FDA Approves Ocrelizumab for Pediatric Relapsing-Remitting Multiple Sclerosis

This approval marks the first high-efficacy B cell–depleting therapy approved for pediatric patients aged 10 years and older.

The FDA has approved ocrelizumab (Ocrevus; Genentech) intravenous (IV) infusion for the treatment of relapsing-remitting multiple sclerosis (RRMS) in pediatric patients aged 10 years and older who weigh at least 55 pounds, marking the first high-efficacy B cell–depleting therapy approved for this age group.[1]

Background: A Longstanding Unmet Need

Pediatric-onset multiple sclerosis (POMS) is defined as MS with disease onset before 18 years of age and accounts for approximately 3% to 5% of all MS cases. Compared with adult-onset MS, POMS is characterized by higher relapse rates, greater MRI lesion burden, and more frequent brainstem and cerebellar involvement at initial presentation, yet patients may reach disability milestones at a younger age due to earlier disease onset. Prior to this approval, fingolimod (Gilenya; Novartis) was the only FDA-approved disease-modifying therapy for pediatric RRMS, leaving clinicians with limited options for this vulnerable population.[1,2]

It is estimated that approximately 5000 to 10,000 children and adolescents in the US live with pediatric-onset MS.[1]

Pivotal Trial: OPERETTA 2

The approval was supported by data from the phase 3 OPERETTA 2 trial (NCT05123703), a double-blind, noninferiority study enrolling 187 pediatric patients aged 10 to 17 years with RRMS. Participants were randomly assigned to receive either 600 mg ocrelizumab IV every 24 weeks or daily oral fingolimod 0.5 mg, with matching placebos, over a double-blind treatment period.[3,4]

In the trial, ocrelizumab demonstrated noninferiority to fingolimod in reducing the annualized relapse rate (ARR). In MRI outcomes, ocrelizumab showed superiority, producing an approximate 48% reduction in new or enlarging T2 lesions and an 87% reduction in gadolinium-enhancing T2 lesions compared with fingolimod. At week 12, the proportion of patients with at least one T1 gadolinium-enhancing lesion was significantly lower in the ocrelizumab group (4.3% vs 15.9%; P = .001).[1,3,4]

Safety Profile

The safety profile observed in pediatric patients was consistent with that established in adult patients treated with ocrelizumab. Serious adverse events (SAEs) and serious infections were infrequently observed and well balanced between treatment groups. Notably, no AEs led to treatment withdrawal in the ocrelizumab group, whereas 3 patients discontinued in the fingolimod group.[1]

Known risks associated with ocrelizumab include infusion reactions, upper and lower respiratory tract infections, herpes infections, decreased immunoglobulins, progressive multifocal leukoencephalopathy, and potential malignancy. Ocrelizumab is contraindicated in patients with active hepatitis B virus infection or a history of life-threatening infusion reactions to the agent.[1]

Clinical Significance

The approval expands ocrelizumab's existing indications, which include relapsing forms of MS and primary progressive MS in adults. The OPERETTA 2 findings are further supported by data from the earlier phase 2 OPERETTA 1 (NCT04075266) study, which demonstrated sustained, near-complete B cell depletion and stable Expanded Disability Status Scale (EDSS) scores with no clinical relapses observed over a median treatment duration of 120 weeks.[1,2]

"This approval represents a landmark for children living with MS in the US and their families, which can help close the longstanding gap in high-efficacy treatment options for children aged 10 and older," Levi Garraway, MD, PhD, chief medical officer and head of Global Product Development at Genentech, said in a news release.[1]

Dosing and Administration

Ocrelizumab is administered by IV infusion every 6 months. The dose is weight-based for pediatric patients, including 300 mg for those weighing less than 40 kg and 600 mg for those weighing 40 kg or more, consistent with dosing established in the OPERETTA program. Researchers noted that patients should be premedicated with a corticosteroid and antihistamine prior to each infusion to reduce the frequency and severity of infusion reactions, and should be monitored during and for at least 1 hour following each infusion.[1]

“Having an FDA-approved, high-efficacy treatment option like [ocrelizumab] available for age-appropriate children and adolescents is a game changer,” Emily Blosberg, founder of Mr. Oscar Monkey, who was diagnosed with MS at 15 years old, said in the news release. “It means the next generation of patients won't have to wait for answers—they have an opportunity to take control of their disease early and potentially stop relapses and brain lesions before they have a chance to take a toll.”[1]

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