EPH85

Estimating Rare Disease Incidence and Prevalence of Essential Thrombocythemia in Major Health Plans in the United States

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KEY FINDINGS & CONCLUSION

- Wide variation exists in both prevalence and incident patient estimates across US commercial health plans.
- This variability can affect eligibility for NCCN recommended ET cytoreductive therapies and formulary policy recommendations.

INTRODUCTION

- Essential thrombocythemia (ET) is a rare myeloproliferative neoplasm (MPN) with risks of thromboembolic events and progression to myelofibrosis or acute myeloid leukemia.
- Current ET treatment landscape is limited despite the lack specificity of prior ICD-10 diagnosis coding and ET patient medical expenditures being 2.3 times greater versus matched comparisons.

OBJECTIVE

- To estimate the ET incident and prevalent patient populations eligible for National Comprehensive Cancer Network (NCCN) guideline driven treatments among major US health plans.

METHODS

- A systematic literature assessment identified epidemiologic estimates to determine the prevalence and incidence of ET, the revised IPSET-thrombosis risk stratification (rIPSET-T) distribution and patients eligible to receive NCCN 1.2025 recommended ET cytoreductive therapy options.
- Health plan membership data from the eight largest commercial health plans was determined from Earning releases, Annual and 10-K reports (**Table 1**).
- The data was incorporated into Microsoft Excel to create model inputs and calculate the base-case analysis of a clinically treatment eligible population that may potentially receive NCCN recommended treatments.

Table 1. United States health plan membership data

US Health Plan	Number of Lives Covered
All Blues Plans	118,000,000[1]
Elevance	45,734,000[2]
United Health Care	49,345,000[3]
CVS	27,095,000[4]
Centene	28,600,600[5]
Cigna	19,147,000[6]
Humana	16,347,100[7]
Molina	5,535,000[8]

RESULTS

- Sixteen references identified the epidemiology[9-14,22], rIPSET-T distribution[15-19], and utilization of NCCN recommended treatment options[15-18,20-21] in separate systematic literature searches.
- Of the 8 health plans assessed, the mean and median lives covered were 38,725,462 and 27,847,800 respectively.
- A mean 426 (SD±362) and 12,164 (SD±14,379) incident and prevalent ET NCCN cytoreductive therapy eligible patients among US health plans were identified (**Table 2**).

Table 2. Mean (SD) incident and prevalent ET NCCN cytoreductive therapy eligible patients among US health plans

US Health Plan	Incident Point Estimate	Prevalent Point Estimate
All Blues Plans	1,298	51,542
Elevance	503	19,977
United Health Care	543	21,554
CVS	298	11,835
Centene	315	12,493
Cigna	211	8,363
Humana	180	7,140
Molina	61	2,418
Mean	426	12,164
Standard Deviation	±362	±14,379

- Among the ET health plan(s) population, a mean of 192 (SD±109), and 8,797 (SD±7,479) in incident high-and prevalent intermediate and high-rIPSET-T eligible patients were identified, respectively (**Table 3**).

Table 3. Mean (SD) incident high-and prevalent intermediate and high-rIPSET-T eligible patients among the ET US health plans population

US Health Plan	Incident High rIPSET-T	Prevalent Intermediate / High-rIPSET-T
All Blues Plans	584	26,806
Elevance	226	10,390
United Health Care	244	11,210
CVS	134	6,155
Centene	142	6,497
Cigna	95	4,350
Humana	81	3,714
Molina	27	1,257
Mean	192	8,797
Standard Deviation	±109	±7,479

- The median percentage of NCCN identified treatment eligible patients was 67.0% representing a mean of 128 (SD±109) and 5,100 (SD±4,335) of incident and prevalent patients, respectively.

LIMITATIONS

- Variability of incidence and prevalence values reported in the literature have not been incorporated into the current analyses.

CONCLUSIONS

- Wide variation exists in both prevalence and incident patient estimates across US commercial health plans.
- This variability can affect eligibility for NCCN recommended ET cytoreductive therapies and formulary policy recommendations.

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