CLINICAL INFORMATION ON HEMANGEOL® AND INFANTILE HEMANGIOMA

To: P&T Committee of the State of Nevada

Hello all, good morning. My name is Dylan Bassett. I am a pharmacist, and I am a part of the Medical Affairs team at Pierre Fabre. I am here today to speak on behalf of Hemangeol, the only FDA approved drug for Infantile Hemangioma and to kindly request the committee to consider placing Hemangeol on the Preferred Drug List.

Infantile hemangioma is the most common tumor of infancy affecting approximately 3 to 5 % of all infants born in the US. Among the 20,000 Infantile Hemangioma patients a year some will present with severe complications that are potentially debilitating and associated with life-threatening conditions. These include but are not limited to airway obstruction, congestive heart failure, functional impairment (such as vision, hearing, or feeding impairment), and ulceration with bleeding and pain. The majority of these patients are at risk of permanent disfigurement. If these lesions are undertreated, untreated, or mismanaged, it is highly likely that they will leave permanent disfigurement and scarring.

HEMANGEOL® is the only FDA-approved drug indicated for these severe forms of Infantile Hemangioma. It was developed in compliance with existing guidelines for pediatric drugs and evaluated to ensure efficacy, safety, ease and acceptability of use for these young pediatric patients. It is the only Beta Blocker that has been evaluated by a randomized, double, blind placebo controlled clinical trial for efficacy and safety, with the primary endpoint being complete or near complete resolution of the target hemangioma. The most common adverse events that were seen were sleep disorders and respiratory infections, but these are not unique to just Hemangeol, but are seen in other oral beta blockers and also in the adult formulation of propranolol.

If the affected Medicaid children in Nevada don't receive Hemangeol, they will have the adult solution of propranolol which is not well-tolerated and not studied for safety and tolerance in the infant population. The adult formulation contains alcohol and sorbitol, two ingredients which are not found in Hemangeol, which are known to induce osmotic diarrhea. This constant and chronic diarrhea is a real struggle for the families during treatment. It is also formulated with flavors such as peppermint, that is not well tolerated by infants so Consequently, adherence is decreased, and the duration of treatment is longer with poorer outcomes. I would also like to point out that the adult formulation of Propranolol has not been evaluated by a randomized double blinded clinical study, and is not FDA approved for the use in Infantile Hemangioma.

I would like the members of the committee to realize that we are referring to small babies, most of the time premature babies, suffering during their first months of life with a condition that can have lasting, detrimental impacts on their social, psychological, and emotional well-being, as well as their immediate family.

In conclusion, Hemangeol is the only safe treatment option for infants affected with severe forms of Infantile Hemangioma. Therefore, we ask that the Committee to consider placing Hemangeol on the PDL. Thank you so much for your time.