

## IPOPI Parliamentary Event

### Campaigning for Severe Combined Immunodeficiency (SCID) to be part of the UK National New Born Screening Programme.

Tuesday 20<sup>th</sup> June, 2017, Attlee Room, House of Lords.

Hosted by Baroness Susan Masham of Ilton and Jim Shannon MP



### 1 Introduction

A range of stakeholders from the patient community, clinicians and Parliamentarians gathered to discuss and to put pressure on key decision makers at the **UK National Screening Committee (UKNSC)** to add **Severe Combined Immunodeficiency (SCID)** to the list of diseases for new-born screening ahead of their meeting on the **23<sup>rd</sup> June 2017**.

The event can be seen as part of a much wider campaign by the patients themselves, by organisations such as the **International Patient Organisation for Primary Immunodeficiencies (IPOPI)** and **Primary Immunodeficiency UK (PID UK)** and by clinicians including **Professor Bobby Gaspar**, an expert in paediatrics and immunology at Great Ormand Street Hospital (GOSH) and the UCL Institute of Child Health. For years now, stakeholders have been lobbying to get SCID screened nationally without success. In 2013 the UKNSC reviewed the evidence of screening the disease but recommended that screening should not be implemented due to lack of evidence of its superiority compared to current practice. This is no longer the case and data is currently available to show the cost-effectiveness and urgency for the implementation of this paediatric emergency.

The event was hosted by Baroness Susan Masham (Cross-Bencher) and Jim Shannon MP (Democratic Unionist Party), who have also been closely involved in the SCID campaign in the past and have worked closely on rare diseases related campaigns as part of their Parliamentary work. Jim Shannon MP was unable to make the event on the day but was able to write and speak to key Government officials working at and with the UKNSC and also raise the issue at Ministerial level, including the new Minister at the UK Department of Health, Steve Brine MP. Baroness Masham has also campaigned on rare diseases issues in the past and is a member of the Lords Science and Technology Committee and has been involved in the Rare, Genetic and Undiagnosed Conditions All Party Parliamentary Group (APPG) in the past. It should be noted that Mike Wood MP (Conservative), also attended the event on the day and has been involved in the follow-up to it.

## 2 Opening Remarks

The event was opened by **Baroness Masham of Ilton**, who welcomed the attendees and gave a brief outline of the purpose of the event and a brief overview of the current status of campaigning for SCID. The Baroness also outlined some of the possible courses of action which Parliamentarians could take in order to help achieve the aim of getting SCID fully recognised by the UKNSC.

The Baroness also read out the UK Call for Action and encouraged attendees to sign it before making the following points:

- **SCID is an issue that both herself and Jim Shannon MP want to hold the Government to account, particularly ahead of the UKNSC meeting on the 23<sup>rd</sup> June.**
- **Pressure should be built up by the attendees in order to effectively campaign on the issue and provide the right response.**

Baroness Masham stressed that SCID should be recognised as a disease to be listed for new born screening by the UK National Screening Committee as soon as possible. She expressed that if the UK Government does not adopt new born screening for SCID, the country is at risk of falling behind other countries. The Baroness concluded that the 23rd June meeting of the UK National Screening Committee is an opportunity for the Government to make good on its commitment to ensure that all UK citizens receive the best possible healthcare and treatment, whatever the disease.

Following the introduction from Baroness Masham, the next speaker, **Professor Bobby Gaspar**, then gave an overview of the disease and went on to introduce some of the patients who were in attendance. Professor Gaspar explained SCID and outlined the huge benefits of new-born screening. He went on to provide an overview of his campaigning for the disease, which began in 2010 when he first wrote to the UKNSC.

Professor Gaspar then went through the four questions that the UKNSC had previously put to him:

### 1. What is the incidence rate of SCID in the UK?

It is hard to tell without screening but the estimation is 1 in 40,000. This means that there are rarer conditions that are currently included in new-born screening.

## 2. Can we successfully test for it?

Yes – the test is being successfully carried out in other countries.

## 3. Could other conditions be detected through SCID screening?

The screening takes place in the US and it has proven that the screening programme can detect other conditions such as Down Syndrome.

## 4. Is it cost effective?

Professor Gaspar said that cost effectiveness is the question that the UKNSC is most concerned about, with its experts saying that the original price of £3.50 per child was too high. According to their review, the test would only have a 65% chance of falling within NICE's QALY threshold. Professor Gaspar also said that he had successfully campaigned for the company to bring down the price of the test, and that it was now £2.50 per child and has a 96% chance of falling within the QALY threshold. Professor Gaspar gave a clear and succinct case why SCID reaches all the criteria that the UKNSC demanded.

## 3 Patient Testimonials

The opening remarks were followed by testimonials from individuals affected by SCID. These included parents of babies lost to SCID at early age and an individual diagnosed with SCID but due to diagnosis at birth and timely treatment, can now live a normal life.

### **Susie Ash and Justin Thorndyke:**

Susie Ash gave a moving account of her experience of losing her son, James, to SCID, which at the time of the event was just 17 weeks ago.

### **Rachel and Danny Carpenter:**

Rachel and Danny lost their son, George, to SCID in 2010. James was diagnosed with SCID at GOSH at seven months and died 10 days before his first birthday.

### **Jonah Howley:**

Jonah Howley was diagnosed at birth with SCID, he received a screening test based on his mother's connections with SCID. He is an example of the importance of diagnosing SCID at birth, and aside from receiving a weekly treatment he lives an active and fulfilling life.



## 4 Subsequent Follow-Up

The UKNSC published their recommendations for SCID screening on 4<sup>th</sup> August 2017. **They have agreed to pilot a SCID screening programme in a number of regions across the country to evaluate its effectiveness.** A working group will shortly be assembled to agree on the scope, logistics and timing of the pilot project.

A consultation has also been launched for relevant stakeholders to add their input into the shape of the pilot programme. The consultation will close on **Saturday 4<sup>th</sup> November 2017.**

## 5 UK Call for Action

During the Parliamentary Event, patients, Parliamentarians, clinicians and other supporters signed a UK Call for Action, with additional supporters adding their support after the event.

The Call for Action document was subsequently sent to the UKNSC, as well as the Government Ministers, Steve Brine MP and Lord James O'Shaughnessy.

This Call for Action was one of the key outcomes from the Parliamentary Event, as well as other tools of support, including Parliamentary Letters and associated correspondence. The Call for Action was successfully acknowledged by the UKNSC and the Ministers and was taken into consideration at the UKNSC meeting on the 23<sup>rd</sup> June.



## 6 Local and National Media Coverage

**Norfolk Eastern Daily Press:** <http://www.itv.com/news/anglia/2017-08-04/i-will-not-let-this-go-vows-mum-as-consultation-into-simple-screening-test-is-launched/>

**ITV News:** <http://www.itv.com/news/anglia/2017-08-04/i-will-not-let-this-go-vows-mum-as-consultation-into-simple-screening-test-is-launched/>