NICE National Institute for Health and Care Excellence

Leniolisib for activated phosphoinositide 3-kinase delta syndrome in people 12 years and over [ID6130]

Draft guidance comments form

Consultation on the draft guidance document – deadline for comments 5pm on **21 November 2024**. Please submit via NICE Docs.

	Please read the checklist for submitting comments at the end of this form. We cannot accept forms that are not filled in correctly. The Evaluation Committee is interested in receiving comments on the following: • has all of the relevant evidence been taken into account? • are the summaries of clinical and cost effectiveness reasonable interpretations of the evidence? • are the provisional recommendations sound and a suitable basis for guidance to the NHS? NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the preliminary recommendations may need changing in order to meet these aims. In particular, please tell us if the preliminary recommendations: • could have a different impact on people protected by the equality legislation than on the wider population, for example by making it more difficult in practice for a specific group to access the technology; • could have any adverse impact on people with a particular disability or disabilities. Please provide any relevant information or data you have regarding such impacts and how they could be avoided or reduced**.**
Organisation name – Stakeholder or respondent (if you are responding as an individual rather than a registered stakeholder please leave blank):	Immunodeficiency UK

Please return to: **NICE DOCS**

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Disclosure Please disclose any past or current, direct or indirect links to, or funding from, the tobacco industry.	None
Name of commentator person completing form:	Dr Susan Walsh, CEO, Immunodeficiency UK.

Comment number	Comments Insert each comment in a new row. Do not paste other tables into this table, because your comments could get lost – type directly into this table.
1	Overall, we are concerned that this initial NICE decision infers that the drug Leniolisib will be not be made available to people affected by APDS. Access to innovative treatments is a fundamental part of the UK Rare Disease Action Plan. This is the only drug that addresses the fundamental cause of APDS and we are pleased that NICE agrees (page 8 'there is an unmet need for an effective treatment that addresses the cause of APDS').
2	We have been asked to specifically comment on the impact of caring for someone with APDS: The impacts reported to the charity are on physical, mental health and quality of life. Carers report stress and anxiety, difficulty with sleeping, depression, feelings of isolation; fears about the future. Dealing with the emotional distress of the affected child/adult. Extra stress is caused by managing treatment, hospital appointments, dealing with unexpected hospital admissions; dealing with schools explaining periods of absence; continually explaining the condition to others. Carers report an inability to hold down a job or having to take reduced working hours leading to financial instability for the family; an inability to socialise with families leading restrictive lifestyles not being able to carry out normal family activities and hobbies. Carers reported an impact on unaffected siblings in terms lack of time to spend with them due to caring for an affected child or adult. As this is a genetic disorder people with APDS may be looking after their affected children so will be struggling with their own health problems as well as the responsibilities of caring.

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	Quotes from carers: 'Tough, exhausting'. 'It's stressful.' 'Emotionally distressing.' 'I'm worried all the time.' 'Concerns about [their] development both socially and academically.' 'The amount of hospital appointments and providing medical care each week at home'. '2-3 physio sessions a day, medicine administered daily, weekly subq infusions, frequent soiling as on antibiotics regularly'. 'delayed development so not potty trained and can't wash himself.' 'Unable to work and socialise. Tired and lack of sleep. Difficult to maintain routine.' 'I have to provide help with day-to-day activities.' 'Significantly, my mother had to give up work, family holidays had to be cancelled, hobbies for my siblings had to be cancelled, time my parents spent with my siblings was compromised as they were always with me.' 'I am unable to work due to xxx's condition as she is constantly getting infections and needs iv medication at least 2/3 monthly', 'Mindful of no soft plays, limit to parks and long walks.' 'Disproportionately caring for child with APDS over other children, lots of holiday from work spent hospital admissions, days work around physio'. 'We all have to know about it, the younger sibling has to fit around the treatment, we have to pay extra for travel insurance, we have to be more conscious of infection, we have to fit in hospital appointments and medical supplies ordering.' .
3.	Page 18 Concerning the emotional benefit of having the drug – the emotional toll on patients and carers of having APDS would be reduced due to the drug causing direct improvements in health such as reduced number of infections, reduced treatment burden, increased energy, increased ability to socialise and have a normal family life.

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	These would be in addition to reducing fears about future health risk of developing lymphoma.
4	Page 20 states 'Many new and existing treatments provide increased hope…'. This is a sweeping statement and unsubstantiated. We ask NICE to validate this statement by defining the 'many new' [treatments] as we are not aware of other new drugs that specifically target the defect in APDS.

Insert extra rows as needed

Checklist for submitting comments

- Use this comment form and submit it as a Word document (not a PDF).
- Complete the disclosure about links with, or funding from, the tobacco industry.
- Combine all comments from your organisation into 1 response. We cannot accept more than 1 set of comments from each organisation.
- Do not paste other tables into this table – type directly into the table.
- Please underline all confidential information, and separately highlight information that is 'commercial in confidence' in turquoise and information that is 'academic in confidence' in yellow. If confidential information is submitted, please submit a second version of your comments form with that information replaced with the following text: 'academic / commercial in confidence information removed'. See the NICE Health Technology Evaluation Manual (section 5.4) for more information.
- Do not include medical information about yourself or another person from which you or the person could be identified.
- Do not use abbreviations.
- Do not include attachments such as research articles, letters or leaflets. For copyright reasons, we will have to return comments forms that have attachments without reading them. You can resubmit your comments form without attachments, it must send it by the deadline.
- If you have received agreement from NICE to submit additional evidence with your comments on the draft guidance document, please submit these separately.

Note: We reserve the right to summarise and edit comments received during consultations, or not to publish them at all, if we consider the comments are too long, or publication would be unlawful or otherwise inappropriate.

Comments received during our consultations are published in the interests of openness and transparency, and to promote understanding of how recommendations are developed. The comments are published as a record of the comments we received, and are not endorsed by NICE, its officers or advisory committees.