

Inquiry into the equitable access to diagnosis and treatment for individuals with rare and less common cancers, including neuroendocrine cancer

Melbourne Hearing –1 February 2024

Question on Notice from Senator Janet Rice

Ipsen

1. Thank you for the opportunity to provide a response to the question from Senator Rice:

CHAIR: What's the definition—I've heard the term 'orphan designation' over the last two days, but we haven't had it defined for us.

Response:

An orphan drug is a medicine, vaccine or in vivo diagnostic agent that meets the requirements of regulation 16J of the Therapeutic Goods Regulations 1990.

Full details of the TGA Orphan Drug Designation eligibility criteria can be found at: <https://www.tga.gov.au/resources/resource/guidance/orphan-drug-designation-eligibility-criteria>

The orphan drug prevalence threshold referred to within the session being that “*the condition affects fewer than 5 in 10,000 individuals in Australia when the application is made*”.