



James B. Bussel<sup>1</sup>, Alexandra Kruse<sup>2</sup>, Caroline Kruse<sup>2</sup>, Jody Shy<sup>2</sup>, Kavita Aggarwal<sup>3</sup>, Michael Vredenberg<sup>3</sup>, Keith McCrae<sup>4</sup>

<sup>1</sup>Weill Cornell Medical College, New York, NY, USA; <sup>2</sup>Platelet Disorder Support Association, USA; <sup>3</sup>Dova Pharmaceuticals, Durham, NC, USA; <sup>4</sup>Cleveland Clinic, Cleveland, OH, USA

## Introduction

- Immune thrombocytopenia (ITP) is an autoimmune disorder causing reduced platelet counts <100,000/ $\mu$ L with potential bleeding consequences and reduced quality of life.
- Treatment is generally reserved for adults with platelet counts <30,000/ $\mu$ L.

## Methods

- ITP patients from the Platelet Disorder Support Association were recruited to complete a one-time, cross-sectional survey online.
- Eligible adults from the United States self-reported that they had been diagnosed with ITP and had received at least 1 treatment.
- Patients completed a 30-45-minute online survey about demographics, diagnosis experience, symptoms, disease management, and treatment.

## Results

### Demographics

- Seventy-six patients completed the survey.
- Diagnosis Experience and Knowledge (Table 1)**
- Two-thirds of patients had been diagnosed at least 10 years previously.
- Events leading to diagnosis were petechiae (21%), general check-up (10%), bleeding events (18%), and bruising (16%).
- Patients with ITP felt well informed about ITP (87.5% reported a score of  $\geq 5$  on a 7-point Likert scale)
- Ninety-seven percent of patients knew their current platelet count.
  - At the time of diagnosis, platelet counts were <10,000/ $\mu$ L in 47% and 10,000–30,000/ $\mu$ L in 27% of participants.
  - At the time of survey response, platelet count was <30,000/ $\mu$ L in 4.62% of participants; 47.69% of respondents had platelet counts >100,000/ $\mu$ L.

**Table 1: Patient Knowledge about ITP as measured on a Likert scale** (1=Very little knowledge to 7=Highly knowledgeable)

Scale	Number of patients	Percentage (%)
1	2	3.13
2	0	0.00
3	2	3.13
4	4	6.25
5	15	23.44
6	25	39.06
7	16	25.00
Total	64	-
Weighted average	5.64	-

### Symptoms

#### Fatigue (Figure 1)

- Based on a Likert scale, 58% of patients reported a score  $\geq 5$  that ITP-related fatigue interfered with their work, family, or social life.
  - Prior to treatment: 35% reported fatigue daily and 13% reported it twice a week.
  - Post-treatment: 39% reported fatigue daily and 16% reported it twice a week.

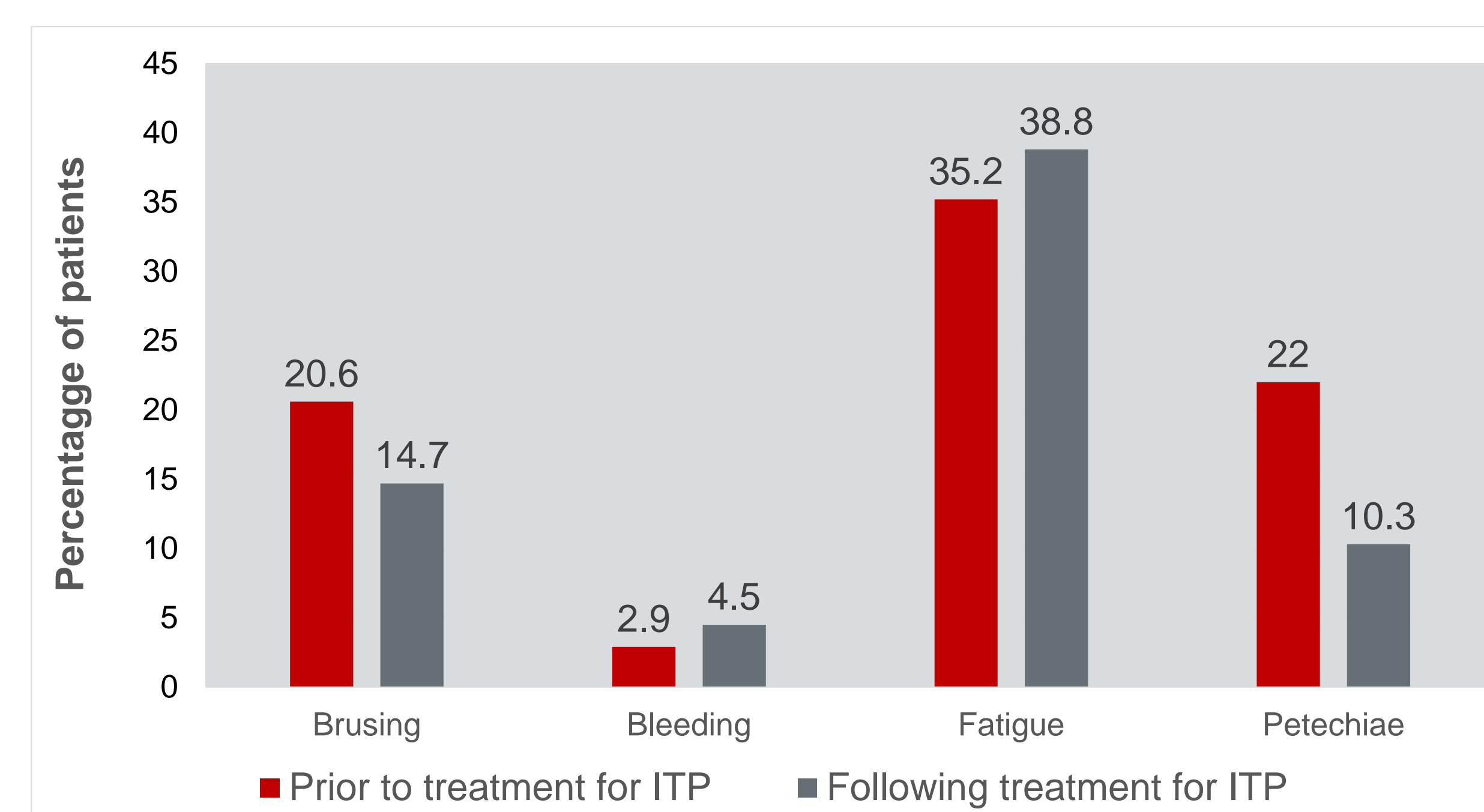
## Results

### Symptoms

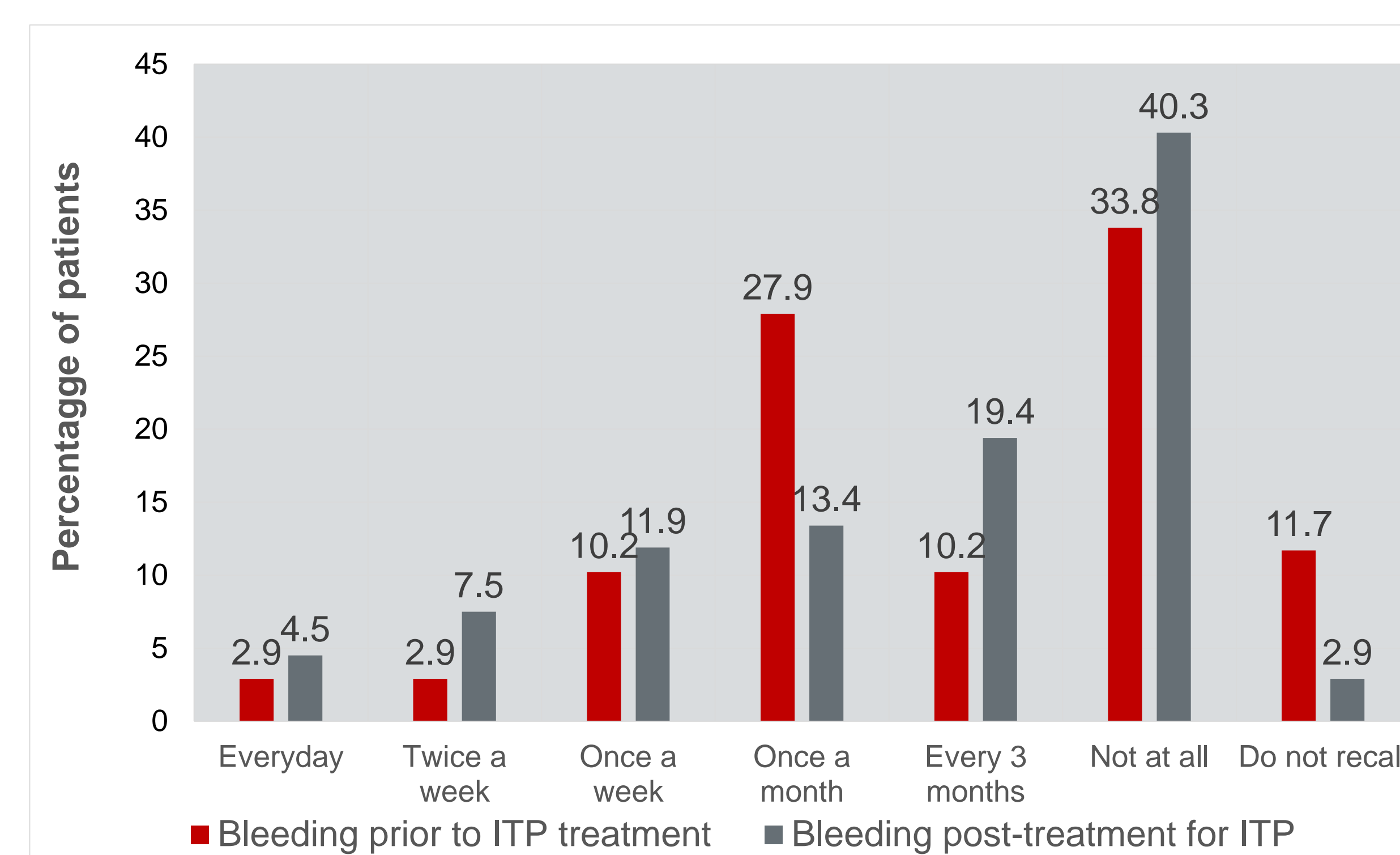
#### Bleeding (Figure 2)

- Based on a Likert scale, 59% reported a score  $\leq 3$  suggesting that bleeding does not interfere with their work, family, or social life.
  - 3% of patients reported bleeding related to their ITP daily or twice weekly prior to treatment.
  - However, fear of bleeding interfered with work, family, or social life in 47% of patients with score  $\geq 5$ ; in contrast, an equal amount, 47%, reported a score  $\leq 3$ .

**Figure 1: Symptoms reported by patients (occurring daily) before and after ITP treatment**



**Figure 2: Frequency of bleeding reported by patients before and after ITP treatment**



### Treatment and Outcomes

#### Splenectomy

- 45% of respondents underwent splenectomy without clinical improvement.
- 31% of respondents were offered splenectomy but declined surgery.

#### Steroids (Table 2)

- Almost 90% of patients received steroids as first line of treatment; 72% received prednisone and 15% received dexamethasone.

## Results

### Treatment and Outcomes

#### TPO-RAs (Table 2)

- Among TPO-RAs, romiplostim was listed as a first line of treatment by only 2 patients.
- Most patients had received rituximab (72%) and eltrombopag (55%) during their course of treatment; 40% received romiplostim.

**Table 2: Treatment Sequencing Pattern in Patients**

Medications	1 <sup>st</sup> line Treatment		2 <sup>nd</sup> line Treatment		3 <sup>rd</sup> line Treatment		4 <sup>th</sup> line Treatment		5 <sup>th</sup> line Treatment	
	%	Nos.	%	Nos.	%	Nos.	%	Nos.	%	Nos.
Patients →										
Did not take any medicine	-	-	3.5	2	8.0	4	25.6	11	43.7	14
<b>Steroids</b>										
Dexamethasone	14.8	9	7.0	3	10.0	5	11.6	5	6.2	2
Prednisone	72.1	44	3.5	2	2.0	1	0	0	0	0
Prednisolone	0	0	1.7	1	2.0	1	2.3	1	0	0
<b>IVIg</b>										
Gamunex	4.9	3	29.8	17	4.0	2	0	0	0	0
Gammagard	1.6	1	17.5	10	2.0	1	0	0	0	0
Gammaplex	0	0	3.5	2	0	0	2.3	1	0	0
Gammaked	0	0	0	0	0	0	0	0	0	0
Privigen	0	0	5.2	3	4.0	2	0	0	0	0
<b>Rituximab</b>										
Rituxan	0	0	15.8	9	34.0	17	6.9	3	9.3	3
MabThera	0	0	0	0	0	0	0	0	0	0
<b>Anti-D Therapy</b>										
RhoGam	0	0	1.7	1	2.0	1	0	0	0	0
WinRho	0	0	0	0	2.0	1	4.6	2	0	0
<b>Thrombopoietin Receptor Agonists (RPO-RA)</b>										
Promacta (eltrombopag)	0	0	1.7	1	16.0	8	18.6	8	9.3	3
Nplate (romiplostim)	3.2	2	1.7	1	10.0	5	16.3	7	3.1	1
<b>Fostamatinib</b>										
Tavalisse (fostamatinib)	0	0	0	0	0	0	2.3	1	3.1	1
<b>Other</b>										
Danazol	1.6	1	3.5	2	0	0	0	0	9.3	3
Mycophenolate mofetil (Cellcept, Myfortic)	0	0	0	0	0	0	2.3	1	9.3	3
Cyclophosphamide (Cytoxan, Neosar)	0	0	3.5	2	2.0	1	2.3	1	0	0
Azathioprine (Imuran, Azasan)	0	0	0	0	2.0	1	4.6	2	3.1	1
Cyclosporine (Sandimmune, Neoral)	1.6	1	0	0	0	0	0	0	3.1	1
Sirolimus (Rapamune)	0	0	0	0	0	0	0	0	0	0
<b>Total</b>	-	61	-	57	-	50	-	43	-	32

## Conclusions

- This survey demonstrates some of the effects of fatigue, bleeding, and treatment on the lives of patients with ITP.
- Almost half the patients had failed splenectomy, although very few patients currently undergo this procedure. This indicates that patients taking the survey were not “random” ITP patients but may likely represent an atypical group that is refractory to treatment or dissatisfied with the course of their ITP despite half the patients having normal platelet counts at the time of the survey.
- The most surprising finding was that, even in patients in whom treatment raised platelet count, fatigue was not always alleviated. These and other issues remain to be clarified as we further unravel the complexity of ITP.