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Draft Guidance on Iptacopan Hydrochloride

May 2025

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Active Ingredient: Iptacopan hydrochloride

Dosage Form: Capsule

Route: Oral

Strength: EQ 200 mg Base

Recommended Study: One in vivo bioequivalence study with pharmacokinetic endpoints

1. Type of study: Fasting
   Design: Single-dose, two-treatment, two-period crossover in vivo
   Strength: EQ 200 mg Base
   Subjects: Healthy males and non-pregnant, non-lactating females
   Additional comments: Monitor for signs and symptoms of infection during the study. Iptacopan hydrochloride oral capsules are approved under a Risk Evaluation and Mitigation Strategy (REMS) with Elements to Assure Safe Use (ETASU), which restricts its use. All pertinent elements of the REMS/ETASU must be incorporated into the protocol and informed consent. Ensure an adequate washout period between treatments in the crossover study due to the long elimination half-life of iptacopan. Alternatively, a parallel study design may be considered.

Analyte to measure: Iptacopan in plasma

Bioequivalence based on (90% CI): Iptacopan

Waiver request of in vivo testing: Not applicable

Dissolution test method and sampling times: The dissolution information for this drug product can be found in the FDA's Dissolution Methods database, http://www.accessdata.fda.gov/scripts/cder/dissolution/. Conduct comparative dissolution testing on 12 dosage units for each of the test product and reference listed drug (RLD).[1] Specifications will be determined upon review of the abbreviated new drug application.

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Unique Agency Identifier: PSG_218276

[1] If the RLD is not available, refer to the most recent version of the FDA guidance for industry on *Referencing Approved Drug Products in ANDA Submissions*.