Inquiry into the current systems of licensing, procurement, commissioning and prescription of treatments for genetic bleeding disorders in the UK

- The inquiry will document the current processes for licensing, procurement and commissioning of treatments for genetic bleeding disorders in the UK
- It will do this with a view to how these impact on the treatments that patients are offered and can be offered by the NHS
- We will seek extensive input from all stakeholders: Government, the NHS, industry, clinicians and people with bleeding disorders
- The purpose is to show where the processes are working well and when they are not
- The inquiry will make recommendations for how the processes could be changed to improve access to treatments and outcomes for people with bleeding disorders

People with genetic bleeding disorders lack certain proteins that enable their blood to clot normally. They tend to require replacement clotting factors or other therapies to manage their bleeding.

The reason for this investigation is that many treatments are expensive which leads to restrictions on the extent they can be prescribed. In some cases new treatments may not be commissioned at all, or restricted to certain doses or patients. This has a detrimental impact on outcomes and quality of life for people with bleeding disorders.

Public opinion supports an improvement in this situation with research by Populus showing that a substantial majority (72%) of people in the UK think that patients should have access to treatment based on clinical need even if this is costly to the NHS because of the rarity of the disease.

How are people with bleeding disorders treated?

Most treatments for severe bleeding disorders in the UK are replacement factor products. This includes plasma-derived clotting factors, recombinant products and some novel therapies. These therapies tend to need to be given intravenously multiple times a week.

People with bleeding disorders will be registered at haemophilia centres where a multi-disciplinary team provide the care required to manage their condition.

How are treatments procured and commissioned

The CMU, part of NHS England, runs tender exercises to produce frameworks which set the prices for most products across the UK.

Treatment for bleeding disorders fall under specialised commissioning in England and similarly in other parts of the UK decisions are made on an NHS wide basis.

The most appropriate treatment will be prescribed and monitored by a specialist haemophilia centre in line with national guidelines and commissioning policies.

How will it gather evidence

The APPG has launched a call for evidence asking all stakeholders to submit their views on the questions the inquiry is considering. This call for evidence will remain open for 12 weeks. Any evidence or queries can be sent to appg@haemophilia.org.uk

The secretariat to the APPG will seek all information published on the current processes and will invite stakeholders to share information. The inquiry would also like to contrast policies in the UK with those in other countries and will seek submissions from aboard.

Who are the key stakeholders

- Patients and Patient Groups
- Treating Clinicians and Clinician Groups
- Other Healthcare professionals
- Companies involved in the development, manufacture or supply of bleeding disorder treatments
- The Departments of Health across the UK
- The Commercial Medicines Unit
- NHS England, Scotland, Wales and Northern Ireland
- NHS Trusts
- NICE, MHRA and other regulators

What will be the outputs

The inquiry will produce a report on the current situation and the views of people with bleeding disorders and other stakeholders.

The report will then make recommendations for the future to improve process and outcomes.

About the APPG

The APPG on Haemophilia and Contaminated Blood is a cross party group of over one hundred MPs. The secretariat to the APPG is provided by The Haemophilia Society. They are a charity funded by individuals as well as corporate funding.

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