December 2, 2019

The Honorable Mitch McConnell
Majority Leader
United States Senate
S-230, The Capitol
Washington, DC 20510

The Honorable Charles Schumer
Democratic Leader
United States Senate
S-221, The Capitol
Washington, DC 20510

The Honorable Lamar Alexander
Chairman
United States Senate
Health, Education, Labor and Pensions Committee
428 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Patty Murray
Ranking Member
United States Senate
Health, Education, Labor and Pensions Committee
428 Dirksen Senate Office Building
Washington, DC 20510

Dear Majority Leader McConnell, Democratic Leader Schumer, Chairman Alexander, and Ranking Member Murray:

On behalf of the 25 to 30 million Americans with one of the over 7,000 known rare diseases, the National Organization for Rare Disorders (NORD) and the undersigned NORD member organizations write to support the nomination of Dr. Stephen Hahn as Commissioner of the Food and Drug Administration (FDA). We urge the Senate Health, Education, Labor, and Pensions (HELP) Committee to swiftly advance his nomination and the full Senate to vote to confirm Dr. Hahn.

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. NORD is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

Ensuring the continuation of FDA's work in support of the rare disease community is more important now than ever. There are over 7,000 rare diseases that afflict almost 30 million people in the United States alone. More than 90% of these diseases still have no FDA-approved therapy. Dr. Hahn has demonstrated, in both his own experience and in the recent HELP Committee hearing, that he understands and has the tools to support the critical public health mission of the agency, including helping deliver innovative, safe, and effective therapies to rare disease patients.

As a practicing oncologist most recently at the University of Texas MD Anderson Cancer Center, Dr. Hahn has first-hand knowledge of patients' experiences and has demonstrated his understanding of the importance of keeping these needs front and center in his work. At MD Anderson, Dr. Hahn also served as the Chief Medical Executive, which allowed him to acquire the requisite skills to lead a large organization like FDA. Further, Dr. Hahn has overseen many

clinical trials, providing him with valuable experience and insight into FDA's regulatory science pertaining to innovative clinical trial designs.

During the HELP Committee hearing, Dr. Hahn repeatedly affirmed his commitment to identifying and employing best practices within the agency to facilitate medical product development for diseases with unmet needs, including rare diseases. He emphasized the need to accelerate innovation and get treatments to patients. Finally, Dr. Hahn demonstrated during the hearing that he appreciates the critical role FDA plays in the stimulation of robust generics and biosimilars pathways, which promote accessibility and affordability of drugs for many rare disease patients.

For these reasons, NORD and its undersigned member organizations support the swift confirmation of Dr. Hahn as FDA Commissioner. Dr. Hahn has shown that he is committed to ensuring that FDA's appropriately high safety and efficacy standards for medical products are both upheld and balanced with the critical need for innovation, particularly in the rare disease space. Dr. Hahn will keep the patient at the center of this work.

FDA needs strong leadership, and we believe Dr. Hahn will provide it. We urge the Senate to swiftly confirm Dr. Stephen Hahn as FDA Commissioner.

Sincerely,

All Things Kabuki
American Behcet's Disease Association
American Multiple Endocrine Neoplasia Support
APBD Research Foundation
Association for Creatine Deficiencies
Avalon Foundation
Children's PKU Network
Congenital Hyperinsulinism International
Cure CMD
Cure VCP Disease
Dreamsickle Kids Foundation
FamilieSCN2A Foundation
Fibromuscular Dysplasia Society of America
Foundation for Prader-Willi Research
Friedreich's Ataxia Research Alliance (FARA)
Glut1 Deficiency Foundation
International Fibrodysplasia Ossificans Progressiva (FOP) Association
International Pemphigus Pemphigoid Foundation
Li-Fraumeni Syndrome Association (LFS Association/LFSA)

Lung Transplant Foundation
Marfan Foundation
Mila's Miracle Foundation
MLD Foundation
Moebius Syndrome Foundation
National Eosinophilia Myalgia Syndrome Network
National Organization for Rare Disorders (NORD)
National PKU News
NBIA Disorders Association
PTEN Hamartoma Tumor Syndrome Foundation
SSADH Association
Turner Syndrome Society of the United States
United Leukodystrophy Foundation