

FOOD AND DRUG ADMINISTRATION (FDA)
Center for Drug Evaluation and Research (CDER)

*Joint Meeting of the Gastrointestinal Drugs Advisory Committee (GIDAC) and the
Pediatric Advisory Committee (PAC)*

DoubleTree by Hilton Hotel Bethesda – Washington, DC, Grand Ballroom
8120 Wisconsin Avenue, Bethesda, Maryland
May 3, 2018

QUESTIONS

1. **DISCUSSION**: The Applicant has submitted a single, adequate, and well-controlled study (Study 64,185-204) as evidence to support the approval of stannosporfin.
 - a. Please discuss the clinical meaningfulness of the primary endpoint of “percent change from baseline in total serum bilirubin (TSB)” at 48-hours post-treatment with stannosporfin.
2. **DISCUSSION**: Please discuss your recommendations for dosing (3 mg/kg or 4.5 mg/kg single dose) based on the available information.
3. **VOTE**: Has the Applicant provided substantial and persuasive evidence of effectiveness for stannosporfin as an adjunct to phototherapy in neonates greater than or equal to 35-weeks gestational age with laboratory evidence of hemolysis and hyperbilirubinemia meeting the American Academy of Pediatrics (AAP) criteria for phototherapy who are at risk for developing complications associated with severe hyperbilirubinemia?
4. **VOTE**: Are the submitted data on long-term safety assessments adequate to characterize the potential risk of stannosporfin-related adverse neurodevelopmental outcomes?
5. **VOTE**: Does the long-term and short-term safety profile of stannosporfin in the proposed indicated population support approval?
6. **DISCUSSION**: Please discuss whether additional interventions beyond FDA-approved labeling, such as a Risk Evaluation and Mitigation Strategy (REMS), are necessary to ensure that the drug’s benefits outweigh its risks.
 - a. Please discuss the REMS proposed by the FDA, which consists of health care setting certification (for dispensing and administration), safe use conditions, and a registry.
7. **VOTE**: Does the overall risk-benefit profile of stannosporfin support approval?
 - A. Yes without a REMS
 - B. Yes with a REMS
 - C. No
8. **DISCUSSION**: Please discuss the necessity of additional studies (clinical or nonclinical) with stannosporfin to assess the potential for adverse neurodevelopmental outcomes. Comment on potential design elements.