Collaboration is Innovation:

Prospects and news in the field of WAGR syndrome research

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Innovative therapy in the field of rare diseases and biotechnology

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What is **WAGR** syndrome?

An Acronym for:

- Wilms tumor
- Aniridia
- Genitourinary abnormalities
- Range 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

11p
Centromere
11q

PAX 6
WT1
0.6 Mb
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
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
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Memphis, Tennessee

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2018 PCORI Conference on Wilms Tumor in WAGR Syndrome
Questions?

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