BLA 125734 donislecel Advisory Committee April 15, 2021

DRAFT CMC Points for Discussion by the Advisory Committee:

CMC Discussion Question # 1:

a) What is the contribution of endocrine, exocrine, or other cell types expected to be in the final drug product to the clinical outcomes and product potency?

b) How might the relative proportions of endocrine, exocrine, or other cell types in the product play a role in clinical outcomes and product potency?

c) What are the specific types of non-β cells that the Applicant should characterize and/or, possibly, control for in the product?

CMC Discussion Question #2:

a) Are the product quality attributes of purity and potency sufficient to evaluate lot-to-lot consistency in manufacturing, product quality, and product strength?

b) If not, what additional product characteristics, not previously identified as CQAs for donislecel, would provide more meaningful measures to assess lot-to-lot product consistency?