Diana DeGette
Energy & Commerce Committee
U.S. House of Representatives
2368 Rayburn House Office Building
Washington, DC 20515

RE: 21st Century Cures - Policy Suggestions from American Society of Transplantation (AST)

Dear Chairman Upton & Congresswoman DeGette:

On behalf of the American Society of Transplantation (AST), representing the majority of professionals engaged in the field of organ transplantation, we applaud your ongoing leadership and steadfast resolve to strengthen our nation's ability to remain a leader in the field of biomedical research. The transplant community is grateful for the opportunity to be involved with the bipartisan Congressional 21st Century Cures initiative and appreciates the opportunity to contribute to this important process and dialogue.

AST is aware of the fiscal and political challenges that annually confront the federal funding process for the National Institutes of Health (NIH). Although consistent and predictable funding streams are without question key to the success of biomedical research, we also believe there are specific policy areas and partnerships that can be supported to enhance, expedite and advance new discoveries and cures. With this approach in mind, AST wishes to focus its comments today on a very important partnership that has served as a backbone and discovery engine for many successful biomedical breakthroughs.

Incentives for Entrepreneurial Investment in American Science

The AST is strongly supportive of the principle behind a Congressional program to advance the future of medical research in America for the 21st Century. We are also very grateful for the opportunity to participate in the early steps of this initiative by introducing some ideas for consideration now. But we also wish to emphasize that we recognize this is just the start of the process and look forward to participating in its evolution.

NATIONAL OFFICE

15000 Commerce Parkway, Suite C • Mt. Laurel, NJ 08054
856.439.9986 • Fax: 856.439.9982 • info@myAST.org • www.myAST.org

GOVERNMENT RELATIONS

William Applegate, Director of Government Relations • Bryan Cave, LLP
1155 F Street, NW • Washington, DC 20004
202.258.4989 • bill.applegate@bryancave.com

WORLD TRANSPLANT CONGRESS 2014

July 26-31, 2014
San Francisco, CA

Page Two

We continue to believe that one part of the solution to making sure the medical research infrastructure of our country remains strong and competitive in a dynamic world is to continue support for the National Institutes of Health. The last decade of essentially unchanged NIH funding in the face of significant increases in the costs of doing science fueled by a literal explosion of new and very exciting technologies has significantly eroded the competitive position of the US and driven many young, promising scientists to other fields. But even more concerning to the AST has been the significant erosion in the ranks of physician/scientists, the leaders in our medical institutions that choose to pursue both laboratory research and clinical medicine. Unfortunately, as NIH funding rates for new grants has continued to fall these physician/scientists are forced out of research by the powerful winds of health care reform and institutional pressures to earn their salaries doing clinical care. The result is abandoning the translational medical research that has brought the cures for disease to patients and written the history of medicine.

The AST proposes a novel solution to this challenge for consideration that we call “entrepreneurial science”.

We note that this solution is not based on demanding additional funding for NIH. While we are certain that Congress would increase NIH funding if that was possible, a pragmatic view of the last decade and the current fiscal realities dictates that we need to change course if we want to succeed in maintaining our place. The AST believes we need to propose and operationalize novel ways to support the future of medical research by capitalizing on the many strengths of the US economic system to create new funding opportunities. Thus, entrepreneurial science starts at the interface between basic laboratory research and clinical trials. The objective is to take promising new therapies or diagnostics based on the latest technologies like deep DNA sequencing, tissue engineering and gene therapy and successfully translate them into tomorrow’s cures. We propose the exploration of creating new ways to foster connections between basic scientists and physician/scientists with our nation’s venture capital and investment banking communities. The entrepreneurial spirit of our country has been a core strength since its founding and we simply wish to tap into this spirit again to create the opportunities for translating the latest scientific research into the latest cures and advance the health and safety of the American people.

First, it is critical to state that there is no future for medicine if we don’t support basic laboratory research. Basic medical research creates the opportunities for translation to clinical medicine. This will remain a primary mission for NIH funding. But when basic research is translated successfully to clinical medical practice it generates huge opportunities for new companies to be created with venture investment. That potential is the basis of entrepreneurial science.

However, the truth is that the majority of physicians and scientists in the US do not have the training or the time or access to the support infrastructure to really facilitate the full transition from laboratory to bedside to a commercially viable product. But only when that full transition is made successfully are our patients actually able to benefit from all the work. In other words, too much great basic and translational research is funded with precious NIH resources, published in our best scientific journals and still never actually benefits a patient. One practical reason is that we don’t have enough expertise available in many of our nation’s universities and medical centers to finish the job. Another reason is that the physician/scientists that have done this work want to go back to the laboratory and concentrate

Page Three

on making the next discovery rather than start the risky and difficult process of translating the work to clinical practice, dealing with the FDA and trying to obtain the considerable funding required to make clinical translation possible. But even in systems with some resources for such entrepreneurialism the current reality is that there are few incentives for scientists and physicians to risk the enormous time and energy required to do this translational work especially when at this time of such limited funding the failure to get their next NIH grant means ending their life's work.

Therefore, entrepreneurial science as a new endeavor must take advantage of creating pools of capital and expertise to invest up front in promising basic research with the agreement that the investors will also have access to commercializing these advances along with the Universities and Medical Centers involved. This is not intended as a passive process but rather an active collaboration between these physicians and scientists with experts in translating science to practice and commercializing it successfully. In this system, promising science would be presented to these teams of translational, commercial and investment experts very early in development as a competitive and managed grant process. Then decisions would be made to join in collaboration with academic physicians and scientists to take their discoveries through the full cycle to a new medicine, diagnostic test or therapeutic strategy. A key point is that these teams can be formed from existing biotechnology and pharma work forces and would instantly be a unique resource to our nation's scientists and also be able to tap into streams of capital from big pharmaceutical companies interested in the science as it develops and shows promise.

We recognize that there are many details to consider to fully develop our proposal for entrepreneurial science. But we also have enormous confidence in the power of the American spirit of entrepreneurialism and simply wish to bring it to medical research now at a time that our nation's leaders realize the critical importance of this work to the future health of all Americans.

The AST represents thousands of physicians and scientists that have dedicated their lives to caring for patients, advancing the practice of transplantation and medicine, and making the scientific discoveries that will create the future cures. We look forward to working with Congressional leadership to understand the details and then operationalize what is now just an aspirational concept of a novel opportunity to fund the next generation of medical miracles with entrepreneurial science.

If you have questions or require any additional information, please do not hesitate to contact me directly or the AST Government Relations Directors, Bill Applegate and Chris Rorick, at [\(202\) 258-4989](tel:2022584989).

Sincerely,

A handwritten signature in black ink that reads "Daniel R. Salomon M.D." in a cursive script.

Daniel R. Salomon, MD
President