TITLE: FOOD AND DRUGS AUTHORITY PUBLIC ASSESSMENT REPORT ON GCP INSPECTIONS

PART 1: Administrative Details	
Title of Clinical Trial:	A Phase 2/3 Adaptive, Double-blind, Placebo-Controlled Study to Evaluate the Efficacy and Safety of VX-147 in Subjects Aged 18 Years and Older with APOL1-mediated Proteinuric Kidney Disease.
Protocol Version used for Inspection:	Protocol Version 3.0 dated 5th April 2022
FDA Clinical Trial Certificate number assigned:	FDA/CT/234
Name of site Investigator:	Professor Sampson Antwi Komfo Anokye Teaching Hospital Department of Child Health P. O Box 1934 Kumasi – Ashanti Region Telephone Number: 0265812061 Email: kantwisampson@gmail.com
Clinical Trial Site Name and Address:	Renal Centre, Komfo Anokye Teaching Hospital
Sponsor Name and Address:	Vertex Pharmaceuticals Incorporated 50 Northern Avenue, Boston, MA 02210-1862 USA Tel: +1 617-341-6100
Inspection Team:	• Mrs. Amma Frempomaa Asare (Lead Inspector) • Dr. Edwin Nkansah • Mr. Jesse Kojo Kuntoh
Date(s) of inspection:	29th February – 1st March 2024
Date of report:	19th June 2024
FAPAR Number:	FDA/CT/PAR/GCP/247

PART 2: Purpose of Inspection	
Purpose:	The inspection was conducted as part of the FDA's mandate to ensure that all approved clinical trials are conducted in compliance with international best practice; the approved protocol, Good Clinical Practice (GCP), ICH E6 R2 standards and applicable regulatory requirements. The conduct of the inspection was as per FDA/CTD/SOP - 06 (SOP for Conducting GCP Inspections).

PART 3: Documents Reviewed and Facilities Inspected	
Documents Reviewed:	The GCP inspection involved document review, study team interviews and a facility visit. Documents reviewed included the version of the trial protocol available on site, Logs (delegation, screening, enrolment, training), Informed Consent Forms (ICFs), Standard Operating Procedures (SOPs), site monitoring visit reports, study-related agreements and Ethics Review Board (IRB) approval. These documents were reviewed to verify traceability, accuracy, consistency, completeness, and reliability of the study data.
Facilities Inspected:	Areas visited during the inspection at the study site, which is the Renal Centre, Komfo Anokye Teaching Hospital, were the consenting area, screening area, phlebotomy room, document storage area and pharmacy room. The inspection team also inspected the VX21-467-301 Unit at the MDS Lancet Laboratory located at Kwadwo Kannin street, Adiebeba in Kumasi.

PART 4: Study Status at Time of Inspection	
Study Status:	At the time of the inspection, the study was actively enrolling participants, and the following details were noted: a) Total number of participants consented = 40 b) Total number of participants screened for APOL1 gene (1st stage of screening) = 40 c) Total number of participants who failed APOL1 gene screening = 23 d) Total number of participants who underwent stage 2 screening = 17 e) Total number of participants who failed stage 2 screening = 16 f) Total number of participants eligible for the study after stage 2 screening = 1

	g) Total number of participants randomized = 1

PART 5: Inspection Findings & Regulatory Actions	
Findings:	Eleven (11) Major findings and Nineteen (19) Minor findings were observed during the inspection.
Regulatory Actions:	Enrolment of new participants in the study was suspended as a regulatory action.

PART 6: Status of Corrective and Preventive Actions (CAPA) & Current Status of Study	
Status of CAPA:	The corrective and preventive actions have been instituted as of 7th June 2024 and all outstanding issues deemed satisfactory.
Current Status of the study:	The suspension of new enrolment of participants into the study has been released. The study is now in substantial compliance with GCP and all FDA-applicable guidelines. The Sponsor and the Investigator site staff have been directed to ensure and maintain compliance with all applicable laws during the entire duration of the study.