

NICE recommends the treatment Leniolisib for APDS



The National Institute for Health and Care Excellence (NICE) has recommended Leniolisib, for treating the rare primary immunodeficiency, activated phosphoinositide 3-kinase delta syndrome ([APDS](#)). Leniolisib is made by Pharming, and it is the first-ever treatment for activated phosphoinositide 3-kinase delta syndrome (APDS) licensed for NHS use in England and indeed in Europe. Currently, the recommendation is for Leniolisib to be available for people aged 12 years and over.

Sophie, whose little boy, Jasper, is affected by APDS, said “***Gives families like ours hope. This is amazing.***”

Susan, our CEO, said “*This is a hugely welcomed decision that will make a massive difference to people with APDS by allowing them to have a better quality of life. I’m delighted that NICE and Pharming were able to reach a mutual agreement on cost-effectiveness and a suitable price point. I would like to thank Sophie, our patient expert at the NICE committee meetings, and all the people who took part in our APDS impact survey and shared their personal stories of living with APDS with us. Your help was invaluable in supporting our extensive advocacy work with NICE over the last year.*”

[First licensed treatment for ultra-rare immune disorder recommended | NICE](#)