

COMMENTARY

I-WISH: A wish list for immune thrombocytopenia quality of life indicators becomes reality

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In this issue of the *American Journal of Hematology*, two articles by Nichola Cooper and colleagues describe results from the immune thrombocytopenia (ITP) World Impact Survey (I-WISH) and the ITP Life Quality Index (ILQI) among 1507 adults (age ≥ 18) with ITP and 313 hematologists and 159 hemato-oncologists treating ITP patients across 13 countries.^{1,2} There are two versions of the I-WISH: the first is for patients with ITP, and the second is for hematologists or hemato-oncologists that treat patients with ITP. The patient version of the I-WISH collects information on demographics, diagnosis, symptoms, the impact of ITP on health-related quality of life (HRQoL), emotional well-being, work and finances, available support, treatment history, and the patient-physician relationship. The physician version of the I-WISH asks about ITP diagnosis and case load, symptoms, impacts of ITP on their patients' HRQoL and well-being, treatment patterns (treatment prescribed following diagnosis and after relapse of ITP, reasons for changing therapy, and frequency of splenectomy), and the patient-physician relationship. Patients also completed the 10-item ILQI, which was developed by the I-WISH Steering Committee specifically for use in the I-WISH study.³ The intent of the ILQI is to promote discussion about HRQoL and fatigue between patients and physicians and inform decisions about treatment. To collect data for the current analyses, surveys were sent out by "mass email" to patient support networks and physicians who were asked to disseminate the survey to their patients, who, in turn, responded online. The authors' goal is to report the results of these surveys on the signs and symptoms of ITP, compare responses between patients and physicians, and explore the impact of ITP on HRQoL and productivity.

ITP has been previously shown to be associated with detriments to HRQoL;⁴⁻¹¹ symptoms can impede physical functioning, social interactions, and emotional well-being. The extent to which ITP impairs HRQoL varies by time since diagnosis and severity of symptoms.^{8,9,11} Some treatments for ITP can also have side effects that negatively impact social and emotional well-being,⁹ but studies have demonstrated improvements to HRQoL when patients are successfully treated.¹²⁻¹⁴ This illustrates that the impact of ITP on HRQoL is multi-faceted and complex.

The patient experience of ITP has been assessed over the years using a variety of tools. However, there is a lack of uniformity regarding how these data are collected. Some studies use generic assessments, like the SF-36, EQ-5D, Functional Assessment of Chronic Illness Therapy fatigue subscale (FACIT-fatigue), and the Functional Assessment of Cancer Therapy thrombocytopenia subscale (FACT-Th6).^{6,7,9} In other cases, patient reported outcomes are collected using ITP-specific tools, such as the Immune Thrombocytopenia Patient Assessment Questionnaire (ITP-PAQ) for adults, and the Kids' ITP Tools (KIT) and ITP-Quality of Life (ITP-QoL) measures for children.^{9,15,16}

The study reported in these two articles produced a significant amount of data on the signs and symptoms of ITP, its impact on HRQoL, and satisfaction with treatment. Among patients who responded, fatigue was cited as the most frequent symptom.² Reductions in energy level, capacity to exercise, and ability to perform daily tasks were common.¹ The patient and physician reports of symptom burden were similar, although patients more often cited fatigue than did physicians.² Patients report substantial impact on their emotional well-being and social life due to ITP: 70% indicated an impact to their social lives, and a large percentage felt concerned, anxious, and nervous about their disease and its potential progression, including 41% who worried about dying.¹ Physicians agreed about the impact of ITP: 69% believed that patients' anxiety about platelet levels had a negative impact on their emotional well-being. Almost half (49%) of patients reduced or seriously considered reducing their working hours (including 29% who had considered terminating employment), and over one-third (36%) of those employed reported that ITP decreased their productivity at work.¹ The percentage reporting negative impacts to work productivity were higher among those aged 18-49 years (40%) than among those aged 50 and older (27%). Younger patients were also more likely to indicate that ITP affected regular activities outside of work (48%) than their older counterparts (39%). The proportion reporting detriments to work productivity and regular activities were similar between men and women.

Many patients experienced delays in diagnosis for a variety of reasons (including waiting to see a specialist, or misdiagnoses), and 63% wished they had received more support at the time of their diagnosis, particularly from their physician.² However, 77% felt their physician considered their needs when planning their treatment goals, which was similar across age and gender.¹ Satisfaction with ITP treatment varied by the type of treatment received: 79% of those receiving anti-CD20 agents reported satisfaction regarding control of their ITP. That percent was 76% for those receiving thrombopoietin receptor agonists, 69% for those receiving intravenous immunoglobulin, 53% for those receiving corticosteroids, and 38% for those who had a splenectomy.² Notably, just 64% felt that their symptoms and HRQoL were considered when treatment decisions were made.² Fifty-four percent of patients reported being on "watch and wait" management at some time; physicians indicated that being asymptomatic, having higher platelet levels, and/or a lack of severe bleeding would make it more likely they would recommend "watch and wait" over pharmacological options. Physicians were also asked about reasons for changing therapies. The most common reasons given were lack of efficacy (87%), worsening disease (74%), and side effects (76%).

These articles add to the existing body of literature demonstrating the wide-ranging impacts of ITP on patient lives. The authors provide evidence of the extent of work and productivity impairment due to ITP, the social and emotional effects, and the level of treatment satisfaction. Further, they reveal how patients feel about their diagnosis and treatment, including the level of support they receive and their level of satisfaction with different therapies.

One of the main strengths of these analyses is the large sample size. Immune thrombocytopenia is a rare disease, with its estimated prevalence between 2.7 and 9.5 per 100 000 individuals.¹⁷ To collect information from over 1500 individuals across 13 countries is commendable. Additionally, the I-WISH and ILQI cover a number of aspects of ITP, including diagnosis, symptoms, HRQoL, emotional well-being, the impact on work, who patients seek support from after diagnosis, and treatment-related goals and satisfaction with therapies. Additionally, a physician version of the I-WISH collected information from those treating patients with ITP. However, the authors note the potential for selection bias and that they were unable to estimate a response rate due to the way surveys were distributed. Further, the physicians included in the study were not linked to patients; a more thorough analysis of the concordance of responses could have been performed if physicians who treated patient respondents could have been identified.

Still, the results presented in these two articles are notable for both the breadth of the aspects of patient experience covered and the size of the sample. The addition of the I-WISH and ILQI to the catalog of applicable tools for assessing the patient experience of ITP is most certainly welcome; the use of disease-specific patient-reported outcome measures can help to promote more uniformity across studies for evaluating how patients experience ITP and its treatment.

CONFLICT OF INTEREST

The author declares no conflict of interest.

DATA AVAILABILITY STATEMENT

Not applicable

REFERENCES

- Cooper N, Kruse A, Kruse C, et al. Immune thrombocytopenia (ITP) World Impact Survey (I-WISH): impact of ITP on health-related quality of life. *Am J Hematol*. 2021;96(2):199-207.
- Cooper N, Kruse A, Kruse C, et al. Immune thrombocytopenia (ITP) World Impact Survey (I-WISH): patient and physician perceptions of diagnosis, signs and symptoms, and treatment. *Am J Hematol*. 2021; 96(2):188-198.
- Grant L, Bonner N, Harper A, et al. Qualitative research to support the content validity of the Immune Thrombocytopenia (ITP) Life Quality Index (ILQI). Annual Scientific Meeting of the British Society for Haematology; April 16, 2018-April 18, 2018, 2018; Liverpool, UK.
- Efficace F, Mandelli F, Fazi P, et al. Health-related quality of life and burden of fatigue in patients with primary immune thrombocytopenia by phase of disease. *Am J Hematol*. 2016;91(10):995-1001.
- Mathias SD, Gao SK, Miller KL, et al. Impact of chronic Immune Thrombocytopenic Purpura (ITP) on health-related quality of life: a conceptual model starting with the patient perspective. *Health Qual Life Outcomes*. 2008;6:13.
- McMillan R, Bussell JB, George JN, Lalla D, Nichol JL. Self-reported health-related quality of life in adults with chronic immune thrombocytopenic purpura. *Am J Hematol*. 2008;83(2):150-154.
- Sanz MA, Aledort L, Mathias SD, Wang X, Isitt JJ. Analysis of EQ-5D scores from two phase 3 clinical trials of romiplostim in the treatment of immune thrombocytopenia (ITP). *Value Health*. 2011;14(1):90-96.
- Snyder CF, Mathias SD, Cella D, Isitt JJ, Wu AW, Young J. Health-related quality of life of immune thrombocytopenic purpura patients: results from a web-based survey. *Curr Med Res Opin*. 2008;24(10):2767-2776.
- Trotter P, Hill QA. Immune thrombocytopenia: improving quality of life and patient outcomes. *Patient Relat Outcome Meas*. 2018;9:369-384.
- Sestøl HG, Trangbæk SM, Bussell JB, Frederiksen H. Health-related quality of life in adult primary immune thrombocytopenia. *Expert Rev Hematol*. 2018;11(12):975-985.
- Yang R, Yao H, Lin L, Ji JM, Shen Q. Health-related quality of life and burden of fatigue in Chinese patients with immune thrombocytopenia: a cross-sectional study. *Indian J Hematol Blood Transfus*. 2020;36(1):104-111.
- George JN, Mathias SD, Go RS, et al. Improved quality of life for romiplostim-treated patients with chronic immune thrombocytopenic purpura: results from two randomized, placebo-controlled trials. *Br J Haematol*. 2009;144(3):409-415.
- Kuter DJ, Mathias SD, Rummel M, et al. Health-related quality of life in nonsplenectomized immune thrombocytopenia patients receiving romiplostim or medical standard of care. *Am J Hematol*. 2012;87(5): 558-561.
- Mathias SD, Li X, Eisen M, Carpenter N, Crosby RD. Blanchette vs a phase 3, randomized, double-blind, placebo-controlled study to determine the effect of romiplostim on health-related quality of life in children with primary immune thrombocytopenia and associated burden in their parents. *Pediatr Blood Cancer*. 2016;63(7):1232-1237.
- Mathias SD, Bussell JB, George JN, McMillan R, Okano GJ, Nichol JL. A disease-specific measure of health-related quality of life for use in adults with immune thrombocytopenic purpura: its development and validation. *Health Qual Life Outcomes*. 2007;5:11.
- Mathias SD, Gao SK, Rutstein M, Snyder CF, Wu AW, Cella D. Evaluating clinically meaningful change on the ITP-PAQ: preliminary estimates of minimal important differences. *Curr Med Res Opin*. 2009;25(2):375-383.
- Michel M. Immune thrombocytopenic purpura: epidemiology and implications for patients. *Eur J Haematol*. 2009;71:3-7.