THE ITP NATURAL HISTORY PATIENT STUDY REGISTRY:
PRELIMINARY RESULTS FROM THE IMMUNE THROMBOCYTOPENIA (ITP) COMMUNITY

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REGISTRY OBJECTIVES

- Collect data on the natural progression of ITP
- Comprehensively understand ITP and its progression over time
- Characterize and describe the ITP population as a whole
- Assist the ITP community with the development of recommendations for standards of care
- Assist researchers studying the pathophysiology of ITP and interventional outcomes
- Support the design of clinical trials for new treatments

This preliminary analysis seeks to provide an abridged overview of registry findings.
SURVEY METHODS

**Adults:** 172 questions across 5 surveys

**Children:** 141 questions across 4 surveys

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<tr>
<th>Enrollment</th>
<th>843</th>
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<tbody>
<tr>
<td>Consented</td>
<td>742</td>
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<tr>
<td>Adults</td>
<td>668</td>
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<td>Children</td>
<td>74</td>
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<th>Surveys</th>
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<td>Participant Profile</td>
<td>473</td>
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<tr>
<td>Treatment and Review of Systems</td>
<td>329</td>
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<td>Medical and Diagnostic Data</td>
<td>378</td>
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<td>Quality of Life</td>
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<td>Adult 1</td>
<td>265</td>
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<td>Adult 2</td>
<td>251</td>
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<td>Pediatrics</td>
<td>38</td>
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<td>All Surveys Completed</td>
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demographics

- 90.3% Caucasian
- 76.4% Female
- 86.5% American
- 9 of 378 patients were diagnosed with an additional disorder
- 38 years Average age of participants
- 12 ½ years Average duration of disease
- Range: 1-100 years
- Range: 0-69 years
- 20 countries represented
Figure 1. A majority of patients were diagnosed within a year of first presenting with symptoms (n=328). 30.7% waited over a year for their official diagnosis (n=145; range 1-42 years). (n=473).
- 86.6% steroids
- 41.6% IVIG
- 36.8% TPO
- 31.9% rituximab

23.7% Splenectomy

54.7% supplements

12% “special diet”

Figure 2. Treatments (n=955) received by patients (n=304) over their current duration of ITP. 25 patients did not receive any therapy.
The impact of ITP on an adult patient’s health-related quality of life (n=265)

- 65% “good/very good” physical health
- 62% “good/very good” mental health
- 90% pain interferes with QoL
- 88% experienced fatigue that week due to ITP
QUALITY OF LIFE: ADULTS

FATIGUE
- None, 12%
- Severe, 13%
- Mild, 32%
- Moderate, 41%
- Very severe, 2%

DEPRESSION
- Never, 15%
- Rarely, 35%
- Sometimes, 32%
- Often, 15%
- Always, 3%

ANXIETY
- Never, 35%
- Rarely, 22%
- Sometimes, 26%
- Often, 13%
- Always, 4%

n=265
QUALITY OF LIFE: PEDIATRIC

84% = Good/Very Good Overall QoL

- 63% = bothered by emotional problems
- 76% = feels nervous about ITP
- 84% = feels sad about ITP

- 71% = tired from ITP
- 39% = experienced pain from ITP in last week
- 7/38 children could no longer participate in their favorite activities

n = 38 children
NEXT STEPS

Continuing to build data set

Examining concurrent medications, patient feelings and responses regarding each treatment, and treatment efficacy and toxicity could guide shifts clinical decision making

Examine differences in disease experience across sub-populations

Comparison between acute, chronic, and patients in remission

Additional surveys in relevant topics

Data access protocol