

The ITP Patient Registry: Educating the ITP Community and Providing a Link Between Treatment Options and Quality Life

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Objective

Immune Thrombocytopenia (ITP) is an immune-based disorder indicated by a significant decrease in platelet count. Natural history studies and patient registries are crucial in identifying patient-reported outcomes, and include patients as active participants in research. These studies address burden of disease and unmet needs of patients.

Methods

The ITP Registry enrolled to date 868 patients (Table 1). Patients complete surveys including treatment and quality of life. Data was extracted from NORD's secure web-based IAMRARE registry platform. Of 765 consented patients, 259 patients (34%) completed surveys pertaining to treatment and quality of life.

Enrollment	868
Consented	765
Adults	691
Children	74
Surveys	
Participant Profile	489
Treatment and Review of System	341
Medical and Diagnostic Data	391
Quality of Life	
Adult 1	274
Adult 2	262
Pediatrics	38
All Surveys Completed	460

Table 1. ITP Registry Participants (n=868).

Results

Treatment Options

ITP patients receive a range of treatments to manage their disease. Patients receive an average of three therapies (range 1-14), with almost half of patients treated with ≥ 3 different therapies over the course of their disease. Only half of survey respondents currently receive treatment, although this does not take into consideration patients who may be in remission because this question was not specifically asked.

Quality of Life

265 adult ITP patients completed the Quality of Life Survey. In general, most participants felt their quality of life is either good or very good; less than a fifth (18%) of respondents said their quality of life was either poor or fair [Figure 1].

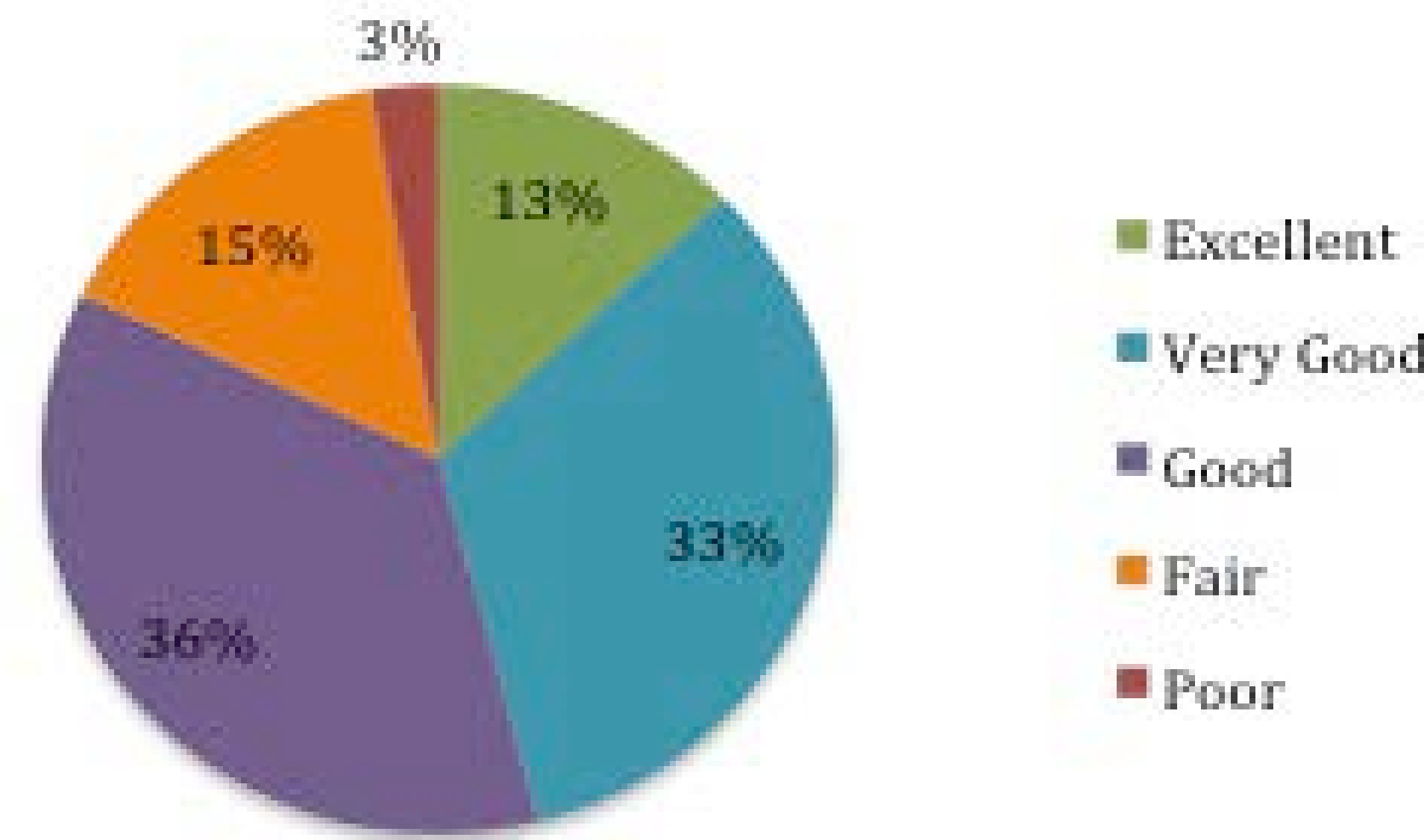
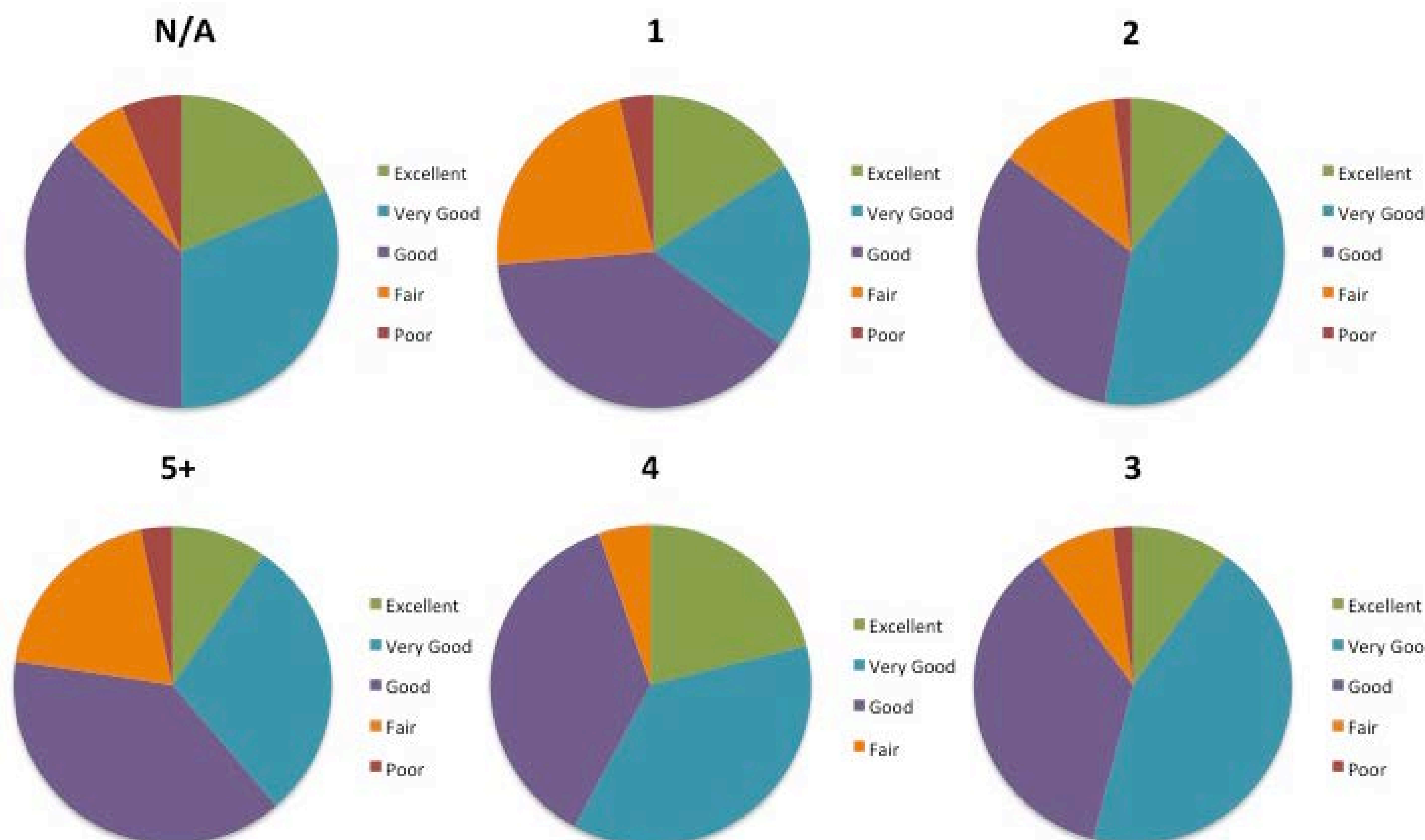


Figure 1 Reported quality of life in all survey respondents (n=259).

Quality of Life Compared To Number Of Treatments

259 registry-consented ITP patients completed both the Treatment and Review of Symptoms and the Quality of Life survey. In general, a higher (very good/good) and centralized consensus of quality of life was seen in patients who received two, three, or four treatments. [Figure 4-6] Patients with less than two treatments or more than five treatments, reported a lower (good/fair) and more divided quality of life [Figure 2,3,7].



Figures 2-7 (L-R, clockwise). The impact on ITP patient's health-related quality of life by the number of treatments the patient has undergone (n=16, 57, 55, 50, 19, and 62).

Discussion

Examining the relationship between disease treatment and quality of life, especially as treatment relates to efficacy and toxicity could guide shifts in current clinical guidelines. In chronic cases of ITP, an average of three treatments is unsurprising considering many patients receive first-line therapies steroids and IVIG before seeking a second-line therapy for longer-term maintenance. These findings suggest that patients who receive a greater number of different treatments may have found a therapy that best works to raise platelet counts through trial-and-error, due to the range of treatment mechanisms used to manage ITP. In addition, these patients may have also become better acclimated to their condition and consider living with their disease a "new normal," or they feel better now in comparison to when they were initially diagnosed.

Our analysis does not take into consideration whether a patient received a therapy more than once, is in remission, or the duration of a treatment, thus the data may be in favor of those patients who have undergone multiple treatments for their ITP or found a treatment that works for them.

A more longitudinal analysis is required regarding patient quality of life. Constructing a complete view of shifts in medication and treatment with combination therapies would give a better indication of the effects of the number of treatments on quality of life.

The ITP Registry allows patients to see the aggregate results of each survey in real time. From this, patients have the opportunity to be further educated about their disease, treatment options, and compare their ITP journey to the ITP community as a whole; this helps patients feel less alone, alleviating some of the burden of living with ITP.

Acknowledgments

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