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Complicated lessons: Lorenzo Odone and **medical miracles**

[Barron H Lerner](#) 

There is no question that Lorenzo Odone lived until the **age of 30 years** because his parents, Augusto and Michaela Odone, defied doctors and developed a mixture of two cooking oils as a possible treatment for their son's devastating disease. **The 1992 film *Lorenzo's Oil***, which commemorated this heroic effort, became an inspirational saga for other patients and families dealing with incurable conditions. Yet Lorenzo's story tells us as much about the limitations of medical research as it does about its triumphs.

By this time, the story of **Lorenzo, who died on May 31, 2008, is well known**. He was an unusually precocious 5-year-old when he began acting oddly in 1983. Doctors eventually diagnosed him with adrenoleucodystrophy (ALD), a devastating genetic neurological disease of young boys that causes confusion, agitation, and eventually unresponsiveness and death within a few years. Doctors told his parents that no treatments existed and that Lorenzo was doomed. Unwilling to believe this, the Odones, neither of whom had medical training, became fixtures at the nearby library of the National Institutes of Health. Within months, they had convened the first international conference of scientists working on ALD. Using insights from this meeting and subsequent research, the Odones, in 1986, developed what became known as Lorenzo's Oil, which successfully lowered their son's concentrations of very long chain fatty acids, presumed to be the cause of neurotoxicity in ALD.

As of **1992, when the film *Lorenzo's Oil* was released, it was not known whether lowering concentrations of very long chain fatty acids had actually helped Lorenzo and his fellow victims**. This fact did not bother the director George Miller, whose film unabashedly celebrated the Odones' accomplishment and depicted ALD specialists as more interested in science than the welfare of their patients. At the end of the film, dozens of boys are shown running and playing sports. They sequentially announce their names and state for how long they have been taking Lorenzo's Oil. The implication was that the oil had either cured them or prevented them from getting ALD.

Not surprisingly, the movie outraged scientists and families affected by ALD, both of whom believed the film played with people's hopes. But the Odones had the last laugh. When the late Hugo Moser, the world's expert on ALD and one of Lorenzo's physicians, finally published data in 2005, it became clear that although the oil did little for already sick boys, it prevented the onset of ALD in two-thirds of susceptible boys who otherwise would surely have died from the disease. It was a breathtaking scientific achievement spearheaded by two laypeople.

History can illuminate Lorenzo's story. **The first voluntary health association in the USA was the National Association for the Study and Prevention of Tuberculosis** (now the **American Lung Association**), founded in 1904. But it was the National Foundation for Infantile Paralysis, also known as the March of Dimes, that cemented the idea that funding for research initiatives could directly lead to medical breakthroughs. In this case, money raised not only supported the research of Jonas Salk and Albert Sabin but also the trials that showed the efficacy of the polio vaccine. Nevertheless, the public remained suspicious of much experimental research which, as a result, was often done on prisoners, orphans, the mentally challenged, and other captive populations without informed consent.

But with the emergence of AIDS in the early 1980s, everything changed. Highly organised groups of gay men, confronted with a mysterious life-threatening disease, aggressively advocated for greater research funding and for early inclusion in experimental trials. Their arguments were persuasive enough for the US Food and Drug Administration to revise its protocols, expediting the drug approval process. Once again, the connection between advocacy, funding, and hope for severely ill patients had been made. "People used to say it doesn't help to throw money at things", Hugo Moser reflected late in his career. "But it does."

This history provides a backdrop for evaluating the Odones' efforts. At first glance, Lorenzo's story seems to be an uncomplicated triumph of devoted parents fighting the medical establishment. Yet although the Odones' accomplishments were remarkable, the actual lessons of Lorenzo's life are much more ambiguous. For one thing, the concerns of the Odones' critics are of utmost importance. Desperate patients with fatal diseases may join risky research protocols believing that the intervention is likely to

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help their case; in reality, the sickest patients may be the least likely to benefit. In other instances, such studies merely seek to accumulate scientific knowledge to benefit future patients. Although groups such as Abigail's Alliance have urged US courts to find a constitutional right for terminally ill patients to get access to unapproved experimental therapies, recent rulings have gone in the other direction.

Some of the judges' scepticism in these cases stems from the fact that highly touted treatments that initially seem promising on the basis of anecdotal information often prove no more effective than standard regimens. For example, the enthusiasm of patients and oncologists for stem-cell transplantation of advanced breast cancer actually led insurance companies to pay for a highly expensive and risky experimental therapy. "I don't feel that I have the right not to offer a transplant to a woman who wants one", one oncologist stated. Ultimately, however, randomised trials showed the procedure to be of limited clinical value. The chief ALD expert in the film *Lorenzo's Oil* warns against this type of false hope when speaking to parents demanding the yet untested oil, asking them "You don't really expect me to endorse a therapy on the basis of one hopeful observation?"

Another pitfall of aggressive experimentation is that activists may disagree over the potential value of specific interventions. For example, in the years after he was paralysed, the actor Christopher Reeve termed himself a "human guineapig" and pushed for embryonic stem-cell research that he believed would enable him and other paralysed individuals to walk again. But other activists believed that Reeve's goals were unrealistic and diverted funding from more promising, and less dramatic, advances.

An additional problem with new medical technologies is that instead of producing cures, they may prolong life in states that are difficult for patients and families. Lorenzo defied all predictions by living to the age of 30 years and was able to enjoy things like music and massages and to interact in limited ways with his family. But because the oil is essentially a preventive agent as opposed to a therapeutic one, Lorenzo remained severely neurologically impaired, bedbound, unable to speak, and fed through a tube. Despite their incredible achievement, the Odone family had only accomplished a small part of what they had dreamed of.

Finally, the strategy for fighting disease that the Odone family helped to construct—challenge the medical establishment, bring researchers together, establish a foundation, and lobby for greater research funding—has yielded only a few dramatic breakthroughs. As of 2009, similar activist campaigns exist for hundreds of other diseases. The National Organization for Rare Disorders lists 139 organisations on its website, ranging from the International Rett Syndrome Association to the [National Fragile X Foundation](#). This increased competition, in the face of finite funding streams, means that some conditions generate more interest than others.

And even when money has been forthcoming, many diseases remain stubbornly resistant to research advances. These conditions include familial dysautonomia, Parkinson's disease, and amyotrophic lateral sclerosis as well as pancreatic, lung, and other solid cancers. In the USA, the entertainer Jerry Lewis has been raising money for muscular dystrophy through a Labor Day telethon since the 1960s but research remains in the "stone age", as one prominent clinician recently stated. Indeed, in the wake of their success in developing Lorenzo's Oil, the Odone family established [The Myelin Project](#) to seek cures for ALD and similar diseases that damage the protective myelin sheaths that surround nerves in the brain and spinal cord. But there have been no comparable breakthroughs.

Ultimately, therefore, while the case of Lorenzo Odone provides a possible blueprint for medical progress, it is not at all a representative story. Aggressive research may as often lead to dead ends and false claims as true advances.

So what can be done? Building on the ill-fated experience with stem-cell transplantation for breast cancer, Richard Rettig and colleagues have proposed a "public-private partnership" of health professionals, insurers, research funders, and patients to evaluate and prioritise cutting-edge research initiatives. This plan would get all interested parties to the table and encourage them to work within the established scientific process.

Yet while Rettig's proposal is sound and crucial, individual stories of dread illness—like that of Lorenzo—will continue to inspire particular patients, families, and scientists to try to manipulate funding, publicity, and the drug-approval process in ways they believe might provide immediate benefits. This will be especially true when anecdotal cases of purportedly successful therapies have emerged for the disease in question. And it will be hard to say no. Since hope is the basis of public support for expensive medical research programmes, to deprive desperate patients and families from grasping at straws is difficult and perhaps even inappropriate. **After all, even though breakthroughs like Lorenzo's Oil happen rarely, they do happen.**

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The art of medicine

Complicated lessons: Lorenzo Odone and medical miracles

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For more on **National Fragile X Foundation** see <http://www.rarediseases.org>

For more on **The Myelin Project** see <http://www.myelin.org>

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