Dear Dr. Robert Califf, Commissioner of Food and Drugs,

We, the undersigned members of Congress, are writing to express our strong support for the development and approval of safe and effective drugs to treat symptoms of the orphan disease of Prader-Willi syndrome (PWS). We believe that the FDA, consistent with its longstanding regulations and guidance, has a rich history of understanding the unique challenges in rare disease drug development and, therefore, exercising flexibility for drugs for rare diseases in the development and approval process.

Other than growth hormone to treat growth failure due to PWS, there are no approved treatments for the disease, which has many varied and challenging symptoms that are both life-limiting and life-threatening. There is thus a tremendous need for novel therapies. As part of our commitment to ensuring that rare disease patients have equitable access to much-needed treatments, we urge the FDA to continue applying its expert judgment and flexibility to accelerate the approval of therapies aimed at alleviating the symptoms of PWS. We are excited that after decades of investment in research efforts and the development of drug development tools by the PWS community, these investments have paid off with new therapies having recently completed development and nearing review for potential approval by the Agency.

The 21st Century Cures Act, which emphasizes the incorporation of patient experience in regulatory decision-making, aligns with our belief that the voices and experiences of PWS patients and their families must be central in the decision-making process. We commend the PWS community for its longstanding engagement with the FDA's patient experience initiatives. Events such as the recent Externally-Led Patient-Focused Drug Development Meeting, which was attended and introduced virtually by Dr. Patrizia Cavazzoni, Director of FDA's Center for Drug Evaluation and Research, exemplify the proactive efforts of the PWS community to collaborate with regulatory authorities in advancing sorely needed treatments for this rare genetic disorder.

In light of these collaborative efforts and in recognition of the urgent need for innovative therapies to treat a multitude of symptoms of PWS, we urge the FDA to prioritize the review and approval of orphan drugs targeting PWS symptoms. By working together, we can ensure that patients with PWS have access to safe and effective treatments that improve their quality of life and provide hope for a better future.

Thank you for your attention to this important matter, and we look forward to your continued support of the PWS community.

Sincerely,

[Members of Congress Signatures]