Global Innovations in Patient-Centered Kidney Care

Panel 7: Government: Agent of Change

May 23, 2019

50th ANNIVERSARY

AAKP
A Legacy of Patient Engagement & Advocacy

School of Medicine & Health Sciences
THE GEORGE WASHINGTON UNIVERSITY

Drug Development for Kidney Diseases: A Regulatory Perspective

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Center for Drug Evaluation and Research

- The **Center for Drug Evaluation and Research (CDER)**, within the US Food and Drug Administration, is tasked with making sure that safe and effective drugs are available to improve the health of people in the United States.

- The **Office of New Drugs**, which resides in CDER, provides regulatory oversight for investigational studies during drug development and makes decisions about marketing approval of new drugs.

Outline

- Observations about the changing landscape of drug development for kidney diseases
- Patient-Focused Drug Development: the importance of the patient voice and initiatives to incorporate

Disclaimers and Disclosures

- The views expressed in this talk represent my opinions.
- I have no financial relationships to disclose.

My sense of things

- Landscape for drug development for kidney diseases has changed dramatically over the last 10 years.
- There is, I think, a sense of hope (born from some recent successes) and greater interest on the part of industry in developing treatments for kidney diseases (or at least some types of kidney diseases).
- It feels as if we are entering a new era for drug development for kidney diseases. The “dog days” are over.*

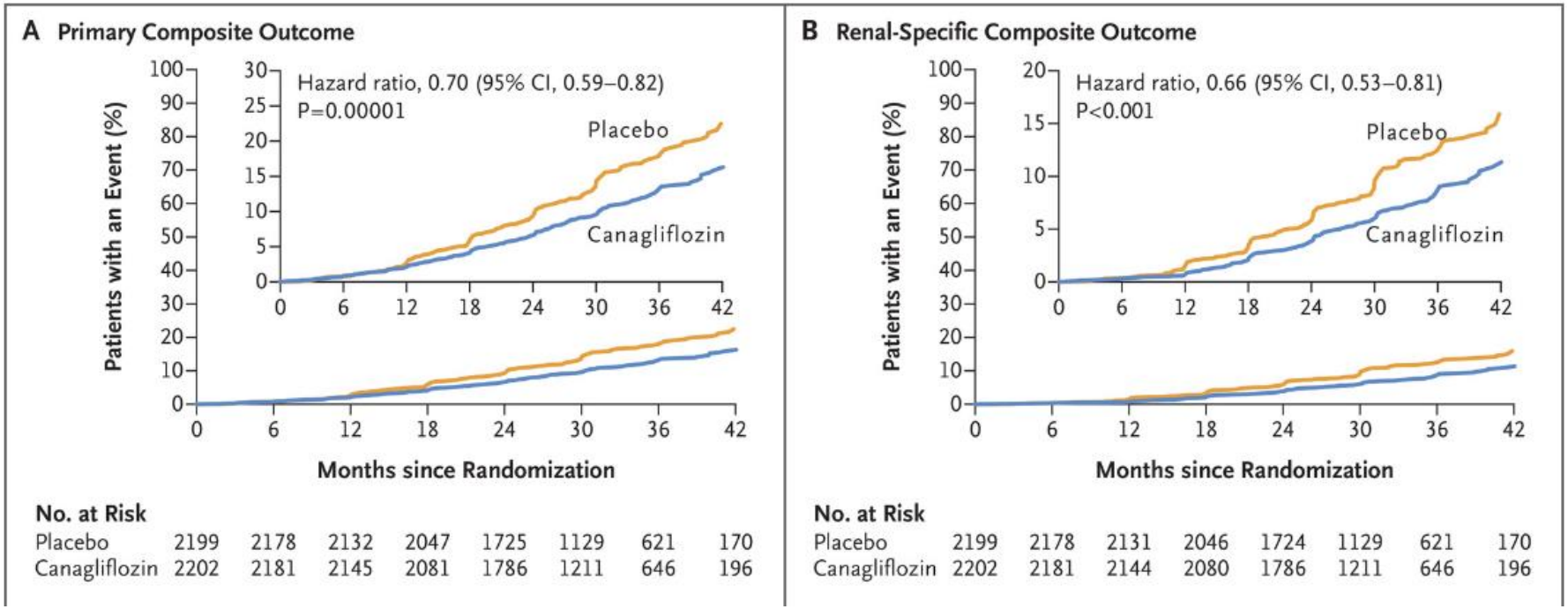
**1 : the period between early July and early September when the hot sultry weather of summer usually occurs in the northern hemisphere; taken to be the hottest, most uncomfortable part of summer.*

2 : a period of stagnation or inactivity

ORIGINAL ARTICLE

Canagliflozin and Renal Outcomes in Type 2 Diabetes and Nephropathy

Vlado Perkovic, M.B., B.S., Ph.D., Meg J. Jardine, M.B., B.S., Ph.D., Bruce Neal, M.B., Ch.B., Ph.D., Severine Bompoint, B.Sc., Hiddo J.L. Heerspink, Pharm.D., Ph.D., David M. Charytan, M.D., Robert Edwards, M.P.H., Rajiv Agarwal, M.D., George Bakris, M.D., Scott Bull, Pharm.D., Christopher P. Cannon, M.D., George Capuano, Ph.D., et al., for the CREDENCE Trial Investigators*



NEJM; April 14, 2019

The NEW ENGLAND JOURNAL of MEDICINE

EDITORIAL

Clinical Credence — SGLT2 Inhibitors, Diabetes, and Chronic Kidney Disease

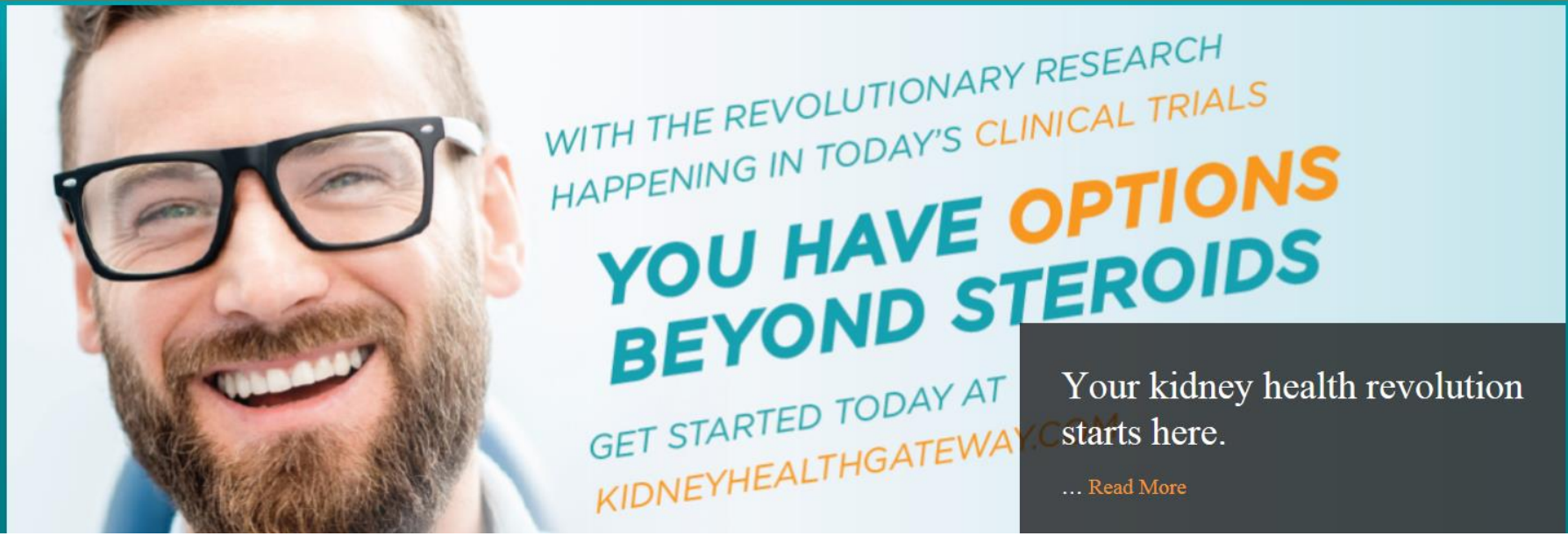
Julie R. Ingelfinger, M.D., and Clifford J. Rosen, M.D.

Overall, the importance of CREDENCE,[9] a well done and large clinical trial, cannot be overstated. The investigators estimated that among 1000 patients treated for 2.5 years, 22 would need to be treated with canagliflozin to prevent the composite primary outcome of end-stage kidney disease, doubling of the serum creatinine level, or renal or cardiovascular death. In addition, among the same number of patients, canagliflozin treatment would prevent 22 hospitalizations for heart failure and 25 composite events of cardiovascular death, myocardial infarction, or stroke. Such data are certain to be welcomed by patients with diabetes and chronic kidney disease and by the clinicians who treat them.

Some metrics, albeit imperfect, of interest in this space

- Steady flow of meeting requests and investigational new drug applications for drugs being developed to treat kidney diseases.
- Drug development programs progressing to later stages of drug development and embarking on well-designed phase 3 trials (*the pivotal efficacy and safety trials supporting a marketing application/drug approval*).
- Increasing number of meetings being organized by the community related to drug development for kidney diseases and initiatives in this space.

KidneyHealthGateway.com - Your kidney health revolution starts here.



Clinical trials and research studies are vital in the mission to find better treatments and cures for Nephrotic Syndrome. When you participate in a clinical trial, not only can you gain access to cutting-edge treatment options and expert care, but **patient participation in clinical trials is the only way that new treatments will become possible.**

Nephrotic Syndrome patients have a great reason to feel hope, because today, there are more clinical trials for Nephrotic Syndrome conditions than ever before.

How did we get here?

NIH National Institute of Diabetes and Digestive and Kidney Diseases

NEPHCURE® Kidney International
Saving Kidneys • Saving Lives

AAKP
A Legacy of Patient Engagement & Advocacy

PKD CONSORTIUM
CRITICAL PATH INSTITUTE

Polycystic Kidney Disease Outcomes Consortium

IT TAKES A VILLAGE

National Kidney Foundation®

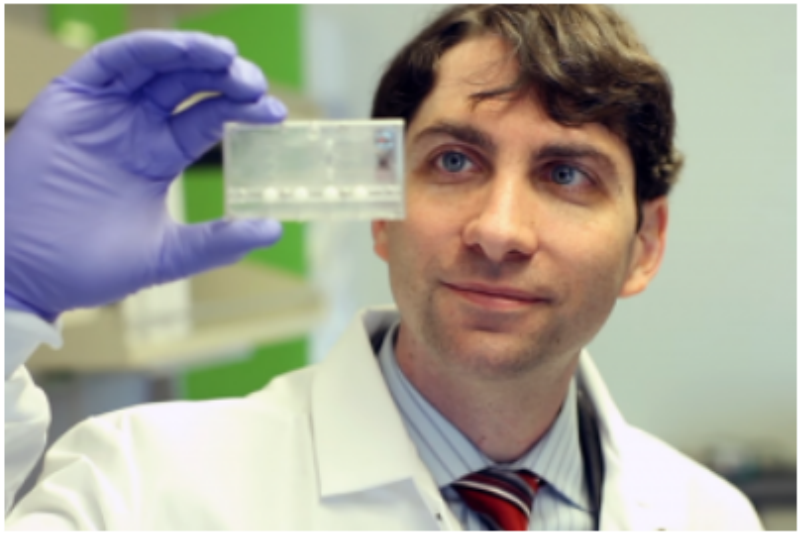
- 2012 NKF-FDA Workshop: GFR Decline as an End Point in Clinical Trials in CKD
- 2018 NKF-FDA-EMA Workshop

KHI
KIDNEY HEALTH INITIATIVE

- Identifying Surrogate Endpoints for Clinical Trials in IgA Nephropathy
- Surrogate Endpoints in Focal Segmental Glomerulosclerosis
- Identification of Appropriate Endpoints for Clinical Trials in Hyperoxaluria

It takes willingness to share data.

Accelerating New Clinical Trials and Treatments for Kidney Disease



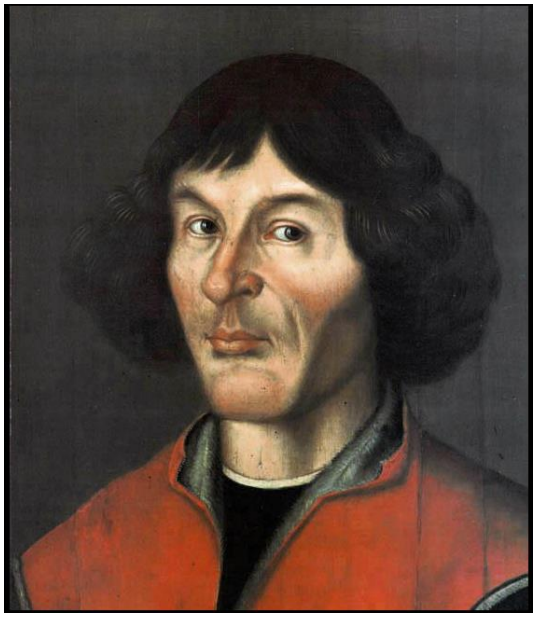
Largest Compilation of Kidney Data Ever Collected is Analyzed and Presented at Joint Scientific Workshop

Silver Spring, MD—March 16, 2018—Today, the National Kidney Foundation (NKF), along with the Food and Drug Administration (FDA) and the European Medicines Agency (EMA) concluded a scientific workshop collaboration to review the results of a major, multi-year meta-analysis examining the largest compilation of data ever collected on chronic kidney disease (CKD). The groups gathered reviewed the data supporting the use of earlier markers of kidney disease progression as endpoints in clinical trials for early stages of CKD. These clinical trials may lead to treatments that will improve the lives of people with kidney disease. The extensive meta-analysis included data on nearly two million participants.

NKF-FDA-EMA Workshop: *Change in Albuminuria and GFR as End Points for Clinical Trials in Early Stages of Chronic Kidney Disease*

Outline

- Observations about the changing landscape of drug development for kidney diseases
- Patient-Focused Drug Development: the importance of the patient voice and initiatives to incorporate



“Finally, we shall place the Sun himself at the center of the Universe.”

-**Nicolaus Copernicus** (1473 –1543), a mathematician and astronomer who developed a model of the universe that placed the sun rather than the earth at the center of the universe. (https://en.wikipedia.org/wiki/Nicolaus_Copernicus)

PATIENT-FOCUSED DRUG DEVELOPMENT

"**Patient-focused drug development** (PFDD) is a **systematic approach to help ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation. As experts in what it is like to live with their condition, patients are uniquely positioned to inform the understanding of the therapeutic context for drug development and evaluation.**"

https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development

Overarching goals

- Facilitating and advancing use of **systematic approaches to collecting and utilizing robust and meaningful patient and caregiver input** to more consistently inform drug development and regulatory decision-making.
- Encouraging identification and use of **approaches and best practices to facilitate patient enrollment** and **minimizing the burden of patient participation** in clinical trials.

https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development

Overarching goals

- Enhancing understanding and appropriate use of **methods to capture information on patient preferences** and the **potential acceptability of tradeoffs between treatment benefit and risk outcomes**.
- Identifying the **information that is most important to patients related to treatment benefits, risks, and burden**, and **how to best communicate** the information to support their decision making.

https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development

21st Century Cures and PDUFA VI

FDA will develop a series of guidance on the collection of patient experience data, and the use of such data and related information in drug development.

FDA-led PFDD Meetings

From 2012 to 2017, under the fifth authorization of PDUFA (PDUFA V), FDA conducted disease-specific PFDD meetings to obtain the patient perspective.

Externally-led PFDD Meetings

Patient organizations identify and organize patient-focused collaborations to generate public input on other disease areas.

https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development

National Kidney Foundation®

Kidney Patients with C3G are Center of FDA Patient-Focused Meeting

Patients share with FDA experience of living with this rare form of kidney disease

Alport Syndrome and Its Challenges Focus of Early August Meeting with FDA

AUGUST 13, 2018 BY IQRA MUMAL, PHD IN NEWS.

National Kidney Foundation's and IgA Nephropathy Foundation's Externally-Led Patient-Focused Drug Development (EL-PFDD) Meeting on IgA Nephropathy

Closing Comments

- It **takes a village** to successfully develop safe and effective treatments.
- There have been **important scientific advances** in the understanding of a number of kidney diseases.
- There is also **interest on the part of industry** in developing treatments for these diseases.
- As the person who is the expert in the disease and who will ultimately use these therapies, **patients need to be involved.**

QUESTIONS?

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