

I have a 42 yr old male friend, misdiagnosed as having osteoporosis for two years, who recently found out that his illness is the rare Gaucher's disease.

Gaucher's disease symptoms include: brittle bones (he lost 9 inches off his height); enlarged liver and spleen; internal bleeding; and fatigue (all the time). The problem (in Type 1) is attributed to a genetic mutation where there is a lack of the enzyme glucocerebrosidase in macrophages so the cells swell up. This will eventually cause death.

Enzyme replacement therapy has been successfully developed and approved by the FDA in the last few years so that those patients administered with this drug (called Ceredase) report a remarkable improvement in their condition. Ceredase, which is manufactured by biotech biggy company--Genzyme--costs the patient \$380,000 per year. Gaucher's disease has justifiably been called "the most expensive disease in the world".

I have researched Gaucher's disease at the library but am relying on netlanders to provide me with any additional information:

****news, stories, reports**

****people you know with this disease**

****ideas, articles about Genzyme Corp, how to get a hold of enough money to buy some, programs available to help with costs.**

****Basically ANY HELP YOU CAN OFFER**

Thanks so very much!

Deborah