Congress of the United States
Washington, DC 20515

May 24, 2021

Janet Woodcock, M.D.
Acting Commissioner of Food and Drugs
Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993

Steven Romano, M.D.
Executive Vice President and Chief Scientific Officer
Mallinckrodt Pharmaceuticals
College Business & Technology Park
Cruiserath, Blanchardstown
Dublin 15, Ireland

Scott Riccio
Executive Vice President, Patient and Community Engagement
Mandos, LLC

Dear Acting Commissioner Woodcock, Dr. Romano, and Mr. Riccio:

We write to you on behalf of our constituent, Woodrow Miller, a 22-month old boy diagnosed with Niemann-Pick Type C1 (NPC) soon after birth. As you know, NPC is a rare, progressive genetic disorder with no known cure. Symptoms of NPC include losing the ability to talk, walk, and swallow. We have gotten to know Woodrow through the tireless efforts of his mother Denise, who has been a fierce advocate on behalf of her son. He is a special little boy and we are pleased that you have responded to the numerous calls by her, NPC advocates, and our offices to both the FDA and Mallinckrodt to allow Woodrow access to the experimental drug adrabetadex.

As you know, adrabetadex, the intrathecal treatment produced by Mallinckrodt Pharmaceuticals, is currently in experimental use through a clinical trial and the Expanded Access Program. The Expanded Access Program was designed for immediate, life-threatening conditions when no other options exist; in order for a patient to gain access to a drug under the Expanded Access Program, there must be agreement among the patient's physicians, the Food and Drug Administration (FDA), and the pharmaceutical manufacturer.

In January, Mallinckrodt announced the discontinuance of adrabetadex and the closing of its clinical trial; no new patients, including Woodrow, were able to gain access to the drug through the Expanded Access Program or through any means after October 2021. Many advocates, family members, and medical providers spoke out to try to reverse this decision and continue access to this potentially lifesaving drug.

We are incredibly encouraged by the news that Mallinckrodt has reached an agreement with Mandos, LLC, to transfer the Investigational New Drug (IND) application for adrabetadex and thus allow its utilization through the Expanded Access Program to continue. As stated in Mallinckrodt's press release, this agreement will allow access to adrabetadex for up to two years for eligible patients upon approval of the U.S. Bankruptcy Court for the District of Delaware. It will also allow Mandos to continue the development of adrabetadex in the future.

We are grateful for your willingness to find a solution for Woodrow and other children and families impacted by NPC. We urge you to take all available steps to ensure this agreement is finalized as soon as possible and that critical research continues into safe, effective treatment for NPC.

Thank you, and we look forward to your earliest response.

Sincerely,

Kyrsten Sinema
United States Senator

Mark Kelly
United States Senator

Debbie Lesko
Member of Congress