

T-4

A Phase II Trial to Evaluate the Safety and Dosimetry of [¹⁷⁷Lu]Lu-DOTA-TATE in Adolescent Patients with Somatostatin Receptor (SSTR)-positive Gastroenteropancreatic Neuroendocrine Tumors (GEP-NETs), Pheochromocytomas and Paragangliomas (PPGLs)

Sue M. O'Dorisio¹, Mark Gaze², Constantino Sábado³, Fazia Brouiri⁴, Ilya Folitar⁴

¹University of Iowa Carver College of Medicine, Iowa City, IA; ²University College London Hospitals NHS Foundation Trust, London, United Kingdom; ³Hospital Universitari Vall d'Hebron, Barcelona, Spain; ⁴Advanced Accelerator Applications, a Novartis company, Geneva, Switzerland

BACKGROUND: In the pediatric population, limited approved therapies exist for patients with GEP-NETs and PPGLs. SSTR subtype-2 is overexpressed by GEP-NET and PPGL tumors; therefore, it is a relevant target for peptide receptor radionuclide therapy with [¹⁷⁷Lu]Lu-DOTA-TATE. [¹⁷⁷Lu]Lu-DOTA-TATE is approved for the treatment of adult patients with SSTR-positive GEP-NETs. It has shown efficacy in several studies evaluating adult patients, but there have been no Advanced Accelerator Applications-sponsored pediatric clinical trials in GEP-NETs and PPGLs to date. The treatment of adolescents with GEP-NETs and PPGLs represents a significant unmet need, providing a strong rationale to evaluate [¹⁷⁷Lu]Lu-DOTA-TATE in these patients.

METHODS: This multicenter, phase II, open-label, single-arm study is designed to evaluate the safety and dosimetry of [¹⁷⁷Lu]Lu-DOTA-TATE in adolescent patients (12 to <18 years old) with advanced, inoperable, SSTR-positive GEP-NETs (grade 1 or 2, well differentiated) in the primary cohort and PPGLs in the exploratory cohort. Eligible patients will receive 4 cycles of [¹⁷⁷Lu]Lu-DOTA-TATE

(activity per cycle: 7.4 GBq), administered every 8 weeks. After the last dose, patients will be followed for 5 years. Radiation dosimetry and pharmacokinetic (PK) assessments will be done after the first [¹⁷⁷Lu]Lu-DOTA-TATE administration. Safety assessments will be performed regularly after each cycle. Primary endpoints are target organ absorbed radiation dose and incidence of adverse events (AEs) after the first cycle. Secondary endpoints are incidence of AEs within 6 months (short-term follow-up) and 5 years (long-term follow-up) after the last dose, and PK and dosimetry compared with predicted values. Efficacy will be assessed as an exploratory objective, including objective response rate, progression-free survival, and overall survival in both cohorts. This study will enroll at least 8 patients with GEP-NETs and as many patients with PPGLs as possible across multiple sites in Europe and North America (NCT04711135).

RESULTS: Study is in progress.

CONCLUSION: Study is in progress.

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