

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (HI-143)

LOVOTIBEGLOGENE AUTOTEMCEL

PRONUNCIATION

loe" voe tye beg' loe jeen aw" toe tem' sel

THERAPEUTIC CLAIM

Treatment of sickle cell disease

PRODUCT DESCRIPTION

The drug substance consists of an autologous CD34+ cell-enriched population from patients with sickle cell disease that contains hematopoietic stem cells transduced with BB305 lentiviral vector encoding the  $\beta^{A-T87Q}$ -globin gene. The properties that are relevant to the drug substance are the same properties that are important to the performance of the drug product (bb1111). Each batch of drug product is tested to confirm that it possesses the desired product quality, including identity, viability, potency and purity. These tests monitor for the presence and quantity of cells expressing CD34+, the percentage of cells containing the lentiviral vector transgene, the ability to express the  $\beta^{A-T87Q}$ -globin protein and the increase in red blood cells exhibiting a normal (anti-sickled) conformation relative to a patient matched cell sample not subjected to transduction

DIAGRAM

Figure 1: BB305 LVV Sequence Diagram

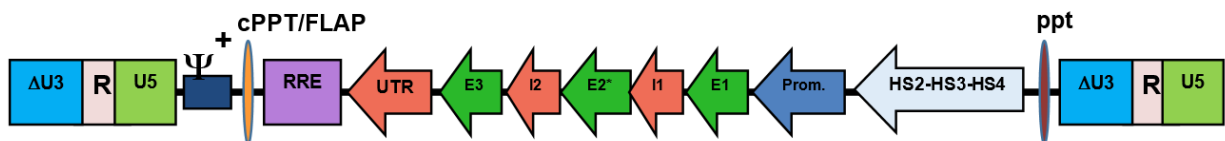


Figure definitions: ΔU3, promoter/enhancer deleted unique 3'; R, repeat; U5, unique 5'; Ψ+, packaging signal; cPPT/FLAP, central polypurine tract; RRE, rev response element; UTR, untranslated region; E3, Exon3; I2, Intron2; E2\*, Exon2 with threonine87glutamine mutation; I1, intron1; E1, Exon1; Prom, promoter; HS2-HS3-HS4, hypersensitive site 2,3,4; ppt, polypurine tract.

TRADEMARK

None as of yet

SPONSOR

bluebird bio.

CODE DESIGNATIONS

bb1111

UNII

2C6A9NH2Z8

WHO NUMBER

11826

SCS