KIDNEY DISEASE CLINICAL TRIALISTS

Virtual Workshop 2020

SEPTEMBER 25-26, 2020

SCIENTIFIC PROGRAM

WELCOME MESSAGE

DEAR COLLEAGUES,

I AM PLEASED TO WELCOME YOU TO THE 5TH KIDNEY DISEASE CLINICAL TRIALISTS WORKSHOP, PREVIOUSLY THE INI-CRCT, TAKING PLACE THIS YEAR IN A VIRTUAL FORMAT, IN RESPONSE TO THE EXCEPTIONAL CIRCUMSTANCES WE FIND OURSELVES IN.

OVER THE NEXT TWO DAYS, THE KDCT WORKSHOP WILL FOSTER AN INTERNATIONAL EXCHANGE OF IDEAS WHERE WE WILL BRAINSTORM ON TRIAL DESIGN, CONDUCT, ETHICS, INTERPRETATION, APPROVABILITY AND IMPLEMENTATION ENCOMPASSING DRUGS, DEVICES, BIOMARKERS AND THERAPEUTIC STRATEGIES FOR KIDNEY DISEASE.

OUR OBJECTIVES ARE TO PRODUCE RELEVANT DATA FROM CONTROLLED KIDNEY DISEASE CLINICAL TRIALS THAT WILL CONTRIBUTE TO BETTER CLINICAL CARE AND TO UNDERSTAND THE PROBLEMS ASSOCIATED WITH MAKING DECISIONS ABOUT WHAT CONSTITUTES RELEVANT INFORMATION, HOW TO IMPROVE KIDNEY DISEASE CLINICAL TRIALS, AND, AS IS COMMONLY THE CASE, HOW TO SATISFY REGULATORY AUTHORITIES AND PAYERS.

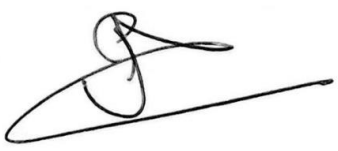
WE ARE DELIGHTED ONCE AGAIN TO WELCOME A NUMBER OF DISTINGUISHED NEPHROLOGISTS, CLINICAL TRIALISTS, PRINCIPAL INVESTIGATORS AND STATISTICIANS FROM ACADEMIA JOINING US FROM EUROPE, NORTH AMERICA AND AUSTRALIA AS WELL AS NIH, EMA, MHRA AND FDA REPRESENTATIVES, PAYERS, PATIENT ADVOCATES AND INDUSTRY ATTENDEES REPRESENTING R&D PHARMA AND DEVICE COMPANIES.

I WILL TAKE THIS OPPORTUNITY TO THANK THE SCIENTIFIC COMMITTEE, ALL SPEAKERS, DISCUSSANTS AND MODERATORS, AS WELL AS PARTICIPANTS FROM INDUSTRY, FOR YOUR COOPERATION AND SUPPORT IN HELPING MAKE THIS WORKSHOP COME TOGETHER.

SHOULD YOU HAVE ANY QUESTIONS, PLEASE REACH OUT TO ME VIA CHAT, EMAIL OR BY PHONE, AS WELL AS TO THE DEDICATED TEAM AT OVERCOME WHO WILL BE READY TO ASSIST YOU.

WITH MY WARMEST REGARDS,

PROFESSOR PATRICK ROSSIGNOL




KIDNEY
DISEASE
CLINICAL
TRIALISTS
Virtual
Workshop
2020
U.S. CAPITOL - WASHINGTON D.C.

DAY 1 - FRIDAY, 25 SEPTEMBER

8.30-11.00 **SESSION 1: TRIALS IN AKI**

Session start time: San Francisco 5.30 / Paris 14.30 / Sydney 22.30

Designing and conducting AKI trials: what are the barriers? - Time for a roadmap
Moderator: Matthieu Legrand (San Francisco, USA)

How to succeed? The objective of this session is to draw lessons from previous trials (including in other fields than AKI) that may inform the design and the conduct of future trials and identify key-items for a roadmap toward AKI trials implementation.

Speakers: (10 min each)

- Alparslan Turan (Cleveland, USA): RCT in AKI patients: issues and limits with traditional RCT?
- Ravindra Mehta (San Diego, USA): AKI is a syndrome, not a disease: Better phenotyping for selective inclusions
- Amit Sharma (Bayer, USA): KHI technology roadmap for biomarkers in AKI
- Juliane Bernholz (AM-Pharma, NED): New therapeutic strategies: an industry viewpoint
- Ivonne Schulman (NIDDK, USA): NIDDK new funding opportunity for a clinical trial on post-hospitalization AKI (COPE-AKI)

Discussants: (5 min each)

- Chirag Parikh (Baltimore, USA): Precision medicine in the field of AKI
- Sachin Kheterpal (Ann Arbor, USA): specific insights in the perioperative setting
- Matthieu Legrand (San Francisco, USA): Which outcome(s) for clinical trials?
- Abigail Ryan (CMS/CM, USA)
- Omar Ali (London, UK): Generating payer evidence to support reimbursement & innovative contracting
- Ivonne Schulman (NIDDK, USA)
- Kimberly Smith (FDA, USA)
- Hrefna Guðmundsdóttir (EMA, ISL)

11.00-11.15 **BREAK**

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11.15-12.45 **SESSION 2: TRIALS IN CKD**

Session start time: San Francisco 8.15 / Paris 17.15 / Sydney 1.15 (Sat)

Endpoints 1: Hot topic session: The universal definition of end-stage renal disease in clinical trials
Moderator: Luis Ruilope (Madrid, ESP)

Speaker: (20 min)

- Rajiv Agarwal (Minneapolis, USA)

Discussants: (5 min each)

- Johannes Mann (Munich, GER)
- Jay Elliott (Bayer, USA)
- Ivonne Schulman (NIDDK, USA)
- Aliza Thompson (FDA, USA)
- EMA
- Patients' perspective

12.45-13.00 **BREAK**

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DAY 1 - FRIDAY, 25 SEPTEMBER

13.00-14.45 **SESSION 2 (continued)**

Endpoints 2: eGFR Slope as a Surrogate End Point for Kidney Disease Progression…..and for which Clinical Trials?
Moderator: Christoph Wanner (Würzburg, GER)

Speaker: (10 min)

- Tom Greene (Salt Lake City, USA)

Discussants: (5 min each)

- Joe Coresh (Baltimore, USA)
- Srinivsasan Beddhu (Salt Lake City, USA)
- Sunil Badve (Sydney, AUS): Insights from the CKDFIX trial
- John Adler (AstraZeneca, USA)
- Fredrik Erlandsson (AstraZeneca, SWE)
- Patients' perspective
- Aliza Thompson (FDA, USA)
- EMA

14.45-15.00 **BREAK**

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15.00-16.45 **SESSION 2 (continued)**

KDCT in the eyes: key methodological and conduct insights from the SGLT2i "game changer" CKD trials
Carte blanche to….
Moderators: Luis Ruilope (Madrid, ESP)

Speakers: (10 min) Discussants (5 min)

- Speaker: Meg Jardine (Sydney, AUS)/Discussant: Yshai Yavin (Janssen, USA)
- Speaker: Christoph Wanner (Würzburg, GER)/Discussant: Sibylle Hauske (Boehringer-Ingelheim, GER)
- Speaker: Hiddo Lambers Heerspink (Groningen, NED)/Discussant: Bergur Stefansson (AstraZeneca, SWE)

Discussants: (5 min each)

- Luis Ruilope (Madrid, ESP)
- Matthew Weir (Baltimore, USA)
- Alain Romero (Relypsa, USA)
- Kimberly Smith (FDA, USA)
- Krishna Prasad (MHRA/EMA, GBR)
- Omar Ali (London, UK): Generating value proposition in CKD and addressing uncertainty for payers
- Patient's perspective: Cynthia Chauhan (Wichita, USA)

16.45 **ADJOURN**

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DAY 2 - SATURDAY, 26 SEPTEMBER

7.30-9.00 **SPECIAL ROUNDTABLE**

Session start time: San Francisco 4.30 / Paris 13.30 / Sydney 21.30

RCTs conduct during the Covid-19 outbreak: lessons learned/ mitigation session
Moderators: Matt Weir (Baltimore, USA), Patrick Rossignol (Nancy, FRA)

Speakers: (10 min each)

- Barbara Gillespie (Covance, USA)
- Yves Rosenberg (NHLBI, USA)

Discussants: (5 min each)

- Meg Jardine (Sydney, AUS)
- Richard Nkulikiyinka (Bayer, GER)
- Christian Milliet (Vifor, SUI)
- Ivonne Schulman (NIDDK, USA)
- Norman Stockbridge (FDA, USA)
- Krishna Prasad (MHRA/EMA, GBR)
- Patient perspective: Caroline Wilkie (Punta Gorda, USA)

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09.00-10.30 **SESSION 3: PRECISION MEDICINE**

Session start time: San Francisco 6.00 / Paris 15.00 / Sydney 23.00

Precision medicine: Standardization of biomarker measurements
Moderator: Joe Bonventre (Boston, USA), Patrick Rossignol (Nancy, France)

Biomarker enabled precision medicine trials warrant a proper standardization of biomarker measurements (e.g. normalization methods (/urinary creatinine, urinary osmolarity, eGFR…) for urine biomarker concentrations; standardization of plasma measurements across cohorts and over time within a cohort to enable a reliable comparison). The aim of this session is to share experience and define the most suitable strategies for standardization.

Speakers: (10 min each)

- Winfried März (Würzburg, GER)
- Chirag Parikh (Baltimore, USA)

Discussants: (5 min each)

- Harold Feldman (from the CKD BioCon project) (Philadelphia, USA)
- Ju Wenjun (Ann Arbor, USA)
- Peter Ganz (proteomics and CKD) (San Francisco, USA)
- Katherine Landschulz (Covance, USA)
- AnnaLotta Schiller (Olink, USA)
- Franck Czerwiec (Goldfinch Bio, USA)
- Aliza Thompson (FDA, USA)
- Krishna Prasad (MHRA/EMA, GBR)
- Patients' perspective

10.30-10.45 **BREAK**

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DAY 2 - SATURDAY, 26 SEPTEMBER

10.45-12.15 **SESSION 4: CKD TRIALS**

Session start time: San Francisco 7.45 / Paris 16.45 / Sydney 00.45 (Sun)

Endpoints 3: Approaches to missing GFR data and optimizing trial design to mitigate risk of missing GFR data
Moderator: Rajiv Agarwal (Minneapolis, USA)

Kidney trials endpoints most frequently include biological parameters (variations in estimated GFR (CKD), and in serum creatinine (AKI) mostly) but this applies also to potassium, phosphorus, hemoglobin… and to any biomarker actually): the aim of this session is to share experience and define the most suitable strategies (e.g. reminders? link with registries?) beyond statistical means to mitigate the risks.

Speakers (10 min)

- Tom Greene (Utah, USA)
- Janet Wittes (Statistics collaborative, USA)

Discussants: (5 min each)

- Hiddo Lambers Heerspink (Groningen, NED)
- Barbara Gillespie (Covance, USA)
- Fred Yang (KBP Biosciences, USA)
- Navdeep Tangri (Winnipeg, Canada)
- Aliza Thompson (FDA, USA)
- Hrefna Guðmundsdóttir (EMA, ISL)
- Patient's perspective: Susan Quella (Rochester, USA)

12.15-12.30 **BREAK**

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12.30-14.45 **SESSION 5: TRIALS/INNOVATION IN ESRD**

Session start time: San Francisco 9.30 / Paris 18.30 / Sydney 2.30 (Sun)

12.30-13.45 PART A: Pathways (from biochemical, electromechanical, biomechanical) to innovation in device trials in chronic hemodialysis, kidney regeneration
Moderator: Prabir Roy-Chaudury (Chapel Hill, USA)

Speakers: (10 min each)

- Charmaine Lok (Toronto, CAN): Vascular access trials
- Joe Bonventre (Boston, USA): Kidney regeneration/repair

Discussants: (5 min each)

- Prabir Roy-Chaudury (Chapel Hill, USA)
- Patient's perspective: Caroline Wilkie (Punta Gorda, USA)
- Robert Lee (FDA, USA)
- EMA
- Abigail Ryan (CMS/CM, USA)
- Omar Ali (London, UK): Innovative contracting with medical devices to support payer reimbursement

DAY 2 - SATURDAY, 26 SEPTEMBER

13.45-14.45 PART B: Specific trial designs and effectiveness endpoints for wearable or implantable renal replacement therapies

Moderator: Prabir Roy-Chaudury (Chapel Hill, USA)

Speaker: (10 min each)

- Melissa West (Washington, DC, USA): Creating a Substrate for RRT Innovation

Discussants: (5 min each)

- William Fissell (Nashville, USA)
- Patient's perspective: Patrick Gee (Chesterfield, USA)
- Abigail Ryan (CMS/CM, USA)
- Omar Ali (London, UK): Innovative contracting with medical devices to support payer reimbursement
- Frank Hurst (FDA, USA)
- EMA

14.45.15.00 BREAK

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15.00-16.30 SESSION 6: PRAGMATIC KIDNEY TRIALS/AI INPUT

Session start time: San Francisco 12.00 / Paris 21.00 / Sydney 5.00 (Sun)

PART A: Pragmatic randomized trials: Updates on the role of cluster randomization for studying interventions in kidney trials, including types of interventions that can/should be studied using such an approach and associated ethical issues

Moderator: Meg Jardine (Sydney, AUS)

Speaker: (10 min)

- Laura Dember (Philadelphia, USA)

Discussants: (5 min each)

- Matthieu Legrand (AKI) (San Francisco, USA)
- Meg Jardine (hemodialysis) (Sydney, AUS)
- Michael Walsh (hemodialysis) (Hamilton, CAN)
- Patients' perspective
- Norman Stockbridge (FDA, USA)
- EMA

PART B: Use of digital health AI in clinical trials

Moderator: Meg Jardine (Sydney, AUS)

Speaker: (10 min)

- Chris Laing (AKI), (London, UK)

Discussants: (5 min each)

- Matthias Kretzler (machine learning) (Ann Arbor, USA)
- Teddy Cha (pulseData, USA)
- Richard Nkulikiyinka (Bayer, GER)
- Patients' perspective
- Norman Stockbridge (FDA, USA)
- EMA

16.30 ADJOURN