KDIGO Controversies Conference

Common Elements in Uncommon Kidney Diseases

CONFERENCE OVERVIEW & OBJECTIVES

KDIGO

Olivier Devuyst & Lisa Guay-Woodford

Amsterdam, June 16-19, 2016

The Global Burden of Rare Diseases

- Rare diseases: ~6,000 – 8,000 disorders
- Affect ~30 M patients in EU and in the USA, > 300 M worldwide
- 80% of rare diseases have a genetic origin
- Typical challenges: variable phenotypes, fragmented data, lack of standards, poor knowledge for disease mechanisms and natural history

KDIGO

Rare Kidney Diseases

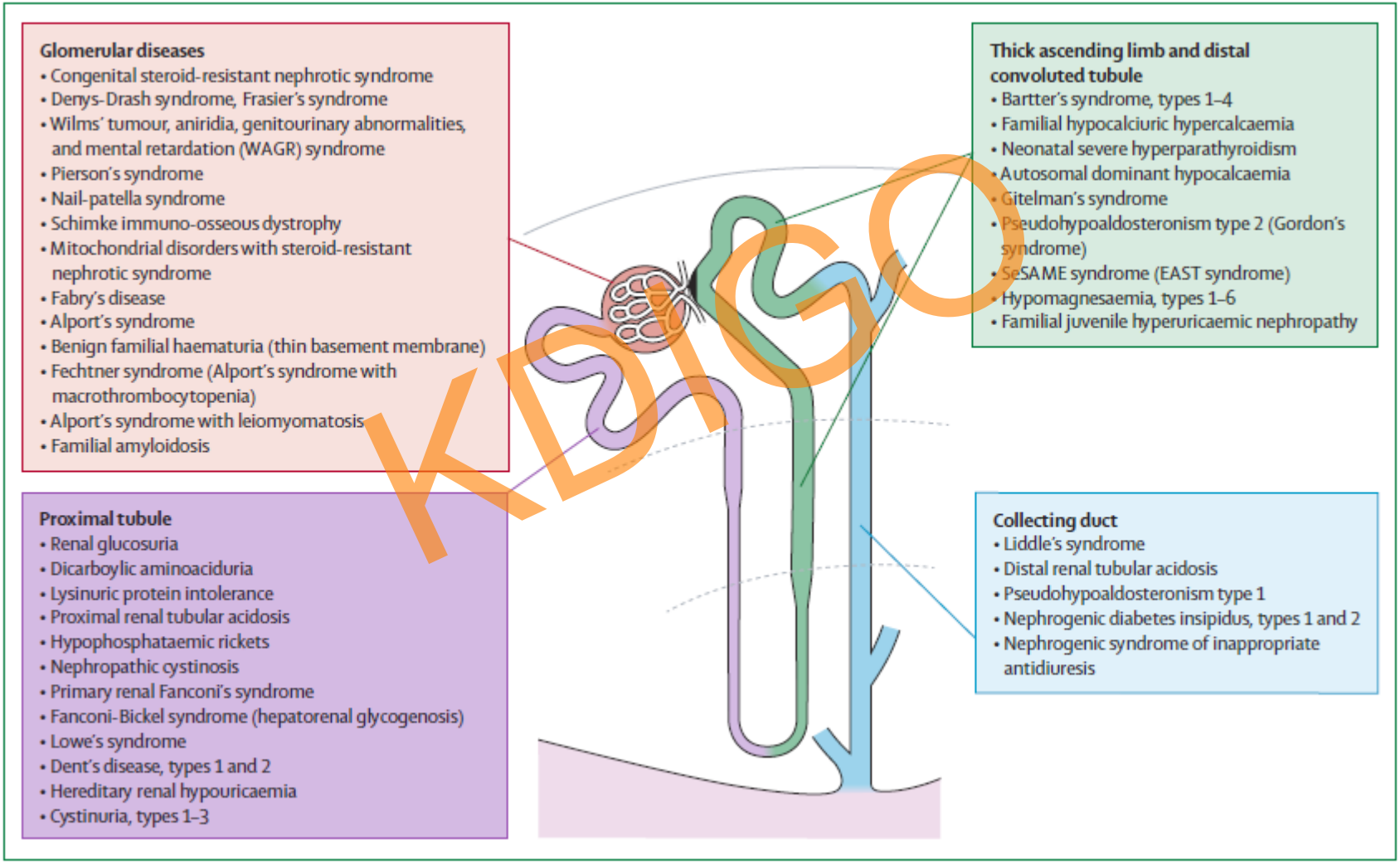
- Rare kidney diseases: > 150 disorders
- Overall prevalence: ~60-80 cases per 100,000
- At least 10% adults and virtually all children on RRT
- Fifth most common cause of ESRD (Diab > HT > GN > PyeloN)

KDIGO

- Patients with inherited kidney disorders rarely die (progresses in RRT)
- However: poor health, poor quality of life, multisystemic complications

→ *children with severe congenital nephropathies can be dialysed from neonatal age onwards, but face many decades of life with ESRD and have a high likelihood of changes in physical, cognitive, and psychosocial development.*

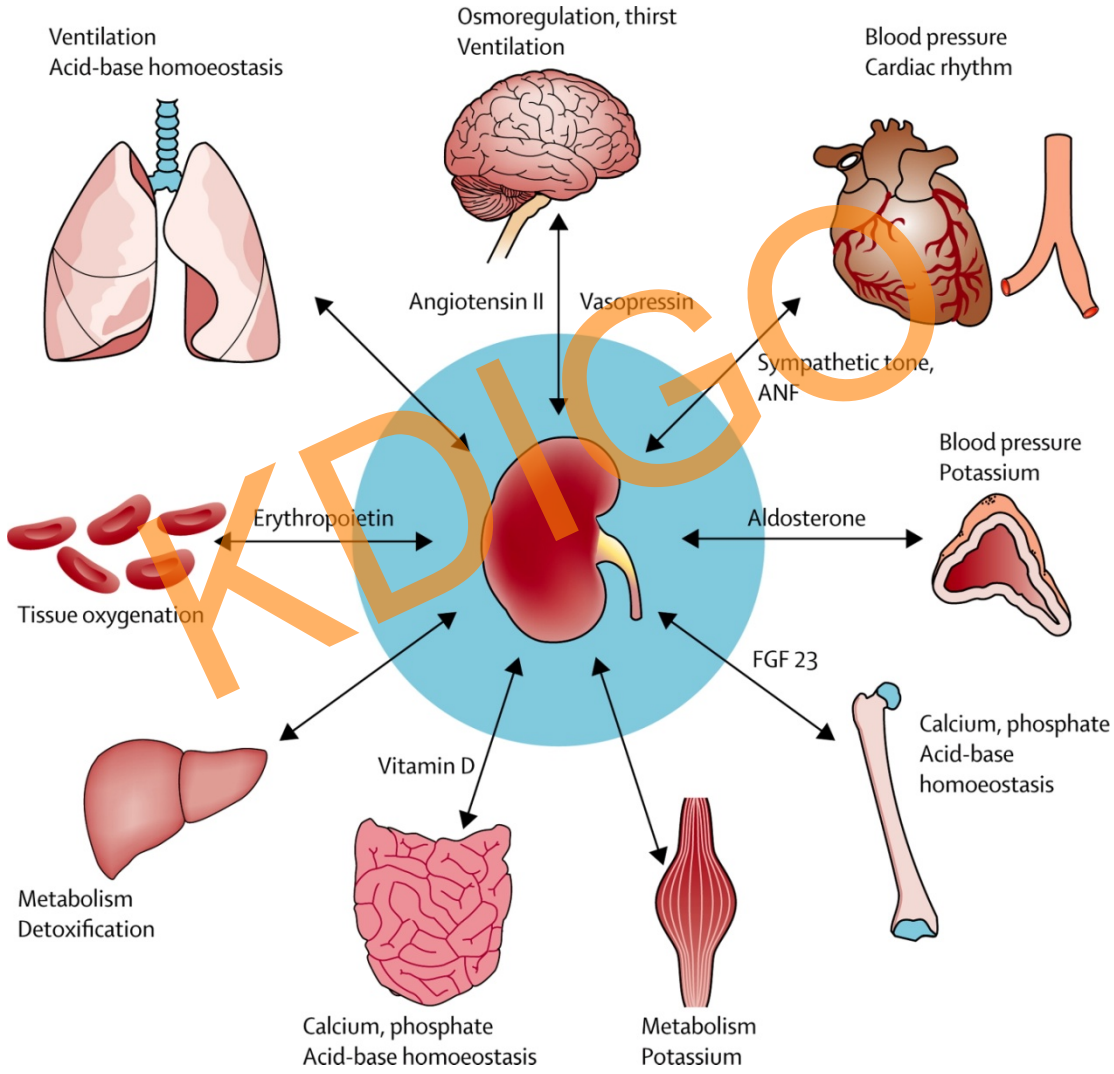
Inherited Kidney Disorders: Segmental Distribution



KDIGO

Devuyst O, et al. Lancet **383**, 2014

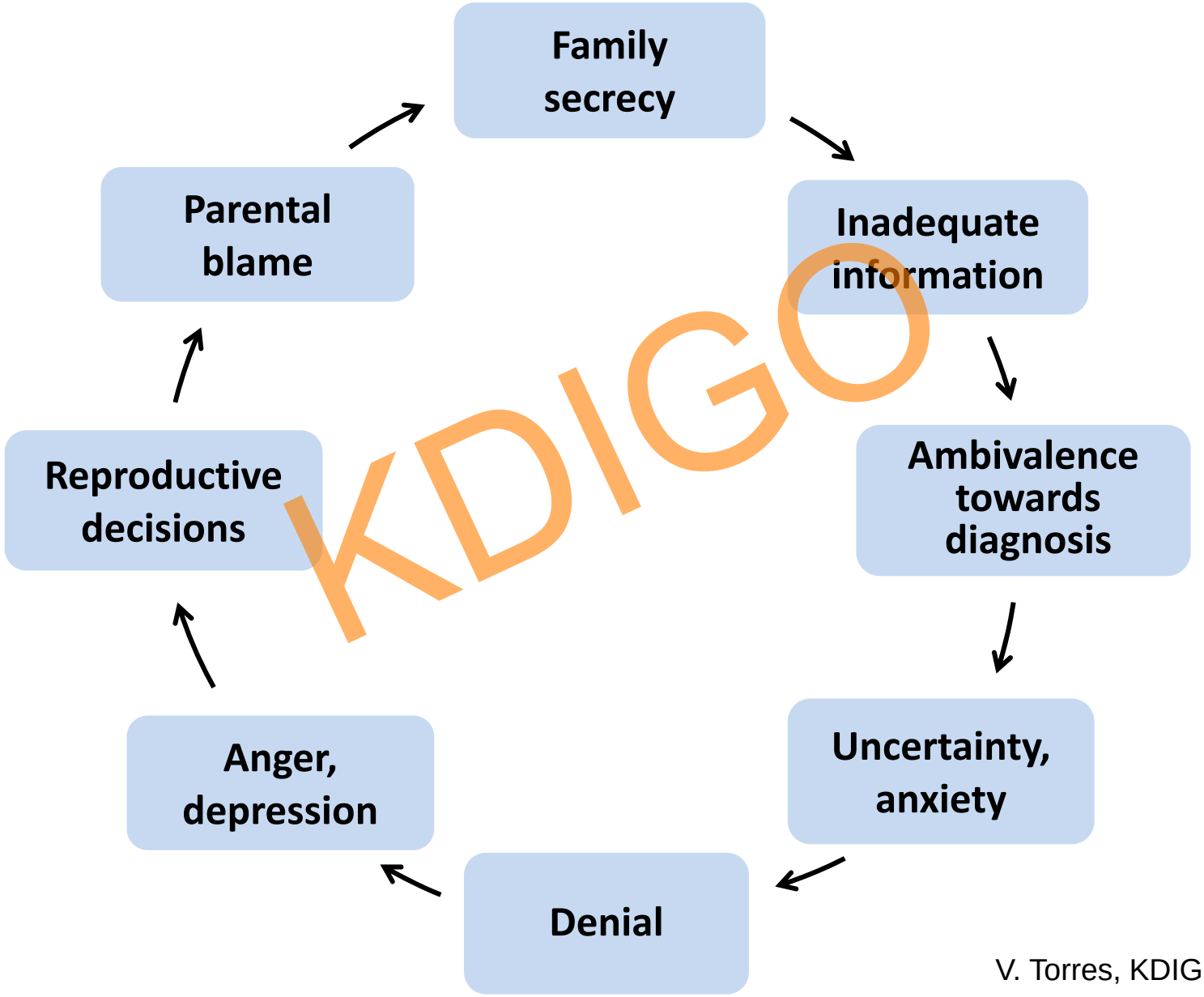
Kidney Function and Homeostasis



KDIGO

Eckardt KU, et al. Lancet **382**, 2013

Emotional Burden of an Inherited Disease



KDIGO

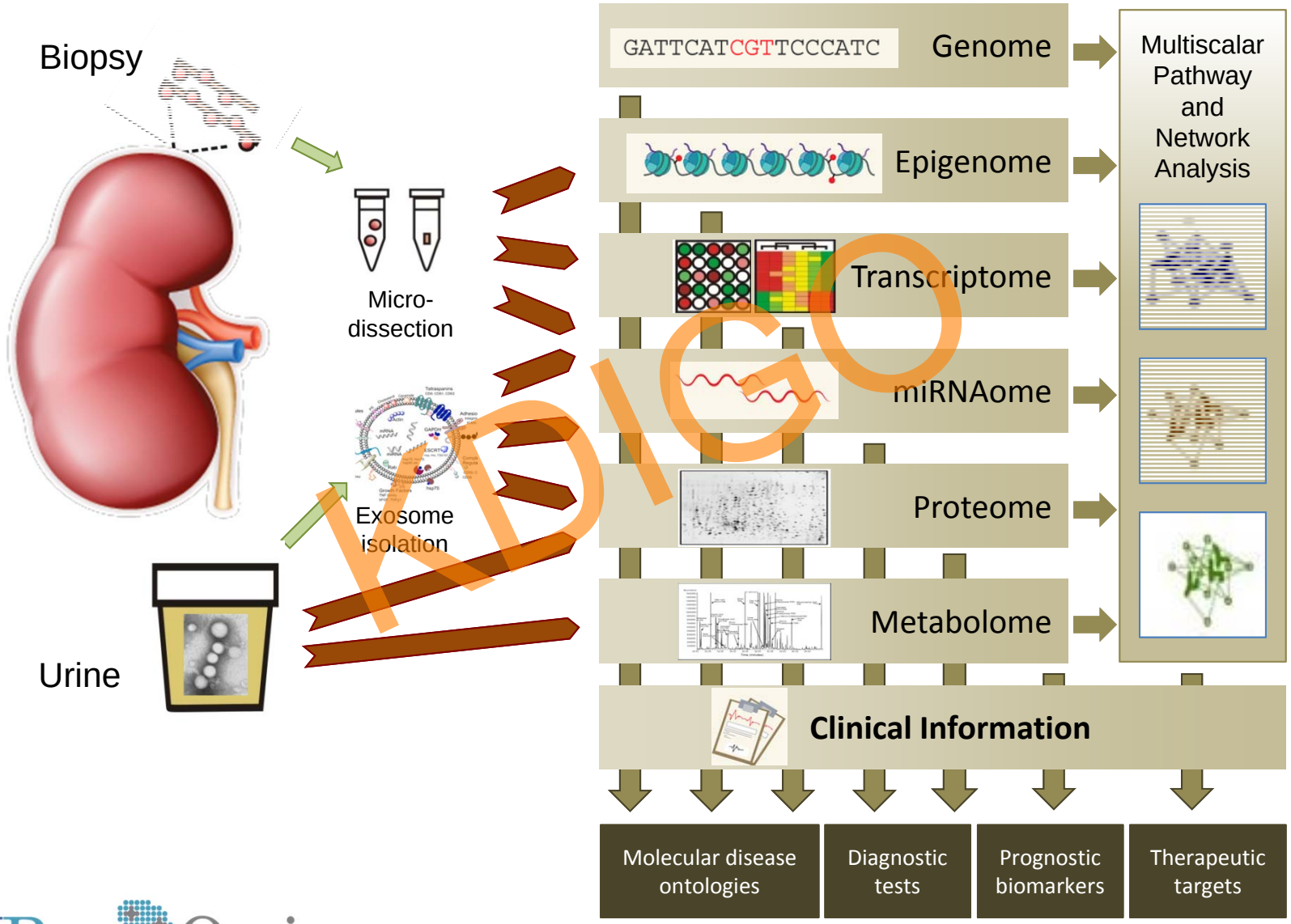
V. Torres, KDIGO-ADPKD

Rare Inherited Kidney Disorders:

Opportunities

KDIGO

Application of -omics technologies in rare kidney diseases



KDIGO

EURenOmics

Research Programmes, Cohorts, Biorepositories

Fragmentation of patient-related information represents a major obstacle for rare disease research.

KDIGO

- EPIRARE: European Platform for Rare Disease Registries (www.epirare.eu)
- PARENT: Patient Registries Initiative (www.patientregistries.eu)
- RD-CONNECT: An integrated platform connecting databases, registries, biobanks and clinical bioinformatics for rare disease research (www.rd-connect.eu)
- IRDiRC: International Rare Diseases Research Consortium (www.irdic.org)
- EURenOmics: European Consortium for High-Throughput Research in Rare Kidney Diseases (www.eurenomics.eu)
- ORPHANET: The portal for rare diseases and orphan drugs (www.orpha.net)
- EURORDIS: The European Organization for Rare Diseases (www.eurordis.org)
- Center for Mendelian Genomics (www.mendelian.org)

Inherited Kidney Disorders: Health Policies

- *To ensure that approaches developed at highly specialized tertiary care centers can be adopted in facilities that cover the majority of population.*

- *To promote the implementation of clinically relevant genetic testing and to ensure delivery and impact of genetic information to physicians, patients and society in general.*

KDIGO

orphanet

EURORDIS
Rare Diseases Europe

NORD
National Organization for Rare Disorders

EDITORIAL

Patient Organizations and Research on Rare Diseases

Julie R. Ingelfinger, M.D., and Jeffrey M. Drazen, M.D.

N Engl J Med 2011; 364:1670-1671 | April 28, 2011 | DOI: 10.1056/NEJMe1102290

Comment on: Efficacy and safety of sirolimus in lymphangioleiomyomatosis.

KDIGO

CYSTINOSIS RESEARCH FOUNDATION

PKD FOUNDATION
Polycystic Kidney Disease

AIRG France
Association pour l'Information et la Recherche sur les maladies Rénales Génétiques

OHF
The Oxalosis & Hyperoxaluria FOUNDATION

The Lowe Syndrome Foundation

FEDERG

GEBERT RÜF STIFTUNG
WISSENSCHAFT.BEWEGEN

Common Elements in Uncommon Kidney Diseases:
Challenges and Topics

- Technological advances in diagnosis
- Consequences of improved genetic diagnosis
- Management of renal function, optimal pediatric transition care
- Challenges in trial design and conduct
- Development of novel biomarkers or surrogates
- Translation of new knowledge into clinical programs
- QOL issues
- Policy initiatives – various parts of the world

→ Implementation of the resources in low-income countries ?

KDIGO

KDIGO Controversies Conference
Common Elements in Uncommon Kidney Diseases

- Address common clinical and patient issues across the field of rare kidney diseases.
- Identify controversial topics and outstanding knowledge gaps
- Propose a research agenda to resolve these issues
- Determine whether there is sufficient evidence base for providing guidance in clinical management
- Systematically integrate patient perspectives

KDIGO

Controversies Conferences examine what is known, what can be done with what is known and what needs to be known on topics of clinical relevance in nephrology.

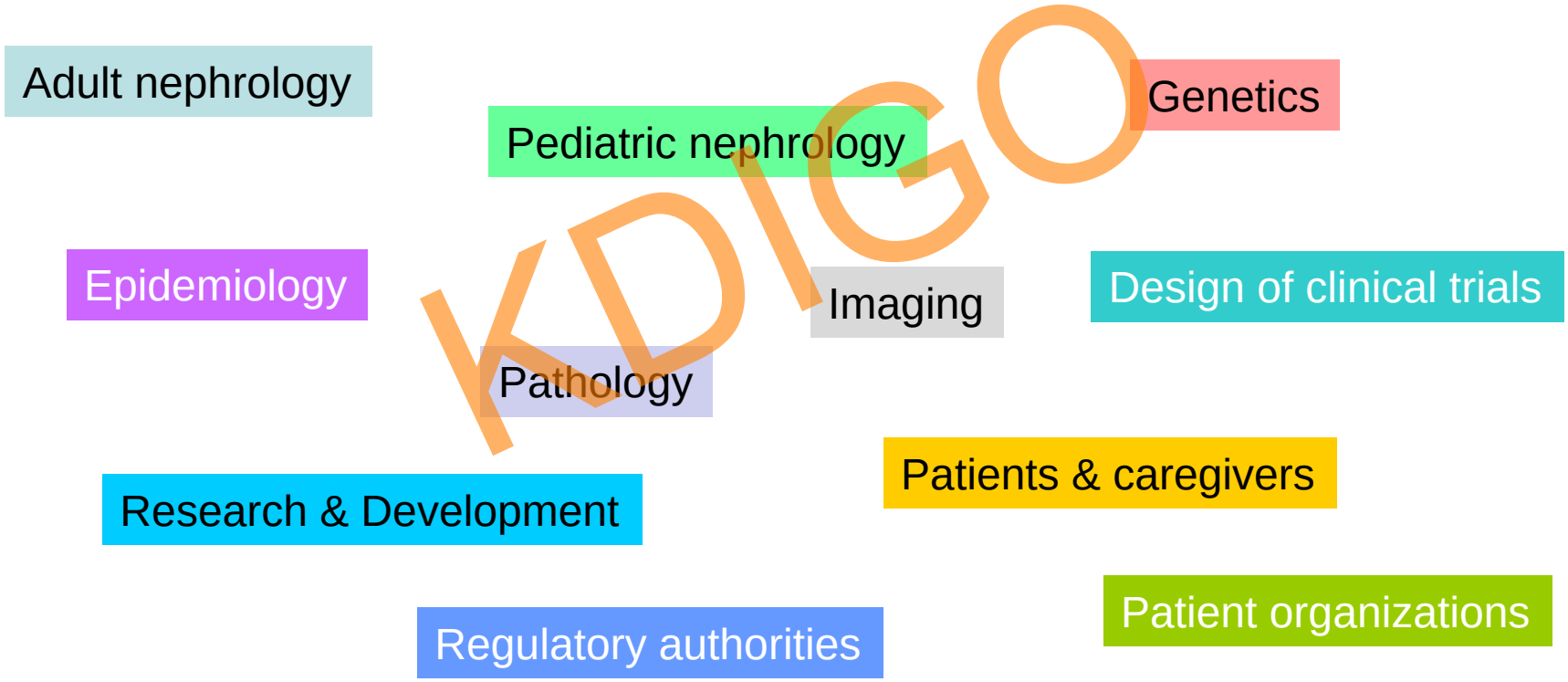
- Current practice recommendations
- Clinical questions and outstanding issues
- Research agenda

- Gitelman Syndrome (Brussels, February 2016)
- Complement-Mediated Kidney Diseases (Barcelona, November 2015)
- Fabry Nephropathy (Dublin, October 2015)
- Nephropathic Cystinosis (Lisbon, December 2014)
- Autosomal Dominant Tubulointerstitial Kidney Disease (ADTKD) (Boston, September 2014)
- Autosomal Dominant Polycystic Kidney Disease (ADPKD) (Edinburgh, January 2014)

KDIGO

KDIGO Controversies Conference
Common Elements in Uncommon Kidney Diseases

Global panel – Multidisciplinary expertise



KDIGO


FROM
CHAOS
ORDER
LETTRE COMPTE DOUBLE
MOT COMPTE DOUBLE

Plenary Sessions 1
Friday June 17, 9:00 to 18:30 hrs

- Plenary lectures covering major themes
- Balanced review of the literature
- Highlighting controversial issues
- Introduction to the prioritized breakout questions

KDIGO

Global Action. Local Change.

KDIGO Controversies Conference on Common Elements in Uncommon Kidney Diseases

June 16-19, 2016

Amsterdam, The Netherlands

KDIGO

Scope of Work

KDIGO Controversies Conference on Common Elements in Uncommon Kidney Diseases
- Breakout Group Roster -

Conference Co-Chairs
Olivier Devuyst - Switzerland
Lisa Guay-Woodford - USA

Diagnostic Challenges *(Amsterdam Room)*		**Management of Renal Function Decline** *(Haarlem - Eindhoven Room)*		**Challenges in Clinical Study Design in Rare Kidney Diseases** *(Rotterdam Room)*		**Translation from Research to Clinical Care** *(Den Haag Room)*		**Practical & Integrated Patient Support** *(Koepelzaal Room)*	
Breakout Group Co-Chairs									
Klein	Jon (US)	Bockenhauer	Detlef (UK)	Day	Simon (UK)	Aymé	Ségolène (FR)	Roberts	Julia (US)
Knoers	Nine (NL)	Perrone	Ron (US)	Schaefer	Franz (DE)	Ingelfinger	Julie (US)	Torres	Vicente (US)
Breakout Group Members									
Antignac	Corinne (FR)	Bleyer	Anthony (US)	Cnaan*	Avital (US)	Angelis	Aris (UK)	Baron*	David (US)
Bae	Kyongtae (US)	Budde	Klemens (DE)	Cornel	Martina (NL)	Bull	Katherine (UK)	Bos	Marjolein (NL)
Bergmann	Carsten (DE)	Chauveau	Dominique (FR)	Flamion	Bruno (BE)	Goodship	Tim (UK)	Cosyns	Etienne (BE)
Goodyer	Paul (CA)	Ding	Jie (CN)	Langman	Craig (US)	Gross	Oliver (DE)	Gear	Susie (UK)
Harris	Peter (US)	Hogan	Marie (US)	Mariz	Segundo (UK)	Horie	Shigeo (JP)	Harr	Nicole (US)
Höfele	Julia (DE)	Hoorn	Ewout (NL)	Rayner	Brian (ZA)	Kashtan	Cliff (US)	Harris	Tess (UK)
Konrad	Martin (DE)	Ong*	Albert (UK)	Remuzzi	Giuseppe (IT)	Kleta	Robert (UK)	Kerecuk	Larissa (UK)
Pei	York (CA)	Pirson	Yves (BE)	Servais	Aude (FR)	Levtchenko	Elena (BE)	McKerracher	Gayle (UK)
Summar*	Marshall (US)	Salomon	Rémi (FR)	Smith	Richard (US)	Ortiz	Alberto (ES)	Nieuwenhoven	Annet (NL)
Torra	Roser (ES)	Stengel	Bénédicte (FR)	van't Hoff	William (UK)	Soliman	Neveen (EG)	Odland	Dwight (US)
Vargas-Poussou	Rosa (FR)			Wanner	Christoph (DE)	Yap	Hui-Kim (SG)	Renault	Daniel (FR)
								Storm	Marjolein (NL)
								Vroom	Elizabeth (NL)

KDIGO

Breakout Session 1
Saturday, June 18, from 8:30 to 12:30 hrs

- Manage time to address prioritized questions
- Discussion leaders: moderate & take notes
- Objective: Reach conclusions regarding
    - Areas of consensus
    - Areas of controversy
    - Gaps in knowledge
    - Need for interactions - expertise
- Prepare report to for Plenary Session

KDIGO

Plenary Session 2
Saturday, June 18, 13:30 to 16:00 hrs

- Preliminary report from Breakout Groups

  (20 min each)

- Questions/comments to Breakout Groups

  (10 min each)

KDIGO

Breakout Session 2
Saturday, June 18 from 16:15 to 18:30 hrs

- Manage time to address prioritized questions
- Objective:
  - Discuss feedback from Plenary Session
  - Finalize areas of consensus, controversy and gaps in knowledge
  - Propose research agenda - controversies & knowledge gaps
  - Determine whether there is sufficient evidence base for providing guidance in clinical management
- Prepare report for last Plenary Session

KDIGO

Final Plenary Session
Sunday, June 19 from 08:00 to 12:30 hrs

- Final reports from Breakout Groups
  (30 min each)
- Discussion (20 min each)
- Final Conference Summation: Wrap up and next steps

KDIGO

Post-Conference Report and Publication

- Medical writer (Jennifer King) to establish a draft, sent to co-chairs of each Breakout Group and conference co-chairs
- Report from co-chairs of each Breakout Group due to the conference co-chairs
- Compiled and revised manuscript by conference co-chairs due to conference participants
- Feedback from conference participants to conference co-chairs
- Manuscript submitted to *Kidney International* by the end of 2016
- Authorship – bloc & names for all participants

KDIGO

Enjoy the meeting - Thank you !