FOOD AND DRUG ADMINISTRATION (FDA)
Office of the Commissioner (OC)

Meeting of the Pediatric Advisory Committee (PAC)
September 19, 2023

QUESTIONS FOR THE COMMITTEE

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1. Given that testing on large animal models (e.g., lamb, piglet, non-human primate) has limitations related to between-species differences in anatomy and physiology which may prevent direct representation of the use of AWT devices in the human neonate, please discuss key safety and proof-of-principle endpoints, including timepoint(s) of assessment (e.g., time of transition from AWT, longer term follow-up), that may be evaluated in animal studies to understand the potential benefits and risks of AWT in the human neonate.

2. Given the limitations of the animal models and the clinical experience data discussed, what additional, if any, existing clinical experience data may be leveraged to help inform the benefit risk assessment of enrollment in a first-in-human trial of an AWT?

3. What challenges do you anticipate in obtaining effective informed consent for an AWT clinical trial? Please discuss potential strategies to address any anticipated challenges.

4. Please discuss critical aspects of safety monitoring in an AWT first-in-human (FIH) trial:

- **a.** What adverse events of special interest should be monitored in an AWT trial to assess subject safety?

- **b.** FDA anticipates that a FIH trial would initially enroll and treat a single subject at a time. When considering enrollment of additional subjects, what safety endpoints, including timepoint(s) of assessment, should be considered before enrolling a subsequent subject(s)?