

April 26, 2018

**Testimony of the Endocrine Society  
Submitted to the  
Food and Drug Administration Endocrinologic and Metabolic Drugs Advisory Committee**

The Endocrine Society is the world's largest professional organization of endocrinologists, representing the interests of over 18,000 physicians and scientists engaged in the treatment and research of endocrine disorders, including rare diseases like acromegaly, adrenal insufficiency, growth hormone deficiency, Cushing's syndrome, and familial chylomicronemia syndrome (FCS). The Society appreciates the opportunity to provide testimony to the FDA as it considers treatment options for such conditions and looks forward to working with the agency as it moves forward in its review.

Many endocrine conditions qualify as rare diseases as they affect fewer than 200,000 people each year, but patients lack effective treatments for many of these diseases. Although the FDA has approved more than 500 treatments since the passage of the Orphan Drug Act, only five percent of rare diseases have a treatment.<sup>1,2</sup> Biopharmaceutical companies are capitalizing on advances in science and technology to develop personalized treatments that build on a greater understanding of molecular or genetic drivers of a disease. Ongoing research to identify new treatments for many of these diseases could have a significant benefit to patients. The Society advocates for increased research funding for rare diseases and supports the FDA's flexibility regarding clinical endpoints, patients sample sizes, and inclusion/exclusion criteria for clinical trials to increase our knowledge of rare diseases and to expand therapeutic options for treating these conditions. We have also proposed to add rare disease experts, when possible, to each review division.

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<sup>1</sup> U.S. Food and Drug Administration, Orphan Drug Designations and Approvals Database.

<sup>2</sup> Global Genes: *Rare Disease: Facts and Statistics*.

The Endocrine Society has a longstanding interest in the diagnosis and treatment of lipid disorders. We have published a clinical practice guideline, *Evaluation and Treatment of Hypertriglyceridemia*<sup>3</sup>, which outlines definitions, primary and secondary causes, and recommendations for managing this disorder. Currently, patients with recurrent pancreatitis secondary to severe hypertriglyceridemia that cannot be controlled by diet have limited treatment options. The FDA's approval of Volanesorsen would offer the first medical treatment specifically for this condition.

The Endocrine Society encourages the FDA to continue support for development of treatments for rare diseases by providing flexibility in study design that accounts for the unique limitations of rare disease research. We urge approval of these therapies if they are found to be safe and effective.

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<sup>3</sup> Lars Berglund et al; Evaluation and Treatment of Hypertriglyceridemia: An Endocrine Society Clinical Practice Guideline, *The Journal of Clinical Endocrinology & Metabolism*, Volume 97, Issue 9, 1 September 2012, Pages 2969–2989