

ALS: Beating Back The Devil

By

[Choctaw Doc*](#)

If you don't believe in a personal Devil AKA Satan, let me introduce you to his Earthly incarnation: ALS (Amyotrophic Lateral Sclerosis). I truly believe that if I could travel back in time to 1942 and challenge the attendees of the infamous [Wansee conference](#) in Berlin (where the Holocaust was mapped out) to come up with a single supremely insidious way to inflict unremitting mental torment on people– they wouldn't come up with anything as monstrous as ALS. Think about it: A disease that whittles away your motor functions until you wind up a puddle of unmoving flesh in a bed or wheelchair – fully conscious – kept alive by a respirator -- lying there day-after-day waiting for your lungs and heart to fail.

The first physician to describe the shriveled motor neurons that characterize ALS was an Englishman named Augustus Waller – in 1850. The first formal description of ALS to appear the medical literature was made by a French doctor named Jean-Martin Charcot during 1869. Eventually scientists figured out that there are 2 forms of ALS: One appears to arise in people who churn out a defective version of an enzyme called copper/zinc superoxide dismutase (SOD1) whose job is to mop up cell damaging free radicals. This form of ALS afflicts 5-10% of those diagnosed with the disease. The other form of ALS is called “sporadic” which is believed to have many triggers and a multitude of molecular, physiologic and genetic players. One major contributor appears to be glutamate overload, in which this excitatory neurotransmitter builds up in motor neurons and induces die-off.

Despite intense research much remains a mystery about the origins and nature of ALS, and only a handful of drugs and other compounds have panned out in terms of impacting the disease. Mind you, the very best FDA approved drug available, glutamate release inhibiting Rilutek® extends the life of some ALS patients by 2-6 months.

Why so little progress? Is it the fault of ALS researchers? The FDA? The pharmaceutical industry?

The scientists doing ALS research are IMO adroitly using all the brains and technology at their disposal to identify ways to slow disease progression in both familial and sporadic forms. More than a few have ALS themselves or afflicted family members or friends and thus have no reason to drag their heels. The slow, gradual pace of progress has more to do with the insidious nature of ALS and the time-consuming nature of doing science itself than anything else. I liken it to trying to contain a large blob of Jell-O with your hands. The harder you try to keep it from oozing between your fingers, the more it seems to do just that. You just can't get the (ahem) upper hand.

As for the FDA: This agency certainly does not have clean hands nor is always a model of efficiency or fairness when it comes to drug approval. Not so many years back officials there [refused to allow doctors who wanted to infuse ALS patients with cord blood from doing so without an IND](#) (New Investigational Drug permit). A few applied but were denied. As the risks were extraordinarily low and there was even some lab and human use indications of clinical benefit, the FDA's stance was puzzling to say the least. More recently there was some seeming foot dragging surrounding allowing ALS patients access to a drug called IPLEX, a combination

of two substances: human insulin-like growth factor 1 (IGF-1) and human insulin-like growth factor-binding protein-3 (rhIGFBP-3); a drug which had shown some efficacy in a [handful of studies](#) conducted mostly in Europe. However, the FDA relented and [signaled a willingness to allow use by ALS patients under either a compassionate use IND or a clinical study conducted by the manufacturer, Insmed](#).

In light of the FDA's action with respect to IPLEX (and many other drugs), it can hardly be said the agency is heartless, unconcerned about the plight of terminally ill people or unable or unwilling to allow dying people access to unapproved drugs (So long as these will not do more harm than good or shorten their lives). But, yes, bureaucracy and more have likely worked to impede progress and even turned it on its head at times. Some critics, in fact, feel the agency is in need of a major overhaul that will better serve the interests of both safety and speedy progress (especially with respect to terminal illnesses).

This brings us to the pharmaceutical industry or "Big Pharma". While it is fashionable in some circles to portray the drug industry as engaged in some kind of carefully orchestrated conspiracy to place profits above human welfare, even to the point of using its resources to suppress or slow approval of viable non-pharmaceutical (especially biologic) treatments that might affect the sale of a particular drug or class of drugs, this overlooks abundant evidence to the contrary. Indeed, it reveals more about information filtering and the biases of "true believers" (in conspiracies) than virtually anything else.

This is not to say the drug industry hasn't had its share of impropriety, illegal activities, scandals and such. Where there is a great deal of money and influence at stake, human foibles and moral weakness will find a way to express itself unless restrained and constrained. So, yes, drug companies are occasionally caught putting their own interests above that of the public their products are supposed to help. But – and this is the big but – there is no credible evidence this is part of some kind of grand conspiracy to ace out all would-be competition by suppressing cures or treatments they do not control or otherwise profit from.

This brings me full circle: So what is the bottom line? Why such a seeming slow rate of progress when it comes to ALS? The scientists are working hard but are undermined by the incredibly insidious nature of ALS itself. Their progress comes by hard won inches not miles. As for the FDA, it has enacted and adheres to a body of regulations, procedures and enforcement policies that place an emphasis on safety and efficacy, but which at times has been anti-progressive insofar as it has moved too slowly when it came to giving dying ALS patients access to experimental drugs and therapies (Some would argue this applies to many, many other health conditions and issues as well, but as ALS is my focus in this article I will forego delving into this). Reform has taken place, but likely more is needed. Some medical advocates argue urgently so. And finally, there is the pharmaceutical industry: No conspiracy, no suppression of a cure for ALS or such (More than a few pharmaceutical company CEOs, directors, staff and scientists have their lives touched by ALS in some way, shape or form, after all) or articulated policy to place profits above the interests of the public they serve. But yes, they are engaged in a highly competitive business enterprise that sometimes tempts them to cut corners or engage in activities that actually do prove an impediment to genuine progress.

In the final analysis, what many are tempted to label some kind of vast conspiracy or premeditated unholy marriage (especially twixt the FDA and the pharmaceutical industry), is merely an emergent system; a confluence of interacting influences, players, regulations and so much that has almost taken on a self-perpetuating and self-sustaining life of its own.

Of course, for those visited by ALS, that wily devil, knowing why progress is sometimes so hobbled is of little consolation. Yes, the system is faulty and beset with bottlenecks and counter-

progressive mechanisms, but waiting for needed reform and repair to not only come to pass but result in speedier access to such highly experimental therapies as genetically engineered stem cells, e.g., cells that churn out growth factors that preserve motor neurons, is simply not an option for the vast majority of ALS patients alive today (And for many other folks with terminal or intractable conditions as well). Most will likely be cold in their graves before substantial “course correction” and “systems redesign” makes its way from debate to action to new treatment options.

Rather than wait for a clinical trial that may never come, many are opting to head to research and treatment centers outside the US for experimental therapies of various kinds. The vast majority involve some form of stem or progenitor cell intervention – some autologous (cells from self) – some allogenic (Donor cells such as cord blood stem cells, fetal cells) – some both. There are risks involved, yes; though these appear “few and far between” (Tens of thousands of cord stem cell, fetal cell and other adult stem cell treatments are done annually across the world with reports of adverse side effects or worse being exceedingly rare).

Of course, critics readily and in many instances justifiably find fault with foreign stem cell medicine programs, much of this based on the fact that some of these operations are basically throwing desperate people a lifeline that is anchored to dubious, even worthless treatments. However, I see a tendency on the part of some stem cell experts to dismiss just about every foreign private stem cell clinical operation they run across or which is brought to their attention as scams, smoke-and-mirrors or worse. Some of these folks actually have ties to private stem cell development companies here in North America and elsewhere. Some actually founded their own stem cell based companies or hold high positions in those founded by other people. One can't help but wonder how often their opinions reflect a confirmation bias at work.

Then, too, I find it telling that some of the most revered stem cell researchers back around 2003 or so were quick to dismiss as “nonsense” reports that cord blood and pure cord blood derived stem cell treatments being done in Mexico were producing neurologic benefits in children with cerebral palsy. Today some of these very scientists are involved with and in some instances running clinical research programs involving the use of cord blood to treat pediatric CP.

Most folks I've spoke to about these disparities label this “politics.” In some instances, perhaps so. But in other instances I see the all-too-human kind of propensity to form *a priori* judgments at work. Consider: Back in the 1920s many people including some experts in aeronautics and medicine dismissed manned space travel to the moon and beyond as simply impossible based on what they felt were clearly established engineering and physical restrictions and contravening factors. More recently, some stem cell authorities and medical consumer advocates have made pronouncements about what specific adult stem cells cannot do or facilitate which have later proved just plain wrong. Naturally one can't help but think this makes them the modern day equivalent of those individuals who confidently declared the prospect of men walking on the moon to be the stuff of science fiction but otherwise highly improbable if not impossible.

I realize, of course, some of these scientific experts were likely motivated to protect the public from getting hoodwinked and their pockets picked by unscrupulous stem cell operators who offered them (*ahem*) the moon and handed them squat. Others may have been quoted out-of-context by journalists.

Thankfully, I am seeing some concerned scientists and others in their orbit not only talking about an openness to explore what is being done at and reported by various private foreign stem cell treatment centers, but actually tackling the gathering of information gathering and then doing analyses that seem balanced on-the-whole.

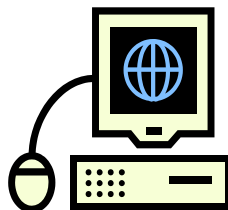
One of these is ALSUntangled which is examining, analyzing and reporting on various, mostly highly web-visible “alternative” ALS treatment programs. They have already posted their findings on at least six treatments, most connected with specific clinical stem cell programs. Here is a link these reports: <http://www.alsuntangled.com/completedinvestigations.html>

What I especially like about ALSUntangled’s handiwork is that their reports do not smack of the often mean-spirited and “jaded” writing I see on many prominent medical consumer web sites. Rather than vilify those doctors and clinics they find are offering treatments that produce little genuine improvements in ALS sufferers, they point out positives where they exist and lace their language with a diplomatic touch that I as a southerner appreciate and believe is vital to communicating issues and opinions to the public. One prime examine is their look at the work of internist Dr. Joe Hickey and his wife at *The Hickey Wellness Center* in South Carolina. Here is a link to this particular write-up: <http://www.alsuntangled.com/pdf/Investigating-The-Hickey-Wellness-Center.pdf>

Bottom line, there is no doubt that some foreign clinics exploit human desperation or exasperation by “selling hope” in the form of worthless cell-based treatments and others of dubious merit. At the same time there are clinics and hospitals that offer experimental adult stem cell therapies that are predicated on findings gleaned from lab and even human studies which are dismissed out-of-hand by critics and skeptics or, worse yet just willy-nilly lumped in with facilities that base their treatments on what appears to be sheer guesswork or nonsensical, even pseudoscientific notions. Rather than toss the baby out with the dirty diaper as many critics do, I would prefer instead to see people armed with the intellectual tools to critically examine clinics, their staffs and claims, and then leave them to reason things out for themselves. And no, it doesn’t take training in the sciences to pull this off. This article of mine is but one example of helping people critically think about the issues surrounding having an adult stem cell treatment outside the US: [For Those Who Are Considering Having Stem Cell Therapy Abroad*](#) The article includes links to all kinds of websites devoted to equipping people to think critically.

Ideally, people with ALS and other terminal or intractable diseases or conditions could count on having access to the means to beat back the devil right here at home, even those deemed highly experimental. This isn’t always the case, of course. So until it is, many will have no recourse but to opt to circle their wagons in foreign lands and do battle using weaponry only available there.

ALS WEB RESOURCES



[ALS Untangled](#)

[Clinical studies: ALS and stem cells](#)

[World Federation of Neurology Research Group on Motor Neuron Diseases/ALS](#)

[Experimental regimen targeting the ependyma slows disease progression in four patients with amyotrophic lateral sclerosis.](#) **NOTE:** High dose lithium carbonate use has bombed as an agent

to slow ALS progression in several recently published clinical trials. This is part of my experimental regimen and, in light of these findings, is obviously in need of revision. However, it may turn out that expunging lithium altogether will prove a case of “throwing the baby out with the dirty diaper.” Instead, I feel that **low dose lithium** may be effective in slowing progression in various motor neuron diseases (Possibly only in a subset of patients possessing genetic variants of this insidious disease). More grist for the research mill. As is <http://bit.ly/wcr7bP> (Amyotrophic Lateral Sclerosis (ALS): Stimulating Proteasome Activity in Motor Neurons to accelerate degradation of misfolded proteins).

THE USUAL NOTICES & LEGAL DISCLAIMERS

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*I am no expert on biology, stem cell biology or such. My focus is on writing and theory.