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FDA approves first oral anticoagulant for pediatric patients

by from the Food and Drug Administration

The Food and Drug Administration (FDA) has expanded the approval of Pradaxa (dabigatran etexilate) to include pediatric patients, making it the first oral anticoagulant approved for use in children.

Pradaxa, a direct thrombin inhibitor, is approved for the treatment of venous thromboembolic events (VTE) in pediatric patients ages 3 months through 17 years who have been treated with parenteral anticoagulants for at least five days. It also is approved to reduce VTE recurrence risk in pediatric patients in the same age group who have been treated previously for VTE.

The studies to support pediatric approval of Pradaxa were conducted under the Best Pharmaceuticals for Children Act (BPCA). First authorized in 2002, BPCA is one tool the FDA can use to improve information available to support the safe and effective use of pharmaceuticals for children. For studies conducted under BPCA, pharmaceutical companies must use an age-appropriate drug formulation. If a formulation is not available, the company must develop and test one and must seek marketing approval for the formulation.

The FDA also approved a new oral pellets dosage form for children who cannot swallow Pradaxa capsules. The pellets are administered twice daily based on age and weight. Due to differences in the absorption and bioavailability of the capsules and pellets, providers are cautioned not to substitute or combine the formulations. Specific dosing recommendations are detailed in the full prescribing information, https://bit.ly/3AgnqYc.

Two phase 3 trials supported approval of Pradaxa in pediatric patients.

One study was a randomized, open-label, active-controlled, parallel-group trial comparing Pradaxa with standard of care anticoagulation therapy in 267 pediatric patients with VTE, stratified by age group. Pradaxa's efficacy was demonstrated based on a composite primary endpoint of complete thrombus resolution, freedom from recurrent VTE and freedom from mortality related to VTE. Of the 267 randomized patients, 81 patients (45.8%) in the Pradaxa group and 38 patients (42.2%) in the standard of care group met the composite success criteria, demonstrating Pradaxa's noninferiority to standard of care.

The other study was an open-label, single-arm trial evaluating Pradaxa for the prevention of recurrent VTE. Subjects included 214 pediatric patients who required further anticoagulation because they were at high risk of a recurrence of VTE.

The study demonstrated a VTE recurrence rate of 1.4% (three patients) while on treatment within the first 12 months after completion of the initial VTE treatment. The overall probability of being free from recurrence of VTE during the treatment period was 0.990 (95% CI: 0.960, 0.997) at three months and 0.984 (95% CI: 0.950, 0.995) at six and 12 months.

The most common adverse reactions to Pradaxa were gastrointestinal symptoms (e.g., dyspepsia, upper abdominal pain, nausea, vomiting and diarrhea) and bleeding. Site-specific bleeding rates were comparable between Pradaxa and standard of care anticoagulation therapy, with the exception of gastrointestinal bleeding,

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which occurred in 5.7% of patients on Pradaxa compared to 1.8% of patients on standard of care.

The FDAOffice of Pediatric Therapeuticsand Center for Drug Evaluation and Research, Division of Pediatric and Maternal Health and Division of Non-malignant Hematology contributed to this article.

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