



# Modified Delphi research to seek consensus on dRTA Disease model, treatment, and management

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## Objectives

To seek insight and validation on the treatment and management of dRTA for the purpose of supporting reimbursement submissions.

## Methods

Distal renal tubular acidosis (dRTA) is a rare disorder of impaired net acid secretion by the distal tubule characterised by hyperchloraemic metabolic acidosis. It can be acquired or inherited, and if left untreated or poorly treated, can be severely debilitating. The research was conducted from September to October 2020. Recruitment was targeted at expert clinicians treating dRTA. 11 nephrologists participated in stage 1 of this Delphi research. Individual telephone discussions were conducted with adult and paediatric clinicians from England, Ireland, Scotland, and Wales during September and October 2020. The objective of the panel workshop on Thursday 22nd October 2020 was to refine the findings and seek consensus where this was lacking with the support of a urologist. The panel represents approximately 204 patients (estimated 179 adults and 25 children).

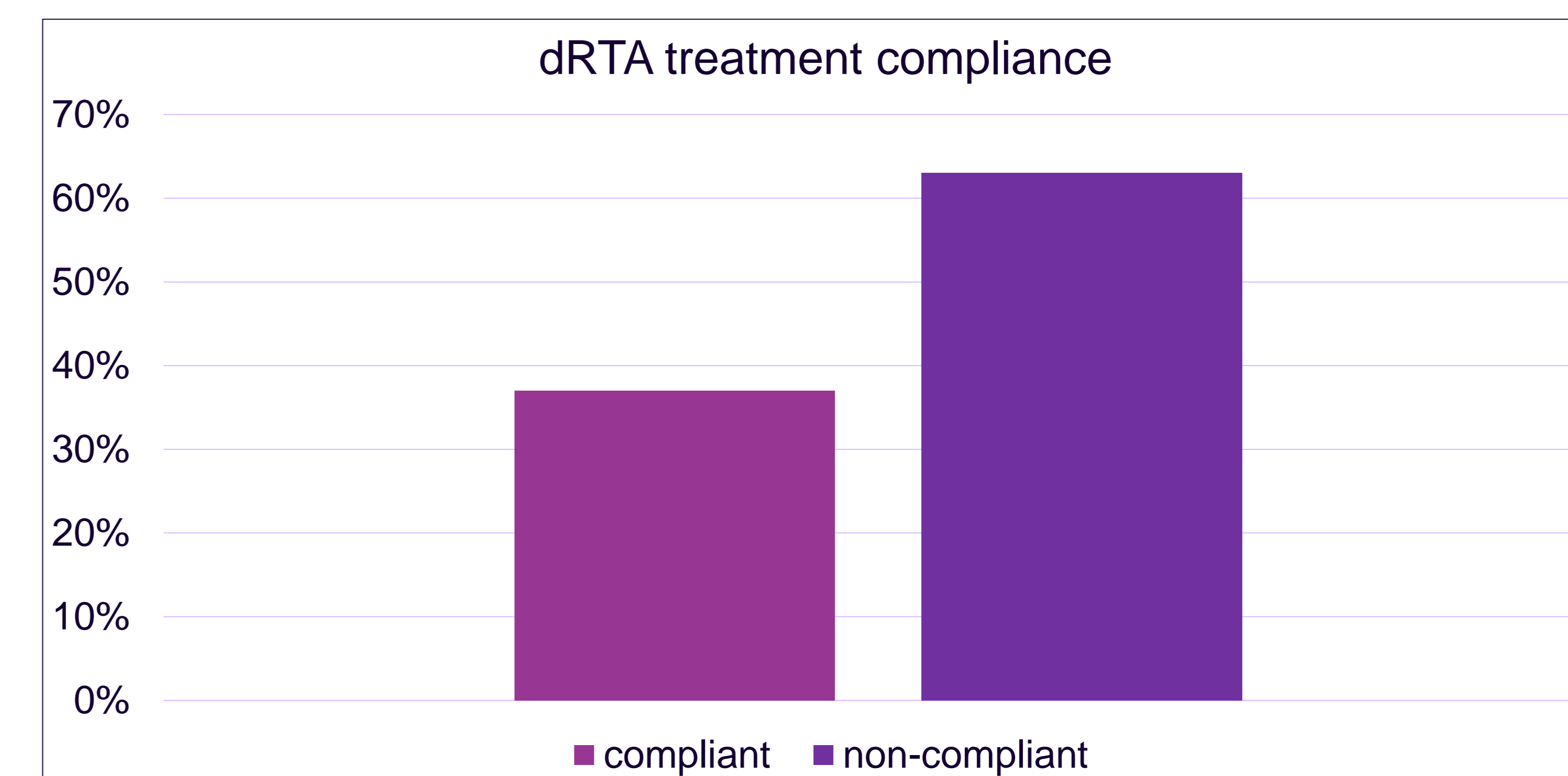
## Results

### Treatment and compliance

- Majority of patients are prescribed bicarbonate or citrate
- Advisors agreed, using the split of treatments described in study B21CS is appropriate for the model
- The estimated average percentage of patients who are fully compliant with their treatment was 37%
- Advisors strongly associate the burden of treatment with the volume and frequency of medication needed

### Modelling assumptions and pathways

- Treatment goals are to correct biochemistry, normalisation of acid-based balance (plasma and urine pH), and prevent disease consequences
- Healthcare resource use depends on disease control and compliance
- It is acceptable to assume that disease control stabilises post-24 months if the patient is compliant with alkali therapy



## Conclusions

Patients with dRTA are a rare population with an unmet need to manage their disease.

Care is considered to be variable and compliance on current treatment is poor (37%) with impacts upon the patient and potential increased economic impact to the healthcare system from lack of compliance.

## Disclosures

This study was funded by Advicenne. JM, SC and AM are employees of Initiate Consultancy who received funding from Advicenne for this work. SD is a consultant employed by Advicenne. Initiate Consultancy received consultancy fees for their role in helping to conduct the original study.