

VIEWPOINT

# Scientists must curb tendency to try untested treatments

BY MATT CAREY

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A large segment of the autism community is interested in research. As a parent of a child with autism, I pay attention to autism research for the simple reason that I'd like to see a better life for people with autism. Whether it is for finding residential placements for adults or improving

**diagnosis in underserved communities**, research can provide information to help a community make decisions, and scientific papers can provide the support needed to advocate for change.

Consider behavior-based therapies, for example. In the 1980s, research support for these therapies fueled efforts to get schools to provide them and, later, for insurance companies to pay for them.

Pharmacological approaches have been less successful. Drug treatments for the core features of autism are, at best, in early-stage clinical trials, although basic research findings have provided leads for treatments. And yet press releases and news articles with quotes from enthusiastic researchers often trumpet the promise of these early-stage treatments.

In some sense, enthusiasm is a good thing. It tells us, the consumers, that researchers are in this to make an impact on the lives of people with autism. But researchers must also be aware that their research and positive words can be misinterpreted — and they **need to try to prevent their misuse**.

The problem lies in the fact that fundamental autism research is an investment in the future, but understandably, consumers would like to see solutions now. And there is a large community of doctors, among others, willing to tell parents that the latest preliminary finding means that we can start treatments now. Doctors may turn to these unproven approaches because of a lack of mainstream ones or because they already tend toward alternative treatments.

## Radical remedies:

Sometimes a doctor might recommend starting off-label use of a drug candidate. In other cases, a clinician might prescribe a treatment regimen that bears little or no resemblance to the one used in research. I remember seeking advice from a nutritionist about a common concern: my child's very limited diet. Although the nutritionist hadn't seen, much less tested, my child, she told me during the consultation that my child was likely suffering from heavy-metal intoxication and I should **consider chelation**, a potentially dangerous approach.

Parents might seek alternative practitioners to help them implement an approach they saw in a news report. Or a parent might attend one of the autism parent conventions, such as held annually in Chicago, in which practitioners pitch alternative approaches in presentations styled as scientific talks. In a research conference, other researchers often challenge their colleagues on questionable data and ideas, but alternative practitioners tend to support each other at parent conventions, even if they disagree.

Consider these examples of legitimate research and the therapies they inspired.

In 2005, a team of researchers led by **Carlos Pardo-Villamizar**, a professor at Johns Hopkins

University in Baltimore, reported finding inflammation in postmortem brains of people with autism <sup>1</sup>. After **news reports** of this work came out, it created a buzz on autism parent discussion forums, and Pardo-Villamizar became “concerned that the study would **raise a lot of controversy and be misused.**” So his team published answers to frequently asked questions **on the laboratory's website**. One of these clearly stated, “THERE IS NO indication for using anti-inflammatory medications in patients with autism.”

Undeterred, some doctors began treating people with autism (mostly children) with anti-inflammatories, including steroids and such potent drugs as Actos, which **has a black box warning of side effects**. On the website, which promotes untested therapies for autism, contributing editor Teresa Conrick floated the idea that **“eradicating” microglia**, critical immune cells in the brain, might be an effective treatment for autism.

## Fallacious franchise:

Others have fashioned treatments from the **‘extreme male brain’ theory of autism**, which suggests that “autism can be considered as an extreme of the normal male profile,” according to British researcher **Simon Baron-Cohen**, who championed the idea <sup>2</sup>. Baron-Cohen hypothesizes that **elevated testosterone in the womb** contributes to the development of autism.

Extrapolating from this proposed testosterone-autism connection, in 2006, doctor Mark Geier created what he referred to as the ‘Lupron protocol.’ Lupron is the trade name for a pricey (\$5,000 per month) sex hormone suppressor that includes, among its uses, chemical castration and delaying puberty. Geier **patented his idea** and, at his peak, was **operating clinics in multiple states** and **‘franchising’** the idea to fellow doctors. The ‘Lupron protocol’ was a key factor in **Geier losing his medical license**.

Some ‘treatments’ have an even more tenuous connection to legitimate research. For example, at least one doctor reportedly used a blood-borne protein called Gc protein-derived macrophage activating factor (GcMAF) to treat children with autism, under the belief that the protein boosts the immune system. The idea was inspired at least in part by research on **immune system differences** in individuals with autism, but there is no good clinical data on GcMAF’s efficacy. Other so-called experts purport that chlorine dioxide, a compound used to treat drinking water and bleach wood pulp, can, when used as a drink or enema, by killing pathogens and neutralizing heavy metals, among other actions.

Researchers need to partner with the autism community to limit the spread of misinformation that supports potentially harmful treatments. An online source of information such as the one Pardo-Villamizar’s team set up was an excellent step, but one that clearly fell short.

## Countering quacks:

Ideally, I would like to see the true autism experts challenge the people pitching these ‘therapies’ in person. Unfortunately, attending many of the less-scientific parent conventions is often impractical. Still, more legitimate autism conventions that focus on serving both individuals with autism and their parents would be great venues for researchers to not only discuss their work, but also to warn parents of faux treatments. Examples include conferences put on by the **National Autistic Society**, the **Autism Society** and, at least as important, local organizations and schools for children with autism.

In some cases, **researchers should counter misinformation** and warn against unproven treatments in the the scientific literature. In a 2015 paper in *Molecular Psychiatry* on the influence of steroid hormones in autism, Baron-Cohen and his colleagues made a comment about the therapy promoted by Geier (and his son, David) <sup>3</sup>. “The androgen-blocker Lupron has already been inappropriately offered as a treatment for autism,” they wrote.

The comment was too little, too late. Published nearly 10 years later and buried deep in the discussion section of the paper, it would not have reached many parents considering the therapy. But a full and timely rebuttal of the Lupron protocol in a scientific journal might have had an impact, as many of the parents considering unsound therapies for their children read these journals.

**Paul Offit**, chief of infectious diseases at Children’s Hospital of Philadelphia, offers one model for successfully countering questionable claims for autism treatments. He took on many of the most dubious therapies in a book, **“Autism’s False Prophets.”** Of course, few researchers have the time to devote to such a project.

## Protecting parents:

The press can also be an agent for good in this context, as it sometimes takes questionable autism therapies to task. In these cases, researchers should make an effort to cooperate with any journalists who reach out to them. The more experts who agree to be quoted in these stories, the better. **A 2009 article in the *Chicago Tribune*** discussed both the neuroinflammation and Lupron findings. The article quotes Pardo-Villamizar as saying, “People are abusing science for the treatment of autism.” And Baron-Cohen says in the story that the Lupron therapy “fills me with horror.” This article likely saved some people with autism from these ‘treatments.’

But even this article would likely not have come to be were it not for the exhaustive work of autism parent **Kathleen Seidel**, who thoroughly documented the exploits of the Geiers **in a series of blogs** published in 2006 and 2007. To publicize more such treatments, researchers could tip off journalists to any such misuses of scientific research that they learn about.

Community-researcher partnerships should focus on ways of improving the lives of those with autism, either through fundamental research, investigating potential treatments or even just by explaining the basic biology of autism. When findings simply suggest new approaches for

treatment, researchers should make an explicit effort to counter the misuse of their results, whether through scientific journals, the media or by speaking directly to parents.

In the end, vulnerable parents and their children must be protected from therapeutic journeys like the one **James R. Laidler**, an anesthesiologist in Portland, Oregon, and his family took several years ago. “Looking back on my experiences with ‘alternate’ autism therapies, they seem almost unreal, like Alice’s adventures in Wonderland. Utter nonsense treated like scientific data, people nodding in sage agreement with blatant contradictions, and theories made out of thin air and unrelated facts,” Laidler wrote in a **2004 column on the website Autism Watch**. “I now realize that the thing the ‘alternative’ practitioners are really selling is hope — usually false hope — and hope is a very seductive thing to those who have lost it.”

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