July 29th, 2020

The Honorable Anna Eshoo
Chairwoman
House Energy and Commerce Subcommittee on Health
2125 Rayburn House Office Building
Washington, DC 20515

The Honorable Michael Burgess
Ranking Member
House Energy and Commerce Subcommittee on Health
2125 Rayburn House Office Building
Washington, DC 20515

RE: Support for H.R. 4439 Creating Hope Reauthorization Act of 2019

Dear Chairwoman Eshoo and Ranking Member Burgess,

The National Organization for Rare Disorders (NORD) applauds the members of the Energy and Commerce Subcommittee on Health for holding this important hearing on the Creating Hope Reauthorization Act of 2019 (H.R. 4439) and urges the Subcommittee to swiftly move to consider the bill in markup so that it can become law at the earliest possible time. H.R. 4439 will cement a powerful incentive, the Rare Pediatric Disease Priority Review Voucher (RPD PRV), that has a proven track record of spurring the creation of treatments for rare diseases in children.

Since its founding in 1983, NORD has been an independent advocacy organization dedicated to patients and families affected by rare diseases and the organizations that serve them. NORD, along with its more than 300 patient organization members, is committed to the identification, treatment and cure of the more than 7,000 rare diseases, of which approximately 90% are still without an FDA-approved treatment or therapy. It is estimated that about half of all rare diseases affect children, and many of these diseases are fatal.

NORD has long supported the Rare Pediatric Disease PRV program. The Creating Hope Act was originally included as part of the FDA Safety and Innovation Act (FDASIA) in 2012 and created the RPD PRV. The priority review voucher is an incentive meant to spur the development of new treatments for diseases that would otherwise not attract interest from companies due to the high cost of development and the lack of market opportunities. The incentive provides the sponsor of a drug, which has been designated as treating a rare pediatric disease, with a voucher for priority review upon approval of that product. The voucher can then be redeemed on another product which can receive priority review. Priority review is a commitment from the Agency to reduce the review time of a product from a 10 month standard review to 6 months.

As of late February, of this year, 22 PRV's have been awarded for Rare Pediatric Diseases since the program was created in 2012. The PRV’s have been awarded for treatments for rare cancers, spinal muscular atrophy, and Batten disease among many other serious conditions.

Recent evidence suggests drug companies are prioritizing pediatric cancer drugs and drugs for rare pediatric illnesses as noted by a 558% increase in requests for rare pediatric disease designations (up from 26 in 2019, to 145 in 2020). This is clear evidence that sponsors have been influenced by the PRV as an incentive.

Drug development is extremely risky and only a small number of drugs make it to market, around 10%. Decisions to begin working on a product take place in the board room, in most cases over a decade before any product is approved. The PRV incentive provides additional assurances to companies that the risk of choosing to move forward with a product for a rare pediatric disease is reduced.

Critics have asserted that evidence of the program's success is limited. NORD submits that any such lack of evidence is due to the short life to date of the program. The program itself is not yet 8 years old which is less than the time it takes to develop a drug for a rare pediatric condition. Renewing the program for a longer term, if not permanently, would provide additional opportunities for robust data collection to support evidence of the program's effectiveness.

NORD appreciates the opportunity to provide these comments and stands ready to be a partner to the Committee to ensure the swift passage of this important program.

Sincerely,

Rachel Sher, J.D., M.P.H.

Vice President, Policy and Regulatory Affairs

National Organization for Rare Disorders