

## STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

|                   |  |
|-------------------|--|
| USAN (GH-141)     | ELIVALDOGENE AUTOTEMCEL                    |
| PRONUNCIATION     | el" i val' doe jeen aw" toe tem' sel       |
| THERAPEUTIC CLAIM | Treatment of cerebral adrenoleukodystrophy |

### DESCRIPTION

Autologous CD34+ enriched cell population that contains hematopoietic stem cells (HSCs) transduced with the Lenti-D lentiviral vector (LVV) that encodes an *ABCD1* cDNA for human adrenoleukodystrophy protein (ALDP).

The CD34+ enriched cell population containing hematopoietic stem and progenitor cells is obtained from autologous peripheral blood mononuclear cells (PBMCs) collected by apheresis from the patient following mobilization of leukocytes. The Lenti-D LVV is a replication-defective, self-inactivating (SIN), third-generation, lentiviral vector derived from the human immunodeficiency virus type I (HIV-I).

|                  |                        |
|------------------|------------------------|
| TRADEMARK        | None as yet            |
| SPONSOR          | Bluebird Bio., Inc.    |
| CODE DESIGNATION | Lenti-D Drug substance |
| UNII             | KUM75TD6SG             |
| WHO NUMBER       | 10901                  |

SCS