

# Understanding the Importance of Using Patient-Reported Outcome Measures in Patients With Immune Thrombocytopenia

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Incorporating patient-reported outcomes (PROs) when studying patients with immune thrombocytopenia (ITP) is essential since treatment decisions are complex and using platelet count only partly explains disease burden. Since most symptoms are only experienced subjectively and are seldom captured during clinician-based evaluations, using self-report is crucial for early symptom detection. However, capturing the patient's illness experience necessitates using well-developed and validated instruments. This article provides insight on the importance of using PROs in ITP, summarizes the methodological steps to develop PRO instruments, and discusses challenges related to integrating PROs into research and clinical practice.

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**I**mmune thrombocytopenia (ITP) affects between 2–6 people per 100,000 per year.<sup>1</sup> While the disease in children generally has a sudden onset but a good prognosis, ITP in adults often presents gradually but tends to be chronic in nature. Choosing the right therapy at the right time is the most challenging task for clinicians. Treatment side effects can be substantial, and are often perceived

by patients as worse than the symptoms of the disease.<sup>2</sup> Traditionally, the assessment of a patient's response to the chosen treatment has been exclusively made by clinicians based on platelet count and clinical bleeding.<sup>3</sup> However, given that many patients with very low platelet counts do not bleed, it is emphasized that treatment choice should rely more on symptoms,<sup>4</sup> underscoring the importance of incorporating the patient's perspective by using patient-reported outcomes (PROs). A PRO is any report of the status of a patient's health condition that comes directly from the patient, without interpretation of the patient's response by a clinician or anyone else. Examples include quality of life (QoL), symptom experience, treatment satisfaction, and adherence.<sup>5</sup>

The importance of PROs in drug development is currently acknowledged worldwide, with the requirement that the PRO instruments are created and validated according to well-described standards outlined in the US Food and Drug Administration (FDA) guidance and the reflection paper on the measures of health-related QoL of the European Medicines Agency.<sup>5,6</sup> This article summarizes the advantages of using PROs in ITP, provides insight into the different methodological steps involved in developing or modifying instruments, and provides examples of how they can be incorporated into research and clinical practice.

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## ADVANTAGES OF THE USE OF PROS IN ITP

First, PROs facilitate better understanding of the impact of the disease and treatment on the patients' life. Assessing the patient's perspective may reveal valuable information that would be missed when relying exclusively on clinician report.<sup>7</sup> For example, current American Society of Hematology treatment guidelines focus on the medical side effects of corticosteroids, including hyperglycemia and osteoporosis, whereas weight gain, mood swings, and puffy face are most bothersome to patients.<sup>8</sup> Second, the patients' perspective might provide unique insights on treatment effectiveness. Directly asking the patient about adherence in the situation of non-response to corticosteroids, for example, might facilitate a deeper understanding why the drugs are not working. Third, PROs can be relevant in decision-making processes. Two drugs can have similar effectiveness but different side effect profiles. In particular, patients report higher treatment-bother with corticosteroids than with other ITP therapies.<sup>9</sup> Patients' preferences might therefore guide treatment choice.

Because of these recognized values, the European Hematology Association Scientific Working Group "Quality of Life and Symptoms" developed the "Patient-Reported Outcomes in Hematology" guidelines, which cover conceptual, methodological, and practical issues surrounding PRO measurement. They provide an overview of existing instruments, and describe state-of-the art studies incorporating PROs, of which some key insights are discussed below.<sup>10</sup>

## WHAT CONSTITUTES A GOOD PRO?

Developing a PRO is not a "do it yourself" project. It is labor-intensive, necessitating meticulous methodology, and requires a collaborative team of clinicians, scientists, statisticians, and patients. Excellent methodological guidance is offered by the article series published in "Value in Health."<sup>11</sup> Before developing a new PRO, clinicians should consider using existing ones. Electronic databases, such as PROQOLID and PROMIS, offer quick and comprehensive overviews of existing instruments. So far, however, instruments capturing the patient's experience of ITP almost exclusively focus on QoL, often applying generic instruments such as the Short Form-36 and the EQ-5D in adults, and the PedsQL and KINDL in children.<sup>10</sup> Three disease-specific QoL measures are also available: the ITP-patient administered questionnaire for adults, the Kids' ITP Tool, and the ITP-Quality of Life for children.<sup>12</sup>

If a PRO instrument is available, each clinician should answer five key methodological questions<sup>5,11</sup> before adopting it in research or practice.

### 1. Does the instrument provide a conceptual definition?

Several PRO instruments are published that do not describe what the instrument aims to measure, or do not provide the conceptual framework that is underpinning the items. One should check that what you are trying to measure fits well with the concept and items outlined in existing PRO instruments. For example, if you would like to understand the impact of ITP on a person's social and professional functioning you should check whether the PRO you are considering addresses these issues. If that is not the case, the search for a more appropriate instrument should continue.

### 2. For which patient population was the PRO instrument developed?

Instead of hastily choosing a self-report instrument off the shelf, one should carefully look at the sample characteristics: for whom was the questionnaire designed? Are these patients similar to the study population one has in mind? Even if the concept measured is the same, a PRO instrument measuring side effects of immunosuppressive drugs in transplantation might not be applicable to patients taking immunosuppressive drugs for rheumatic conditions. Also, will subjects be able to complete the questionnaire? Think of vision problems, cognitive impairments, or literacy levels. If questionnaires are designed in a different language, culturally sensitive translations, following rigorous protocols are mandatory, to make sure items and instructions are clear to patients with a different geographical or cultural background.

### 3. Was there sufficient patient input in the PRO instrument development process?

Strictly speaking, if no patients were involved in the development process, it is not really a PRO instrument. Patient involvement is recommended at three possible occasions.<sup>5</sup> First, if no conceptual definition exists, qualitative interviews with the patient group of interest, are helpful to understand how, for example, patients conceptualize side effects of pharmacological treatment (eg, patients might talk about frequency of occurrence, distress experienced and impact on their daily functioning as dimensions of the concept "side effects"). Also, interviewing patients allows to identifying the symptoms which they deem to be important. Second,

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