As of October 29, 2022, 54 patients with BBS enrolled in an index trial. The mean (SD) age of patients was 20.4 (11.8) years; 55.6% of patients were female. This trial supports the continued efficacy and long-term use of setmelanotide in BBS.

At the time of index trial enrollment, patients were ≥6 years old with obesity. BBS is a rare genetic disease of obesity characterized by multiorgan dysfunction; includes obesity with severe, early-onset obesity; and insatiable pathologic hunger (hyperphagia).

Patients <18 years old (n=28; 37.8 kg/m² at index baseline)

 Patients ≥18 years old (n=25; 47.2 kg/m² at index baseline)

Mean (SD) body weight in patients aged ≥18 years (n=25) was 132.3 (20.9) kg.

Six of 15 patients (40.0%) and 5 of 6 patients (83.3%) achieved ≥10% weight reduction at Month 18 and 24, respectively.

Mean change in BMI, %

Results

Patient Disposition and Baseline Characteristics

- As of October 29, 2022, 54 patients with BBS enrolled in an index trial (≥18 years old; n=28; 35.2 kg/m² at index baseline), of which 42 continued enrolled in the LTE trial
- Among patients enrolling in the LTE, 30 and 19 received at least 18 and 24 months of setmelanotide treatment, respectively
- Parents were receiving ongoing treatment at the time of data analysis, and not all patients have reached the 18- and 24-month time points

Efficacy Outcomes

- Mean (SD) BMI at index baseline was 44.2 kg/m² (n=53)
- Patients received up to 15 months of setmelanotide as part of their index trial
- All at time of index trial enrollment, patients were ≥6 years old with obesity
- Patients began the LTE immediately following completion of an index trial and continued on the same dose of setmelanotide (Figure 1)
- Trial visits occurred approximately every 3 months in the LTE

Conclusions

- The long-term efficacy of setmelanotide treatment in patients with BBS was well tolerated and maintained weight-related improvements in adult and pediatric patients with BBS, with no new safety concerns
- Further evaluation of the different characteristics (eg, age, sex, genotype) on setmelanotide response is ongoing
- This trial supports the continued efficacy and long-term use of setmelanotide in patients with BBS

Summary

- Setmelanotide demonstrated continued clinical benefit on body weight-related outcomes in patients with Bardet-Biedl syndrome (BBS) for up to 2 years

Table. Adverse Events Occurring During the Index and LTE Trials in the Safety Population (N=54)

- Adverse events in the overall safety population across the index and LTE trials

Figure 1. Study design.

Figure 2A. Mean change in BMI across all patients at 18 and 24 months of setmelanotide treatment of patients ≥18 years old, showing a greater decrease in BMI at 24 months than at 18 months.

Figure 2B. Mean change in BMI Z score from index trial baseline by months of setmelanotide treatment for patients <18 years old.

Note: Data are presented as mean ± SD. BMI, body mass index; LTE, long-term extension; SD, standard deviation.

Risk assessment and measures include: *P < .05; **P < .01; ***P < .001.

See the Phase 3 index trial full report published in October 2021; Data from Phase 1, Phase 2a, and Phase 2b trials presented at ObesityWeek 2021; Data from Phase 2b trial presented at ObesityWeek 2022; Data from Phase 3 trial presented at ObesityWeek 2022; Data from LTE trials presented at ObesityWeek 2022; Data from Phase 2 index trial presented at ObesityWeek 2021; Data from LTE trials presented at ObesityWeek 2022.

References:


2. Guay-Woodford LM, Roberts CE Jr, Lee MA, et al; NCT03013543a or NCT03746522b; November 1-5, 2021; Virtual.


5. Guay-Woodford LM, Roberts CE Jr, Lee MA, et al; NCT03013543a or NCT03746522b; November 1-5, 2021; Virtual.