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Mr William Powell AM
Assembly Member for Mid and West Wales
Chair Petitions Committee
Ty Hywel
Cardiff Bay
Cardiff
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28 January 2015

Dear Mr Powell,

Response to letter from David Rees AM, Chair of the Health and Social Care Committee, regarding the Petition P-04-570 – Inequitable access to treatments that have not been nationally appraised in NHS Wales

The petitioners extend thanks to David Rees AM for his response to the request to review the current 'exceptionality' criteria within the IPFR process and acknowledgement that the Health and Social Care Committee has limited capacity to conduct a review of this criteria.

In his written statement on next steps following a review of the Individual Patient Funding Request (IPFR) Process dated 5th November 2014, the Minister for Health and Social Services, Mark Drakeford, outlined that, 'it is not appropriate to routinely use the IPFR process to determine access to orphan and ultra-orphan medicines'. The petitioners support this statement, as it is recognised that the exceptionality criterion is a barrier that prevents patients being able to access these medicines due to deficiencies within the rest of the system. We are committed to working with the All Wales Therapeutics and Toxicology Committee (AWTTC) to ensure that the new process for appraising these medicines is timely and robust, drawing on both clinical and patient expertise. It is also critical that mechanisms for developing patient cohort policies are developed by the Welsh Health Specialised Services Committee (WHSSC) to ensure that the current gaps in process are addressed.

In response to a written question by Darren Millar AM on 18th November 2014, Mark Drakeford stated that he expected work on the revised process to be completed in full by September 2015. It is essential that interim commissioning measures are put in place to enable access to orphan and ultra-orphan medicines for patient cohorts whilst new processes are in development. Patients with great clinical need are currently being denied access to life-enhancing treatments because they have no route of access to these medicines. They are denied access through the IPFR process as they are not considered 'exceptional', even though their clinician agrees that they require access to the therapy.

The petitioners request that the Minister outlines timescales for developing interim commissioning policies for patient cohorts who are currently denied equitable access to potentially life-saving therapies. It is critical that patients receive interim decisions whilst processes are developed. This issue affects two groups of patients. Firstly, patient cohorts who are denied access to therapies as a result of insufficient routes to make them available to the patient community. Secondly, those patients who

are at risk of withdrawal of treatment received via compassionate grounds from pharmaceutical companies.

Denial of access to or withdrawal of a therapy for a patient with a rare disease often results in an immediate decline in the health of the individual and has a significant, detrimental impact on the patient and their family. It is therefore essential that interim measures are put in place to prevent decisions based on inappropriate and inequitable processes and, to ensure that therapies are available to those who need them.

Yours sincerely,

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

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.