

# Conference on Wilms Tumor in WAGR Syndrome

October 18-20, 2018  
Ann Arbor, Michigan

## Summary and Highlights

**INTERNATIONAL WAGR SYNDROME ASSOCIATION**  
Awareness. Research. Support.





## 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

*Emma Gunckle and her mother Jenny*

# 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 (IWSA)**, 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 Gunckle** Michigan

*front left to right:*

**Kelly Trout** Texas  
**Brittany Gelsomino** Illinois  
**Adam Gunckle** Michigan  
**John and Beth Morris** Pennsylvania



**Peter Ehrlich**  
Surgery  
University of Michigan  
C.S. Mott Children's Hospital



**Jeffrey Dome**  
Oncology  
Children's National  
Health System



**Elizabeth Mullen**  
Oncology  
Dana Farber Cancer  
Institute



**David Malkin**  
Oncology/Genetics  
University of Toronto  
Hospital for Sick Children



**Andrew Davidoff**  
Surgery  
St Jude's Children's  
Research Hospital



**Jenny Gunckle**  
Michigan  
Parent



**Adam Gunckle**  
Michigan  
Parent



**Kelly Trout**  
Texas  
Parent



**Janna Hol**  
Oncology (PhD candidate)  
Princess Maxima  
Pediatric Oncology Center  
Netherlands



**Kathleen Kieran**  
Urology  
Seattle Children's Hospital  
University of Washington



**Daniel Green**  
Oncology  
St Jude's Children's  
Research Hospital



**Marry van den Heuvel-Eibrink**  
Oncology  
Princess Maxima Pediatric  
Oncology Center  
Netherlands



**Dolly Carlson**  
Michigan  
Parent



**Shari Krantz**  
Maryland  
Parent



**Alper Dogan**  
Turkey  
Parent



**Rhonda Sena**  
New Mexico  
Parent



**Fred Hoffer**  
Radiology  
Washington State



**Vicki Huff**  
Genetics  
MD Anderson Cancer Center



**Joyce Turner**  
Genetics  
Children's National  
Medical Center  
GW University



**Norbert Graf**  
Oncology  
Saarland University



**Cathryne Cansler**  
Michigan  
Parent



**Beth Morris**  
Pennsylvania  
Parent



**John Morris**  
Pennsylvania  
Parent



**Brittany Gelsomino**  
Illinois  
Parent



**Conrad Fernandez**  
Oncology  
IWK Health Centre  
Dalhousie University



**Andrew Murphy**  
Surgery/Oncology  
St Jude's Children's  
Research Hospital



**Joan Han**  
Endocrinology  
La Bonheur Children's Hospital



**Robert Shamberger**  
Surgery/Oncology  
Harvard Medical/Boston  
Children's Hospital

# Researchers and IWSA Parent Attendees

# Conference Goals



*Parents John and Beth Morris with Andrew Murphy MD*

- 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





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



*Adam Gunckle, parent*

# Parents of children with WAGR syndrome shared their personal stories and experiences with Wilms tumor researchers



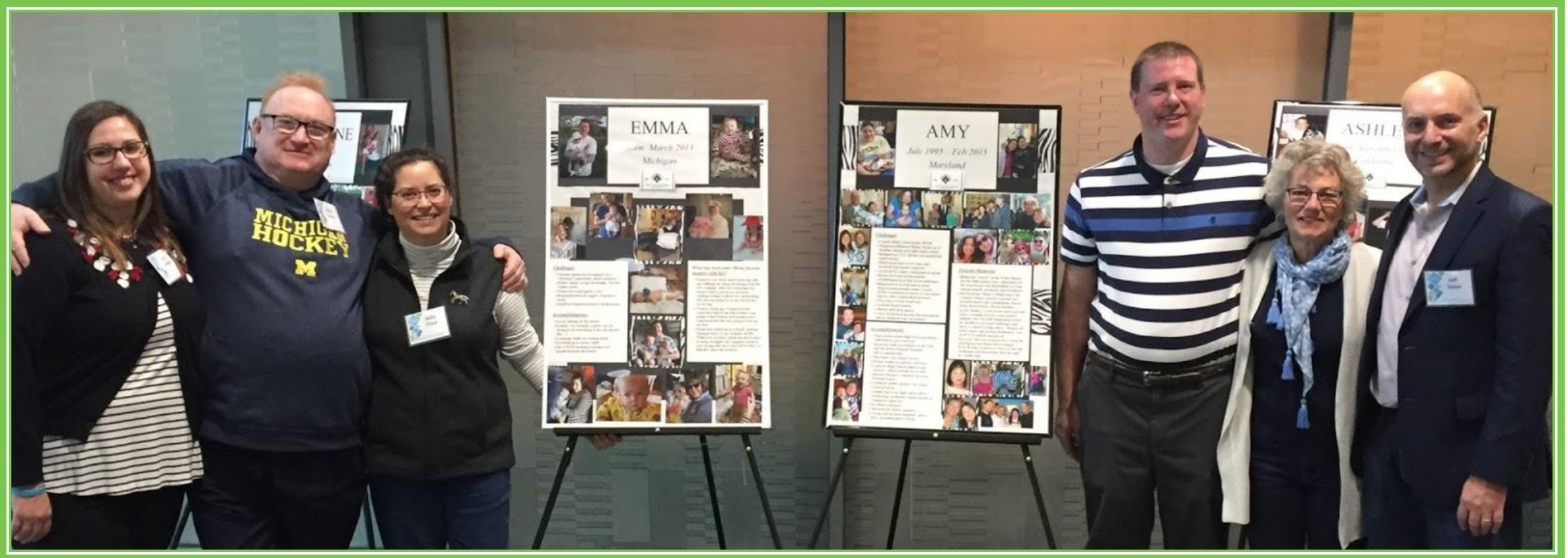
*Parents Brittany Gelsomino and Jenny Gunckle*



*Parents Kelly Trout and Adam Gunckle, PhD candidate Janna Hol, Parent Jenny Gunckle, and Vicki Huff, MD*

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

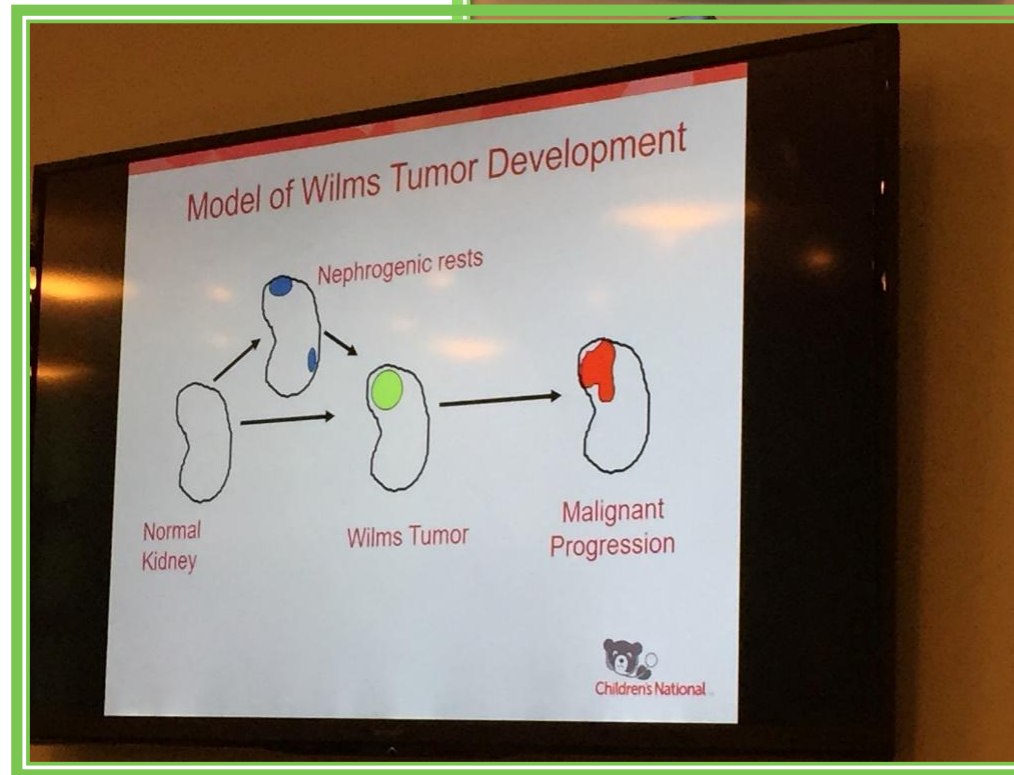


The presentation on WAGR syndrome by Joan Han, MD was critical to Wilms tumor project planning and design

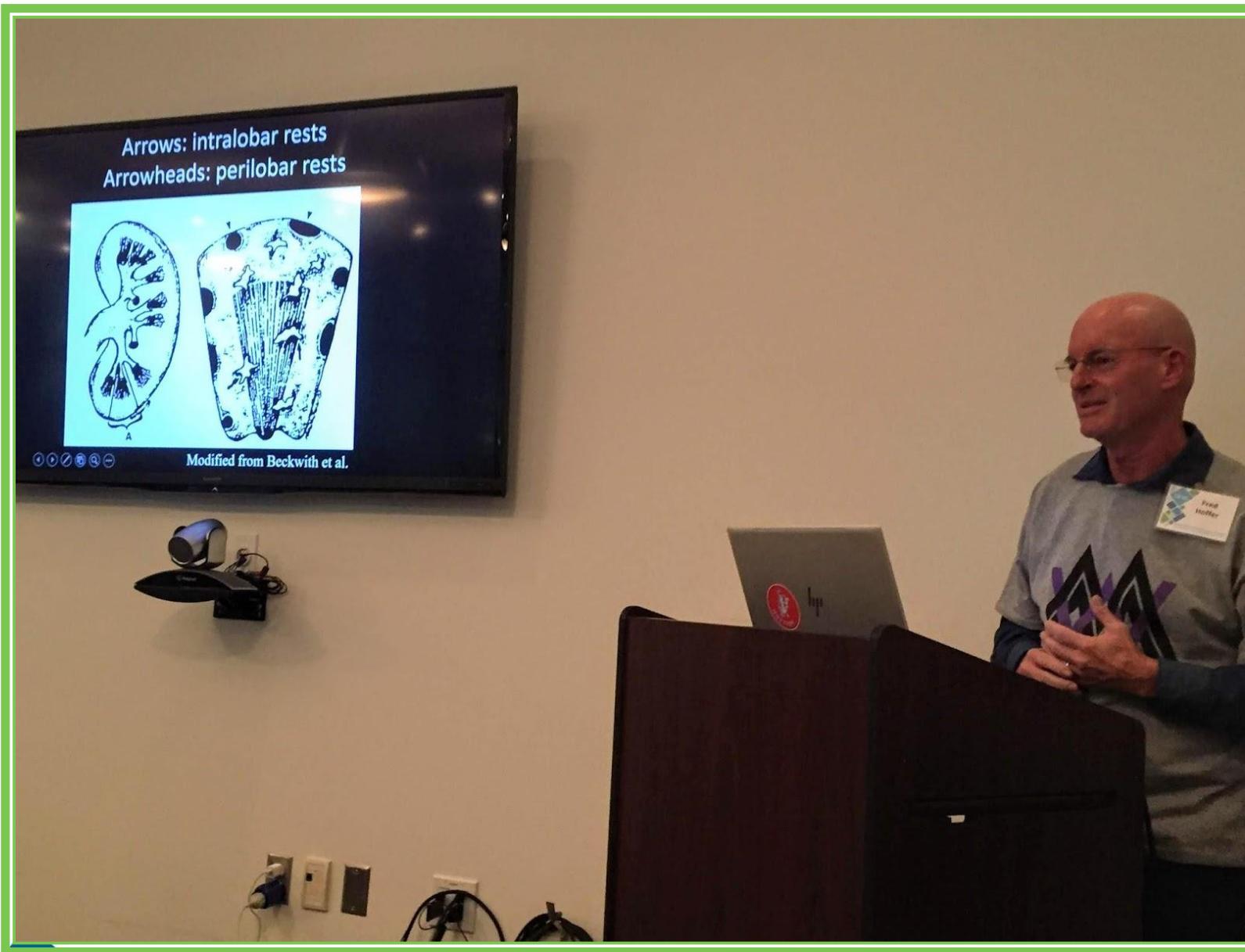


Parents Kelly Trout and Shari Krantz with Joan Han, MD

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



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

The Conference was designed to maximize interaction and discussion



*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



*Parent Brittany Gelsomino*

- **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**

# 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 Research 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***

*Fred Hoffer, MD and Jeff Dome, MD*



*Parents Shari Krantz, Brittany Gelsomino, Cathryne Cansler, Rhonda Sena*

***“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**

