



DATE September 10, 2019

FROM Christine Drabick, Bioresearch Monitoring Branch
Division of Inspections and Surveillance
Office of Compliance and Biologics Quality

THROUGH Dennis Cato, Branch Chief, Bioresearch Monitoring Branch

THROUGH Carrie Mampilly, Director, Division of Inspections and Surveillance

TO Thomas Finn, Chair, STN 125685/0
Winson Tang, Clinical Reviewer
Jean Gildner, RPM

SUBJECT Bioresearch Monitoring Final Review Memo
SPONSOR: Enzyvant therapeutics GmbH
PRODUCT: Allogeneic Processed Thymus Tissue.
BLA : STN: 125685/0

FINAL SUMMARY STATEMENT:

A Bioresearch Monitoring (BIMO) inspection of three clinical studies conducted by the IND sponsor did not reveal substantive problems that impact the data submitted in the application.

BACKGROUND:

The clinical studies submitted in support of this application were conducted by the sponsor investigator of the IND. A BIMO inspection assignment was issued for three protocols in support of this Biologics License Application (BLA).

Eighteen subjects were consented for Protocol 668-1. Two subjects were screen failures, 16 were enrolled and 14 received transplants. Six subjects died before the 12 month time point and eight completed the study. Records for 12 of the 14 transplanted subjects were reviewed during the inspection.

Twenty one subjects were consented for Protocol 668-2. Twelve subjects received transplants. Three subjects died before the 12 month time point and nine completed the study. Records for 12 of the 12 transplanted subjects were reviewed during the inspection.

Thirty one subjects were consented for Protocol 25966. Twenty six subjects received transplants. Six subjects died before the 12 month time point and 20 completed the study. Records for 12 of the 26 transplanted subjects were reviewed during the inspection.

The inspection was conducted in accordance with FDA's Compliance Program (CP) 7348.810, Inspection Program for Sponsors, Contract Research Organizations, and Monitors and CP 7348.811, Inspection Program for Clinical Investigators. Information submitted in the BLA was compared to source documents at the site. The inspection assignment included specific questions concerning the clinical study.

PROTOCOLS: The conduct of the following protocols was evaluated:

668-1 Thymus Transplantation in Complete DiGeorge Syndrome

668-2 Phase II Study of Thymus Transplantation in Complete DiGeorge Syndrome

25966 Safety and Efficacy of Thymus Transplantation in Complete DiGeorge Anomaly

BIMO INSPECTION SUMMARY:

Protocol	Location	FDA Form 483 Issued?	Inspection Classification
668-1	Duke University Health System Room 109 B Research Park IV Durham, North Carolina 27710	No	NAI = No Action Indicated
668-2	Duke University Health System Room 109 B Research Park IV Durham, North Carolina 27710	No	NAI = No Action Indicated
25966	Duke University Health System Room 109 B Research Park IV Durham, North Carolina 27710	No	NAI = No Action Indicated

SIGNIFICANT INSPECTIONAL FINDINGS: No significant inspectional findings were observed.

SPONSOR ISSUES: No sponsor issues were identified.

FINANCIAL DISCLOSURE:

The Clinical Investigator Compliance Program directs the FDA investigator to ask the clinical investigator if and when he/she disclosed information about his/her financial interests to the sponsor and/or interests of any sub-investigators, spouse(s) and dependent children, and if and when the information was updated. The information submitted to the BLA was verified for each of the inspected clinical sites.

ADMINISTRATIVE FOLLOW-UP:

An information letter was issued to the sponsor investigator.

Should you have any questions or comments about the contents of this memo or any aspect of Bioresearch Monitoring, please contact me at 240-402-8928.

Christine J. Drabick
Consumer Safety Officer

Distribution

Electronic Copy:

EDR BLA 125685/0
IND 9836, Letter to Sponsor
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Eileen Bannerman, FDA Investigator

History:

Draft: Drabick: August 30, 2019