News: FDA Reviews NDA for ^{18}F-FDOPA in Congenital Hyperinsulinism

The U.S. Food and Drug Administration (FDA) in early December released notification of its intention to provide priority review of a New Drug Application (NDA; #212531) for marketing authorization of ^{18}F-fluorodopa injection (^{18}F-FDOPA) for PET imaging in congenital hyperinsulinism. The NDA is the product of a collaborative effort among SNMMI, the National Cancer Institute (NCI), Washington University St. Louis (MO), and Children's Hospital of Philadelphia (PA). These groups worked together to submit the NDA in September 2018, with a goal of enabling wide availability of ^{18}F-DOPA PET for preoperative anatomic localization of focal hyperfunctioning areas of the pancreas in infants and children with congenital hyperinsulinism.

Congenital hyperinsulinism is the most common cause of persistent hypoglycemia in infants and, although affecting only 1 in 50,000 newborns, may result in death, seizures, and neurodevelopment abnormalities. Up to 50% of surviving children with the disease suffer from long-term neurodevelopment disabilities. ^{18}F-FDOPA PET is the only imaging modality currently able to provide direct focal imaging of insulin hypersecretion, a capability with special relevance at the very small scale of infants and young children.

In a September 5, 2018, letter in support of the NDA, SNMMI leadership noted that ^{18}F-DOPA PET has long been in use at specialized centers seeing infants with this rare and complex disease to assist in anatomic evaluation of the pancreas for potential surgical resection of adenomatous tissue. As a result, several centers have published results of their experience, which served as key efficacy and safety data for the NDA. The NDA also includes reports of post-marketing clinical experience with levodopa (an approved drug), the ligand component of ^{18}F-DOPA. Also included is a systematic review of published reports. More than half of the patients included in this review (155/289) were seen at the Children's Hospital of Philadelphia, which has published reports on their experience and made associated data available for review. The NDA also includes a request for a Rare Pediatric Disease Voucher.

Paula Jacobs, PhD, from the NCI's Cancer Imaging Program, commented: "Not only is this NDA poised to benefit infants and children with a very rare disease, it demonstrates the power of collaboration among government and academic groups to advance the approval of drugs that might otherwise never occur for lack of commercial return."

Under the FDA priority review, the original Prescription Drug User Fee Act date for a decision on the NDA was May 24, 2019. NDA reviews may be delayed as a result of the January partial government shutdown.

SNMMI