Precision Medicine: Beginning of the End of Orphan Drugs

In [Regulation & Government](http://www.orphan-drugs.org/category/regulation-government/) by Chris HackettJanuary 29, 2015[Leave a Comment](http://www.orphan-drugs.org/2015/01/29/precision-medicine-orphan/#comments)



If every disease is a rare disease, none of them are.

It’s the unspoken elephant in the room as the U.S. and the world makes headway into the long-sought advance of precision medicine.

The Orphan Drug industry exists as it does today on the back of old science: some diseases happen to occur very, very infrequently. Every other disease is, of course, by the very definition, “common”. For these “common” diseases, people should just be able to take one treatment developed by one lab to eliminate it, or at the very least mask the symptoms. The “blockbuster drugs” of old.

Science has moved on. And now that Obama has made it clear he plans to at least begin the discussion on precision (personalized) medicine, industry will have no choice but to begin preparing to move on as well.

2014 happened to be a great year for rare diseases, and pharma, as record numbers of new drugs were approved and a large number of them were for orphan designations. It doesn’t look like this is going to die down anytime soon, either, [if financial predictions are to be believed](http://www.orphan-drugs.org/2014/11/26/orphan-drugs-set-worth-176-billion-2020/). Those rare diseases are now easy money for the pharma companies – often, the patients are more than willing to pay the hefty price in order to improve their way of life. All good things, however, must come to an end someday. But that doesn’t necessarily mean they need to be replaced with something worse.

By its definition, precision medicine, which The President highlighted in his State of the Union, is the antithesis of the aforementioned discrepancy between “common” and “rare”. Once people begin to look at diseases – specifically, genetic diseases – not as a foreign threat to the body but as an internal error within it, every individual becomes their own patient population.

**EVERY MAN IS AN ISLAND**

Obama’s remarks were important, but far more important is the implication it has for thinking in general. The average person still thinks of medicine as a treatment for symptoms rather than for a cause. Therein lies the paradigm shift:  people’s *symptoms*may be the same. But the cause can be entirely different.

That is because each of us is, well, different. As cliche as it might be, our genetic codes truly makes each of us our own island. Our genome, proteome, metabolome, and eventually every single feature on our body are as unique as our fingerprints. We as a culture enjoy embracing our differences culturally. It is time we brought that to the next level – we have to embrace our differences biologically if we are ever to develop medicines that truly target the things that make us sick.

To many, that statement sounds bad: “We are all different on a genetic level and must be treated differently.” It has been ingrained to us since we were children in our liberal-minded education system that treating people differently based on genetics is wrong. But that is counter-intuitive in science. When it comes to medicine, treating people differently based on biology is how we are going to save more lives.

**ORPHANIZATION AND THE PRICE WARS**

A long time ago in a country far, far away, the Orphan Drug act was passed to spur development in “rare” diseases, which we just established is based on an old methodology of thinking. The high priced drugs that the act has spurred have brought out the ire of those people who see them as egregious, even leading to a [debate](http://www.orphan-drugs.org/2014/09/19/orphan-drug-industry-sustainable/) on the subject at this years WODC 2015. This includes the payers, who are concerned about the proliferation of these drugs.

Obviously, this has made orphan drugs a profitable business model. Companies are nearly falling over themselves trying to develop the next drug for a rare indication, and some larger diseases such as cancer, are being “orphanized”. (Which, [as I have mentioned previously](http://www.orphan-drugs.org/2015/01/21/obamas-precision-medicine-initiative-means-orphan-drugs/), is not in and of itself a bad thing.)

However, this is where things get tricky. In their haste to capitalize on this viable business model and expensive drugs, pharma is in a way destroying it. Pharma will mention the benefits of “orphanization” in that this is the scientific method of finding new drugs, and they are right. Every time they reduce the patient population it allows a smaller subset of the gene pool to be treated more “precisely”. Continuing this into its natural progression, the optimal situation would be that every patient is their own gene pool from which a drug is developed. Which leads me to precision medicine.

**PRECISION MEDICINE AND THE END OF ORPHAN DRUGS**

Obama touched on precision medicine in the [SOTU](http://www.cnn.com/2015/01/20/politics/state-of-the-union-2015-transcript-full-text/), but it was around long before he brought it up. Now, the government will be focusing on it too. This is the same government that created the orphan drug industry in the States with the ODA, so we already know what the power of incentives can do.

In a prior article I mentioned that the government emphasizing precision medicine means that payers will have to accept that high drug prices are here to stay. But there is an alternative – if Obama’s initiatives put certain things into motion, it could very well mean that the ODA, the very iron from which this industry has been forged, will become redundant – or worse, entirely irrelevant. As I mentioned previously, the ODA existed in the “common/uncommon” world. Obama has indicated that his government is moving us into the “individualized” world. Like certain Amendments in the Constitution, the ODA is going to rapidly become a vestigial limb of an otherwise potently evolved system.

Even if you take a step backwards and acknowledge that we may never get to individualized medicine, and that we will only have medications based on certain genotypes or particular SNPs that may be more widespread than one person, we are still headed in a direction where every medication will fall below the 200,000 threshold. Reducing that threshold will slow the decline of the terminology, but will not eliminate it. By the time that happens, it will be inevitable that every drug pharma is researching will be trending in this direction. If every drug has an incentive – well, that defeats the entire point of it in the first place.

**HOW TO PREPARE**

This is (hopefully) coming, and we can all rejoice when cancer is cured because of it. The best thing Pharma can do to prepare is to keep doing exactly what they are doing – whether they mean to or not, their recent focuses are driving the drug industry in this direction, and to back away now would not only do them a disservice but also the people who need these new age medicines. Oh, and don’t forget to come to industry events like the World Orphan Drug Congress USA to keep your finger on the pulse of where we are headed.

As for payers, look at it from the lens of making the overall market cheaper. Once this is the norm, preventive medicine and diagnostics will be so potent that it may negate the need for expensive treatments at all. It’s always darkest before the dawn.

For the consumer at large, sit back and enjoy the ride. We are living in a great time for medicine, and it can only get better from here. We are developing more medicines than ever before, the key will be getting them into the hands of the patients without breaking the bank as they become more common. We are living in a great time for drug research.

Antibiotics on the other hand…well, that is a story for a different day.