

Interview on Phenylketonuria (PKU)
Proposed Discussion Guide for National Policy-Makers

Good morning / afternoon. I am conducting a market research survey on behalf of Weber Shandwick among policymakers in INSERT COUNTRY. If you are interested in taking part, I would like to ask you a few questions to see if you fit the profile of policy makers we are looking to take part.

Before I start, I would like to assure you that we act in accordance with the ABPI, MRS and BHBIA codes of conduct regarding anonymity and confidentiality – the aim of this market research is to gain your views and is not in any way promotional. We are also fully compliant with the Data Protection Act.

Any information you disclose will be treated in the strictest confidence and the results are pooled so no answers are attributable to any individuals.

The topic of the market research is policy makers perception and awareness of issues relating to Phenylketonuria (PKU) . The research is strictly non-promotional and the main objective is to understand how Public Policy Makers currently see PKU on the general government agenda and specifically on the national health policy agenda health.

The market research is being conducted on behalf of INSERT NAME. The market research study itself is strictly non-promotional. The company who is commissioning this research do not hold or see any information regarding respondents.

GENERAL GOVERNMENT AGENDA ON HEALTH POLICY

Experience of working on rare diseases

Objective: Examine the policy-maker's experience of working in the field and how they consider rare diseases are perceived amongst fellow health policy-makers.

- (1) Please could you describe the work you have carried out on rare diseases and the current policies/ initiatives you are working on?

- (2) Are policies on rare diseases, such as PKU, visible among your peers working in the health arena?

- (3) Do you feel other policy-makers in health are aware of your work in the field? Please expand on your thoughts.

Visibility of rare diseases at national level amongst the public

Objective: Assess how visible rare diseases are amongst the national public and which mediums/ channels/ stakeholder groups are used to help raise awareness.

- (1) What campaigns – government or charity-funded – are run at national level to raise awareness of rare diseases?

- (2) What mediums, if any, are used to raise awareness of rare diseases:
 - Parliamentary debates

 - Televised/ radio campaigns

 - Newspapers/ magazines/ weekly publications

 - Social networking sites

 - Events

 - Other:.....

- (3) On a scale of 1 to 5, with 5 being the highest level, how visible do you think rare diseases are amongst the general public?
 1. Not visible
 2. Little visibility
 3. Some visibility
 4. Visible
 5. Very visible

Priority of rare diseases on the health agenda

Objective: determine the perception of rare diseases as a public health policy priority and the state of play of implementing the Council Recommendations on rare diseases. (Note: Council Recommendations, although not legally binding, are the strongest political statement that can be made by national ministers at the EU level .)

- (1) On a scale of 1 to 5, with 5 being the highest level, to what extent is tackling rare diseases a national health priority?
 1. Not a priority
 2. Low priority
 3. Medium priority

4. Priority
5. High priority

(2) Are the Council Recommendations (published in 2009) on rare diseases incorporated or being implemented via policies at national level? Please state the state of implementation of the following key Recommendations by the Council:

- Establishment and implementation of plans or strategies for rare diseases
- An adequate definition, codification and inventorying of rare diseases research in rare disease
- Centres of expertise and European reference networks for rare diseases
- The gathering the expertise on rare diseases at European level
- Empowerment of the patient organisations
- Sustainability of infrastructures developed in the field of information, research and healthcare for rare diseases.

(3) Is the issue of rare diseases coupled with other public health initiatives at national level such as HTA policy or policies for paediatric treatments? If yes, please provide details and if no, then please could you outline any future possibilities?

(4) Will the government be looking at the issue of distinguishing orphan drugs from other medicines during HTA evaluations and ensuring budget allocation for orphan drugs are adapted to take into account this distinction.

Awareness of Phenylketonuria (PKU) and visibility among national policy-makers

Objective: determine policy-maker’s knowledge and understanding of PKU, its effect on the patient and available treatments; determine visibility of PKU as a rare disease amongst national policy-makers and which mediums/ channels/ stakeholder groups are used to help raise awareness.

- (1) Have you come across Phenylketonuria (PKU) and if yes, please could you describe the context?

- (2) What is your understanding of the effects PKU has on the patient, when it can be detected and how it effects the patient?

- (3) Do you know which treatments (amino acids supplements, Phe free food, medication BH4) can be used for patients with PKU? If yes, please could you expand.

- (4) On a scale of 1 to 5, with 5 being the highest level, how visible is PKU as a rare disease amongst the general public?
 1. not visible
 2. little visibility
 3. some visibility
 4. Visible
 5. Very visible

- (5) If visibility has been established, through which medium has awareness been raised?
 1. Via third parties raising awareness (e.g. patient associations, health NGOs)
 2. Via media
 3. Via implementation of policies regarding rare diseases
 4. EU or national campaigns (if yes, please specify)
 5. Other:.....

- (6) On a scale of 1 to 5, with 5 being the highest level, how knowledgeable of PKU are policy-makers , its effects on patients and how it can be treated?
 1. No knowledge

2. Little knowledge of PKU
3. Some knowledge of PKU
4. Knowledgeable of PKU
5. Highly knowledgeable of PKU

(7) Do you feel PKU should be more visible amongst fellow policy-makers and if so, why?

National plans/ initiatives for PKU

Objective: determine whether there are national plans for the management of PKU (or for rare diseases including PKU) and possible future initiatives.

(1) Have any national plans/ initiatives been adopted for the management of PKU and, if so, please provide details?

(2) If no national plans/ initiatives are underway, will there be any upcoming in the near future on treatment of PKU? If so, please could you provide details?

(3) Are there any national plans/ initiatives currently on the agenda regarding other rare diseases?

(4) Are there any national policies for the testing/ detection of rare diseases in young babies?

Pricing and reimbursement

Objective: determine current pricing and reimbursement policies at national level for patients with PKU.

(1) Reimbursement of amino acids supplements, Phe-free food, medication BH4, for PKU:

1. Is treatment for amino acid supplements reimbursed?

If yes:

- a. How is it reimbursed?
- b. Up to what age is it reimbursed?
- c. Are there any restrictions on reimbursement policy?

2. Is treatment of Phe-free foods reimbursed?

If yes:

- a. How is it reimbursed?
- b. Up to what age is it reimbursed?
- c. Are there any restrictions on reimbursement policy?

3. Is treatment of medication BH4 reimbursed?

If yes:

- a. How is it reimbursed?
- b. Up to what age is it reimbursed?
- c. Are there any restrictions on reimbursement policy?

(2) Is reimbursement via a mutual/ insurer, and if so, up to what percentage is reimbursed?

(3) If treatment is available via prescription, up to what age group?

(4) Does reimbursement vary between geographical areas/ regions?

(5) Do you know the cost of PKU on the healthcare system? Is this information publicly available?

- (6) Have HTAs identified cost savings from early detection and treatment of PKU? If yes, please could you provide information and if no, please could you alert us to future research which could help identify savings?

Stakeholder engagement

Objective: determine the policy-maker's engagement with third parties and the frequency of contact with stakeholders.

- (1) Is the Government (Ministry of Health) currently engaged in discussions with third parties regarding treatment for PKU and if yes, please could you provide details? Do they include:

1. Patient organisations
2. Industry
3. Other policy-makers (at national or European level)
4. Clinicians
5. Health economists
6. HTA experts
7. Other:.....

- (2) How often do you engage with any of these stakeholders and through which mediums?

1. One to one meetings
2. Events
3. Via other parties
4. Policy debates

Centres of Excellence/ Networks of Expertise

Objective: to assess whether there are any (or planned) 'Centres of Excellence' for PKU or rare diseases in children, in the target country. (To note: at EU level the Council Recommendations on Rare diseases raises the need for Centres of Excellence and this is complemented by the EU Directive on Cross Border Healthcare which recognises the need to pool expertise between Member States to create Networks of Expertise on rare diseases and create 'Centres of Expertise'.)

- (1) Could you please describe the state of play for implementing at national level EU initiatives on rare diseases:
 - a. Council Recommendations to establish 'Centres of Excellence' focused on rare disease?
 - b. The EU Directive on Cross Border Healthcare which establishes the need to set up Networks of Expertise for rare diseases?

- (2) What do you define as a 'Centre of Excellence'?

- (3) What do you define as a 'Network of Expertise'?

- (4) Is there a Centre of Excellence for rare diseases established in your country or a neighbouring country? If yes, please provide details. Please state also if you do not know.

- (5) Do you know who to contact at EU level to find out further information about the nearest Centre of Excellence for rare diseases? If yes, please provide details.

- (6) How is the Centre of Excellence nearest to you funded?

- (7) Under your current health system, do you feel patients have a high level of access to the nearest Centre of Excellence for rare diseases? How are referrals made?

- (8) Are policy makers and third parties in your country actively engaged in dialogue with the Centre of Excellence?

- (9) Has a Network of Expertise been established on PKU and is information/ data from your country provided to the network?

- (10) Can patients with PKU travel to another EU Member State to receive treatment and care or is sufficient treatment/ care provided in the home country?

Health and Expertise Promotion

Objective: to determine the policy-maker's awareness/ contact with PKU experts.

- (1) Are there PKU experts in your country and if so could you please provide details?

- (2) Are PKU experts high-profile such as participating in national campaigns, or via the media, etc?

EU-funded projects

Objective: to determine policy-makers awareness of current/ potential EU-funded projects focused on PKU and on any rare diseases.

- (1) Are you aware of any EU-funded projects on PKU which involve organisations from your country? If yes, are national policy-makers aware of the research underway and kept informed of developments?

- (2) If you are aware of EU-funded projects, please could you provide details and the parties participating from your country? Please also provide any financial details/ funding provided that you may be aware of.

- (3) Are you aware any organisations (e.g., research institutes) in your country which may want to participate in an EU-funded project?