



DATE September 22, 2022

FROM Colonious King, Consumer Safety Officer  
Bioresearch Monitoring Branch (BMB)  
Division of Inspections and Surveillance (DIS)  
Office of Compliance and Biologics Quality (OCBQ)

THROUGH Dennis T. Cato, Chief BMB

THROUGH Carrie M. Mampilly, MPH, Director DIS

TO Anna Kwilas, PhD, Chair  
Shelby Elenburg, MD, Clinical Reviewer  
Leah Crisafi, MD, Clinical Reviewer  
Colleen Caldwell, MS, MPH, RPM

SUBJECT Bioresearch Monitoring Final Discipline Review Memo

SPONSOR: Bluebird bio, Inc.

PRODUCT: elivaldogene autotemcel (Skysona)

BLA: STN: 125755/0

### **FINAL SUMMARY STATEMENT**

Bioresearch Monitoring (BIMO) inspections were performed for the Sponsor and two Clinical Investigators (CI) participating in the conduct of Protocols ALD-102 and ALD-104 in support of this original Biologics License Application (BLA). The inspections did not reveal problems that impact the data submitted in the application.

### **BACKGROUND**

BIMO inspection assignments were issued for the following two protocols in support of this BLA:

**Protocol ALD-102:** A Phase 2/3 Study of the Efficacy and Safety of Hematopoietic Stem Cells Transduced with Lenti-D Lentiviral Vector for the Treatment of Cerebral Adrenoleukodystrophy (CALD)

Protocol ALD-102 was conducted at seven centers worldwide with four sites in the United States. Thirty-five subjects were screened, and 32 subjects enrolled in Protocol 102. The inspection covered 31% of the enrolled subjects.

**Protocol ALD-104:** A Phase 3 Study of Lenti-D Drug Product After Myeloablative Conditioning Using Busulfan and Fludarabine in Subjects  $\leq 17$  Years of Age with Cerebral Adrenoleukodystrophy (CALD)

Protocol ALD-104 was conducted at nine centers worldwide with four sites in the United States. Thirty-five subjects were screened, and 28 subjects were enrolled in Protocol 104. The inspection covered 57% of the enrolled subjects.

The BIMO Sponsor and CI inspections were conducted in accordance with FDA's Compliance Program (CP) 7348.810, Inspection Program for Sponsors, Monitors and Contract Research Organizations (CRO) and CP 7348.811, Inspection Program for CI, respectively. Information submitted in the BLA were compared to source documents at the site, and the inspection assignments included specific questions concerning the clinical study.

Summary of BIMO Inspections

<b>Site Number</b>	<b>Protocol Number</b>	<b>Location</b>	<b>FDA Form 483 Issued</b>	<b>Final Classification</b>
105	104	Massachusetts General Hospital	No	No Action Indicated (NAI)
110	102, 104	University of Minnesota Masonic Children's Hospital	No	NAI
Sponsor	N/A	Bluebird bio, Inc. Cambridge, MA	No	NAI

SIGNIFICANT INSPECTIONAL FINDINGS: No significant inspectional findings were observed.

SPONSOR ISSUES: No sponsor issues were identified.

FINANCIAL DISCLOSURE:

The CI CP directs the FDA investigator to ask the CI 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 last updated. The information submitted to the BLA was verified for each of the inspected clinical sites.

ADMINISTRATIVE FOLLOW-UP:

Should you have any questions or comments about the contents of this memo or any aspect of BIMO, please contact me at 240-402-8759.

Colonious King  
Consumer Safety Officer