- Transthyretin familial amyloid polyneuropathy (TTR-FAP) is a rare, progressive and ultimately fatal disease wherein malnutrition, wasting, and cachexia are the inevitable and cardinal signs of disease progression.
- This article describes a post-hoc analysis of nutritional status in patients with TTR-FAP due to the Val30Met variant, who participated in an 18-month randomized, double-blind study of tafamidis versus placebo followed by a 12-month extension trial.
- Overall nutritional health was measured by use of the modified body mass index (mBMI; the product of BMI and serum albumin), an easy-to-use physiologic indicator reporting on gastrointestinal function and disease progression monitor.
- Tafamidis was associated with improving or maintaining nutritional status in Val30Met TTR-FAP patients over 30 months of continual treatment as well as reversing the decline in mBMI among patients who switched from placebo treatment after the randomized, double-blind study.
- BMI increases were most pronounced in patients with low BMI at entry into the studies.

This summary slide represents the opinions of the authors. Sponsorship for this study was funded by Pfizer Inc., New York, USA. Medical writing assistance for this study was provided by Malcolm Darkes of Engage Scientific Solutions, Horsham, UK. For a full list of acknowledgments and conflicts of interest for all authors of this article, please see the full text online. 