NIH Starts to Spend $4.8 Billion in “Extra” Cures Drug Research Money

Stephen Barlas

The National Institutes of Health (NIH) launched 110 new brain research projects in the fiscal year ending last September (2017) with the first portion of the $1.5 billion over 10 years it will hopefully receive from the 21st Century Cures Act, which spread a total of $4.8 billion over four NIH programs. That is money over and above the NIH annual appropriation from Congress. The other three “Innovation Funds” are: Precision Medicine, Cancer Moonshot, and Regenerative Medicine.

The $1.5 billion in new Brain Research through Advancing Innovative Neurotechnologies (BRAIN) research money seems particularly important given the announcement in January that Pfizer was ending its Alzheimer’s research program after years of fruitless development efforts, which have plagued other companies as well. Some companies, such as AstraZeneca, Eli Lilly and Co., and Biogen, Inc., are still plowing that field. Perhaps some of the new basic research the NIH will be funding with its BRAIN money from Congress will open some knowledge windows in chemistry and biology that will lead to a greater number and more successful Alzheimer’s clinical trials. That is the hope, anyway.

The NIH is already spending Cures money from the three other Innovation Funds, too, in some cases paralleling some of the work the Food and Drug Administration (FDA) is doing as a result of its new Cures directives (see article on page 149). A prime example is the “All of Us” initiative, which is the primary focus of the Precision Medicine Initiative (PMI). While the FDA is using Cures money to aggregate patient post-marketing data, the NIH will be collecting patient data in order to learn about medical conditions and to assemble a critical mass of potential clinical trial participants. The NIH is partnering with five companies to create a participant technology center.

“Getting all these partners on board would have been nearly impossible had not the Cures Act included something called Other Transactions Authority for PMI, making it possible for NIH to move forward with unprecedented speed and flexibility to carry out beta testing of all the many components, and now a planned launch in the spring of 2018,” NIH Director Francis Collins, MD, PhD, told the House Energy and Commerce Committee on November 30, 2017. “It will also be a platform where many clinical trials can also get started because these participants will have been pre-consented for contact to see if they would be interested in taking part in a clinical trial, say, for diabetes or Alzheimer’s risks.”

There is also some NIH/FDA crossover with Cancer Moonshot funding and the FDA’s Oncology Center for Excellence. The Cancer Moonshot was supposed to send some of its $1.8 billion (the largest of the four Innovation Funds) to the FDA annually, but that hasn’t happened because of what FDA Commissioner Scott Gottlieb, MD, termed “legal” problems, so far unspecific. Moonshot money is going to cancer prevention, cancer diagnosis, cancer treatment, and care through the Beau Biden Cancer Center.

One of the highest-visibility initiatives within the Moonshot program is a partnership with 11 leading biopharmaceutical companies announced on October 12, 2017, that is aimed at pushing immunotherapy forward. Called the Partnership for Accelerating Cancer Therapies (PACT), the five-year public–private research effort totaling $215 million will initially focus on efforts to identify, develop, and validate robust biomarkers to advance new immunotherapy treatments that harness the immune system to attack cancer. The FDA is also charged with getting biomarkers qualified and available for use in any clinical trial.

There is additional FDA Cures synergy with the NIH’s Regenerative Medicine Innovation Project, which with a $30 million budget is by far the smallest of the four Innovation Funds at the NIH. Just $2 million was spent in fiscal 2017, but the NIH kicked in $3 million from its central account, almost all of that supporting research on adult stem cells. In September, NIH made eight clinical research awards that cover a broad spectrum of science and new technologies.

The Cures money provided to the NIH is separate from the annual appropriation, which, for the NIH, has been falling over the past decade. According to a commentary in the January 3, 2018, issue of Stat, “…adjusted for inflation, in terms of purchasing power, the NIH budget is down 19.2% from 2003.” In that sense, the $4.8 billion is a godsend. However, that Cures “add-on” also has to be appropriated annually, and there is no guarantee Congress will provide all the cash it promised.

Moreover, neither the FDA nor NIH portions of the Cures Act provide any leverage for either agency to reduce the high costs of specialty drugs. At the House hearings, U.S. Representative Jan Schakowsky (D-Illinois) referred to the $4.8 billion and told NIH Director Collins and FDA Commissioner Gottlieb, “If we are spending billions to incentivize the development of new drugs, I think we also have to ensure that patients can afford those drugs. The development of new drugs and devices is meaningless unless the discoveries are affordable to patients. It is almost cruel to find a cure and then have it priced so high that a patient can’t afford it.”

A month after those hearings, Sparks Therapeutics introduced its new blindness cure called Luxturna (voretigene neparvovec-rzyl), a gene therapy of the type the Cures Act hopes to further, for $425,000 per eye. continued on page 179
REFERENCES


