

Dear NPC Community,

FDA has received more than one hundred emails from patients with NPC and their families calling on FDA to find a path forward for approval of treatments for NPC. FDA's leadership thanks patients and families for taking the time to share their views with us, especially when there are disagreements. We heard loud and clear your risk tolerance and preferences at the recent patient listening session and will continue to factor that into our decision-making. Please know that we are not ignoring the needs of the NPC community – we see a devastating, relentless, disease that robs people of their lives and children. We will promptly approve any drug submitted to FDA that establishes effectiveness using flexible, feasible, efficient, and scientifically valid approaches and when those benefits outweigh the risks. We remain committed to working with you and the drug companies to advance therapies for this devastating disease.

Sincerely,  
Center for Drug Evaluation and Research  
U.S. Food and Drug Administration

