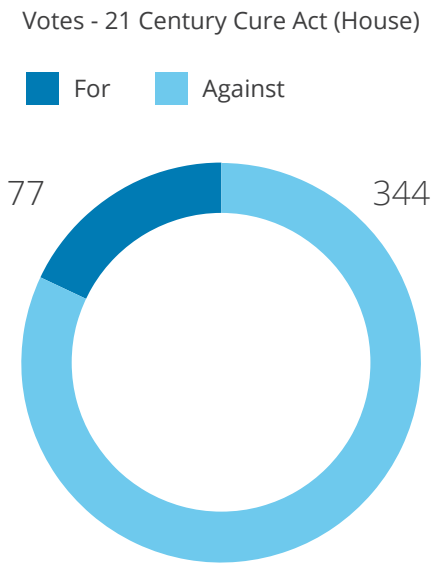
Precision Medicine, and the 21st Century Cures Act: 5 Scenarios on the Future of the Pharmaceutical Sector

You may have heard of the 21st Century Cures Act (H.R. 6) and its current draft. If not, now would be a good time to lay out its elements one by one. The central idea of the bill is to speed up the approval process for drugs and devices. As John Mack, marketing blogger and pharmaceutical marketing expert explains: "The act is a way for the pharmaceutical industry to get more drugs approved in a quicker and cheaper fashion". Mack who runs the blog pharma-mkting.com knows the pharmaceutical space well and has been analyzing US and global pharmaceutical affairs for many years. On July 10, 2015, the bill passed the house and has now been sent to the Senate. As the US

national health innovation pipeline is working under increased pressure the National Institutes of Health (NIH) is running on a historical funding low. In a campaign video Diana DeGette, a Democrat from Colorado who co-developed the draft, says that if laws don't keep up with the pace of innovation we will all lose out.

NIH's director Dr. Francis Collins has a great passion for precision medicine and when appearing at a round table at a medical school recently he said the funding situation at the NIH has driven the number of grants his organization is able to fund down by half and allowing it now to only approve 1 in 6 proposals (while the Guardian writes it could be as low as 5%, depending on institute). Now with this bill and its funding promise NIH's situation could dramatically change for the better. It would effectively be able to build a serious NIH innovation fund and would allow more research and technology projects to move into development. The act promises to provide an additional $2 billion per year for 5 years.



Graphic: Bill passed the House with 344 votes for and 77 against it

But there is more. The implication and background of the bill is slightly larger and more complicated that one might hope for. The New York Times writes that the bill aims at spurring biomedical innovation, and wants to change the way federal health officials approve drugs and medical devices. On the flipside, if the bill would finally pass as legislation it would instructs the administration to cover its costs by selling $8.75 billion worth of oil from the US strategic federal reserve, which "is not a piggy bank", as the New York Times comments cynically.

Precision Medicine and the 21st Century Cures Act

Precision medicine is a program announced by the Obama administration last January. With an already approved budget its official description says that it is an emerging approach for disease treatment and prevision which includes a patient's variability in their genes, environment, and a person's lifestyle.

What has the precision medicine initiative to do with the 21st Century Cures Act? Precision medicine would allow the inclusion of much more technology

driven healthcare innovation and would permit the introduction of additional personal data and information into health science. The bill mentions and defines precision medicine specifically again in the section "FDA Advancement of Precision Medicine" as an advanced analytical subset approach.

If you wonder how such diseases are identified, the bill mentions that this would occur via two possible options. The first is via the genotype, or genotype in combination with other biological characteristics. The second is via any other biological characteristic or means of identifying such a characteristic of the disease. Under this definition, the secretary should be allowed to update the definition to whatever is needed to meet the criteria of a precision medicine drug in the future and would also be allowed to work much closer with drug companies to meet the requirements.

"The term 'precision medicine' or 'precision drug' means a drug that, either alone or in combination with other therapies, targets a subset of individuals with a disease, which subset can be used to address the underlying cause of the disease in order to modify the progression of the disease, prevent the disease, or cure the disease".

Pharmaceutical Companies and the 21st Century Cures Act

One central questions remains. What would happen if the bill would really pass as a law and how would pharmaceutical companies respond? There are a few possible scenarios that could result from the content currently written in the discussion draft.

1. Increased Use of Biomarkers for Precision Medicine Drugs

The bill speaks specifically about allowing pharmaceutical companies to maximize the use of scientific tools or methods to incorporate biomarkers into non-clinical and clinical development of a precision drug in order to evaluate how such drugs modify the progression of diseases beyond well-established primary clinical endpoints (and identifying such surrogate endpoints). With this in place, pharmaceutical companies could determine the artificially calculated outcome endpoints without actually having to wait for the end of a trial. This saving in time could cause problems and John Mack is suspicious about it. He says the danger is that you might not see the final side effects, which may only come into force later after the patient has taken the drug for several months. As a result of this danger pharmaceutical companies may respond with solutions to increase the reliability and safety of such endpoint calculations. But how?

This is the billion dollar question. Monitoring software technology and personal mobile health applications for patients might be of great assistance in order to reduce the risk for trial patients.

2. Even More R&D Investments Towards Personalized Medicine

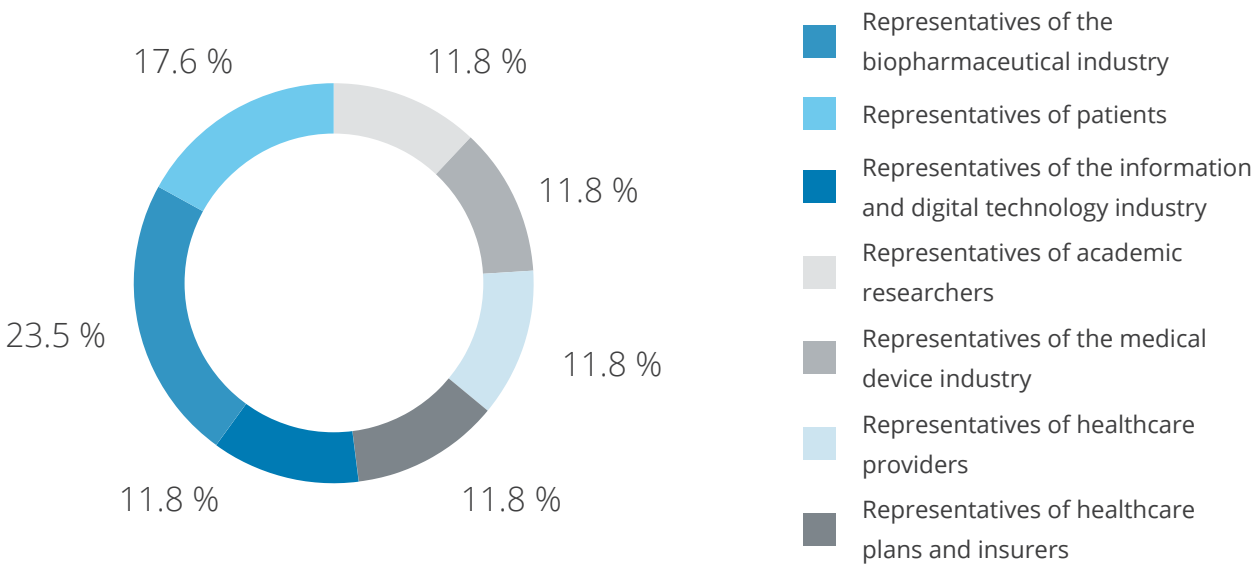
According to a report by Tufts Center for the Study of Drug Development, new research appeared that suggests the biopharmaceutical sector is making significant progress in the personalized medicine space. The problem for this sector is that it apparently still faces scientific, regulatory and reimbursement challenges regarding clinical adoption in its efforts to get targeted therapies approved.

The 21st Century Cures Act could help to remove these barriers. In the last 5 years, Biopharma companies have increased their research and development investments into personalized medicine by almost 100% according to a paper by Genoweb. If the Cures Act bill would really turn into law, the future investments could possibly even exceed the already expected increase of one-third for personalized medicine.

3. More Collaborations Between Digital Technology Industry and the Biopharma Sector

The draft requires the formation of a board of appointed members. Two of 17 members should be from the information and digital technology industry and four members are supposed to originate from the biopharmaceutical space. Although a bit of an unequal distribution the bill might drive collaboration between the technology and the biopharmaceutical sectors, as both sectors would now sit on the same table. In response to challenges around the data privacy debate for the bill, the technology sector might also bring their solutions to the table. Fierce Health IT wrote about unanswered privacy questions the bill causes. In particular, two sections in the bill would be of great concern. One is that it would make it easier for researchers to be able to access information to develop study protocols while making that process available electronically. The bill would also allow patients to give consent for their data to be used for future research, and not just for one study alone. The pharmaceutical sector and representatives from the technology industry, in collaboration with patient privacy advocates, would need to come up with a solution for how to respond to these question marks with clear and transparent strategies. The technology industry had to find solutions for similar challenges previously in other sectors such as banking and retail. The pharmaceutical and the research sectors might possibly be able to leverage knowhow gained by the tech sector by working closer together.

Appointed Members (21 Century Cure Act)



Graphic: 2 of the 17 members would represent the digital technology industry

4. More Health Initiatives with the Word "Precision" in the Title

Just recently, the US National Cancer Institute (NCI) launched the Precision Cancer Initiative and with it, its NCI-MATCH trial. It enables more information to be identified for particular cancers based on signals the research finds in molecular profiling from the trial. The initiative allows additional clinical trials to be undertaken for precision medicine drugs for oncology. The NCI writes on their website that oncology would be a natural choice as precision medicine would concentrate mainly on genes. Cancers would be a disease of the genome and a suitable first choice. Once the bill passes more precision medicine clinical trials could follow and with it more initiatives that use genome research for additional diseases.

5. Drug Development for Children with Rare Diseases

Lisa Gillespie writes in a KHN piece that advocates say more research needs to be conducted with children, that there is a shortage of good therapies for rare and often deadly pediatric diseases and that children would miss out on having access to pediatric specific drugs (particularly in the rare disease category). The bill would extend the national pediatric drug voucher program, Gillespie writes. On the flipside, it would do so only for an additional three years. This would not be enough for drug companies, which generally need 10 years or more to win investors over to grant research projects for

new drugs. After all, if the bill doesn't permit a longer extension for the pediatric drug voucher program the economic incentives may not be there to boost drug development for children with rare diseases. If this is understood correctly by policy makers they would do well to consider an amendment to this part of the bill at some point.

Pro and Cons of the Bill

The mentioning of precision medicine in the bill is far-reaching and could affect the pharmaceutical, research and healthcare hospital sectors significantly. With advocates for the bill on one side, such as Cresent Hardy, representative of Nevada's 4th Congressional District, there is also a determined set of people who have criticized the bill vehemently. One is Dr. Jerry Avorn, a professor at Harvard Medical School who calls the bill a Trojan horse. Hardy says it is a $12 billion saving opportunity.

Hardy writes that the bill would keep jobs in the US. It would also reduce the deficit by $500 million and would increase chances to find cures for the 7,000 rare diseases which he thinks currently lack economic incentives to be looked at.

In an interview published on the website of the New England Journal Dr. Avorn says that the pace of the drug approval process wasn't a problem in the first place. "For the FDA it takes between 6 and 10 months to take a completed dossier a company provides and to turn it into a commonly informative decision on a drug. There is not a problem with the speed in the drug approvals today", he says. But a whitepaper by the FDA says that the agency would be the fastest drug review agency in the world.

Avron is most concerned with the lowering of standards the bill suggests, and how approvals could be based on biomarkers instead of outcome, essentially opening possibilities for drug makers to disregard the current gold-standard for clinical trials. "It is not always the case that relying on biomarkers is going to be a good way to bring new drugs to market if they replace more conventional assessments of patients benefit", he says.

Besides the criticism one has to recognize that already early on in the development process the 21st Century Cures initiative managed to receive significant support by credible medical bodies such as the American Medical Association (which helped to outline reforms for the bill in areas such as tele- and personalized medicine, antibiotics development, EHRs and the protection of patient data).

Which parties would gain most in the end would only be clear after it had passed all legal stages. It's clear is that the bill could force more sectors to work closer together and that might be not a bad thing. The technology industry could become an important ally for the biopharmaceutical space and we would expect to see more collaboration between these two very different sectors if this bill is successfully implemented.

The Emergence of New Technologies If the Act Passes

Digital health is now JUST health and understanding bits and bytes is key to the technology organizations – but are they ready?

For the technology sector this act could be a real blessing. To the question of whether the US healthcare market would all of the sudden be swamped with new health technology applications if the act passes, John Mack explains that the bill already talks about telemedicine and that pharmaceutical companies would possibly gain a lot more confidence in developing, supporting and sponsoring these kinds of health applications. "I am not completely sure whether that is the kind of business the pharmaceutical companies would explore alone", he says in the interview.

In fact more pharmaceutical companies have started to consider working with technology companies. A PWC report also mentions that access to patient data will raise the stakes of the game and that the convergence of technology and market forces in healthcare will make patient data a powerful factor in the cost/benefit equation. If US pharmaceutical companies would really want to venture out on their own they would need to understand every bit and bite of the technology they want to use, how its data can be protected and the impact on patients. We are particularly likely to see a response from pharmaceutical companies in terms of data protection. As health data breaches increase - some surveys suggest that nearly half of all breaches took place in healthcare in 2014 – the pharmaceutical companies would really need to find ways to protect data effectively and they likely won't come up with a solution all by themselves.

Personalized in "personalized medicine" means more security and regulation

Further Mack says that there are lots of other technological companies already out there, including Apple and Google, developing apps that connect to devices with a healthcare purpose. "These companies don't want to go through the expense to get FDA approval and the act is mainly focused on helping them not to have to go the

difficult way of navigating through usual FDA regulations", he says. With the act and these new applications, personal medicine would emerge. Now, not only Apple and IBM are playing this game but also pharmaceutical giants such as Merck are in for the win. Back in 2012 the company was a leading investor in health technology startups according to the US health startup accelerator RockHealth and the act could cause more investments to flow into seed level health technology projects.

With more personalized medicine on the US market would this mean more regulations too? The FDA defines personalized medicine as precision medicine and a way to tailor medical treatments to the individual characteristics. If the bill would now allow more health technologies in personal medicine the act would trigger a whole lot of new regulations. In fact a whole chapter in the H.R.6 text is dedicated to ruling the sensible oversight for technology, which advances regulatory efficiency.

Big data will only get bigger – are you ready pharma?

Finally, what about big data you may ask. Big data is another great reason for pharmaceutical companies to seek support and collaboration with the technology sector if the bill happens to pass. The big challenge is no longer just to sequence a human's genome. Instead the trouble comes from organizing the data value chain effectively and store the data. Technology companies such as Tute Genomics create cloud-based solutions for precision genome-guided medicine. How to get such information analyzed as quickly as possible and then stored, protected and connected to outcome? It's the billion-dollar question.

For genomic information alone, even at the moment medical research faces major problems in doing it on a small scale and working with healthcare providers effectively. Now imagine what could happen if every trial patient would be connected and monitored in real time. The data volume would explode, and the act could help to do exactly that.

Pharmaceutical companies such as Roche already work in other parts of the world with mobile tech and personal monitoring technologies. The company's latest announcement revealed that it intends to start using a Samsung Galaxy S3 Mini smartphones during a phase I drug trial to track Parkinson's disease patients' symptoms. If the act would pass, we are likely to see a lot more similar trials in the US.

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USA HQ
Toll Free: 866-687-3588
Tel: +1-512-516-8880

Ukraine HQ
Tel: +380-32-240-9090

Bulgaria
Tel: +359-2-902-3760

Germany
Tel: +49-69-2602-5857

Netherlands
Tel: +31-20-262-33-23

Poland
Tel: +48-71-382-2800

Sweden
Tel: +46-844-685-084

UK
Tel: +44-207-544-8414

EMAIL
info@softserveinc.com

WEBSITE:
www.softserveinc.com