A call for action for PKU: 6 key demands for change

1. BH4 (Kuvan) is the only licensed non-dietary treatment for PKU, which works for about 30%-50% of people with PKU. BH4 allows patients to eat more natural protein and keep their phenylalanine levels within safe limits, thus leading to good neurocognitive outcomes. BH4 has been licensed in the EU since 2008 and is used in the majority of EU countries. The NHS does not commission BH4 treatment despite good evidence of clinical efficacy. NHS England is due to re-consider commissioning BH4 treatment this year. **We are calling for MPs to ensure that the NHS commissioning process for BH4 and any future non-dietary treatments for PKU is fair, transparent and ensures equitable access to treatment. The UK must no longer lag behind the rest of Europe in access to treatments for PKU.**

2. The European guidelines for PKU care state that all patients should be tested for BH4 (Kuvan) responsiveness. Clinics in the UK fail to test patients, so they are kept in the dark about their ability to tolerate a better diet, which in turn would lead to a better life. **MPs should ensure that all patients with PKU are entitled to BH4 responsiveness testing.**

3. Currently patients with PKU suffer patchy care depending on location. **We are calling for MPs to ensure that all children should be treated and followed up in specialised metabolic centres, with an experienced metabolic physician and dietitian.**

4. Many adults with PKU are “lost to treatment” – they are failing to receive adequate metabolic care or any care at all. Without adequate treatment, adults with PKU are at higher risk of neurocognitive, mental health and nutritional problems and may not meet their full potential as citizens. **We are calling for MPs to ensure that all adults with PKU have life-long, systematic follow-up in specialised metabolic centres.**

5. Many patients with PKU suffer psychological or neurocognitive issues related to their condition. Children may have learning difficulties and do not receive proper help. Adults commonly experience problems such as anxiety and depression related to inadequate management of their PKU. **MPs should ensure that all patients with PKU have access to psychological support in metabolic clinics. Patients should receive age appropriate neurocognitive testing and follow-up support.**

6. Patients with PKU need to eat prescribed low phenylalanine food and dietary supplements to stay healthy. However, these items are prescribed through GP surgeries who lack a specialist understanding of the complex nutritional needs of patients with PKU. As a result, many patients experience unacceptable restrictions or difficulties in accessing these foods and supplements which can endanger their health. **MPs should ensure that specialist metabolic centres should administer prescriptions for PKU foods and dietary supplements to achieve better patient outcomes and a more efficient NHS service.**