U.S. FOOD & DRUG ADMINISTRATION

September 10, 2024

Dear Ms. Lantz,

Thank you for your July 31, 2024, letter to Drs. Farchione and Buracchio regarding the petition on diazoxide choline (DCCR) for the proposed treatment of those with Prader-Willi syndrome. Your letter was received by the Center for Drug Evaluation and Research for response.

First, we appreciate your efforts to bring together members of the PWS community and provide a platform for those with PWS and their caregivers to share their experiences. We would also like to thank you for collating and sharing the summary of community feedback on DCCR. Organizations like yours are very important for helping find solutions for new treatments for rare diseases. Please know that we believe in the incorporation of patient input in decision-making.

The Food and Drug Administration (FDA) understands that PWS is a rare condition with physical, emotional, and social impacts, and we recognize the substantial burden this disease places on both patients and caregivers. We recognize the lack of therapeutic options for patients and the unmet medical need that exists. One of our top priorities is to support people affected by rare diseases by accelerating, supporting, and facilitating the process of getting drug products to market. The FDA remains steadfast in our work to facilitate the development and approval of safe and effective therapies to treat PWS, and we will continue to work closely with drug developers to bring treatments to patients that meet regulatory requirements as soon as possible.

Consistent with federal statutes and FDA's implementing regulations concerning the confidentiality of commercial information, and to protect the integrity of the review process, we generally cannot disclose information about an unapproved application, including the status of the Agency's review of a particular drug product[1]. Therefore, we are unable to comment on specific pending applications. If you have additional questions or concerns that are not directly related to a drug application, we recommend you contact CDER's Professional Affairs and Stakeholder Engagement (PASE) Staff at CDERPASE@fda.hhs.gov. PASE is available to engage with stakeholders about drug development, drug review, and drug safety. More information about PASE is available on the FDA website at https://www.fda.gov/about-fda/center-drug-evaluation-and-research-cder/professional-affairs-and-stakeholder-engagement.

Thank you, again, for sharing your thoughts with us regarding this matter.

Sincerely,

[1] Relevant laws include the Freedom of Information Act (FOIA) (5 U.S.C. 552); the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 331(j); and FDA regulations (e.g. 21 CFR 20.61(c), 21 CFR 312.130(b), 21CFR 314.430(c), and (d)(l))).

CDER Executive Operations
Center for Drug Evaluation and Research
U.S. Food and Drug Administration