Y Pwyllgor Deisebau
Petitions Committee

Cynulliad Cenedlaethol Cymru
National Assembly for Wales

David Rees AM
Chair of the Health and Social Care Committee
Ty Hywel
Cardiff Bay
Cf99 1NA

Bae Caerdydd / Cardiff Bay
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Our ref: P-04-570

December 2014

Dear David

The Petitions Committee is currently considering the following petition from Genetic Alliance UK, Tuberous Sclerosis Association, Association of Glycogen Storage Disorders, which has collected 1089 Signatures.

P-04-570 Inequitable Access to Treatments That Have Not Been Nationally Appraised in NHS Wales

We the undersigned call on the National Assembly for Wales to review the use of the "exceptionality rule" in determining whether a patient can access a treatment through the Individual Patient Funding Request process.

***Additional Information**: To access treatments through the IPFR process, a patient population must demonstrate its exceptionality. For common illnesses, it may be possible to identify a subset of patients within the larger population who are more likely to respond to a particular therapy. For rare disease patients, demonstrating that you are a unique patient when you are part of a small group of patients whose condition is considered rare is practically impossible. The exceptionality criteria place an onus on clinicians to provide evidence that the patient's clinical condition is significantly different to the general population of patients with the same condition and is likely to gain significantly more benefit from the intervention than might normally be expected. This evidence requirement is too onerous to apply to patients with rare diseases due to small patient numbers within rare disease*

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Croesewir gohebiaeth yn y Gymraeg a'r Saesneg/We welcome correspondence in both English and Welsh

populations. Patients with great clinical need are prevented from accessing life-changing/ life-saving treatments.

At our meeting on 11 November, the Committee considered correspondence from the Minister for Health and Social Services and the Petitioner. I attach copies of this correspondence for your information.

We agreed to draw the petition to the attention of the Health and Social Care Committee and ask whether you would be prepared to consider carrying out an inquiry as requested by the petitioners.

I would be grateful if you could draw this letter to the attention of the Committee and let me know their views.

Yours sincerely

William.

William Powell AC / AM
Cadeirydd / Chair

Enclosures:
Correspondence from the Minister for Health and Social Services dated 31 August 2014; and
Correspondence from the Petitioner dated 5 November 2014.

Mark Drakeford AC / AM
Y Gweinidog Iechyd a Gwasanaethau Cymdeithasol
Minister for Health and Social Services

Llywodraeth Cymru
Welsh Government

Eich cyf/Your ref P-04-570
Ein cyf/Our ref MD/03174/14

William Powell AM
Assembly Member for Mid & West Wales
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31 August 2014

Dear William,

Thank you for your letter of 14 August on behalf of the Petitions Committee, regarding Petition P-04-570 - Inequitable access to treatments that have not been nationally appraised in NHS Wales.

As you are aware, I announced a review of the IPFR process on 16 October last year. The review group produced a report which concluded the current IPFR process in Wales supports rational, evidence based decision making for those treatments which are not routinely available. They also made a number of recommendations aimed at strengthening the process. On 30 April 2014 I issued a Cabinet Written Statement regarding access to medicines, including the recent IPFR review, which can be accessed at:

http://wales.gov.uk/about/cabinet/cabinetstatements/2014/?lang=en

The Written Statement also announced we will have a new appraisal system specifically tailored to address those medicines that treat rare diseases; often referred to as orphan and ultra orphan medicines. I will be issuing a further statement on access to new medicines in September.

I hope this information is helpful and clarifies the current position.

Best wishes,

Mark.

Mark Drakeford AC / AM
Y Gweinidog Iechyd a Gwasanaethau Cymdeithasol
Minister for Health and Social Services

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Mr William Powell AM
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Chair Petitions Committee
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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 31st 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

Registered charity numbers: 1114195 and SC039299
Registered company number: 05772999

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[1]. 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[2]. 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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[1] 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

[2] 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