



ANDREW FRANCIS WALLACE/TORONTO STAR

Toni Vernon has a rare blood disease and must undergo dialysis three times a week. She needs a drug that costs \$500,000 a year.

A rare disease, a lonely struggle

Health ministry holds back on costly Soliris, which could treat her unique condition

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Toni Vernon is sick, but not sick enough in the eyes of the government to get access to the medication she needs.

The 59-year-old Don Mills mother of four suffers from atypical Hemolytic Uremic Syndrome (aHUS), a rare and potentially deadly blood disease that attacks the body's organs and affects less than 100 Canadians a year. She spends about 10 hours a week receiving dialysis, the disease having ravaged her kidneys. She gets severe headaches, feels weak, and can no longer work.

The good news: Vernon has a friend who is a perfect match for a kidney transplant, which would allow the former realtor and gym enthusiast to go back to her active lifestyle.

The bad news: the transplant won't proceed until she receives Soliris, the only drug approved by Health Canada to treat aHUS and keep it from damaging the new kidney.

The very bad news: Ontario does not fund Soliris for aHUS.

"I was basically told that without (Soliris), I have to be on dialysis for the rest of my life," said Vernon.

The only way to get access to Soliris, which costs \$500,000 a year, is to apply for it on compassionate grounds. Because the province is in

negotiations with the drug's manufacturer, "requests will be considered on a case-by-case basis for individuals who have been urgently hospitalized due to an immediate life-, limb-, or organ-threatening complication" resulting from their illness, said Gabe De Roche, a spokesman for Health Minister Eric Hoskins.

The ministry limits temporary funding to six months, with further funding to be reassessed after that. Another aHUS sufferer, Johann Kellow, featured in the Star last month, is getting Soliris through this policy after being hospitalized due to pancreatitis linked to aHUS. She questions why the government would temporarily fund a drug she will need for life, and why they won't do so for others with the same diagnosis.

Vernon is considered to be in remission, despite her damaged kidneys. She's now become the latest Ontarian to go public with her plea for an expensive drug to treat a rare illness. She's meeting with her local MPP next week. She's also emailed Hoskins and Premier Kathleen Wynne, but has yet to get a response.

"I'm a very private person; I didn't want this to be made public," she said, "but I figured if I didn't do anything, nothing would get done, and I would go unnoticed. Then I realized I had to help myself, because I didn't want to sit here and wait to die."

Vernon was hospitalized in the summer of 2012 for several weeks. Her bloodwork was off and she received transfusions. She ultimately

developed renal failure, but it took weeks to get the aHUS diagnosis.

"In the beginning it was very, very scary. They didn't know what to do with me," she said. "I felt like I was getting sicker by the moment. I thought I was going to die."

At first, the dialysis gave her severe headaches that would last seven to eight hours, to the point where she had to go to the ER. Her body handles it better now, but she's far from her old self. She has been off work two years, has difficulty attending social functions, and travelling long distances is out of the question.

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TONI VERNON

"You could almost call it a life on hold," said her physician, Dr. Michelle Hladunewich, director of nephrology at Sunnybrook Health Sciences Centre.

"She really can't do the things she wants to do. She's bound to a dialysis machine."

While the disease has yet to attack any of Vernon's other organs, there's a chance it will. She wants to know Soliris will be there should another organ fall prey to the illness.

"I may not be able to survive, because if it attacks another organ like my kidneys, who knows what will happen," she said. "That's the fear I have to live with."

In a previous email to the Star, De Roche said Soliris is not permanently funded for aHUS because of "unresolved concerns regarding diagnostic criteria, appropriate use, lack of long-term efficacy and safety data, high cost, and uncertainty in cost-effectiveness." Connecticut-based Alexion, the drug's maker, submitted extra information in June, which the health ministry is reviewing.

Soliris is funded by the province for the treatment of another rare illness, paroxysmal nocturnal hemoglobinuria (PNH), through a bulk-buy deal reached by the premiers in 2011.

Alexion spokesperson Catherine London said Soliris is pricey due to the rarity of illnesses it treats and the cost of research and development.

Vernon, who has been married for 17 years, said she hasn't applied for disability assistance yet — "to be quite honest, I kept thinking I was going to get my kidneys back and I was going to be normal again" — but now says she may have to start selling property to stay afloat.

Holding back tears, she says she can't help but feel frustrated and angry at the bureaucracy that is essentially waiting for her to be on death's door before helping.

"I'm disgusted and devastated by the fact that they are playing, in my opinion, with my life and others like me," she said. "They're not treating us like human beings. I feel if it was one of them, one of their family members, ... this would not be an issue."