Rituxan® (rituximab) – Expanded orphan indication

- On September 27, 2019, the FDA announced the approval of Genentech's Rituxan (rituximab), in combination with glucocorticoids, for the treatment of adult and pediatric patients 2 years of age and older with Granulomatosis with Polyangiitis (GPA) (Wegener's Granulomatosis) and Microscopic Polyangiitis (MPA).
    - Rituxan was previously approved for this indication in adults only.
- Rituxan is also approved for the treatment of adult patients with non-Hodgkin's lymphoma, chronic lymphocytic leukemia, rheumatoid arthritis, and pemphigus vulgaris.
- GPA and MPA are two types of ANCA-associated vasculitis (AAV). AAV is a form of vasculitis that primarily affects small blood vessels. In general, GPA and MPA affect the small blood vessels of the kidneys, lungs, sinuses, and a variety of other organs, but the diseases may affect each person differently.
    - The estimated overall prevalence of GPA and MPA in the U.S. is up to 3 cases per 100,000 people.
    - Cases of pediatric onset GPA and MPA are considered even rarer and are associated with severe, potentially life-threatening symptoms.
- The approval of Rituxan for the expanded indication was based on an open-label, single arm study in 25 pediatric patients with active GPA and MPA. The study design consisted of an initial 6-month remission induction phase, and a minimum 12-month follow-up phase up to a maximum of 54 months. Patients received Rituxan or non-U.S.-licensed rituximab. The primary objectives of this study were to evaluate safety and pharmacokinetic parameters in pediatric GPA and MPA patients. The efficacy objectives of the study were exploratory and principally assessed using the Pediatric Vasculitis Activity Score (PVAS).
    - After 18 months, all 25 patients achieved PVAS remission.
- Rituxan carries a boxed warning for fatal infusion-related reactions, severe mucocutaneous reactions, hepatitis B virus reactivation, and progressive multifocal leukoencephalopathy.
- The recommended induction dose of Rituxan for the treatment of GPA and MPA in pediatric patients is 375 mg/m^2 administered by intravenous (IV) infusion once weekly for 4 weeks.
    - Prior to the first Rituxan infusion, IV methylprednisolone 30 mg/kg (not to exceed 1g/day) should be administered once daily for 3 days. Following IV methylprednisolone administration, oral steroids should be continued per clinical practice.
    - For follow up treatment of pediatric patients with GPA/MPA who have achieved disease control with induction treatment, Rituxan should be administered as two 250 mg/m^2 IV infusions separated by two weeks, followed by a 250 mg/m^2 IV infusion every 6 months thereafter based on clinical evaluation.

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- Refer to the Rituxan drug label for dosing for all its other indications.