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Obama Signs Bipartisan Bill To Speed Miracle Cures to Market. But Who Will Have Access to Expensive New Technology? Who Won't?

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In a city that's witnessed trench warfare between Congress and the White House during the last six years of the Obama Administration, this was a rare moment: a bipartisan love fest. On Dec. 13, 2016, President Obama signed the 21st Century Cures Act," which includes expanded funding to push medical technology through the development pipeline. "We are bringing to reality the possibility of new breakthroughs to some of the greatest health-care challenges of our time," Obama said. "It is wonderful to see how well Democrats and Republicans in the closing day of this Congress came together around a common cause."ⁱ

The legislation was backed by a coalition of interests, including the powerful pharmaceutical industry, academia, and consumer groups supporting speedier medical research. Its few critics have mainly argued that the popular funding provisions "mask a worrisome loosening of regulations at the Food and Drug Administration that could put patients at risk."ⁱⁱ

Hardly anyone, however, is asking the million-dollar question: Which Americans will end up having access to new miracle cures, many of which promise to be extremely expensive? And, who will not? The country's patchwork of health insurance already is rationing expensive new technology to some populations, particularly low-income people. Congress, meanwhile, has begun a fractious debate over repealing, and possibly replacing, the Affordable Care Act (ACA). So, while lawmakers have just put their collective foot on the technology gas pedal, they may soon slam the brakes on funding for expanded coverage, potentially throwing millions of Americans into the ranks of the uninsured.

The pitch: 10 Areas Where Big Breakthroughs Can Happen

It's a Thursday afternoon at an influential think tank in Washington, D.C., a week and a half after Donald Trump won the presidential election. The director of the National Institutes of Health, Dr. Francis Collins, is making a spirited pitch for the lame duck Congress to increase NIH funding as part of the 21st Century Cures bill.ⁱⁱⁱ At a session entitled "Realizing the Promise of Medical Innovation," Dr. Collins highlights 10 areas where researchers will be able to make breakthroughs, including cures for major diseases, over the next five to 10 years. What's needed from American taxpayers' right now is the funding for NIH to do the basic science to empower the medical technology industry to keep moving forward, he says.^{iv} The new technology would open a door of hope for millions of Americans, including those with mental health disorders, Alzheimer's, spinal cord injuries, diabetes, cancer, and heart disease.

The 10 areas where science is poised for breakthroughs and cures include the following, Dr. Collins says:

1. Single-cell biology. Researchers now can look at a single cell and the genes on it (before they had to study clumps of cells). This huge step forward in developing cancer therapies.
2. The brain. By 2025, scientists should be able to understand how information is processed by the brain's neural pathways. This could be the foundation for advances in treatment of schizophrenia and other mental illnesses; traumatic brain injuries; and autism.
3. Alzheimer's. There are recent advances in understanding both the cause of the disease and possible interventions. There is evidence that we can now remove amyloid plaque from the brain (the plaque is a marker for the disease).
4. Spinal cord injuries. New treatments are being developing using both bioengineering and electrical intervention.
5. Diabetes. Researchers are developing artificial pancreases that can deal with both Type 1 and Type 2 diabetes. Some of these are "completely engineered" and some built from stem cells taken from blood or skin.

6. Cardiology. Scientists are working on a way to use a person's own stem cells to build them a new heart (this is a "reach," the NIH director says, but it could happen in a decade).
7. Pandemic flu prevention. The recent Ebola and Zika epidemics have scared the public, but the "big scare" remains return of the pandemic flu which killed tens of millions in 1918. It's hard to prepare for this risk because we now need to develop a new vaccine for every flu strain -- and that takes time to make. Most vaccines create antibodies that attack proteins on the surface of a virus. If flu viruses can be attacked below the surface, we could make a single vaccine that could prevent all types of flu. That's possible because scientists have found "invariant protein strands" below the surface of flu viruses.
8. Pain. There are new ideas on creating non-addictive painkillers to replace opioids that have led to epidemic levels of addiction.
9. Immunotherapy for cancer. For some patients, doctors now have ability to fire up the immune system to fight cancer (Dr. Collins mentioned successful immunotherapy provided to former president Jimmy Carter during the late stage of cancer treatment).^v In next five years, we will figure out why this approach works only for some patients and expand its reach.
10. Precision medicine. A massive long-term study is under way to find out who gets sick, who stays healthy, and why.

Research funded by NIH and other-public sector agencies powers the basic scientific advances needed for medical breakthroughs. The private sector, in turn, uses this knowledge to develop technology that it distributes to Americans through a complex web formed by market dynamics and public policies. Who has access to the new technology depends on decisions made by corporations (including pricing); health plans (including coverage decisions); and Congress, state legislatures, and government agencies (including access to affordable health coverage). In our patchwork system, not everybody has access to new medical technology. If it is needed for survival, some will live and some will die.

Case Study: the Miracle Cure for Hepatitis C

About three years ago, Gilead Sciences introduced a breakthrough hepatitis C treatment, Sovaldi, which can cure up to 90 percent of patients within three months. The price set by the manufacturer -- \$1,000 for each pill or about \$84,000 for a course of treatment -- has been difficult for health plans and public programs to absorb.^{vi} Medicaid programs in many states are rationing Sovaldi and two competing hepatitis C drugs that have since entered the market, often by limiting access to only those patients with advanced liver disease.^{vii}

American health plans have objected to hepatitis C drugs' price tag. The head of Molina Health Plans, which covers two million Medicaid patients nationwide, noted that the price of curing one hepatitis C patient roughly equaled the cost of insurance coverage for 25 Medicaid enrollees.^{viii} More than three million Americans are estimated to have hepatitis C -- a number that has made universal access to the miracle cure difficult to afford. Treating all lower-income hepatitis C patients enrolled in Medicaid or held in prison would cost the government about \$55 billion.

But not treating people with hepatitis C also is costly. The disease can be spread from person to person through blood contact, including small amounts on shared shaving items and unsterilized tattoo needles. About one in 30 Baby Boomers has the disease, which can be dormant for many years without symptoms. Untreated, hepatitis C can be fatal and drastically reduce the quality of life.^{ix} Consequences of infection include scarring of the liver, cirrhosis, liver cancer, liver failure and death. For some, the only option for survival is a liver transplant.^x In 2014, more people died of hepatitis C than HIV/AIDS, according to the U.S. Centers for Disease Control and Prevention.

Before the cure came to market, the lifetime cost of treating a person with hepatitis C was estimated to be about \$65,000 (a bit less expensive than the original price of the cure).^{xi} In 2011, researchers figured that hepatitis C prevalence had peaked in 1994, with between 2.8 and 4 million Americans infected, and would decline by two-thirds by 2030. But the prevalence of advanced liver disease in later stages of the illness was expected to rise, as well as

the total cost associated with chronic infection, which would reach about \$9.1 billion in 2024.

The new hepatitis C drugs have been a significant factor driving up U.S. drug spending in 2014 and 2015 and drug costs for many health insurance programs, including Medicare.^{xii} The Centers for Medicare and Medicaid Services reported that national spending on drugs rose 12.4 percent in 2014, well above the 2.4 percent increase in 2013. The agency attributed the jump in part to the introduction of hepatitis C treatments, which accounted for \$18 billion in new spending in 2014 and 2015 combined. The degree to which the new drugs might reduce future medical costs is an open question.

Solvaldi's \$84,000 price tag (for Americans) was not the most costly novel treatment when put on the market. According to one report, more expensive therapies, targeted to smaller populations, included the following:

- 1. Soliris.** For about \$440,000 a year, this treats paroxysmal nocturnal hemoglobinuria, in which the immune system destroys red blood cells. The condition affects up to 10,000 people in North America.
- 2. Cinryze.** At \$417,000 a year, this treats hereditary angioedema, an immune system disorder affecting 6,000 Americans.
- 3. Vimizim.** At \$380,000, this treats Morquio A syndrome, an enzyme deficiency interfering with skeletal development. There are about 3,000 such patients in the developed world.
- 4. Elaprase.** At \$375,000, this is for Hunter syndrome, a serious genetic disorder that primarily occurs in males and affects about 2,000 people worldwide.
- 5. Naglazyme.** At \$375,000, this treats Maroteaux-Lamy syndrome, a metabolic disorder with symptoms that include an enlarged head and short stature. It affects about 1,100 people worldwide.^{xiii}

Drug and device manufacturers say that they need to charge enough to make a profit and continue investing in expensive and risky research that most often leads to dead ends in the search of more cures. Balancing the need to maximize profits with affordability has resulted in a wide range of prices across nations.

Many Americans were outraged by Gilead's decision to charge \$84,000 for the hepatitis C cure in the United States, somewhat less in other advanced countries, but only \$900 in Egypt and other poor countries.^{xiv} Is that fair? It depends on how you measure. In the U.S., average gross domestic product (GDP) for person is about \$56,000.^{xv} As the cost of the hepatitis C medication comes down in price, it will roughly equal per-person GDP, and then probably drop further. In poorer countries, despite differential pricing, the drug remains very expensive in relative economic terms. In Egypt, per person GDP is \$3,600. In Afghanistan, it's \$590. No doubt, an underground cross-border market already is in play. Both pills and medical tourists easily can cross national boundaries.

Back at the Bipartisan Policy Center session medical innovation, a panel discussion follows Dr. Collins' opening remarks. The panel includes an industry lobbyist, a consumer advocate, and medical technology experts. From what they say, some of the cures in the pipeline may cost upwards of \$1 million per patient. A member of the audience asks: Shouldn't access to these treatments be on the table as Congress considers more funding for NIH research? How can the country afford these cures for everybody? If the public pays for research enabling technology development, doesn't it have the right to access it? Not necessarily, the industry representative says: the government pays for research, but the private sector develops and markets the cures. One panelist suggests developing innovative funding mechanisms, including new types of "mortgages" for medical technology, presumably so taxpayers, health plans, or individuals could pay some or all of future costs to pharmaceutical manufacturers in advance.

Who Will Live?

Which of us are most likely to gain access to expensive new medical technology? In the current American health care financing patchwork, access is determined largely by the type of insurance a person has as well as available cash. If survival depends on expensive new technology, the odds will be best for the wealthy and people covered by private health insurance, bought by employers or individuals, and Medicare, the federal program covering the elderly and disabled. But even those with what's now considered to be "good coverage" could face unaffordable

out-of-pocket costs. As health care costs continue growing faster than the economy as a whole, many private plan sponsors are increasing copayments and deductibles. After a cancer diagnosis, Medicare beneficiaries without additional wrap-around coverage already pay an average of \$8,115 a year out of pocket, or 23.7 percent of their incomes, according to researchers at Johns Hopkins University. They found that Medicare beneficiaries with cancer are paying up to 63 percent of their incomes toward their health care – with hospitalizations the major factor for their high expenses.^{xvi}

As evidenced by the cure for hepatitis C, people relying on Medicaid, the federal-state program for the low-income, are more likely to face some kind of rationing. People with no health coverage, no money, or limited financial resources can expect to be last in line. This creates a policy challenge. Over the past decades, the American middle class has shrunk and the income and wealth gaps between those at the top and bottom have widened.^{xvii} About half of Americans have virtually no net assets.

The number of Americans with no health insurance could soon double if Congress and the president follow through on promises to repeal the ACA. If the law enacted in 2010 were repealed, the Congressional Budget Office and Joint Committee on Taxation (CBO/JCT) estimated that the number of uninsured Americans would increase by 22 million or 23 million in 2017, 2018, and 2019; and by about 24 million in all subsequent years through 2025.^{xviii} (So far, the ACA has cut the percentage of uninsured Americans in half.)

Repealing the ACA (which might rival the pancreas in complexity) and its various funding sources also would increase federal budget deficits by **\$137 billion** over the 2016–2025 period, according to CBO/JCT. Incidentally, losing this revenue would make it harder for Congress to find the dollars to increase NIH funding for medical research. According to its website, the NIH invests nearly \$32.3 billion annually in medical research “for the American people.”^{xix} NIH investments of American taxpayers’ money span 265 “research/disease areas.”^{xx}

As new medical technology comes to market, it’s likely that the cost and coverage tensions that arose with the launch of the hepatitis C cure will repeat, and

perhaps magnify. It will be interesting to see how American voters respond. It's a fair bet that people who voted for Donald Trump are less likely than those who voted for Hillary Clinton to have health insurance, and more likely to lose it, should the ACA's coverage sections be repealed.

In any event, budget pressure is expected to grow. U.S. government debt already exceeds the nation's annual GDP and is expected to rise as social programs, primarily Medicare and Social Security, absorb the costs of an aging population. The U.S. already spends more than 17 percent of GDP on health care, far more than any other country.^{xxi} France and Germany, for example, spend about two-thirds as much, enjoy similar access to medical technology, and provide near-universal coverage to their citizens.

As American taxpayers continue underwriting research pushing miracle cures through the technology pipeline, will the cost pressures they bring lead to a more inclusive health care financing system -- more like those in Europe and Canada? Or will cost pressure and political decisions make the system less equitable?

In 10 years, who will have health insurance and who won't? Will the near-poor have less coverage than those on Medicaid? Who will be left on the outside looking in? Will standard health insurance coverage be good enough to save your life? How much will one of those stem-cell hearts or artificial pancreases cost anyhow? Where will you be able to get one?

Conclusion

Accelerating the speed at which million-dollar cures come to market is likely to open a Pandora's Box of intensifying political conflict over who has access to life-extending technology and who does not. As Congress invests in new medical technology, it should consider how its decisions shaping the health care financing system, including the fate of the ACA, will determine which Americans will have access to what comes through the pipeline. Whether Republicans or Democrats, lawmakers need to take steps to bring the cost of medical technology down. Developers of medical technology need to be more efficient and prices need to come down to levels that are affordable for all Americans, regardless of what type

of coverage they happen to have, if any. Policymakers and industry leaders would be wise to make sure that what's coming to market includes vaccines, drugs, and procedures that will lower medical costs in the long run.

ⁱ https://www.washingtonpost.com/news/powerpost/wp/2016/12/13/obama-paying-tribute-to-biden-and-bipartisanship-signs-21st-century-cures-act-tuesday/?utm_term=.e51e3310e95c. "Obama, paying tribute to Biden and bipartisanship, signs 21st Century Cures Act Tuesday," Washington Post, Dec. 13, 2016.

ⁱⁱ https://www.washingtonpost.com/news/powerpost/wp/2016/12/13/obama-paying-tribute-to-biden-and-bipartisanship-signs-21st-century-cures-act-tuesday/?utm_term=.e51e3310e95c. "Obama, paying tribute to Biden and bipartisanship, signs 21st Century Cures Act Tuesday," Washington Post, Dec. 13, 2016.

ⁱⁱⁱ <http://www.help.senate.gov/chair/newsroom/press/21st-century-cures-bill-released> On Nov. 26, 2016, House and Senate health committee leaders announced that they had released the final "21st Century Cures" bill, and that House would vote on a \$6.3 billion landmark medical innovation package to accelerate the discovery, development, and delivery of new cures and treatments and "provide new funding for the National Institutes of Health and Food and Drug Administration."

<http://khn.org/morning-breakout/congress-sends-cures-bill-to-obama-in-rare-bipartisan-feat/> On Dec. 7, the Senate overwhelmingly passed the legislation and sent it to President Obama.

^{iv} <http://bipartisanpolicy.org/events/realizing-the-promise-of-medical-innovation/> "Realizing the Promise of Medical Innovation," Bipartisan Policy Center, retrieved from the web, Nov. 24, 2016

^v https://www.washingtonpost.com/news/to-your-health/wp/2016/09/28/cancer-immunotherapy-is-moving-fast-heres-what-you-need-to-know-now/?tid=a_inl "A consumer's guide to the hottest field in cancer treatments — immunotherapy," Washington Post, updated version retrieved from the newspaper web site on Nov. 24, 2016.

^{vi} <http://www.cbsnews.com/news/1000-a-day-miracle-drug-shocks-us-health-care-system/> "\$1,000-a-day miracle drug shocks U.S. health care system," CBS Money Watch, April 3, 2014.

^{vii} <http://www.theatlantic.com/health/archive/2015/09/an-expensive-medications-human-cost/407299/> "The True Cost of an Expensive Medication," The Atlantic, Sept. 25, 2015.

^{viii} <http://www.sfgate.com/health/article/Cost-of-Gilead-s-hepatitis-C-pill-Sovaldi-spurs-5398315.php> "Cost of Gilead's hepatitis C pill, Sovaldi, spurs revolt," SFGATE, April 13, 2014.

^{ix} <http://www.hepchope.com/> See HEPCHOPE.com.

^x <http://www.pewtrusts.org/en/research-and-analysis/blogs/stateline/2014/02/28/could-new-hepatitis-c-drugs-bust-state-budgets> Pew Charitable Trusts Stateline, “Could New Hepatitis C Drugs Bust State Budgets?,” Feb 28, 2014.

^{xi} <https://www.ncbi.nlm.nih.gov/pubmed/23280550> *Hepatology*. 2013 Jun;57(6):2164-70. “Chronic hepatitis C virus (HCV) disease burden and cost in the United States,” [Razavi H](#), [Elkhoury AC](#), [Elbasha E](#), [Estes C](#), [Pasini K](#), [Poynard T](#), [Kumar R](#).

^{xii} <http://healthaffairs.org/blog/2016/11/03/the-cost-of-a-cure-revisiting-medicare-part-d-and-hepatitis-c-drugs/> Health Affairs Blog, “The Cost of a Cure: Revisiting Medicare Part D and Hepatitis C Drugs,” Jack Hoadley, Tricia Neuman, and Juliette Cubanski, Nov. 3, 2016.

^{xiii} <http://www.sfgate.com/health/article/Cost-of-Gilead-s-hepatitis-C-pill-Sovaldi-spurs-5398315.php> “Cost of Gilead's hepatitis C pill, Sovaldi, spurs revolt,” SFGATE, April 13, 2014.

^{xiv} <http://www.sfgate.com/health/article/Cost-of-Gilead-s-hepatitis-C-pill-Sovaldi-spurs-5398315.php> “Cost of Gilead's hepatitis C pill, Sovaldi, spurs revolt,” SFGATE, April 13, 2014.

^{xv} <http://data.worldbank.org/indicator/NY.GDP.PCAP.CD> Current GDP Per Capita, retrieved from the World Bank web site, Nov. 24, 2016.

^{xvi} https://www.washingtonpost.com/news/to-your-health/wp/2016/11/23/many-medicare-cancer-patients-hit-by-high-out-of-pocket-costs/?hpid=hp_regional-hp-cards_rhp-card-national%3Ahomepage%2Fcard “Many Medicare cancer patients hit by high out-of-pocket costs,” Washington Post, Nov. 24, 2016.

^{xvii} Growing inequality in the United States has been widely documented. See “Trends in Family Wealth, 1989 to 1913,” Congressional Budget Office, Aug. 18, 2016 <https://www.cbo.gov/publication/51846> ; Stiglitz, J. (2013). *The Price of Inequality*. New York, NY: W.W. Norton & Company; Piketty, T. (2014) *Capital in the 21st Century*. Cambridge, MA & London, UK: The Belknap Press of Harvard University Press; Saez, E. & Zucman, G. (2014, October). Wealth Inequality in the United States since 1913. Retrieved March 14, 2016, from <http://gabriel-zucman.eu/files/SaezZucman2014Slides.pdf> with author’s permission; and Jesse Bricker, et al., “Changes in U.S. Family Finances from 2010 to 2013: Evidence from the Survey of Consumer Finances” (*Federal Reserve Bulletin* 100, no. 4, September 2014).

^{xviii} <https://www.cbo.gov/publication/50252> ‘Budgetary and Economic Effects of Repealing the Affordable Care Act,’ Congressional Budget Office, June 19, 2015.

^{xix} <https://www.nih.gov/about-nih/what-we-do/budget#note> See “Research for the People,” retrieved from NIH web site Nov. 24, 2016.

^{xx} https://report.nih.gov/categorical_spending.aspx “Estimates of Funding for Various Research, Condition, and Disease Categories,” table published Feb. 10, 2016, retrieved from NIH web site Nov. 24, 2016.

^{xxi} <http://data.worldbank.org/indicator/SH.XPD.TOTL.ZS?page=1> Health Expenditures as % of GDP, retrieved from the World Bank web site, Nov. 25, 2016.