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Safeguarding science

Changing political and funding landscapes in the US create an uncertain environment for biomedical research. The research community must insist that scientific policy follow from science, not political partisanship.

With the inauguration of US president-elect Donald Trump comes a sense of instability and uncertainty, not only for overall governance and policy, but also for biomedicine in particular. New leadership and new legislation have the potential to disrupt both research and safeguards for drug approval.

There is as yet no clarity on how the Trump administration, together with the Republican-controlled legislature, will shape federal funding and policy for research in the US. Certainly, discussion of these topics was not a prominent aspect of the presidential campaign. The incoming administration recently took its first step toward the oversight of biomedicine, with the nomination of Republican Congressman Tom Price for the head of the US Department of Health and Human Services, but, at the time of this writing, nominees to lead the National Institutes of Health (NIH) and Food and Drug Administration (FDA) have not been named.

Although it is not yet clear who will run these agencies, continued federal support for embryonic stem cell research—essential for advances in regenerative medicine—might be under threat. Both Price and vice-president-elect Mike Pence, currently governor of Indiana, have previously opposed this type of research, and it doesn't seem farfetched that the new administration could seek to reinstate rules dating from the George W. Bush administration that restrict federal funding for such research to a very limited number of embryonic stem cell lines.

Researchers whose experiments depend on access to fetal tissue—essential for studies of fetal development and used heavily for studies of infectious diseases, such as HIV and Zika virus—also have cause for worry. Since covertly filmed videos in July 2015 brought public attention to the role of Planned Parenthood in providing fetal tissue to researchers, this topic has been in the bull's-eye of the Republican Party, which created a congressional panel to investigate relationships between abortion service providers, tissue-procurement agencies and research institutions. Echoing Republican lawmakers, Trump has called for a ban on federal funding of Planned Parenthood, and the Republican Party platform goes further in calling for the criminalization of research that uses fetal tissue from elective abortions. The biomedical community and patient groups must mobilize to fight any new restrictions on both embryonic stem cell and fetal tissue research.

Alongside the governmental transition, a wide-ranging piece of new US legislation called the 21st Century Cures Act was signed into law last month. This Act will provide $4.8 billion over the next 10 years for three large-scale NIH research projects—the Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative, the Cancer Moonshot and the Precision Medicine Initiative. Although this law offers welcome funding, it also contains provisions, intended to facilitate drug approval, that have the potential to weaken the drug-review process.

The Cures Act directs the FDA to consider "real world evidence" as it decides whether to approve drugs—defined as "data regarding the usage, or the potential benefits or risks, of a drug derived from sources other than randomized clinical trials"—and a provision to raise the profile of patient-experience data. Although such data can be valuable, this provision risks tipping the review process toward anecdotal evidence and away from clinical trial data. Many in the biopharmaceutical community expressed concerns of this type after the recent controversial approval of the drug eteplirsen for Duchenne muscular dystrophy (*Nat. Med.* **22**, 1193, 2016). The Cures Act also allows companies to submit summaries of study data for supplemental applications for a drug or biologic, which has the potential of allowing companies to cherry pick the data that they present to the FDA. Although it isn't yet clear how these provisions will play out in practice, it is imperative that the FDA maintain rigorous standards for drug approval. The interpretation and implementation of these new provisions will be influenced greatly by the head of the FDA, which raises the stakes for the selection of a nonpartisan, scientifically and medically qualified individual for this post.

Biomedical science requires continuity of resources to thrive—research programs cannot be easily paused and restarted. In these early days, it is unclear what priority the government will give to funding of the NIH. The Cures Act provides some assurance for continued funding of some of the NIH's large projects, but funding for the type of single-investigator-led grants that are the staple of NIH funding also needs to be safeguarded. With the launch of these ambitious new projects—aimed in part at revolutionizing disease treatment—and the budget increase that the NIH enjoyed in 2016, it would be a huge loss to science and medicine if this momentum were not sustained under the new administration.

Amid the uncertainty of these times, the biomedical community must insist that scientific funding, policy and regulatory decisions be made on the basis of strong scientific evidence. Certainly, Trump's recent waffling on the existence of climate change does not inspire confidence that scientific reality will carry the day. For scientific and medical progress, what is needed is not the staking out of ideological positions on the basis of politics, campaign promises or personal religious belief, but a free and open process of scientific exploration and discussion.