



Renal Failure and WAGR Syndrome

Renal (kidney) failure is a common feature of WAGR syndrome. Up to 60% of individuals with WAGR syndrome may develop renal failure at some point in their lives, most often during adolescence or early adulthood.

Who Is At Risk?

Currently there is no method for predicting which individuals with WAGR syndrome will develop renal failure. Anyone with WAGR syndrome is at risk for this complication, *including those who have never had Wilm's tumor.*

What Causes Renal Failure in People with WAGR Syndrome?

The type of renal failure associated with WAGR syndrome is called "Focal Segmental Glomerulosclerosis," or FSGS. This type of renal failure can occur with a number of other medical conditions, such as diabetes. It is not known why people with WAGR syndrome develop FSGS, but it is thought to be the result of the genetic defect that causes WAGR syndrome.

FSGS refers to a condition in which the glomeruli, the tiny tubes which filter the blood to make urine, become scarred. Instead of filtering properly, these damaged glomeruli allow protein to appear in the urine. Over time, the kidneys lose their ability to function properly, and kidney failure is the result.

How Is FSGS Treated?

The course and outcome of FSGS varies from one patient to another. Some patients with FSGS respond well to medication, and their kidney function may remain relatively stable for long periods of time. Other patients may go quickly from diagnosis to the need for dialysis or kidney transplant.

Many patients with FSGS respond well to treatment with a class of drugs called angiotensin converting enzyme (ACE) inhibitors. ACE inhibitors are not a cure, but they do appear to slow the progression of the disease in some patients. The same may be true for patients with WAGR Syndrome and FSGS.

Signs of renal failure may include hypertension (high blood pressure) hyperlipidemia (very high cholesterol) and/or proteinuria (protein in the urine) Early diagnosis of renal failure is important, because appropriate treatment may help prolong the time between diagnosis of the condition, and the need for

dialysis or kidney transplant. Guidelines for testing and monitoring at each stage of life are available here:

[Health Supervision for Individuals with WAGR Syndrome](#)>

To learn more about the function of the kidneys, FSGS, or living with chronic renal failure, please visit the following websites:

<http://www.nephron.com>

<http://www.renalnet.com>

<http://www.kidney.org>

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Characteristics and outcomes of children with the Wilms tumor-Aniridia syndrome: a report from the National Wilms Tumor Study Group.

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PURPOSE: Children with the rare Wilms tumor (WT)-aniridia (WAGR) syndrome have not had systematic evaluation of their clinical and pathologic features. We compared demographics, disease characteristics, and treatment outcomes in a large cohort of WT patients who did or did not have the WAGR syndrome.

PATIENTS AND METHODS: Clinical and pathology records were reviewed for 8,533 patients enrolled between 1969 and 2002 by the National Wilms Tumor Study Group.



RESULTS: Sixty-four patients (0.75%) had the WAGR syndrome. For WAGR and non-WAGR patients, respectively, the average birth weights (2.94 and 3.45 kg), median ages at diagnosis (22 and 39 months), and the percentages with bilateral disease (17% and 6%), metastatic disease (2% and 13%), favorable histology (FH) tumors (100% and 92%), and intralobar nephrogenic rests (ILNR; 77% and 22%) all differed. Survival estimates for WAGR and non-WAGR patients were 95% +/- 3% and 92% +/- 0.3% at 4 years but 48% +/- 17% and 86% +/- 1.0%, respectively, at 27 years from diagnosis. Five late deaths in WAGR patients were from end-stage renal disease (ESRD).

CONCLUSION: The excess of bilateral disease, ILNR-associated FH tumors of

mixed cell type, and early ages at diagnosis in WAGR patients all fit the known phenotypic spectrum of constitutional deletion of chromosome 11p13. Despite a favorable response of their WT to treatment, WAGR patients have a high risk of ESRD as they approach adulthood. The renal pathology associated with this apparent late manifestation of WT1 deletion, and the explanation for the abnormally low birth weights in patients with del 11p13, have yet to be determined.

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End stage renal disease in patients with Wilms tumor: results from the National Wilms Tumor Study Group and the United States Renal Data System.

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PURPOSE: We sought to assess accurately the full spectrum of end stage renal disease (ESRD) in Wilms tumor survivors by combining the unique resources of the National Wilms Tumor Study Group (NWTSG) and the United States Renal Data System (USRDS), and to confirm preliminary reports of an increased incidence of ESRD in patients with the Wilms tumor-aniridia syndrome (WAGR).

MATERIALS AND METHODS: ESRD was ascertained in 5,910 patients enrolled in NWTSG studies during 1969 to 1994 by record linkage to USRDS and by direct followup. Cumulative ESRD incidence was estimated accounting for intercurrent mortality.

RESULTS: Of 115 cases of ESRD 10 (9%) were ascertained by the NWTSG alone, 13 (11%) by USRDS alone and 92 (80%) by both. Cumulative incidence of ESRD at 20 years from diagnosis of unilateral Wilms tumor was 74% for 17 patients with the Denys-Drash syndrome, **36% for 37 patients with WAGR, 7%**

for 125 male patients with hypospadias or cryptorchidism (genitourinary [GU] anomalies) and 0.6% for 5,347 patients with none of these conditions. The incidence of ESRD after diagnosis of bilateral Wilms tumor was 50% for the Denys-Drash syndrome (6 patients), **90% for WAGR (10), 25% for GU anomaly (25) and 12% for other (409).** ESRD in patients with WAGR or GU anomalies tended to occur relatively late, often during or after adolescence.

CONCLUSIONS: The risk of ESRD is remarkably low for the majority of patients with Wilms tumor. However, those with WAGR or associated GU anomalies are at higher risk and should be screened indefinitely to facilitate prospective treatment of impaired renal function.

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