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Can we afford the costs of orphan disease treatments?

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At Risk for Huntington's Disease

HD is a genetically caused brain disorder that causes uncontrollable bodily movements and robs people's ability to walk, talk, eat, and think. The final result is a slow, ugly death. Children of parents with HD have a 50-50 chance of inheriting the disease. There is no cure or treatment.

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Can we afford the costs of orphan disease treatments?

Millions of people in America suffer from rare, or "orphan," diseases, conditions defined by the government as affecting fewer than 200,000 people. With an estimated 30,000 affected individuals, Huntington's disease is one of the more common of these disorders.

The pharmaceutical industry has largely ignored these diseases, which number several thousand, because each disease promises too few customers/patients to enable companies to recoup investments in drug research and development and therefore generate a profit. The market usually doesn't work for people with these diseases.

News about a lawsuit by Arkansas cystic fibrosis (CF) patients against the state's Medicaid program for its refusal to pay for a highly effective but extremely expensive drug – Vertex Pharmaceutical's Kalydeco – shined light on this predicament.

In an article titled "The $300,000 Drug," *New York Times* columnist Joe Nocera recognized Kalydeco as a "wonder drug" but questioned whether the country can afford the personalized medicine approach that enables scientists to design specialized treatments for very small and specific groups of patients.

With an annual wholesale cost of $311,000, Kalydeco was developed for a subgroup of about 1,100 CF patients with specific genetic mutations. The subgroup numbers about 2,150 patients worldwide in an overall CF population of 70,000 individuals.

"Because patients will likely be taking the drug for the rest of their lives, it could cost millions of dollars to keep just one patient on Kalydeco," Nocera speculated. "That raises another important question about the coming of personalized medicine. How are we, as a society, going to pay for it?"

Same question for the HD community

The HD community could face this very same question. Because the U.S. has only 30,000 HD patients and 150,000 to 250,000 people at risk of carrying the gene, a potential treatment could cost a lot.

Boston-headquartered Vertex has sought to develop HD treatments since mid-2008. Though the company has made a substantial effort, it doesn't yet have plans for a clinical trial. (Click here to read more.) Isis Pharmaceuticals, Inc., of Carlsbad, CA, has also worked about as long and is planning to launch a clinical trial in the next year or two.

It's still too early to project the costs of treatments that have yet to be tested or even fully designed. Other potential remedies are in trials but at best likely remain years from reaching the market.

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Furthermore, an HD treatment regimen will likely involve a cocktail of remedies, meaning that patients – via their insurers – will probably have to pay for more than one drug.

Vertex vice president of research Paul Negulescu (left), Gene Veritas (aka Kenneth P. Serbin), and Vertex vice president of biology Beth Hoffman at the company's San Diego facility, September 2010 (photo by Heather Farr, Vertex)

Patient assistance programs

The HD community must remain vigilant regarding the cost of potential treatments. However, failing to consider a number of factors, the coverage of the Kalydeco costs was perhaps too pessimistic about the future.

First, as I commented regarding the impatience with California's stem cell institute after ten years of operation without a drug, biomedical research is slow by nature. And it's expensive, with the average cost of developing a new drug in the U.S. at $1.2 billion. Only one in ten clinical trials results in a marketable drug, although the research from the unsuccessful projects provides highly valuable information on what does *not* work.

In the case of CF, Vertex is at work on another treatment that would reach thousands more patients with different kinds of mutations.

As Nocera himself noted, Vertex provides Kalydeco for free to patients without insurance.

Lundbeck, the pharmaceutical firm that markets Xenazine, which diminishes some of the involuntary movements caused by HD (chorea), provides financial assistance to patients who qualify. Depending on the dosage, the annual wholesale cost of this treatment can reach $50,000 or more, but, according to the Lundbeck website, "85 percent of U.S. patients taking Xenazine have a monthly co-pay of $50 or less before requesting co-pay assistance."

It's highly conceivable that the developers of future HD treatments will provide similar kinds of assistance – especially because these firms will have relied on the good will and extensive cooperation of HD families who participate in research studies and clinical trials. However, it's not clear what the drug companies will charge insurers.

CHDI and pharma giants

After the founding in 2003 of the CHDI Foundation, Inc., a non-profit virtual biotech firm backed by wealthy donors who wish to remain anonymous, pharmaceutical firms small and large started to gain interest in developing Huntington's treatments.

As a result, the network of firms working on HD now includes pharmaceutical giants such as Pfizer, Roche, and Medtronic.

As a non-profit with the sole purpose of finding HD treatments, CHDI promotes research on Huntington's and the diffusion of scientific knowledge about the disease. With more researchers and firms involved, the chances for treatments have grown. Having more options could very well mean that treatments would cost less.

By pouring hundreds of millions of dollars into HD drug research, CHDI has created an incentive to produce cheaper drugs.

As it states on its website, CHDI seeks to connect academic research, drug discovery, and clinical development in order to avoid "costly delays to therapeutic development" and make potential treatments a "good investment" that will result in "full clinical development, including licensure and marketing to get drugs to HD patients."

Similarly, the Hereditary Disease Foundation and the Huntington's Disease Society of America (HDSA) have supported research that could yield yet additional drugs.

Patient-driven medicine

Thanks to this level of support for HD research, the HD community stands in perhaps a better position than those facing even more rare diseases.

Nevertheless, orphan disease communities in general have reason to feel optimistic about both the development of treatments and their cost, if the vision of one key medical leader becomes reality.

Lee Hood, M.D., Ph.D., one of the scientific giants behind the Genome Project and the recipient in 1987 of the Lasker Basic Medical Research Award (the American equivalent of the Nobel Prize), has developed a plan for more effective and affordable medicine. In 2000, Dr. Hood founded the Institute for Systems Biology (ISB). Located in Seattle, the non-profit ISB teams scientists and technologists from many disciplines to pioneer the future of research in biology, biotechnology, medicine, environmental science, and science education.

In a 2012 speech at the Seventh Annual HD Therapeutics Conference, sponsored by CHDI, Dr. Hood outlined the importance of systems biology – what I think of as the "big picture" of disease – for HD research. Dr. Hood also advocated for the adoption of P4 medicine: predictive, preventive, personalized, and participatory. (Click here to read more.)

"Patients and consumers will be a major driver in the realization of P4 medicine through their participation in medically oriented social networks directed at improving their own healthcare," Dr. Hood and Mauricio Flores, J.D., wrote in the March 2012 issue of the journal New Biotechnology.

ISB and several collaborating organizations have run some pilot programs in P4. If it is implemented on a wide scale, Dr. Hood predicts that it will revolutionize our healthcare system. Everybody will carry a health-monitoring device, and diseases will be predicted and prevented long before onset as the result of tiny blood samples taken from a pin prick, the article states.

Predicting falling medical costs

Significantly, costs could plummet.

"P4 medicine will require that all healthcare companies rewrite their business plans in the next 10 years or so," Dr. Hood and Flores wrote. "Many will not be able to do so and will become 'industrial dinosaurs.' There will be enormous economic opportunities for the emergence of new companies tailored to the needs and opportunities of P4 medicine."

The authors projected that savings will result from a series of factors, including earlier and more effective diagnosis of disease; better matching of drugs with diseases and their subtypes; better identification of genetically based adverse reactions to drugs; the ability to "re-engineer" disease-affected biological networks within people in order to reduce the cost of drug development; an increasing ability to deal effectively with cancer; the use of stem cells for replacement therapy and diagnostics; the routine extension of effective mental and physical health into people's 80s and 90s; an improved understanding of microbes in the body; a deeper understanding of neurodegeneration (the cause of HD, Alzheimer's, Parkinson's, and other disorders); and the digitalization of medical and genetic information.

"On another tact, our prediction is that there will be a 'wellness industry' that will emerge over the next 10-15 years that will in time far exceed the size of the healthcare industry," Dr. Hood and Flores affirmed. "P4 medicine is an area replete with economic opportunities."

Dr. Hood and Flores believe that P4 medicine will "democratize" healthcare.

"The patient (consumer), through social networks, will drive the emergence of P4 medicine," they wrote. "Because of intrinsic conservatism and sclerotic bureaucratic systems, physicians, healthcare specialists and the healthcare industry will take a back seat to the power of patient-driven social networks in bringing change to the healthcare system. Indeed, patients may be the only driving force capable of truly changing our contemporary healthcare system to the proactive P4 mode."

This scenario serves as a serious alternative to the dim view that orphan disease communities will remain relegated to high-cost solutions.

Guaranteeing proper care standards

Indeed, a "revolution" has occurred over the past two decades in how patients have related to their doctors and the pharmaceutical industry (click here to read more).

Nowadays, people enter the healthcare system as both patients *and* advocates for their well-being.

This outlook led the Arkansas patients to sue for the right to have their Kalydeco costs covered.

Their lawsuit offers a striking similarity with the HD community's pressure on the Social Security Administration and Congress to update the decades-old, inaccurate government criteria for determining disability benefits for Huntington's patients (click here to read more). The Arkansas plaintiffs in effect have demanded that the state recognize Kalydeco as the standard treatment for their type of CF.

Negotiating the price

The competition of the marketplace, greater efficiency in drug development, and the revolution in medicine outlined by Dr. Hood should

put downward pressure on the cost of drugs.

Patient advocates must play a crucial role in this process.

As the late San Diego biotech leader Duane Roth had told me during a dinner with California stem cell leaders in 2008, patient advocates must find ways to appeal to pharmaceutical companies' primary interest in profits. Advocates need to lobby and court these business leaders.

At the same time, disease organizations such as HDSA and its network of advocates can pressure pharmaceutical companies and government agencies to assure new drugs' accessibility and affordability.

In some circumstances, government can join in the process of persuasion and even play hardball, as the Brazilian Ministry of Health did in the 1990s in order to convince multinational pharmaceutical firms to dramatically reduce the price of HIV/AIDS medications. The Brazilian government provides HIV/AIDS drugs for free.

"Local production of generics, the possibility of breaking patents, and the offer of technology transfer became instruments for price negotiations with other countries and the pharmaceutical industry, leading to a real reduction in prices on the Brazilian and international markets," wrote the coordinator of the country's National STD/AIDS Program.

The marketplace exists, but it is susceptible to politics.

The rhetoric about the $300,000 drug can scare a lot of people. But in the long run, such a cost is not a foregone conclusion.

Posted by Gene Veritas at 12:11 AM

Labels: advocacy , AIDS , CHDI , clinical trials , cystic fibrosis , drug , HDSA , Huntington's disease , Isis , Kalydeco , Lundbeck , orphan disease , P4 medicine , pharmaceutical industry , systems biology , treatments , Vertex , Xenazine

1 comment:

Nancy Liccione said...

I post this on my son's birthday. He has Huntington's Disease which he inherited from me. I will mention how devastating the dual diagnosis of us both was beyond description of the degree of agony it caused. This leaves two adult siblings also facing the extremely real and horrible possibility of also testing positive.How do you describe the level of fear and agony? There is so much talk about how much it will cost over a lifetime of the patient to treat them . This is only if a cure was available. There is very much talk about what it costs for research to even attempt to find the cure. There is much talk about how the money for research for a cure is often spent unwisely. I fully agree with the approach of P 4 medicine...PREDICTIVE, PREVENTIVE,, PERSONALIZED, and PARTICIPATORY medical approach to this disease. It must be fought against with compassion for it's victims and extreme commitment on every level!!! P 4 is a great start , but the greed of those who see an opportunity to make money at the expense of the patients by holding up research ,MUST STOP!!!I am suggesting PARTICIPATION in every way possible. I am also throwing down a challenge. You have my willingness to participate,

ABSOLUTELY. The problem is how do you connect the suffering patient(and family) with personalized medicine and participation?

11:46 PM, July 22, 2014

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