

Autism Research Should Be Financed Like Venture Capital

By John Rodakis

We still don't know what autism is, despite decades of research and billions of dollars spent. We don't know what causes it or how to treat it. This lack of progress is partly the result of structural deficiencies in how autism research is funded. Fortunately, lessons from financial markets and the venture-capital industry can help solve these problems and accelerate the pace of discovery—for autism and perhaps other medical conditions.

Consider recent research by Robert Naviaux, a professor at the University of California, San Diego. Earlier this year he announced results from a clinical trial involving 10 boys with autism. Half were given the drug suramin and showed significantly improved language and social behavior. The study lends further support to Dr. Naviaux's theory that a treatable metabolic condition may underlie autism. This promising lead is welcome news, but it reinforces my view that the scientific understanding of autism is years, possibly decades, behind where it would be if the handful of groups that control virtually all funding for autism research had taken a more-diversified approach. In finance, markets that are deep—made up of many investors with varying opinions—are more efficient and better at price discovery. Similarly in science, many “investors” funding multiple approaches in parallel should lead to more-rapid advances. Therein lies the problem. In the U.S. just three organizations control 99% of all funding for biomedical research on autism: the federal government (primarily the National Institutes of Health); Autism Speaks (which does commendable work raising awareness); and a large foundation funded by a family. Everyone else collectively makes up less than 1% of funding.

These three organizations almost exclusively support research that aligns with the conventional view of autism as primarily a genetic disorder of brain wiring. The problem is that this “genetics-first” paradigm does not fit the emerging research, including Dr. Naviaux's, and has failed to produce answers. Research that does not fit neatly within this view—or that dares to contradict it—has little chance of being funded.

Case in point: None of the three organizations have supported Dr. Naviaux's recent research or the clinical trial, even after he successfully reversed autism-like behaviors in multiple mouse models. Thankfully, a grassroots effort by parents and small nonprofits, including the one I run, was able to supply most of the funding. For the rest, Dr. Naviaux went into debt.

A similar story is what led me to start a nonprofit in 2014. While trying to understand my son's unexpected improvement in autism symptoms while taking a common antibiotic, I was surprised to discover results from a clinical trial published 15 years before. In that study, 8 of 10 boys with severe autism showed significant improvements while taking the antibiotic vancomycin. I met with the researchers years later to find out why they had not followed up on this novel, intriguing finding. They all said the same thing: They could not get funding because their results did not fit the established paradigm.

Portfolio theory teaches that diversification reduces risk, but there is little diversification in autism research funding. In finance, the risk is of capital loss or increased volatility; in autism the risk is a continued epidemic robbing children of their childhood and the prospect of an independent life. Beyond the personal toll, the economic costs of autism in the U.S. have been estimated at between \$300 billion and \$500 billion a year. Our lack of answers has a high price tag.

As Dr. Naviaux's recent success shows, radical ideas have great value in science, but to be proven, they need to get funded. At the NIH, grant proposals are scored by small committees of *prior grant recipients*, a system that virtually enshrines the status quo. Incrementalism, at best, is the result. Playing it safe almost never produces breakthroughs. I'm sure the people on those committees would like to see progress as much as I would. The problem is not intent, but structure.

What we need is for the "market" that allocates capital to medical research to more closely resemble the risk-taking financial and venture-capital markets. Researchers should be rewarded for stretching beyond conventional views in search of breakthroughs. The obvious need is for more funders with adequate capital and diverse views. This could be fostered by formally combining the power of the venture-capital model with the passion of the medical nonprofit, but that will take time.

Meanwhile, some stopgaps may help. To ensure that the NIH and other government agencies diversify their autism research efforts, I propose a hard cap—say, no more than 25%—on how much of their grants can go to genetics-related studies, thus mandating diversification.

Another approach would be to start something akin to what Israel's Directorate of Military Intelligence created to counter groupthink: an office of "devil's advocate," staffed by analysts whose job is to identify and challenge conventional points of view. At the NIH this group could fund studies that run counter to the prevailing paradigm.

This is the approach that the nonprofit I founded, N of One: Autism Research Foundation, takes by committing its limited funds to small studies that buck the conventional view in the hopes of seeding a breakthrough. In finance, we call it seed venture capital. It's time we apply the lessons and approaches of a system that works to one that has not.

Mr. Rodakis is founder and president of the nonprofit N of One: Autism Research Foundation, which supported Dr. Naviaux's suramin study.

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