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Adipose Tissue, Appetite, & Obesity

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Long-term Efficacy of Setmelanotide in Patients With POMC or LEPR Deficiency Obesity

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The melanocortin-4 receptor (MC4R) pathway in the brain is responsible for energy regulation, affecting appetite and body weight. Impairments of this pathway can result in
hyperphagia (insatiable hunger) and obesity. The MC4R can be impacted by the POMC, PCSK1, or LEPR genes. Variants in these genes that are inherited from both parents (biallelic) can result in MC4R pathway signaling deficiencies. Setmelanotide is a drug that activates the MC4R. In earlier Phase 3 studies, setmelanotide was shown to improve hunger and reduce weight in patients with POMC, PCSK1, and LEPR biallelic deficiency. The present study aims to examine the long-term efficacy of ~3 years of setmelanotide treatment in patients with POMC, PCSK1, and LEPR biallelic deficiency in a long-term extension after a previous trial. Setmelanotide treatment continued to provide sizable reductions in body weight and body mass index while being generally well tolerated. These findings provide strong support of the safe and effective long-term use of setmelanotide to treat obesity in patients with POMC, PCSK1, and LEPR biallelic deficiency.

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