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DELAYED RELEASE VERSUS IMMEDIATE RELEASE CYSTEAMINE FOR PATIENTS WITH NEPHROPATHIC CYSTINOSIS: MODELLED DIFFERENCES IN LIFE EXPECTANCY AND QUALITY OF LIFE

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Objectives: Nephropathic cystinosis is a rare, systemic disease that is progressive, severe and characterized by renal failure and death. Lifelong treatment with Immediate Release Cysteamine (IRC) has been shown to reduce cystine levels, preserve organ function, and improve survival. Due to intrinsic properties of IRC (strict 4 times daily dosing), inadequate adherence to treatment is very common and associated with poor disease control. Twice daily dosing of Delayed Release Cysteamine (DRC) mitigates some of these properties and drug adherence is demonstrated to be higher. We compared IRC with DRC within a Markov partition model to estimate the differences in life years (LYs) and Quality-Adjusted-Life-Years (QALYs).

Methods: Survival curves for mortality, end-stage-renal-disease (ESRD), diabetes and neuromuscular disorder were constructed from a cohort study, DRC clinical trials and expert opinion. Utility values were derived from the Pediatric Quality of Life Inventory and literature.

Results: A patient with poor adherence (30%) to IRC, DRC gave additional 27.35 LYs and 22.6 QALYs (0% discounting) over IRC or additional 10.05 LYs and 10.48 QALYs (3% discounting). For a 75% adherent patient with IRC DRC gave additional 5.83 LYs and 10.85 QALYs (0% discounting) or additional 1.51 LYs and 5.39 QALYs (3% discounting). QALY gains are greater than LYs gained due to lifelong reductions in drug disutility with DRC. Median times to ESRD, diabetes and neuromuscular disorder were significantly delayed with DRC.

Conclusions: Improving DRC adherence leads to substantial gains in LYs and QALYs. Discounting of health benefits appears to significantly reduce the perceived benefits of DRC over IRC.

Biography

Goran Medic earned his Masters of Pharmacy degree at the Faculty of Pharmacy, University of Belgrade in Serbia and specialised in pharmacoeconomics and pharmaceutical legislation. He has more than 9 years of hands-on experience in performing systematic literature reviews, meta-analysis, writing reimbursement and health economic dossiers, developing and adapting health economic models for reimbursement and international marketing. He has gained experience in numerous disease areas, including orphan diseases, oncology, cardiology, pulmonary diseases, infections, psychiatric diseases, epilepsy and musculoskeletal disorders amongst others. Goran is now working as Market Access Manager Europe in Raptor Pharmaceuticals B.V in the Netherlands.

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