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Pharma's Paradox: Cure A Deadly Childhood Disease And Then Get Attacked On Price

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It's a diagnosis that no parents want to hear - their child has acute lymphoblastic leukemia (ALL). While a treatable cancer of the bone marrow and blood, traditional drug treatment regimens can be brutal and not always successful. But, in August the FDA announced the approval of a new gene therapy to treat ALL. Known as Kymriah (tisagenlecleucel), this Novartis product is the [first gene therapy approved](#) in the U.S., thereby opening a new era in medicine.



SEBASTIEN BOZON/AFP/Getty Images)

The [value of Kymriah](#) has been wonderfully captured by Dr. Lisa Rosenbaum in the *New England Journal of Medicine*, where she outlines the development of CAR-T technology – the science on which Kymriah is based. Dr. Rosenbaum, however, does more than talk about the underlying science. She puts this research into context with the story of Emily Whitehead who, at the age of five, was diagnosed with ALL. Emily

was put through the rigors of multiple rounds of chemotherapy to no avail. Emily's physicians told her parents to consider hospice, but instead, they entered her in a clinical trial for Novartis' Kymriah. The procedure worked and Emily is now a thriving 12 year-old.

Kymriah is not a pill. CAR-T therapy is based on removing T-cells from a child's blood, genetically modifying them to respond to specific targets on the child's cancer cells, and then reinfusing these modified cells. These new T-cells then seek out and destroy one's cancer cells. The results to date have been pretty striking. Of the 63 evaluable children who received Kymriah, 83% had complete elimination of malignant cells at three months.

However, Rosenbaum is pretty balanced in her appraisal of the work to date with Kymriah. CAR-T therapy can cause a severe side-effect known as cytokine release syndrome and so this treatment needs to be carried out at appropriate medical institutions that are prepared to handle not just cell reinfusions but also any subsequent issues.

But the launch of curative gene based therapies has brought along another concern – drug pricing. How much is such a treatment worth? For Emily Whitehead's parents, Kymriah's value is priceless. It saved their daughter. But not all share that view.

As Kymriah neared FDA approval, many analysts speculated that [Novartis would charge](#) anywhere between \$600,000 - \$750,000. Instead, Novartis set the price at \$475,000. Furthermore, Novartis took the stance that payment wouldn't be made unless the patients had responded to Kymriah at the end of the first month after treatment. Nevertheless, critics assailed Novartis for this price. Not surprisingly, payers were first to weigh in with outspoken biopharma critic Steve Miller claiming that this [price is "dramatically higher](#) than other specialty drugs." Actually, this statement is not true. There are drugs that cost more than twice Kymriah. Kymriah barely makes [the top ten](#). But Miller's posturing is to be expected. After all, Express Scripts and other payers are facing the approval of other gene therapies in the coming years.

It has been disappointing, however, to see the views of certain physicians on this topic. In a recent "Viewpoint" in the *Journal of the American Medical Association*, Drs. Peter Bach, Sergio Giralt and Leonard Saltz of Memorial Sloan Kettering Cancer Center (MSKCC) [lash out at Novartis](#) at its price for Kymriah. The MSKCC group has done some nice work on the topic of pricing in the past, particularly in getting Sanofi/Regeneron to [bring down the price of the colon cancer](#) drug, Zaltrap. Unfortunately, their stance on Kymriah, unlike Dr. Rosenbaum's analysis, focuses way more on pricing, the side-effects of CAR-T therapy and on the "hype" surrounding this technology. Those patients whose lives have been saved by Kymriah will have little to relate to in the MSKCC essay.

So, how do we deal with the pricing of cures? What value do we put on life? Taking a child with ALL and truly enabling her to lead a full and normal life adds a great deal of value to society. Dr. Bach has said that “Every other country uses a formal technology assessment to figure out how much to pay for drugs – we don’t. And this is the only sector where **the monopolist sets the price.**” He’s right. But if you look at how other countries set drug prices, \$475,000 looks pretty good. That’s because countries use “Quality-Adjusted Life Years” (QALYs) to help in guiding the price of a new drug. The U.K., for example, pegs a QALY at about \$50,000. So, using that figure, one would argue that a drug that restores life to a child could be worth \$50,000/year for the rest of that patient’s life. Such a figure would bankrupt the healthcare system. But, the Novartis Kymriah price really accounts for less than 10 years of a productive life if measured this way. That doesn’t sound so unreasonable.

Critics do have a valid concern over payment models. It seems reasonable that drug makers could arrange a payment system whereby a company is paid gradually over a 5 year period. That would ease the immediate burden. However, executing against such a plan would not be simple. For example, would ALL be considered a pre-existing condition and thus not covered should a patient change insurance providers? What happens when a patent expires during the payment period – would the drug maker be out of luck? These and other issues will need to be worked out.

What is most disappointing in all of these discussions about price is the fact that these gene therapies are major medical breakthroughs. Regardless of what critics say, these ARE CURES for the many who benefit. Just ask Emily Whitehead and her parents. Indeed, let’s begin to figure out how best to reward the innovators who are restoring life to patients. But let’s not denigrate the biopharmaceutical industry in doing so.