Conference on
Wilms Tumor in WAGR Syndrome
October 18-20, 2018
Ann Arbor, Michigan

Summary and Highlights

INTERNATIONAL WAGR SYNDROME ASSOCIATION
Why a Conference?

Fifty percent of children with WAGR syndrome develop **Wilms tumor**, a type of kidney cancer.

Wilms tumor has not been studied before in people with WAGR syndrome.

A conference would bring scientists and parents together to develop research projects.
The Conference on Wilms Tumor in WAGR Syndrome was held at the University of Michigan, Ann Arbor
The conference was created by the International WAGR Syndrome Association (IWASA), a not for profit 501(c)(3) organization, whose mission is to promote awareness, stimulate research, and support families affected by WAGR syndrome.

The conference was funded through a Patient-Centered Outcomes Research Institute (PCORI) Eugene Washington PCORI Engagement Award.

PCORI helps people make informed health care decisions, and improves health care delivery and outcomes by producing and promoting high integrity, evidence-based information that comes from research guided by patients, caregivers and the broader health care community.
Eleven parents of children with WAGR syndrome and eighteen researchers came from around the world to attend the Conference.
Parent Attendees

rear left to right:
Alper Dogan Turkey
Shari Krantz Maryland
Cathryne Cansler Michigan
Rhonda Sena New Mexico
Dolly Carlson Michigan
Jenny Gunuckle Michigan

front left to right:
Kelly Trout Texas
Brittany Gelsomino Illinois
Adam Gunuckle Michigan
John and Beth Morris Pennsylvania
Conference Goals

- Engage patient representatives and researchers in planning clinical studies
- Identify gaps in knowledge
- Identify barriers to participation in clinical studies
- Develop consensus on research questions
- Develop a committed team for future projects
- Develop a patient-centered model for this research
- Develop a roadmap for future research

*Parents John and Beth Morris with Andrew Murphy MD*
A major goal was to ensure that the research would be “patient-centered”
Patient-centered research allows patients and caregivers to help choose what is studied based on their concerns and priorities.
Parents of children with WAGR syndrome shared their personal stories and experiences with Wilms tumor researchers.
Researchers also learned about patients with WAGR syndrome through poster presentations.
Parents Jenny and Adam Gunckle, Kelly Trout, and Shari Krantz reviewed posters with Conference Committee members Peter Ehrlich, MD and Jeff Dome, MD
Parent and Conference Project Lead Kelly Trout provided training on Patient-Centered Outcomes Research
The presentation on WAGR syndrome by Joan Han, MD was critical to Wilms tumor project planning and design.
Jeff Dome, MD provided a comprehensive review of current Wilms tumor research.
Radiologist Fred Hoffer, MD discussed the challenges of distinguishing between nephrogenic rests and Wilms tumor in patients with WAGR syndrome.
Peter Ehrlich, MD identified important considerations in surgical management of Wilms tumor.
Janna Hol, PhD student, shared an exciting project she is working on at the Princess Maxima Pediatric Oncology Center, Netherlands.
The Conference was designed to maximize interaction and discussion

Parent Dolly Carlson, Genetic Counselor
Joyce Turner, Parents Adam Gunickle and Rhonda Sena

Elizabeth Mullen, MD
Conrad Fernandez, MD
Conference Outcomes: Research Projects

- Consideration of a medication for prevention of Wilms tumor in children with WAGR syndrome
- Options for biospecimen collection and storage
- Clinical characteristics and outcomes of children with WAGR syndrome and Wilms tumor registered in SIOP 93-01 and 2001
- Development of a Clinical Center of Excellence for WAGR syndrome
- Publication of guidelines for diagnosis and treatment of Wilms tumor in patients with WAGR syndrome

Conference attendees continued discussions over dinner
Conference Outcomes: Topics for Future Consideration

- Late effects of treatment
- Imaging and surveillance: nephrogenic rests vs Wilms tumor
- Including patient advocates on the Children’s Oncology Group Pediatric Cancer Predisposition Syndromes Committee
- Growth curves in WAGR syndrome patients
- Further characterization of WAGR syndrome features and conditions

Parent Brittany Gelsamino
Key Parent TakeAways

- Researchers are very interested in parents’ knowledge and experience
- Learned how research projects are developed
- Gained confidence in their ability to help design these projects
- All have become enthusiastic research ambassadors

Jenny, Cathryne, Kelly, Beth, and John assemble Conference bags
Key Researcher TakeAways

- Patient and caregiver concerns yield valuable clinical insights
- Patient advocate input is instrumental in research project creation and design

Left and above, l-r: Marry van den Heuvel-Eibrink MD, Norbert Graf MD, Janna Hol PhD student, Daniel Green MD, Peter Ehrlich MD
From “hasn’t been studied before” to multiple projects now in the pipeline, the Conference on Wilms Tumor in WAGR Syndrome demonstrated that collaboration between patient advocates and researchers can achieve remarkable results.
“It was a great meeting, well organized, wonderful atmosphere, great discussions, new knowledge gained, perspectives for the future developed. Altogether an amazing effective meeting”
~ Research Attendee
“This style of conference needs to happen more often. These top researchers were able to see our children from our view. This was an amazing platform that allowed all these researchers to communicate and build on each other’s ideas…I walked away feeling proud to have advocated for children with WAGR Syndrome and like we actually are making a difference.”

~Parent Attendee
The IWSA wishes to thank
Miranda’s Mission and WAGR Warriors
and everyone who supports the
INTERNATIONAL WAGR SYNDROME ASSOCIATION