

Highly Specialised Technology Committee Interests Register

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

Publication Date: 23 April 2025

Name	Role with NICE	Type of interest	Description of interest	Interest declared	Comments
Emtiyaz Chowdhury	Committee Member	Non-financial professional	Emtiyaz declared the following: I am employee of Parexel. Parexel provides services to the manufacture. I've not been involved in direct work associated with either technology.	10/10/2024 06/02/2025	It was agreed that Emtiyaz's declaration would not prevent them from participating in discussions on this evaluation.
Susan Walsh	Clinical Expert	Indirect	Susan Walsh declared the following: Organisation interests (Immunodeficiency UK): Immunodeficiency UK received £12,324 in funding from Pharming in Sept 2024. The funding was restricted to admin support, website, database and IT costs.	17/12/2024	It was agreed that Susan's declaration would not prevent them from providing expert advice to the committee.

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Austen Worth	Clinical Expert	<p>Direct non-financial</p> <p>Indirect</p>	<p>Austen declared the following:</p> <p>I am UK chief investigator on a trial of Leniolisib as treatment for patients with APDS between the ages of 1 year and 6 years of age, which is soon to open. This group of patient at present have limited evidence for the efficacy of this drug.</p> <p>Pharming have sponsored a national clinical audit meeting we hosted last year, providing £3000.</p>	05/10/2023	It was agreed that Austin's declarations would not prevent them from providing expert advice to the committee.
Siobhan Burns	Clinical Expert	Direct financial	<p>Siobhan declared the following:</p> <p>I have been invited to be on Pharming's UK expert advisory programme for leniolisib in activated PI3K delta syndrome (APDS).</p> <p>I have an Advice Agreement for ad-hoc consultancy work with Pharming, set up through UCL Consultants.</p>	14/09/2023	It was agreed that Siobhan's declarations would not prevent them from providing expert advice to the committee.

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			<p>Pharming provided financial support for an educational event that I organised for Primary Immunodeficiency (UCL/Newcastle University Winterschool 2023).</p> <p>I have participated in a conference (AIPID workshop, Barcelona 2023) in part sponsored by Pharming and had travel expenses paid for by the conference organisers.</p> <p>I have received grant support from the European Union, National Institute of Health Research, the UK Research Institute, the Medical Research Council, the Wellcome Trust, the Royal Free charity, Rosetrees charity, the Jeffrey Modell Foundation, UCLH and GOSH/ICH Biomedical Research Centers and CSL Behring. I have received personal fees or travel expenses from Immunodeficiency Canada/IAACI, CSL Behring, Baxalta US Inc, Grifols, Pharming, GSK and Biotest. I undertake a small amount of private clinical</p>		

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		Direct non-financial	<p>practice in Clinical Immunology at the Royal Free London.</p> <p>I am:</p> <ul style="list-style-type: none"> • Chair of the European Society of Immunodeficiency (ESID) Clinical Working party and a Board member of ESID • A member of the NHS SCID Newborn Screening Diagnostic Reference Panel • Chair for the ERN-RITA Transition Working Group • A member of the UK Clinical Immunodeficiency Professional Network A member of the Chronic Granulomatous Disease Society Research Advisory Panel 		

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		Indirect	<ul style="list-style-type: none"> • A member of the International Primary Immunodeficiencies Congress (IPIC) Scientific Committee • Associate Editor for Frontiers in Immunology, section Primary Immunodeficiencies <p>I have published several articles in APDS. I co-supervise a PhD aimed at developing gene editing treatments for APDS. My research group is developing functional assays to test variants of unknown significance in the APDS genes, to assist with diagnosis.</p> <p>I provide advice and expert opinion to the International Patient Organisation for PID (IPOPI) and recently was invited to the IPOPI Global Stakeholders Summit 2023.</p> <p>I lead the Clinical Working Party for ESID which produces guidance around Management of Primary Immunodeficiency for clinicians</p>		

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			managing patients with PID and secondary immunodeficiency.		