



**Genetic Alliance UK**  
Supporting. Campaigning. Uniting.

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Mr William Powell AM  
Assembly Member for Mid and West Wales  
Chair Petitions Committee  
Ty Hywel  
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05 November 2014

Dear Mr Powell,

[Response to letter from the Minister for Health and Social Services regarding the Petition P-04-570 – Inequitable access to treatments that have not been nationally appraised in NHS Wales](#)

In his letter dated 31<sup>st</sup> August 2014, the Minister for Health and Social Services, Mark Drakeford, outlined his response to a petition supported by Genetic Alliance UK, Tuberous Sclerosis Association and Association of Glycogen Storage Diseases, and signed by over 1,000 individuals and patients which called for a review into the use of the 'exceptionality' criterion when determining whether a patient with a rare condition is able to access a treatment through the Individual Patient Funding Request (IPFR) process.

In his response, the Minister referred to the recent review of this process by the National IPFR Review Group in October 2014. The remit of that review did not include a consideration of the appropriateness of the exceptionality criterion, and instead focused on improving the transparency and consistency of the process. Genetic Alliance UK sat as a member of that review group and at no point was the appropriateness of the exceptionality criterion to IPFR applications from rare disease patients discussed or considered.

While we accept that the IPFR process is not designed as a means through which rare disease patients can access the medicines their clinician's agree they need, due to a lack of alternative options this route is the only one available to them. The exceptionality criterion is a barrier that prevents them from being able to take these medicines as a result of deficiencies within the rest of the system.

As our petition states, a review of the current IPFR criteria by the Health and Social Care Committee, including an investigation into the use of the exceptionality criterion when considering IPFR applications from patients affected by rare conditions, is necessary to finally address this issue.

Yours sincerely,

Alastair Kent OBE, Director of Genetic Alliance UK and Chair of Rare Disease UK

### Why are so many patients with rare conditions making IPFR requests?

Currently, many rare disease medicines have not been through a national health technology appraisal. As Genetic Alliance UK's recent work revealed, this is a particular issue for patients affected by rare conditions because the National Institute for Health and Care Excellence (NICE) has historically appraised very few rare disease medicines: less than 10% of the 47 rare disease medicines launched between 2002 and 2013 for the treatment of non-cancer indications were appraised by NICE<sup>1</sup>. This is unlikely to change in the future as NICE's new process for appraising highly specialised technologies only has capacity to appraise three medicines a year<sup>2</sup>. The duty of evaluation of the vast majority of medicines for rare diseases will pass to AWMSG in Wales.

As a result of these factors, no national commissioning policy exists for many rare disease medicines and so patients in Wales currently have no alternative but to try to access potentially life changing medicines through the IPFR process.

In this context, it is clear how the 'exceptionality' criterion creates a barrier to medicine access for patients with rare conditions as in many cases it is a whole group of patients who are applying for access to a treatment and, by definition, do not differ from each other. In these cases it is the whole patient population that could benefit and a thorough appraisal of the evidence would be ideal. The urgency of the medical need and the risk of avoidable progression is great and therefore waiting for such an appraisal to be carried out is not an option.

### Why do patients with rare diseases believe that the 'exceptionality' criterion is not appropriate?

The report published by the IPFR Review Group defines the term 'clinical exceptionality' as: 'the patient's clinical condition is significantly different to the general population of patients with the same condition and as a result, the patient is likely to gain significantly more benefit from the intervention than might normally be expected.'

Aside from the fact that in the absence of any commissioning policy the exceptionality criterion becomes a barrier to cohorts of patients accessing a treatment through the IPFR process as a last resort, there are two additional reasons why this criterion can disadvantage patients with rare conditions seeking to access medicines through IPFRs:

1. Rare diseases often vary in the nature and severity of the associated symptoms. It is therefore difficult to identify one patient as 'exceptional'.
2. The patient population affected by a single rare condition is small. As a result there can be little information about the natural history of the condition and/or limited evidence available. It can be difficult to prove that one patient is different from a population about which little is known.

**Genetic Alliance UK** is the national charity working to improve the lives of patients and families affected by all types of genetic conditions. We are an alliance of over 180 patient organisations. Our aim is to ensure that high quality services, information and support are provided to all who need them. We actively support research and innovation across the field of genetic medicine.

**Rare Disease UK** is a multi-stakeholder campaign run by Genetic Alliance UK, working towards the delivery and implementation of the UK Strategy for Rare Diseases, which was published by the Department of Health in November 2013.

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<sup>1</sup> Genetic Alliance UK's Patient Charter on NHS England's commissioning of medicines for rare conditions (October 2014)  
Accessed here: [www.geneticalliance.org.uk/docs/hst-patient-charter\\_final.pdf](http://www.geneticalliance.org.uk/docs/hst-patient-charter_final.pdf)

<sup>2</sup> Genetic Alliance UK's Patient Charter on NICE's Highly Specialised Technology Evaluation Programme (April 2014)  
Accessed here: [www.geneticalliance.org.uk/docs/hst-patient-charter\\_final.pdf](http://www.geneticalliance.org.uk/docs/hst-patient-charter_final.pdf)