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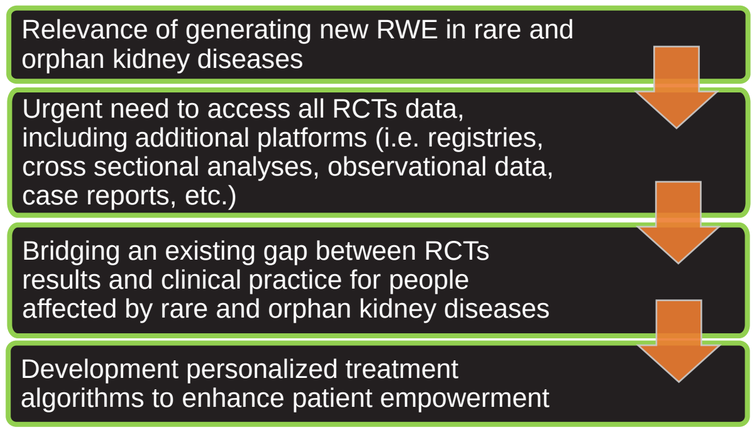
`The Urgency of Generating Real World Evidence Data to Bridge an Existing Gap between Randomized Clinical Trials and Clinical Practice in Rare and Orphan Kidney Diseases: An Ongoing Research from Main Available Platforms'

Domenico Merante, CSL Vifor, Global Clinical Development, Glattbrugg, Switzerland
Henrik Schou, CSL Vifor, Global Medical Affairs, Glattbrugg, Switzerland

Background and Objectives:

This research describes tools and methodologies to fill an existing data gap between randomized phase 3 clinical studies (RCTs), Real World Evidence (RWE) data and clinical practice when a drug is near to be/already approved, but yet to be reimbursed with an assigned price for a rare or orphan kidney disease. The background and main scope of this research are outlined below.



Methods:

This original research advocates the urgent use of new technologies to predict when patients suffering from rare and orphan kidney diseases, can achieve a more adequate benefit/risk assessment profile of an approved/to be approved drug. The research is based on an extensive literature review from main available sources, including PUBMED, EMBASE, COCHRANE, CLINICALTRIALS.GOV, Dr.EVIDENCE and similarly available platforms with posted clinical study results.

Results:

Expected outcomes from this research are linked to execution of the following and sequential activities:

- Developing a novel methodology to fill the highlighted clinical gap, such as a risk assessment tool to assist Health Care Professionals (HCPs) to support kidney rare/orphan disease management.
- Developing a predictive/treatment algorithm based on both RCTs, RWE data from available literature/web-based platforms.
- Conducting a subsequent clinical study with most appropriate biomarkers for improving therapeutic appropriateness of available and/or future treatments ('testing the algorithm').
- Producing HCP feedback, based on truly patient-focused data, regulatory and payers' feedback.
- Incorporating new clinical evidence in current scientific disease management and treatment guidelines.

Conclusions:

Initial results of this ongoing qualitative research are expected later in 2023. The main final objectives of the research are the following:

- to use accredited biomarkers in kidney rare/orphan disease area to generate treatment/risk disease-specific algorithms.
- based on available and new technologies, the approach aims to support 'patient empowerment and disease management'.
- to increase disease awareness and patient education based on personalized treatment/risk disease developed algorithms.
- to generate results expected to better reflect clinical practice and support patient empowered disease management.

Main references:

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