?Parliamentary debate calls for an end to the deadlock in failure to provide drug treatment for the rare disease PKU

Friday 23 March, 2018

Vicky Ford MP (Chelmsford, Conservative) spoke at an adjournment debate in Parliament this afternoon about her constituent Cait Cotter. Ten year old Cait has the rare disease phenylketonuria (“PKU”) which was diagnosed as a newborn baby by the heel prick test.

PKU is a rare genetic metabolic disorder affecting around 1:10,000 people in the UK. People with PKU cannot metabolise phenylalanine, an amino acid found within protein foods. Currently, the only treatment funded in the UK is an extremely restricted dietary therapy in which almost all natural protein is removed from the diet. The majority of PKU patients in other European nations have access to the drug BH4 (Kuvan). The NHS does not currently commission Kuvan treatment.

Vicky Ford told Parliament that her constituent Cait struggles with the strict PKU diet. Her parents say she is permanently hungry, and her whole day is ruled by limited food and having to drink artificial protein supplements. Even vegetables such as potato and cauliflower need to be restricted.

For one month Cait enjoyed a one month free trial of the drug Kuvan, which is the only licensed non dietary treatment for PKU. Cait’s protein tolerance increased threefold and she could enjoy food without fear or caution. Her energy, mood, alertness and focus improved.

It was noted that Kuvan is available in the majority of European countries already. However the appraisal of the drug by the NHS has stalled. Vicky Ford stated that the UK is a world leader in the life sciences, but if we want to stay at the forefront of world research we must make sure that British patients like Cait can benefit from these discoveries. Ford called for an end to the deadlock, asking the drug manufacturer BioMarin and NICE to "engage with each other in a transparent way that has the full support of the patient organisation. The whole patient population should be considered." Ford noted that patients taking the drug would need less dietary, medical and educational interventions, and these costs savings need to be considered against the cost of the drug. It was also noted that BioMarin, has indicated a willingness to offer substantial discounts to the NHS.

Jackie Doyle-Price MP, on behalf of the Ministry of Health, stated that “I am happy to report that NHS England has received a preliminary policy proposal for the use of Kuvan in the management of PKU for adults and children, as new evidence has now been published to support its use… NHS England is now working with NICE to agree the best approach and has asked it to consider developing advice on the use of Kuvan.”

Eric Lange, Chairman of NSPKU, the national charity for PKU, said “We are grateful for Vicky Ford for highlighting the UK’s failure to provide access to Kuvan treatment for PKU. Patients and families are told to place hope in a fair system to make a decision on drug funding. It has often seemed that the NHS is determined to keep ‘kicking the can down the road’ rather than make a fair decision. We hope that real progress is made on this issue as a matter of urgency.”

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