PD171 National Health Service Wales - First UK Nation To Approve Dostarlimab For The Treatment Of Rectal Cancer

Rachel Jonas (rachel.jonas@wales.nhs.uk), Rosie Spears, Gail Woodland and James Coulson

Introduction: Dostarlimab is a monoclonal antibody that blocks the programmed cell death protein 1 receptor. Emerging evidence has shown complete clinical response with dostarlimab in patients with locally advanced, treatment-naïve stage II or III rectal cancer where the tumor is either mismatch repair deficient or has high microsatellite instability. Dostarlimab has the potential to replace standard treatments such as chemoradiotherapy and surgery.
Methods: As part of the One Wales medicines assessment process, a literature search was performed and the marketing authorization holder was contacted to ensure the most up-to-date information was available. Clinical experts were consulted to advise where the medicine would sit within current therapy pathways in National Health Service Wales and to ensure that outcome data could be collected if dostarlimab were approved. After reviewing the evidence, an evidence summary report was written by the All Wales Therapeutics and Toxicology Centre that included the clinical and cost effectiveness, safety, and budget impact of dostarlimab.
Results: The One Wales Medicines Advisory Group assessed the evidence in June 2023, recommending access to dostarlimab for patients meeting the criteria for treatment. This recommendation was endorsed by the All Wales Medicines Strategy Group and ratified by the Welsh Government in August 2023. Starting and stopping criteria for dostarlimab were developed in collaboration with clinical experts to complement the One Wales decision. All patients in Wales who meet the agreed starting criteria will now be given the option for routine treatment with dostarlimab. To date, two patients have started treatment with dostarlimab, both of whom have reported treatment response at three months.
Conclusions: Wales is the first nation in the UK to approve routine access to dostarlimab for rectal cancer. The One Wales process allows access to dostarlimab, with the associated potential for avoiding life-changing surgery. Monitoring patient outcomes will provide real world data to enhance the immature dataset currently available on the clinical and cost effectiveness of dostarlimab for rectal cancer.

PD172 Off-Label Rituximab As A First-Line Immunosuppressant Treatment For Generalized Myasthenia Gravis In Wales

Clare Elliott (clare.elliott@wales.nhs.uk), Gail Woodland, David Haines and James Coulson

Introduction: Generalized myasthenia gravis (gMG) is a chronic autoimmune disorder that leads to muscle weakness and fatigue. Initial treatment is with pyridostigmine and corticosteroids, but if these are ineffective off-label immunosuppressants are used. There is some published evidence that rituximab, an anti-CD20 monoclonal antibody, may be effective in the treatment of gMG, although it is unlicensed for this condition.
Methods: Evidence for rituximab as a fourth-line or later immunosuppressant treatment for refractory gMG was assessed using the One Wales Medicines (OWM) process and made available in 2019. OWM provides an access route to medicines for specific patient cohorts where no licensed treatments are routinely available. A review in 2022 identified new evidence suggesting that lower dose rituximab could be an effective first-line immunosuppressant treatment for gMG. Clinicians confirmed an unmet need, so a reassessment by OWM was considered appropriate. Clinical and cost effectiveness were assessed through a literature search, budget impact analysis, and clinical expert opinion.
Results: The OWM Advisory Group assessed the evidence outlined in an evidence summary report. Clinical experts also provided the clinical context and current treatment options for patients with gMG. Overall, it was found that rituximab as a first-line treatment provided a potential improvement in patient outcomes and value for money, compared with current therapy. It was, therefore, supported for routine use in April 2023. Starting and stopping criteria for rituximab were developed in collaboration with clinical experts. All patients in Wales who meet the agreed starting criteria will now be given the option of routine treatment with rituximab.
Conclusions: The OWM process allows routine access to rituximab as a first-line immunosuppressant treatment for gMG. The OWM team are collaborating with clinicians across Wales to instigate a new systematic method for collecting and analyzing patient outcomes. These real-world data will be used to assess benefit to ensure continued access to the best treatments for Welsh patients.

https://doi.org/10.1017/S0266462324003982