

Dear Friends in the UK,

It is nice to see you again. I always appreciate your kind words and encouragement. I hope you and your family are safe and well during this COVID-19 pandemic. I felt so relieved that Prime Minister Boris Johnson and Prince Charles had recovered from the COVID-19.

Three things are always on my mind: Improve clinical care, Raise awareness, and Provide a cutting-edge treatment for Wolfram syndrome. I am determined to make 2020 the game-changing year for us despite this challenging time. Today, I would like to share the good news with Wolfram Syndrome UK and my friends in the UK.

We have been testing if gene editing by CRISPR-Cas9, in combination with patient-derived induced pluripotent stem cells (iPSCs), can be utilized for the treatment of Wolfram Syndrome. I am glad to inform you that gene editing worked in Wolfram patient iPSC-derived beta cells. We were able to use these cells to cure one of the problems, making normal beta cells by correcting WFS1 gene mutation. We could cure diabetes in cells and mice. This is a proof of concept demonstrating that correcting gene defects that cause or contribute to medical problems— in this case, in the Wolfram syndrome gene — we can cure the problems. This is a major discovery in the gene therapy field, and it has been just published in a high-profile medical research journal, Science Translational Medicine. My next step is to bring this personalized gene therapy to our patients. We still have multiple hurdles, but I will do my best to accelerate the process.

It is possible that by correcting the genetic defects in these cells, we may correct other problems Wolfram Syndrome patients experience, such as visual impairment and neurodegeneration. So we are currently working on eye and brain cells derived from iPSCs of patients with Wolfram Syndrome to replicate this success for other problems. Many, many thanks to my patients, colleagues at Washington University and supporters in the UK and the world. As always, please feel free to contact me with any questions (urano@wustl.edu). I would like to know what you think and how you feel.

Thank you again, Wolfram Syndrome UK and my friends in the UK, for your continued support and encouragement, especially in this very trying time for the world. We will work as one team and change history together.

“That's one small step for us today, leading to one giant leap toward a cure for Wolfram Syndrome.”

Sincerely,
Fumi Urano, MD, PhD
Sunday, April 25, 2020