**Methods**

Introduction

BBS is a rare syndromic form of obesity associated with renal abnormalities; it is caused by mutations in several genes that code for proteins involved in the cellular regulation of energy homeostasis and cell cycle control.

**Objective**

To assess the long-term, on-treatment outcomes over 3 years of setmelanotide therapy in patients with BBS.

**Study design**

Patients aged 26 years were eligible for this long-term extension (LTE) trial (NCT02657565) if they

- Completed a prior (index) trial in which they received setmelanotide
- Demonstrated clinical benefit from treatment on the basis of efficacy results
- Patients with BBS enrolled in the LTE after completing a Phase 2 or 3 trial of setmelanotide
- Patients received ≥12 months of setmelanotide treatment as part of their index trial at the time of enrollment in the LTE trial.

Results

**Patient disposition and baseline characteristics**

Overall, 35 patients had a clinically meaningful age-appropriate weight response at 1 year in the index trial.

**Efficacy outcomes**

**Safety outcomes**

No new safety concerns emerged during long-term treatment, and setmelanotide was generally well tolerated in both the index and LTE trials (N=54).

Conclusions