

From Marlene Haffner, interviewed by Suzanne Junod and Robert Tucker, 13 March 2012:

MEH: The movie “Lorenzo’s Oil” was made about a disease called adrenoleukodystrophy, and had to do with a drug that is a combination of two oils which will lower very-long-chain fatty acids. The trouble is it lowers it well in the peripheral blood, but it needs to get across the blood-brain barrier. But that particular movie was about a little boy, who died only a year or so ago, with adrenoleukodystrophy, and Dr. Hugo Moser, one of our grantees for a long time, was developing this product. He discerned that it simply was not working. The family did not want to believe that, and in the movie Dr. Moser is depicted as an ogre. He was anything but. He has since died. But it was an interesting movie. The father came to me, the father of the little boy came to me long after and said, “You know, I made a mistake.” It was a well-received movie with Susan Sarandon and Nick Nolte, and all of us in the Office of Orphan Products Development took an afternoon and went to see the movie, because we were sure we would just be bombarded with questions. Quite frankly, I don’t think we got any.

SJ: It certainly brought orphan products to the minds of the public in a way that I don’t think had ever happened before.

MEH: It did.

SJ: It showed clearly how much one’s hopes can dictate a lot of what is seen by patients and practitioners alike.

MEH: Absolutely.

SJ: Did Lorenzo live anywhere near a normal life?

MEH: Oh, no, no, no. He was severely mentally . . .

SJ: But in terms of longevity?

MEH: No. He died at the age of 21.

SJ: And that would have been predicted without treatment?

MEH: He would have died earlier without it, but he lived basically in a family-derived ICU, so I don't think that his life was made any longer even with that level of care. He certainly didn't lead a quality of life.