A Phase 3, 2-part, multicenter, randomized, double blind, placebo controlled study to evaluate combination BL-8040 + G-CSF vs placebo + G-CSF in mobilizing CD34+ cells for auto-HCT for MM

RESULTS of Part 1

- 11/11 patients (100%) treated at 1.25 mg/kg dose collected the target goal of ≥6 x 10^6 CD34+ cells/kg.
- 9/11 patients (82%) reached the primary endpoint of ≥6 x 10^6 CD34+ cells/kg with 1 dose of BL-8040 and up to 2 apheresis sessions.
- 11/11 patients (100%) treated at 1.25 mg/kg dose collected ≥6 x 10^6 CD34+ cells/kg in 4 or less apheresis days.
- 6/11 patients (55%) collected ≥6 x 10^6 CD34+ cells/kg in 1 apheresis day.
- 6/11 patients (55%) mobilized ≥6 x 10^6 CD34+ cells/kg in 1 apheresis session.
- BL-8040 + G-CSF vs placebo + G-CSF in mobilizing CD34+ cells for auto-HCT for MM

Primary Objective
- To demonstrate the superiority of one dose of BL-8040 + G-CSF over placebo + G-CSF to mobilize ≥6 x 10^6 CD34+ cells/kg in 1 apheresis day

Secondary Objectives
- To demonstrate the superiority of one dose of BL-8040 + G-CSF over placebo + G-CSF to mobilize ≥6 x 10^6 CD34+ cells/kg in 2 apheresis days
- To demonstrate the superiority of one dose of BL-8040 + G-CSF over placebo + G-CSF to mobilize ≥6 x 10^6 CD34+ cells/kg in 3 apheresis days
- To assess comparability between BL-8040 + G-CSF and placebo + G-CSF in terms of neutrophil engraftment, platelet engraftment, and hospital length of stay

METHODOLOGY
- Screening
- Mobilization
- Primary Collection
- Optional Collection

OBJECTIVES

Primary Objective
- To demonstrate the superiority of one dose of BL-8040 + G-CSF over placebo + G-CSF to mobilize ≥6 x 10^6 CD34+ cells/kg in 1 apheresis day

Secondary Objectives
- To demonstrate the superiority of one dose of BL-8040 + G-CSF over placebo + G-CSF to mobilize ≥6 x 10^6 CD34+ cells/kg in 2 apheresis days
- To demonstrate the superiority of one dose of BL-8040 + G-CSF over placebo + G-CSF to mobilize ≥6 x 10^6 CD34+ cells/kg in 3 apheresis days

RESULTS

- 11/11 patients (100%) treated at 1.25 mg/kg dose collected the target goal of ≥6 x 10^6 CD34+ cells/kg in 4 or less apheresis days
- 6/11 patients (55%) collected ≥6 x 10^6 CD34+ cells/kg in 1 apheresis day
- 6/11 patients (55%) mobilized ≥6 x 10^6 CD34+ cells/kg in 1 apheresis session
- Administration of BL-8040 in a 8.4-fold average increase in circulating peripheral CD34+ cells (range 0.5-18.3, median 7.5, n=9)
- Common BL-8040-adverse related events consisted primarily of Grade 1-2 injection site reactions and transient systemic reactions
- The DMC recommended the adverse study proceed to Part 2 after the data from the 1st cohort of 11 patients was assessed

CONCLUSIONS

- The GENESIS Part 1, lead-in results demonstrate that BL-8040 is a potent mobilizer of HSCs, with the potential to increase mobilization success rates while reducing healthcare utilization and cost, in preparation of multiple myeloma patients for autologous hematopoietic cell transplantation.
- BL-8040 is well tolerated and most commonly associated with mild, transient injection site reactions.
- Time to engraftment and graft durability appear to be comparable to standard of care mobilization regimens.

Table 1: Most Common AEs, Frequency & Grade

Table 2: Platelet engraftment defined as first of 3 consecutive measurements of platelet count ≥20 x 10^9/L without platelet transfusion support for 7 days following the conditioning regimen associated nadir. ANC engraftment defined as ANC ≥0.5 x 10^9/L for 3 consecutive days or ≥1.0 x 10^9/L for 1 day following the conditioning regimen associated nadir. Final engraftment defined as whichever is the later of the two. Graft failure defined as maintaining  2 of 3: platelet ≥50 x 10^9/L, ANC ≥1.0 x 10^9/L, Hgb ≥10 g/dL and ANC ≥0.5 x 10^9/L or ANC ≥1.0 x 10^9/L for 1 day following the conditioning regimen associated nadir.

Table 3: Most Common AEs, Grade

BL-8040:
- Genome-wide activation of CXCR4 (C-X-C motif receptor type 4) with high affinity (IC50 4.42 nM) and a long receptor half-life
- Produces a subpopulation of Homer-1a, a factor which regulates CXCR4 expression
- Binds to CXCR4 with high affinity (IC50 4.42 nM) and a long receptor half-life
- Leads to increased CXCR4 expression and increased mobilization success rates while reducing healthcare utilization and cost, in preparation of multiple myeloma patients for autologous hematopoietic cell transplantation.

OBJECTIVES

Primary Objective
- To demonstrate the superiority of one dose of BL-8040 + G-CSF over placebo + G-CSF to mobilize ≥6 x 10^6 CD34+ cells/kg in 1 apheresis day

Secondary Objectives
- To demonstrate the superiority of one dose of BL-8040 + G-CSF over placebo + G-CSF to mobilize ≥6 x 10^6 CD34+ cells/kg in 2 apheresis days
- To demonstrate the superiority of one dose of BL-8040 + G-CSF over placebo + G-CSF to mobilize ≥6 x 10^6 CD34+ cells/kg in 3 apheresis days

RESULTS

- 11/11 patients (100%) treated at 1.25 mg/kg dose collected the target goal of ≥6 x 10^6 CD34+ cells/kg in 4 or less apheresis days
- 6/11 patients (55%) collected ≥6 x 10^6 CD34+ cells/kg in 1 apheresis day
- 6/11 patients (55%) mobilized ≥6 x 10^6 CD34+ cells/kg in 1 apheresis session
- Administration of BL-8040 in a 8.4-fold average increase in circulating peripheral CD34+ cells (range 0.5-18.3, median 7.5, n=9)
- Common BL-8040-adverse related events consisted primarily of Grade 1-2 injection site reactions and transient systemic reactions
- The DMC recommended the adverse study proceed to Part 2 after the data from the 1st cohort of 11 patients was assessed

CONCLUSIONS

- The GENESIS Part 1, lead-in results demonstrate that BL-8040 is a potent mobilizer of HSCs, with the potential to increase mobilization success rates while reducing healthcare utilization and cost, in preparation of multiple myeloma patients for autologous hematopoietic cell transplantation.
- BL-8040 is well tolerated and most commonly associated with mild, transient injection site reactions.
- Time to engraftment and graft durability appear to be comparable to standard of care mobilization regimens.

Table 1: Most Common AEs, Frequency & Grade

Table 2: Platelet engraftment defined as first of 3 consecutive measurements of platelet count ≥20 x 10^9/L without platelet transfusion support for 7 days following the conditioning regimen associated nadir. ANC engraftment defined as ANC ≥0.5 x 10^9/L for 3 consecutive days or ≥1.0 x 10^9/L for 1 day following the conditioning regimen associated nadir. Final engraftment defined as whichever is the later of the two. Graft failure defined as maintaining  2 of 3: platelet ≥50 x 10^9/L, ANC ≥1.0 x 10^9/L, Hgb ≥10 g/dL and ANC ≥0.5 x 10^9/L or ANC ≥1.0 x 10^9/L for 1 day following the conditioning regimen associated nadir.

Table 3: Most Common AEs, Grade

BL-8040:
- Genome-wide activation of CXCR4 (C-X-C motif receptor type 4) with high affinity (IC50 4.42 nM) and a long receptor half-life
- Produces a subpopulation of Homer-1a, a factor which regulates CXCR4 expression
- Binds to CXCR4 with high affinity (IC50 4.42 nM) and a long receptor half-life
- Leads to increased CXCR4 expression and increased mobilization success rates while reducing healthcare utilization and cost, in preparation of multiple myeloma patients for autologous hematopoietic cell transplantation.