

VECTORS FOR STUDY OF WOLFRAM SYNDROME

<u>Abreu, Damien, Urano, Fumihiko</u>

Gill, John

T-019395

<u>Dr. Fumihiko Urano</u> is director of the <u>Wolfram Syndrome International Registry</u> at WU and has developed many research tools for studying Wolfram Syndrome.

The following constructs contain the wildtype WFS1 gene with various flags, or mutations of WFS that cause mild forms of Wolfram Syndrome (019395):

- WT WFS1 Flag
- pECFP-WT WFS1
- pcDNA3 G695V WFS1
- pcDNA3 P724L WFS1 (Addgene)

Gene therapy constructs for treating Wolfram Syndrome:

- pD-WFS1 human WFS1 cDNA for gene therapy
- AAV-CMV-WFS1
- AAV-CMV-IRES-GFP
- AAV-CMV-WFS1-IRES-GFP
- AAV-CMV-WFS1
- AAV-CMV-MANF-IRES-GFP
- AAV-CMV-MANF
- hWFS1-Flag-pLenti CMV (019426)