Speakers Bureau for Roche. Dr. Paul has received personal compensation in the range of $500-$4,999 for serving on a Speakers Bureau for Pfizter. Dr. Paul has received personal compensation in the range of $500-$4,999 for serving on a Speakers Bureau for Merck Serono. The institution of Dr. Paul has received personal compensation in the range of $500-$4,999 for serving as an Editor, Associate Editor, or Editorial Advisory Board Member for Springer. Dr. Levy has received personal compensation in the range of $500-$4,999 for serving as a Consultant for Mitsubishi Pharma. Dr. Levy has received personal compensation in the range of $500-$4,999 for serving as a Consultant for UCB Pharma. Dr. Levy has received personal compensation in the range of $5,000-$9,999 for serving as a Consultant for Sanofi. Dr. Levy has received personal compensation in the range of $5,000-$9,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Alexion. Dr. Levy has received personal compensation in the range of $5,000-$9,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Horizon. Dr. Levy has received personal compensation in the range of $5,000-$9,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Genentech. Dr. Levy has received personal compensation in the range of $500-$4,999 for serving as an Editor, Associate Editor, or Editorial Advisory Board Member for Elsevier. Dr. Levy has received personal compensation in the range of $10,000-$49,999 for serving as an Expert Witness for Various law firms. The institution of Dr. Levy has received research support from National Institutes Health.

FDA Approved Treatments for Neuromyelitis Optica Spectrum Disorder in Clinical Practice: A Survey of Academic Neuroimmunologists

Jesse Thon, Robert Sharkus, Richa Thakkar, Krystal Hunter, James Siegler, Olga Thon

Objective

To assess utilization and barriers to use of three recently FDA-approved treatments for neuromyelitis optica spectrum disorder (NMOSD) by academic neuroimmunologists.

Background

Since 2019, eculizumab, inebilizumab, and satralizumab have received FDA approval for the treatment of aquaporin-4 IgG positive (AQP4+) NMOSD after showing efficacy in reducing attack frequency. However, little is known regarding the adoption of these therapies into practice or barriers to their use. We conducted a survey of academic neuroimmunologists to identify current treatment practices.

Design/Methods

An electronic survey study was conducted of US neuroimmunologists. Recipients were identified on department websites of university-associated hospitals with affiliated neurology residencies. The survey was administered via email, and included questions regarding clinical background, patient cohort, treatment use in different scenarios, and barriers. The authors were blinded to the identity of respondents.

Results

383 neuroimmunologists from 105 institutions were identified, of whom 33 from 18 states completed the survey. Nearly all (88%) reported treating patients with the newly FDA-approved therapies (NFTs). None reported discomfort discussing any NFT with their patients. Respondents reported uncommonly switching clinically stable patients to NFTs (69% never switch, 22% switch 1-25% of the time). For newly diagnosed AQP4+ NMOSD patients, NFT initiation rates varied (16% initiate none, 42% 1-25% of the time, 6.5% 25-50%, 19% 50-75%, 16% 75-100%). For patients with a relapse, responses were dichotomized regarding switching to NFTs - respondents either switch 75-100% of their patients (60%) or none or 1-25% (16%, 24% respectively). Nearly half (16/33) of respondents reported being unable to start NFTs, with insurance/cost issues being the most cited barrier.

Conclusions

Among academic neuroimmunologists, FDA-approved therapies for AQP4+ NMOSD are being utilized for newly diagnosed patients and those with disease recurrence, although individual practice patterns vary. The main perceived barriers to NFT use are insurance/cost-related issues.

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Safety and Effectiveness of Eculizumab in Japanese Patients With Aquaporin-4 Antibody-Positive Neuromyelitis Optica Spectrum Disorder: Interim Analysis of a Post-marketing Surveillance Study

Ichiro Nakashima, Yo Hoshino, Kazumi Okamura, Hidekazu Kikui, Kazuo Fujihara, Yuriy Edwards

Objective

NA.

Background

The terminal complement C5 inhibitor eculizumab is approved in Japan for the prevention of aquaporin-4 antibody-positive (AQP4+) neuromyelitis optica spectrum disorder (NMOSD) relapse and undergoing mandatory post-marketing surveillance (PMS) of real-world use.

Design/Methods

This PMS interim analysis assessed eculizumab's safety and effectiveness in Japanese patients AQP4+ NMOSD patients from approval (November 2019) to interim data cut-off (1 April 2021).

Results

At data cut-off, 79 patients treated with eculizumab were registered under PMS. Data were available for 20 patients who provided consent for publication and constituted the safety data set; three patients from the phase 3 PREVENT study were excluded from the effectiveness data set. Two patients discontinued before data cut-off owing to physician or patient decision (one each). In the effectiveness data set, 16/17 (94%) patients were female, mean illness duration was 9.2 years (standard deviation [SD] 7.5 years, range 0.3–23.8 years) and mean age at eculizumab initiation was 48.6 years (SD 11.9 years, range 27–77 years). In the 2 years before eculizumab, 12/17 patients experienced relapse, and 6/17 experienced =2 relapses. Importantly, no relapses were reported in the effectiveness data set (10.7 patient-years [PY] of treatment); this compared favorably with a 0.65/PY relapse rate in the 2 years before treatment (34.0 PY). Three adverse events (malaise, eyelid oedema, erythema) and one serious adverse event (urinary tract infection [UTI]) were observed in three patients in the safety data set: eyelid oedema and erythema (one patient) were deemed treatment-related; malaise and UTI were deemed unrelated to treatment. No meningococcal infections were reported, and the safety results were consistent with those from PREVENT.

Conclusions

For the first time in a real-world setting, eculizumab was effective in preventing relapses and well tolerated in Japanese patients with AQP4+ NMOSD, consistent with its efficacy and safety profile in the global phase 3 PREVENT study.

Disclosure: Dr. Nakashima has received personal compensation in the range of $500-$4,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Alexion. Dr. Nakashima has received personal compensation in the range of $500-$4,999 for serving on a Scientific Advisory or Data Safety Monitoring board

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Long-Term Eculizumab in AQP4+ NMOSD: Relapse-Risk Reduction and Safety in PREVENT and its Completed Open-Label Extension

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Objective

NA.

Background

Eculizumab is well tolerated and significantly reduces relapse risk versus placebo in patients with aquaporin-4 immunoglobulin G-positive (AQP4+) neuromyelitis optica spectrum disorder (NMOSD). We report eculizumab's long-term relapse-risk-reduction efficacy and safety in AQP4+ NMOSD during PREVENT (NCT01892345) and its completed open-label extension (OLE; NCT02003144).

Design/Methods

After receiving eculizumab or placebo during PREVENT, adults with AQP4+ NMOSD could enter the OLE (eculizumab maintenance dose, 1200 mg/2 weeks, with/without concomitant immunosuppressive therapy). Combined PREVENT and OLE (final data cut, 12 July 2021) data were analysed.

Results

During PREVENT and/or the OLE, 137 patients received eculizumab for a median (range) of 183.4 (0.1–342.0) weeks (3.5 years) and a total of 449.2 patient-years (Table 1). The estimated proportion of adjudicated relapse-free patients at week 216 (4.1 years) was 92.9% (95% CI: 85.9–96.5%; Figure). Nine patients experienced 10 adjudicated relapses (seven during the OLE, including one since the last interim analysis; Table 2). The adjudicated annualized relapse rate was 0.022 (95% CI: 0.012–0.041; Table 1). Rates of treatment-related adverse events and serious adverse events (SAEs)/100 patient-years were 165.3 and 7.0, respectively, versus 167.5 and 24.5 with placebo in PREVENT. The most common SAE was urinary tract infection (5.1% of patients). The serious infection rate was 10.5/100 patient-years with no meningococcal infections. No patients died during the OLE.

Conclusions

The proportion of relapse-free patients remained high (92.9%) through 4.1 years' eculizumab treatment. Long-term eculizumab was well tolerated with no new safety signals. These long-term data confirm eculizumab's sustained benefit/risk profile in AQP4+ NMOSD.

Disclosure: Achim Berthele has received personal compensation in the range of $500-$4,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Biogen. Achim Berthele has received personal compensation in the range of $500-$4,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Bayer. Achim Berthele has received personal compensation in the range of $500-$4,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Celgene. Achim Berthele has received personal compensation in the range of $500-$4,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Merck. Achim Berthele has received personal compensation in the range of $500-$4,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Roche. Achim Berthele has received personal compensation in the range of $500-$4,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Alexion. Achim Berthele has received personal compensation in the range of $500-$4,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Novartis. Achim Berthele has received personal compensation in the range of $500-$4,999 for serving on a Speakers Bureau for Biogen. Achim Berthele has received personal compensation in the range of $500-$4,999 for serving on a Speakers Bureau for Alexion. Dr. Wingerchuk has received personal compensation in the range of $10,000-$49,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Viela Bio. Dr. Wingerchuk has received personal compensation in the range of $5,000-$9,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Genentech. Dr. Wingerchuk has received personal compensation in the range of $10,000-$49,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Roche. Dr. Wingerchuk has received personal compensation in the range of $500-$4,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Biogen. Dr. Wingerchuk has received personal compensation in the range of $10,000-$49,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Horizon. Dr. Wingerchuk has received personal compensation in the range of $500-$4,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Mitsubishi Tanabe. Dr. Wingerchuk has received personal compensation in the range of $500-$4,999 for serving on a Scientific Advisory or Data Safety Monitoring board for UCB Pharma. Dr. Wingerchuk has received personal compensation in the range of $10,000-$49,999 for serving as an Editor, Associate Editor, or Editorial Advisory Board Member for Wolters Kluwers. The institution of Dr. Wingerchuk has received research support from Alexion. Dr. Fujihara has received personal compensation in the range of $500-$4,999 for serving as a Consultant for Abbvie. Dr. Fujihara has received personal compensation in the range of $500-$4,999 for serving as a Consultant for Merck Boipharma. Dr. Fujihara has received personal compensation in the range of $500-$4,999 for serving as a Consultant for Japan Tobacco. Dr. Fujihara has received personal compensation in the range of $5,000-$9,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Novartis. Dr. Fujihara has received personal compensation in the range of $5,000-$9,999 for serving on a Scientific Advisory or Data Safety Monitoring board for Mitsubishi-Tanabe. Dr. Fujihara has received personal compensation in the range of $5,000-$9,999 for serving on a Scientific Advisory or Data Safety Monitoring

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