

# Collaboration is Innovation:

Prospects and news in the field of  
WAGR syndrome research



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International WAGR Syndrome Association

Innovative therapy in the field of rare diseases and biotechnology

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# What is **WAGR** syndrome?



## An Acronym for:

- **W**ilms tumor
- **A**niridia
- **G**enitourinary abnormalities
- **R**ange of developmental delays

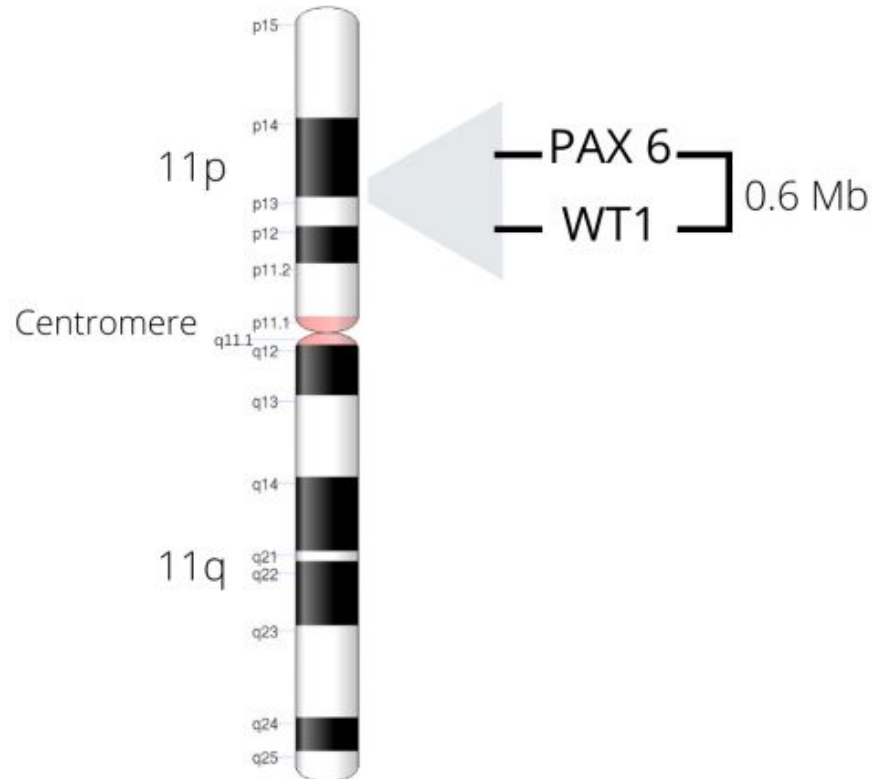
# Associated conditions: **WAGR syndrome**

- Auditory and Sensory Processing
- Behavioral and Psychiatric
- Cardiopulmonary
- Gastrointestinal
- Head, Ears, Nose, Throat
- Metabolic and Endocrine
- Musculoskeletal
- Neurologic
- Sleep

- BDNF gene deletion
  - obesity
  - hyperphagia
  - decreased response to pain
- Chronic kidney disease
  - FSGS
  - hypertension, proteinuria
- Pancreatitis
  - **No Propofol!**

# Diagnosis and Treatment: **WAGR syndrome**

## Chromosome 11: WAGR Syndrome



# International WAGR Syndrome Association

## Mission:

Promote Awareness

Stimulate Research

Support Families

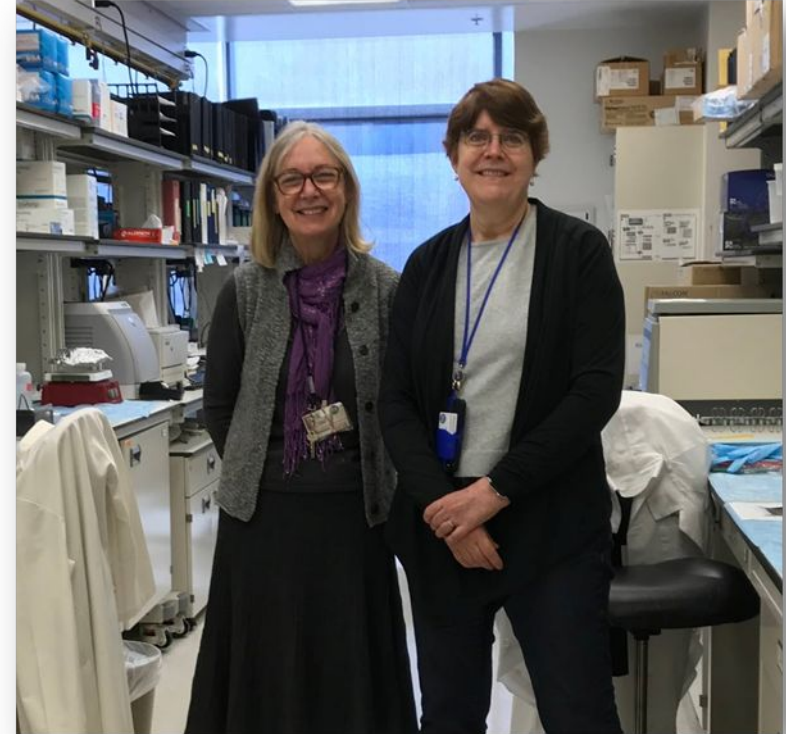




# Research involving WAGR syndrome

- **Wilms tumor**

- International Scientific Symposium
- Publications
- Imaging Study
- Textbook Chapter
- Mouse Model
  - “Chemo-Preventative Agent for Wilms tumor in Children with WAGR syndrome”



Vicki Huff, PhD  
Cristy Ruteshouser, PhD  
MD Anderson Cancer Center  
Houston, Texas

Collaboration

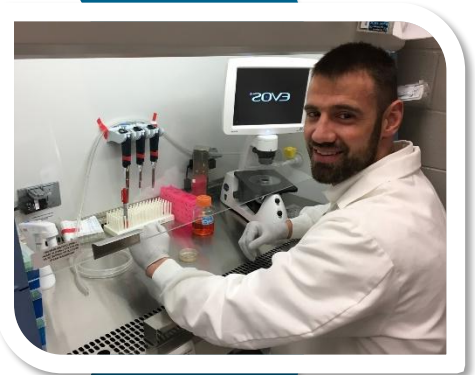


Patients

IWSA



Funders



Researchers



Patient Groups

# Patients and Families

- **Patient Registry**
  - Patient-reported data
  - Recruitment
  - Opportunity for novel data collection
- **“Research-ready” patient community**
  - Informed
  - Motivated
- **Dissemination of results**
  - WAGR.org website
  - Social media





# Researchers



Prof. Dr. Marry van den Heuvel-Eibrink  
Princes Maxima Centrum  
Utrecht, Netherlands

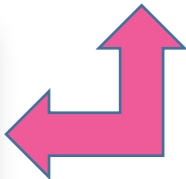
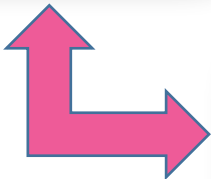
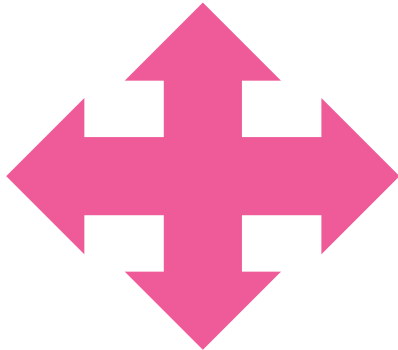
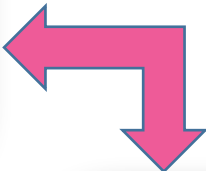
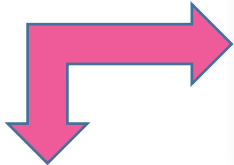


Andrew Murphy, MD  
St Jude Children's Research Hospital  
Memphis, Tennessee



Peter Ehrlich, MD  
CS Mott Children's Hospital  
Ann Arbor, Michigan

**Patient Organizations**





# Funders



2018 PCORI Conference on Wilms Tumor in WAGR Syndrome



# Questions?

International WAGR Syndrome Association

**wagr.org**

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