



AMGEN

UNDERSTANDING THE CHALLENGES

FACED BY PATIENTS WITH ITP (IMMUNE THROMBOCYTOPENIA)

Summary of a Policy Roundtable Discussion

2020

ABOUT THE ORGANISERS

The ITP Support Association is a UK registered charity which aims to promote and improve the general welfare of patients, and the families of patients, with Immune Thrombocytopenia (ITP). We work with the medical profession in order to advance the knowledge and treatment of ITP.



For Amgen, the key to serving patients is by being the best possible partner to the NHS and the professionals that fuel it. Over the last 40 years, Amgen has developed innovative medicines in cancer and long-term conditions and is immensely proud of the difference made to the lives of people in the UK through these and the services we deliver alongside them, enabling even more value for the system and patients. Committed to driving sustainable solutions that can adapt to an ever-evolving health system, Amgen is proud to serve its people, customers, and patients every day.



This non-promotional roundtable was jointly hosted by Amgen UK Ltd and the ITP Support Association and both organisations worked together to shape the scope of the roundtable. Amgen UK Ltd provided funding for the roundtable by way of agency services to manage the meeting logistics and the report drafting.

EXECUTIVE SUMMARY

ABOUT ITP

Immune Thrombocytopenia (ITP) is an autoimmune condition present in adults and children that causes a shortage of platelets in the bloodstream. For patients, fewer platelets can result in more frequent and severe bruising and internal and external bleeding. Without the right treatment and care, ITP can significantly impact upon patients' quality of life and can also result in acute admissions to hospital and, more rarely, fatalities.¹

ITP occurs when the immune system mistakes platelets as foreign bodies and destroys them. The condition can follow a virus, vaccination or the use of certain medications, but for most people the cause is unknown. Approximately 3 to 4,000 people in the UK will have ITP at any one time but it remains a condition that is relatively unknown by the general population.²

Through patient groups and ITP clinical networks there is now a greater understanding of the patient experience and the challenges and issues they encounter during the diagnosis and care pathway. This understanding has increasingly translated into clinical guidance and best practice models of care, however, there is significant variation in the experience of patients and the treatment and support they are provided. It is important that the NHS learns from evidence and feedback to ensure that a full range of holistic support services are offered to patients throughout England.

BACKGROUND AND OBJECTIVES OF THE ITP POLICY ROUNDTABLE

These issues were highlighted in the ITP Support Association's Patient Perception Survey 2020 and the inconsistencies identified warranted further exploration, with a view to identifying why best practice guidance is not translating to the patient experience. With this in mind, the ITP Support Association and Amgen UK Ltd convened a group of patient and clinical representatives for a policy roundtable discussion.

The objectives were to explore and define the challenges faced by ITP patients and to identify best practice models of care – more relevant now than ever given the far reaching impact of COVID-19 on traditional care frameworks.

Discussion focused on reviewing examples of the patient experience in 2020, reviewing updated clinical best practice and interpreting how these should benefit patients, and identifying common issues relating to the diagnosis, treatment and management of the condition.

As a relatively low profile condition it was also important that roundtable participants provided recommendations to NHS leadership and wider health policymakers as to how patient experience and Quality of Life can be improved, and unwarranted variation reduced.³

The coalition in attendance agreed that the points of consensus and recommendations provided should form the basis of further discussion, research and activity.

¹ Can I Die from ITP? An American Perspective. The ITP Support Association Platelet Reprint Series. George et al (2012)

² Immune Thrombocytopenia (ITP). Guy's and St Thomas' NHS Foundation Trust. (2020)

³ Global ITP Awareness Week (21st - 27th September). ITP Support Association. (2020)

POINTS OF CONSENSUS & RECOMMENDATIONS



NEXT STEPS: BUILDING ON THE ROUNDTABLE DISCUSSION

- Roundtable attendees to consider forming a coalition to help advocate and progress the recommendations outlined in this report.
- ITP stakeholders to work together to undertake further research into the factors behind the most pertinent issues affecting patients that were identified in the ITPSA Patient Survey.

CONTENTS

- 1 About the organisers
- 2 Executive Summary
- 3 Points of consensus and recommendations
General recommendations to take forward
- 5 **THE CHALLENGES FACING ITP PATIENTS
(SUMMARY OF DISCUSSION)**
 - 1. THE ITP PATIENT PERSPECTIVE IN 2020**
Reflections from the ITP Support Association Patient Perception Survey 2020
Patient awareness and involvement in the development of a patient treatment plan for ITP
Inconsistencies in clinical support on offer to ITP patients
Lack of knowledge of ITP in primary care settings
 - 6 THE DEBILITATING IMPACT OF ITP ON PATIENTS'
PHYSICAL AND MENTAL WELLBEING**
Treatment related issues
 - 2. UPDATED CLINICAL GUIDANCE**
 - 7 3. DIAGNOSIS AND SYMPTOMS**
Awareness of ITP in primary care
 - 8 Impact of ITP on mental health
 - 9 Fatigue and ITP
- 4. TREATMENT AND CARE**
What is important to patients
- 10 The importance of CNSs
Treatment and care settings
- 5. ITP IN A COVID-19 WORLD**

THE CHALLENGES FACING ITP PATIENTS

1. THE ITP PATIENT PERSPECTIVE 2020

REFLECTIONS FROM THE ITP SUPPORT ASSOCIATION PATIENT PERCEPTION SURVEY 2020

Attendees discussed the themes and key findings of the ITP Support Association's Patient Perception Survey 2020⁴. It was explained that over a number of years, the association has carried out a number of surveys covering the subject of ITP, utilising its growing network of Local ITP Support Groups around the UK. The ITP Support Association carries out its Patient Perception Survey every 2 to 3 years, providing the association with an up to date assessment of how ITP patients see their treatment experience. This provides information on areas needing improvement which is published and shared as appropriate. The 2020 patient perception survey builds upon the previous survey undertaken in 2017 and has expanded its scope from focusing solely on ITP specialist centres to all treatment settings. The 2020 results are particularly relevant due to the recent changes in NHS service provision since the onset of the COVID-19 pandemic. A total of 194 responses were received.

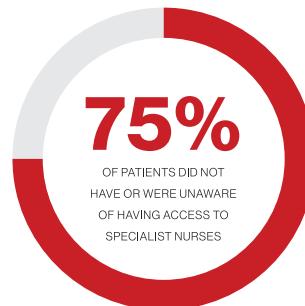
A NUMBER OF KEY THEMES AND CHALLENGES EMERGED FROM THE SURVEY, INCLUDING:

Patient awareness and involvement in the development of a patient treatment plan for ITP. The survey reveals that while the majority of patients said that their doctor has a treatment plan for their condition, 35% of respondents said they do not have, or were unaware of their doctor having a plan to manage ITP.



INCONSISTENCIES IN CLINICAL SUPPORT ON OFFER TO ITP PATIENTS

The survey highlights that almost 75% of patients either do not have or are unaware of having ITP specialist nurses in the clinic that they attend. Feedback from patients suggests that they are more likely to feel empowered when their treatment and care options are discussed with ITP CNSs, emphasising the importance of shared decision making.



LACK OF KNOWLEDGE OF ITP IN PRIMARY CARE SETTINGS

The survey found that 55% of patients said that their GP had no knowledge of ITP when they first sought advice for symptoms. Indeed, feedback highlighted that patients had experienced serious problems regarding the diagnosis of their condition, inconsistent information regarding the side-effects from treatment options. There was widespread agreement that this question was particularly pertinent due to the ongoing coronavirus pandemic, which has seen a number of changes in the availability of GP services for patients.

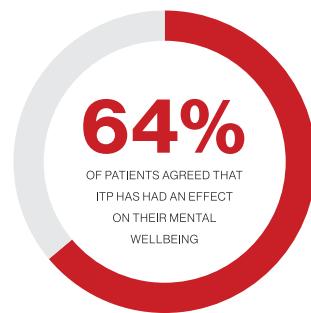
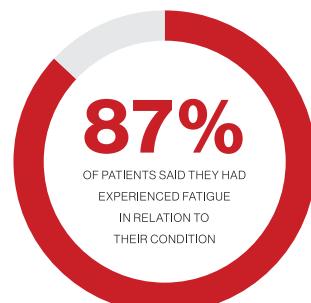


THE DEBILITATING IMPACT OF ITP ON PATIENTS' PHYSICAL AND MENTAL WELLBEING

The survey shows that 87% of patients have experienced fatigue in relation to their condition, while 64% of patients agreed that ITP has had an effect on their mental wellbeing.

TREATMENT RELATED ISSUES

Several survey questions were highlighted outlining patient feedback relating to treatment information and lack of shared decision making. One question found that over a quarter (26%) of respondents said they were not told about possible side effects from treatment. Other questions in the survey highlighted situations where patients described being deterred from a treatment or put onto a treatment option against their preference. Roundtable attendees outlined that sometimes patients may forget or misplace treatment information and one solution for retaining this information is more frequent dialogue with a clinician that patients feel at ease with, such as a clinical nurse specialist (CNS).



2. UPDATED CLINICAL GUIDANCE

CHANGES IN THE UPDATED INTERNATIONAL CONSENSUS GUIDELINES

Roundtable attendees discussed the 'Updated international consensus report on the investigation and management of primary immune thrombocytopenia'.⁵ The rationale for the update was outlined by a number of its contributors, before overall sentiment of the new guidance as well as some specific changes were highlighted.

The guidance, which is an update of the 'International Consensus Report' published in 2010⁶, provides recommendations on the diagnosis and management of ITP in children, adults and during pregnancy. The overall ethos of the new guidance was described as trying to adequately reflect quality-of-life considerations, to ensure the individualisation of care and to incorporate patients more in treatment decision-making.

Attendees focused on the changes contained within the new guidelines, noting that the previous consensus guidelines provided "vague unrealistic treatment goals", and were not reflective of best practice. It was acknowledged that the last decade had seen numerous developments and changes in treatment practices for the management of patients with ITP, and that the update was "much needed".

⁵ Updated international consensus report on the investigation and management of primary immune thrombocytopenia. Provan et al (2020)

⁶ International consensus report on the investigation and management of primary immune thrombocytopenia. Provan et al (2010)

There was strong agreement that the updated consensus guidelines represented a “dramatic shift” in emphasis, away from arbitrary platelet targets, and towards a holistic approach to treatment and management in which a patient’s quality of life should be the primary focus. It was noted that patients must be involved in the decision-making process in terms of the management and treatment of their ITP. It was acknowledged that patients should be treated as individuals, and that shared decision making, in which the patient is empowered to make decisions when discussing treatment options, is vitally important.

It was recognised that over the past ten years there had been a move away from clinicians focusing solely on increasing a patient’s platelet count. While still important, ITP is now regarded as much more than simply the patient’s platelet count.

Findings from the ITPSA Patient Perception Survey and the ITP World Impact (I-WiSh) Study⁷ highlight that patients view quality of life as the most important factor in the treatment and management of the condition.

There was widespread agreement that the long-term use of steroids was not best practice and that healthcare professionals should seek to prescribe drugs with minimal toxicity. While the use of steroids as initial treatment is accepted the importance of reducing the time patients should spend on steroids was noted, and that TPO-R agonists should be used earlier. It was noted that medical therapy with robust evidence should be explored before surgical therapy is considered.

3. DIAGNOSIS AND SYMPTOMS

Following the reflections from the most recent ITP Support Association Patient Perception Survey 2020, the discussion moved onto the issues related to the diagnosis of ITP, and the symptoms that patients experience as a result of their condition. During this part of the discussion, a number of key themes and challenges emerged, including:

AWARENESS OF ITP IN PRIMARY CARE

Patients do experience problems and delays in diagnosis. It was noted that part of the solution for an improvement in ITP education was to raise GP awareness in primary care, especially in relation to a clearer understanding of diagnostic pathways.

There was recognition that many patients feel that their GP has little knowledge of ITP. Anecdotal evidence was provided of patients at an NHS Hospital Trust requesting information on ITP that they could share with their GP to increase understanding of the condition. A theme of patients increasingly requesting information from ITP specialists on the condition that could be shared with practitioners in primary care was re-emphasised. It was noted that there was a need to build good relationships across the ITP network, including specialist centres, District General Hospitals (DGHs), the ITP Forum and patient support groups.

It was highlighted that, before the onset of the coronavirus pandemic, a framework document was being developed in collaboration with the British Society for Haematology (BSH) in order to highlight best practice in ITP care. There was general agreement that there was a need for a document to be produced to outline “what best practice ITP care could look like in the UK”, particularly in relation to paediatric care, where patients are predominantly managed by non-ITP specialists.

Attendees discussed the merits of producing a one-sided 'What is ITP' educational document as a tool to increase knowledge of the condition in primary care settings. Such a tool could be distributed by patients, with the support of the ITP Support Association and the ITP Forum, to individual primary care centres. Efforts also should be made to engage national primary care organisations such as the Royal College of GPs and the National Association of Primary Care, alongside key stakeholders, including the National Clinical Director of Primary Care at NHS England, to encourage them also to promote awareness of ITP.

IMPACT OF ITP ON MENTAL HEALTH

There was a strong recognition of the impact to patients' mental health following a diagnosis of ITP. Patient representatives emphasised that once a patient realises they have a long-term condition to manage for the rest of their life, it is a "tremendous blow". It is important that patients have support groups such as the ITP Support Association, who can provide information and support to individuals and families.

There was a recognition that there may be some instances where ITP patients may face extreme anxiety regarding the risk of a gastrointestinal bleed (GI) or a brainbleed. It was agreed that this has a significant impact on patients' mental wellbeing and everyday life, especially those that are newly diagnosed with ITP. The importance of patient support groups, and their ability to provide information to reassure patients on the likelihood of such events, was emphasised.

It was acknowledged that patients who have experienced mental health problems should be given access to a clinical psychologist. Attendees discussed that, at present, just 20% of specialist ITP Centres have a dedicated clinical psychologist for ITP patients. There was consensus that this percentage should increase in order to provide more comprehensive and consistent mental health support.

It was also noted that many ITP Specialist Centres lack the finances to employ a clinical psychologist and highlighted that patients who are unable to access Specialist Centres and receive treatment at DGHs face even bigger challenges. Attendees were updated that an NHS Trust had recently had a business case passed to employ a clinical psychologist. It was acknowledged that this was not exclusive to ITP patients, but would provide direct access for ITP patients as required.

There was agreement that the majority of trusts have access to psychology support and are able to make a referral for a patient. It was suggested that in terms of improving outcomes for ITP patients, there was a need to embed a clinical psychologist into ITP specialist services. Attendees discussed the example of Red Cell Services for patients with red blood cell disorders, which are specially commissioned and have an integrated patient feedback mechanism. There was recognition that the ITP Support Association could play a role in advocating for a similar type of service to include a dedicated clinical psychologist for patients.

There was agreement that the issue of ITP patients' mental health emphasises the need for ITP diagnosis and subsequent care to move beyond the standard patient – doctor interaction and decision, and towards a process of holistic care characterised by more frequent and honest discussions. It was noted that the issue highlights the importance of a multi-disciplinary team (MDT) in treating and supporting patients with ITP.

FATIGUE AND ITP

Fatigue impacts the majority of patients and can have a significantly detrimental impact on their quality of life. It was recognised that a number of treatments for ITP cause patients to experience fatigue. Attendees noted that there is a correlation between an increase in platelet count and a reduction in fatigue.

There was consensus that the ability of clinicians to control the platelet count is not a precise science. The example was given of how many haematologists now would consider a platelet count of 50 to be acceptable as haemostasis is relatively normal at this level. However, this may not reduce levels of fatigue or improve quality of life in all patients although the causation is very multifactorial.

It was acknowledged that fatigue is a multifactorial issue. Attendees noted that while the quality of life data shows that a patient will likely experience less fatigue with a higher platelet count, this may also be dependent on factors such as having treatment in a community setting, avoiding travelling to hospital to take an ITP drug regularly.

There was agreement between attendees that it was important to be measuring fatigue either by Health-Related Quality of Life (HRQOL – individuals perceived physical and mental health) or fatigue specific questionnaires. This is particularly important in paediatric ITP and is built into routine practice. Attendees explored replicating this across all ITP patient populations.

4. TREATMENT AND CARE

Following the discussion on diagnosis and symptoms, the conversation moved to the treatment and care of ITP. It was outlined to attendees that this section of the discussion would provide important perspectives on what matters most to patients, touching on the key role of CNSs, alongside the importance of the treatment setting.

WHAT IS IMPORTANT TO PATIENTS

In line with the previous discussion points on the impact of ITP on patients' mental wellbeing, attendees agreed that there is a need for ITP treatment and subsequent care to move beyond the standard patient – doctor interaction. A shift towards a process of shared decision-making is required, with patients empowered to make decisions related to their condition.

It was suggested that patients could advocate for a move to such an interaction through the support from the ITP Support Association, the ITP Forum and the ITP Registry. In addition, there was strong consensus that patient support groups are paramount for providing patients with inter-patient support, which is key to their physical and mental health.

It was noted that while these patient support groups traditionally take place via face-to-face meetings of small groups of patients, meetings had moved to a virtual setting during the COVID-19 pandemic. Attendees highlighted the positive impact of such meetings, with patients able to discuss their own personal issues with each other and to pose questions to CNSs and clinicians who are able to allay any fears and anxieties they may have.

THE IMPORTANCE OF CNS

There was a strong recognition that, particularly in the context of the COVID-19 pandemic, patients are increasingly anxious about the treatment and management of their condition. CNSs in attendance outlined that since the onset of the pandemic they have been increasingly asked to provide high levels of reassurance to patients.

Attendees agreed that patients greatly value having access to an ITP CNS in a community setting. Anecdotal evidence from attendees suggested that patients are often more comfortable discussing their condition with a nurse than a GP. They are able to share their perspective, ask more detailed questions and feel more empowered about managing their condition. Examples and evidence was provided to support this.

Anecdotal evidence was shared regarding frequent discussions between patients and CNSs regarding side effects. It was outlined that a CNS will monitor for side effects of a particular treatment, before relaying any reported instances at a multidisciplinary team (MDT) meeting. Options will then be outlined to patients before an agreement is made, either to reduce or change treatment plans. This type of monitoring is not consistent across the NHS, but it should be.

There was strong agreement that the role of a CNS was "crucial" to an ITP patient's treatment and care. It was noted that not all ITP patients manage to get access to a CNS, and that there is a need for all patients to have routine access to such services to enable greater shared decision making on treatment and care decisions.

TREATMENT AND CARE SETTINGS

Attendees agreed on the importance of joined-up relationships between specialist ITP centres and District General Hospitals (DGHs) at a local level to optimise treatment and care of ITP. It was noted that the development of new long-term community diagnostic hubs (CDHs) had the potential to transform the way ITP patients receive treatment and care for their condition.

It was highlighted that the role of CDHs would be particularly helpful given the current COVID-19 pandemic, as they would ensure that ITP patients can have their diagnostic tests in a COVID-free environment. Indeed, attendees agreed that it was important for clinicians, NHS managers and patient groups to consider the development of a framework document that outlines new effective models of ITP diagnosis, treatment, and care as a result of COVID-19 disruption. Further exploration is also required into the viability and success of more ITP patients being treated in the community and in their homes.

5. ITP IN A COVID-19 WORLD

Attendees agreed that the diagnosis, treatment and ongoing management of ITP has been significantly affected by the implications of COVID-19 and the disruption of "business as usual" delivery of healthcare services.

It was highlighted that since April, guidance from the World Health Organization (WHO) has been adopted by many hospitals and specialist centres. Specific advice was noted regarding that the use of high dose steroids and immunosuppressive treatment may increase the risk of complications if COVID-19 is present⁸. Conversely, the use of some forms of steroid (generally dexamethasone) in more severe cases has been deemed more effective in serious cases of COVID-19 than milder cases and this has been borne out in the recent RECOVERY trial.⁹

⁸ Advice if you take immunosuppressive medication. Gloucestershire Hospitals NHS Foundation Trust. (2020)

⁹ Low-cost dexamethasone reduces death by up to one third in hospitalised patients with severe respiratory complications of COVID-19. RECOVERY. (2020)

Further work is also being done via a clinical audit to capture an understanding of prescribing practices during the pandemic. Attendees agreed they would welcome this analysis as well as further clarity from NHS England regarding the funding for certain types of treatment used in first line.

During the COVID-19 pandemic there has been anecdotal evidence that some ITP patients had experienced difficulties in obtaining treatment and getting blood tests. The ITPSA therefore ran a survey focused on how patients had been impacted by disruption since March 2020. The responses demonstrated that approximately 80% of responders outlined that their treatment had continued as close to normal as possible but with the use of remote consultant sessions. Haematologists in attendance explained that not all patients could have their appointments done remotely and it remains a challenge to decide which patients need to be seen face-to-face and how to ensure they are not put at an increased risk of the virus.

More concerningly, 20% of the patients who responded outlined that they had not continued with ITP treatment during this period due to a range of factors, including anxiety about contracting the virus and difficulty in confirming appointments. Roundtable participants shared examples of patients who avoided appointments and going on to requiring acute care in hospital. All agreed that this is a concerning situation and coordination is required to ensure that the fear is removed from patients who are reluctant to see a clinician, and that there is not a “postcode lottery” in access to support.

Examples of new processes for managing ITP in response to COVID-19 were shared, including the roll-out of community diagnostic hubs and samples being taken in a range of settings including vacated live entertainment venues. It was noted that through the development of community diagnostic hubs and home drug delivery services, there was an opportunity for patients to take greater ownership of the management of their condition. It was agreed that moving the management and care of their condition to a community setting and closer to home has the potential to empower patients and improve their quality of life.

It was highlighted that the sharing of best practice regarding the implementation of community hubs, alongside feedback from ITP support groups could provide a framework for ‘improved ITP pathways and models of care in the future’.

This non-promotional roundtable was jointly hosted by Amgen UK Ltd and the ITP Support Association and both organisations worked together to shape the scope of the roundtable. Amgen UK Ltd provided funding for the roundtable by way of agency services to manage the meeting logistics and the report drafting.



APPENDIX

ROUNDTABLE PARTICIPANTS

- Professor Adrian Newland (Chair), Professor of Haematology at Barts and the London School of Medicine and Dentistry, Queen Mary University of London
- Mervyn Morgan, Chief Executive, ITP Support Association
- Rhonda Anderson, Senior Patient Mentor, ITP Support Association
- Dr Drew Provan, Medical Adviser ITP Support Association. UK ITP Forum Executive Committee Member
- Dr Nicky Cooper, Faculty of Medicine, Department of Immunology and Inflammation, Imperial College, London
- Dr Jecko Thachil, Consultant Haematologist, Manchester University NHS Trust
- Dr Quentin Hill, Chair, UK ITP Forum, ITP Centre Director and Consultant Haematologist Leeds University Hospitals
- Dr Vickie McDonald, Director, Adult ITP Registry, ITP Lead Royal London Hospital
- Dr John Grainger, Consultant Paediatric Haematologist, Manchester University NHS Foundation Trust, National Lead for Paediatric ITP
- Dr Sue Pavord, Consultant Haematologist, Oxford University Hospitals
- Louise Taylor, ITP Nurse, Royal London Hospital, Nursing Representative, UK ITP Forum
- Rosie Davies, ITP Nurse, Royal Liverpool and Broadgreen University Hospital NHS Trust