Building a Cure Machine

Sometimes in life an event occurs that is so transforming it forces us to take inventory of everything we know or believe to be true. It makes us reframe our priorities and redefine our purpose. It tests what we are made of in mind and body and soul. But through the pain, such an event can also provide us an opportunity to find the best part of ourselves—and apply it for good.

Just over a decade ago, my daughter was stricken out of the blue by a disease so rare that none of her doctors even knew its name, let alone its severity. Neuromyelitis optica. Even more frightening, there was no approved therapy and no hope for a cure. I was forced to make a choice between two roads—each of which could test my limits in every way. One path was to simply accept that fate had dealt my 14 year-old daughter a death card that would soon take her away from her mother and all who loved her. I would never walk down that road. The other path would be to transform myself and build a cure machine for my daughter—but much bigger than just the two of us—to make its blueprint adaptable as a model to solve other diseases as well.

Most ideas that end up changing the world for the better begin with one person who refuses to take no for an answer. It takes someone who is relentlessly focused on finding a new way. Part desperation and part determination, such breakthroughs often emerge from life-or-death circumstances. Of course the brains and brawn of cutting-edge researchers would be needed to build this engine for cures. But in this life-or-death challenge, something even more daunting loomed ahead on this life-saving road: no genius or eureka or wish or will would mean anything unless pharmaceutical companies would commit to turn molecules into medicines that are safe and effective.

Generating and turning big data into real solutions takes money—and cultivating pharma takes savvy. Convincing a drug company to invest in clinical trials that often exceed costs of the science by 10- to 50-fold is no simple business proposition. And if true in even the most marquis of diseases such as COVID-19 or cancer, imagine how difficult it would be in a rare disease that at the time was thought to afflict 1 in a million.

Academic researchers, biotech start-ups and pharmaceutical giants usually exist in series. Researchers often make discoveries, but are not experts in drug development. Biotech start-ups separate the science into go versus no-go drug candidates, but are not experts in clinical trials, large-scale manufacturing or marketing. Big pharma is capable of manufacturing and testing new drugs in the real-world crucible of FDA review, but is risk averse and not best in reconnecting to patients who will benefit from the very drugs they produce. There was something missing in this life-saving continuum, and it was all the more apparent through the lens of a rare disease. To build our cure machine—we needed to reinvent this baton-pass series that could take decades and end in delayed failure—into a streamlined accelerator where failures are ruled out early and key advances beget further successes. We needed to crosswire traditional research with business savvy—and we needed to do this at the speed of life. And that is exactly what we did through The Guthy-Jackson Charitable Foundation.

Usually research that leads to medicine takes a path of twists and turns that is as long as it is anguishing. When my daughter was diagnosed, neuromyelitis optica (NMO) had been known for over 100 years. And for over 100 years there was no formal clinical trial or therapy proven safe and effective in preventing catastrophic relapses. That all changed in just one decade. We brought experts together—within and beyond medicine— who would never have done so otherwise—because finding cures is as much about solving problems as it is medical intellect. We aligned small but mighty biotech with rigid but experienced pharma. The results were phenomenal—and most surprising to those who said it could never be done. Four international clinical trials were conducted in just five short years. Today, there are three FDA-approved drugs each of which confers 90% relapse-free remission. These are miraculous leaps that are saving & improving lives. But they did not come to be without blood, sweat and tears—and there is more to do to cure NMO once and for all.

Necessity is the mother of invention—and a mother is a necessity to a child. My daughter helped me re-invent myself to help her. As the mother of a rare disease patient and a global cosmetics firm, I had a once in a lifetime chance to help save countless lives. My heart told me how to help my daughter—my head told me how to get pharma to invest hundreds of millions of dollars to create the first-ever treatments for a rare disease. A diagnosis of NMO was a transforming event that made me reframe my priorities and redefine my purpose—from mascara to medicine. Through our Foundation, we built a cure machine, proved it works and then shared it through The Power of Rare: A Blueprint for a Medical Revolution to help solve other diseases— rare and not so rare. Like my daughter and I, academia and biotech and pharma are stronger together than apart. Whether NMO or COVID-19 or cancer or beyond, sometimes events in life make us find the best part of ourselves—and apply it for good.