An Update on New Therapies for Wolfram Syndrome

Translational Science Benefits Model
IMPACT PROFILE

This research works toward developing diagnostic tests and therapeutics for patients with Wolfram Syndrome, a rare inheritable condition. This is accomplished by testing treatments in models, trialing drugs in patients, and analyzing donated samples from patients. In addition to this research, Dr. Urano has also opened a new clinic to improve care access.

The Challenge

Wolfram Syndrome is a rare inherited condition associated with juvenile-onset diabetes, vision loss, deafness, and neurodegeneration. This condition affects approximately one in 500,000 people globally. Because of its **impact on multiple organs**, Wolfram Syndrome is usually **fatal by mid-adulthood**. The condition is typically attributed to pathogenic causes affecting the **WFS1 gene**.

The Approach

To better understand Wolfram Syndrome and develop treatments, Dr. Urano has:

- Continued studying the disorder's progression in rodent and cell models of Wolfram Syndrome.
- Tested potential therapeutics in rodent and cell models of Wolfram Syndrome.
- Developed a partnership with other WashU specialists to provide personalized treatments for Wolfram Syndrome patients.
- Initiated a clinical study to collect donated samples from Wolfram Syndrome patients.

The Impact

Dr. Urano's research has contributed to potential therapeutics and diagnostic tests for Wolfram Syndrome. His investigative research with drugs has resulted in a clinical trial where the drug has been tested for patient safety and efficacy. This drug has the potential to radically change treatment and quality of life for patients. In addition to drug research, Dr. Urano has also helped establish a clinical registry and a Wolfram Syndrome clinic. The clinic streamlines treatment for Wolfram Syndrome patients who often experience inefficient care for this rare disorder. Patients of this clinic may choose to donate biological samples to be analyzed in the clinical study. These findings may shed light on new treatments and diagnostic tests as well. This body of work has the potential to significantly change the lives of patients with Wolfram Syndrome and their families.

RESEARCH HIGHLIGHTS

- Obtained medical records from **300** patients
- Received donations of **100** biological samples from patients
- Engaged **50** patients in longitudinal study
- Filed **2** license agreements
- Completed foundational work for **1** clinical drug trial

Key Benefits

Dr. Urano's work on Wolfram Syndrome has resulted in ***clinical***, ***community***, and ***economic*** benefits.

Identified dulaglutide as a potential therapeutic for Wolfram Syndrome in mouse and iPSC models.

Continued research on AMX0035, a drug that has concluded a Phase II clinical trial.

Data analysis from the clinical registry may lead to new therapeutic procedures.

Created a new clinic for evaluation, education, and counseling for Wolfram Syndrome patients.

Filed a license agreement for a humanized rat model to study Wolfram Syndrome.

The team:
Dr. Urano, MD, PhD, Samuel E. Schechter Professor of Medicine, Professor of Medicine and Pathology & Immunology, Division of Endocrinology, Metabolism and Lipid Research

Find out more:
Read the full case study
Learn more about the registry

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