

Dear Friends in the UK,

It is nice to see you again. The winter is ending, and the spring is approaching. I am determined to make 2020 the game-changing year for us. Three things are always on my mind: Improve clinical care, Raise awareness, and Provide a cutting-edge treatment for Wolfram syndrome. I would like to share my three goals for 2020 with you.

1. Set up a new clinical trial for Wolfram syndrome using a new drug (more specifically, get an orphan drug designation, create a trial protocol, and secure funds). I have been working with a biotech and National Institutes of Health in the US on this.
2. Complete preclinical studies for gene therapy for optic nerve atrophy in Wolfram (more specifically, complete studies in rodent and iPSC models) and start setting up a clinical study. I have a clear plan for this.
3. Set up genetic testing for genetic forms of diabetes and ER stress-related disorders, including Wolfram syndrome and WFS1-related disorders, and create a clinical service for those patients through a newly established endocrinology genetics clinic. A cutting-edge genetic testing has already launched at the Washington University Medical Center in the US.

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 for your continued support and encouragement. I am determined to make a difference in the future of our patients. We will work as one team and change history together.

Sincerely,
Fumi Urano, MD, PhD
Samuel E. Schechter Professor of Medicine
Barnes-Jewish Hospital
Washington University School of Medicine