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Underreporting of Patient-Reported Outcomes in Myeloproliferative Neoplasms Clinical Trials

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There are no relationships to disclose.

1 | Background

Patient-reported outcomes (PRO) provide unique and meaningful insight about the effect of a treatment from a patient’s view. Patients with Myeloproliferative Neoplasms (MPN) can live with their disease for long periods of time. Therefore a comprehensive evaluation of treatment effectiveness should consider the patient burden of the disease and treatment effect.

2 | Objective

To evaluate the frequency at which PRO measures are utilized as study endpoints and are made publicly available when trial results are published.

3 | Methods

- We searched Citeline\textsuperscript{1} Trialtree database for randomized clinical trials including patients with myeloproliferative neoplasms, initiated between 2006-2016, utilizing at least 1 PRO measure.
- We searched PubMed, Medline (Ovid), Google Scholar, ClinicalKey and ONAHLE databases for publications associated with the trial, and recorded date of publication, year, number of randomized patients and reported outcomes.
- We then conducted a comprehensive evaluation of treatment effectiveness should consider the patient burden of the disease and treatment effect.

4 | Results

- 19 trials (19/30; 63%) included at least one PRO assessment as an endpoint (Table 1).
- Among these 19 trials, a variety of PROs were utilized to measure symptoms, functional health, and psychological and somatic HRQOL (Figure 2).

<table>
<thead>
<tr>
<th>Trial Characteristics</th>
<th>n/N</th>
<th>%</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total number of MPN studies identified</td>
<td>35/35</td>
<td>100</td>
</tr>
<tr>
<td>Potentially included studies</td>
<td>30</td>
<td></td>
</tr>
<tr>
<td>Disease states</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Chronic Myelogenous Leukemia (CML)</td>
<td>20/35</td>
<td>57.1</td>
</tr>
<tr>
<td>Polycythemia Vera (PV)</td>
<td>2/35</td>
<td>5.7</td>
</tr>
<tr>
<td>Primary Myelofibrosis</td>
<td>5/35</td>
<td>14.3</td>
</tr>
<tr>
<td>Myelosclerosis</td>
<td>2/35</td>
<td>5.7</td>
</tr>
<tr>
<td>Essential Thrombocytosclerosis</td>
<td>1/35</td>
<td>2.9</td>
</tr>
<tr>
<td>Combination of MPNs</td>
<td>3/35</td>
<td>8.6</td>
</tr>
</tbody>
</table>

- Seven studies (36.8%) that included a PRO failed to publish PRO data.
- 13/19 trials that had a PRO measure (68.4%) were sponsored by industry, three (15.8%) by industry-academic and three (15.8%) solely by academic institutions.

5 | Conclusion

- A substantial number of MPN randomized trials did not include a PRO measure.
- Disease-specific PRO measures were used in 52% of the studies that included a PRO.
- In 37% of trials, PRO data was not reported.
- Even when reported, PRO data often came late after the initial report and partially reported.

6 | Recommendations

The collection of PRO data should result in routine, timely and appropriate reporting as part of the trial outcome publication to allow for a thorough assessment of investigational drug treatment effects. Investigations should focus on causal factors that influence the underreporting of PRO data inclusion in trial publications such as: (1) logistics with trial design (2) missing data or difficulty with data collection in patients with advanced or progressive disease (3) utilization of disease-specific PRO measures.