

**Testimony Submitted by Dr. Frederick Askari to the
United States Senate Special Committee on Aging**

**“Valeant Pharmaceuticals' Business Model: the Repercussions for Patients and the Health
Care System.”**

Hearing on April 27, 2016

Good afternoon and thank you Chairman Collins, Ranking Member McCaskill, and distinguished members of the Committee for holding this hearing. My name is Dr. Fred Askari, and I serve as Director of the Wilson Disease Center of Excellence at the University of Michigan. I directly treat around 400 Wilson disease patients, and consult on dozens of other cases.

Wilson disease is a rare genetic disorder of copper processing that is fatal if not diagnosed and treated. Copper is in the food we eat, and it is an essential trace element necessary for life. In people with Wilson Disease, due to a genetic defect, copper accumulates to toxic levels. Copper overwhelms the body, chiefly damaging the liver and brain.

Wilson disease is completely manageable with proper treatment; however it is a uniformly fatal disease if left untreated. It can be a crippling disease if copper levels are not well controlled or if the diagnosis is not made early enough. Risks of going untreated vary and depend on state of disease control, but toxicity can onset in as few as several weeks after stopping treatment. Risks of not treating Wilson disease or gaps in treatment include liver failure, brain damage, and death.

While there is no known prevention or cure for Wilson disease, there are treatment options, and people managing the disease with medication are often able to live full, healthy, and productive lives. The medications must be taken daily for life. Treatment options utilize two types of action: (1) Chelating agents that prompt the organs to release copper into the bloodstream to be filtered by the kidneys and eliminated through urine; and (2) Zinc-based therapies which prevent the body from absorbing the copper. The standard of care has called for utilizing a chelating agent at least initially to remove the excess copper. When copper levels are stabilized, patients move to a daily maintenance therapy either through continuing on a chelating agent or switching to zinc.

Historically, the first line of treatment for Wilson disease was penicillamine, known by the trade name Cuprimine. This is a chelating agent that works by removing excess copper. It has been used to treat Wilson disease since 1956. While penicillamine continues to work for many, it is no longer the default for every patient because approximately one third of patients

experience adverse side effects. The gold standard for treatment today is trientine, known by the trade name Syprine, which causes fewer side effects.

Once the patient has been stabilized with Syprine, some patients can be switched to zinc treatment. The FDA approved zinc acetate is called Galzin and prevents the body from absorbing copper. In some patients, Galzin causes extreme stomach upset and gastrointestinal problems.

The persistently increasing price of Valeant's Wilson disease drugs poses a problem for up to half of my patients. One patient was denied coverage, and left off treatment completely for several weeks. Another, a 17 year old patient, lives in fear of losing coverage when he turns 24, as his mother was forced to take early retirement. Access to appropriate treatment is especially a problem for seniors with Medicare.

I have worked with dozens of patients to obtain Syprine through Valeant's patient assistance program. It is time-consuming and frustrating. My clinic has had to hire two full time employees just to deal with the red tape caused by the price hikes, such as the paperwork for the patient assistance program and associated insurance claims. Even when patients are approved for patient assistance, they cannot be certain they can stay in the program—they have to reapply every year.

While the process of applying for patient assistance programs is difficult enough as is, it is especially difficult for Wilson disease patients. Some have neurological conditions, which can make it even more difficult for them to navigate the programs. Many patients who are able to get the drug they need worry they may lose access in the future, and may hoard pills or skip doses to prevent being caught without.

Finally, I am not here to cast blame on the entire drug industry. Ethical pharmaceutical companies do support research, which provides new and improved treatments for diseases. Wilson patients have many unmet needs with current treatments. Based on an expectation of reasonable investment returns, companies invest in developing these new treatments, such as gene therapy, once daily dosing regimens, and novel therapies such as one being investigated, TM, which offers hope for improved neurological outcomes. We are fortunate that there are companies which safely manufacture, test, and distribute medications for rare diseases. One should not confuse companies which institute sudden and dramatic price increases on longstanding critical drugs with those which are truly developing new ones. There is an enormous human cost associated with these practices. I urge Congress to work diligently to arrive at policies that will protect patients, while maintaining incentive for new lifesaving therapies.

I thank the Committee for investigating this important issue, and for the opportunity to share my concerns. I look forward to answering your questions.