Research Paper

Real-world clinical characterisation of polycythemia vera patients from a prospective registry in Portugal: is resistance to hydroxyurea a reality?

Maria Sarmento1, Marta Duarte1, Juan Sanchez2, Sandra Ponte2,\*, Diana Roriz3, Laura Fernandes3, Maria José Monteiro Silva4, Judite Pacheco4, Gisela Ferreira5, Jorge Freitas6, Inês Costa7, Daniel Brás7

1Unidade Local de Saúde de Matosinhos, Hospital Pedro Hispano, Senhora da Hora, Portugal;

2Centro Hospitalar Lisboa Ocidental, Hospital de São Francisco Xavier, Lisboa, Portugal; smponte@chlo.min-saude.pt

3 Fundação Champalimaud, Lisboa, Portugal;

4 Centro Hospitalar de Vila Nova de Gaia/Espinho, Vila Nova de Gaia, Portugal;

5 Centro Hospitalar do Baixo Vouga, Aveiro, Portugal;

6 Instituto Português de Oncologia do Porto, Porto, Portugal;

7 Novartis Farma, Produtos Farmacêuticos S.A, Porto Salvo, Portugal;

\*Correspondence: smponte@chlo.min-saude.pt

SUPPLEMENTARY INFORMATION

**TABLES**

**Table S1. Gender and mean disease duration of low- and high-risk PV patients.**

|  |  |  |
| --- | --- | --- |
| **Baseline characteristics** | **Low-risk (n=21)** | **High-risk (n=104)** |
| Gender | n=21 | n=104 |
| Male | 13 (61.9%) | 46 (44.2%) |
| Female | 8 (38.1%) | 58 (55.8%) |
| Disease duration, years | n=20 | n=102 |
| <1 year | 4 (20.0%) | 29 (28.4%) |
| 1 - 4 years | 6 (30.0%) | 34 (33.3%) |
| 5 - 9 years | 7 (35.0%) | 21 (20.6%) |
| 10 - 14 years | 1 (5.0%) | 10 (9.8%) |
| ≥15 years | 2 (10.0%) | 8 (7.8%) |
| Mean ± SD | 5.2 ± 4.9 | 4.7 ± 5.1 |
| Missing | 1 (4.8%) | 2 (1.9%) |

**Table S2. Types of thrombotic and hemorrhagic events since PV diagnosis**

|  |  |
| --- | --- |
|  | n=134 |
| Thrombotic events | 10 |
| Arterial |  |
| Extensive myocardial ischemia | 1 |
| Pulmonary embolism thrombus | 1 |
| Retinal vascular thrombosis | 1 |
| Venous |  |
| Deep vein thrombosis | 2 |
| Portal vein thrombosis | 1 |
| Unspecified myocardial infarction venous insufficiency | 1 |
| Splenic vein thrombosis | 1 |
| Superficial venous thrombosis | 1 |
| Thrombosis | 1 |
| Hemorrhagic events | 8 |
| Stroke | 3 |
| Ischemic infarction | 2 |
| Gastric ulcer - towel hemorrhage | 1 |
| Upper gastrointestinal bleeding | 1 |
| Acute myocardial infarction | 1 |

**Table S3. Probabilities of thrombo-haemorrhagic events for the first 5 years after PV diagnosis.**

|  |  |
| --- | --- |
|  | % [95% CI] |
| 6 months | 5.0% [0.0, 11.5] |
| 12 months | 5.0% [0.0, 11.5] |
| 24 months | 8.0% [0.0, 16.3] |
| 36 months | 11.3% [0.1, 21.2] |
| 48 months | 11.3% [0.1, 21.2] |
| 60 months | 17.2% [0.9, 30.8] |

NOTE: only data from the first five years after PV diagnosis was considered.

**Table S4**. **Clinical outcomes of PV**

|  |  |  |  |
| --- | --- | --- | --- |
|  | Baseline | 12 months | 24 months |
| MF progression | n=66 | n=29 | n=22 |
| No | 65 (98.5%) | 29 (100.0%) | 21 (95.5%) |
| Yes | 1 (1.5%) | 0 (0.0%) | 1 (4.5%) |
| AML transformation | n=66 | n=29 | n=22 |
| No | 66 (100.0%) | 29 (100.0%) | 22 (100.0%) |
| Yes | 0 (0.0%) | 0 (0.0%) | 0 (0.0%) |
| Non-hematological toxicity | n=66 | n=29 | n=23 |
| Not available | 21 (31.8%) | 17 (58.6%) | 14 (60.9%) |
| No | 44 (66.7%) | 12 (41.4%) | 8 (34.8%) |
| Yes | 1 (1.5%) | 0 (0.0%) | 1 (4.3%) |
| Tinnitus | 1 (1.5%) | 0 (0.0%) | 0 (0.0%) |
| Bradycardia | 0 (0.0%) | 0 (0.0%) | 1 (4.3%) |

**Table S5**. **Clinical outcomes of PV**

|  |  |  |
| --- | --- | --- |
| Baseline | 12 M | 24 M |
| Neutrophils <10  Phlebotomies  Total symptom score ≥20 Individual symptom score >5 | Neutrophils <10  Individual symptom score >5 | Neutrophils <10 |
| Neutrophils <10  Phlebotomies  Total symptom score ≥20 | Phlebotomies | Neutrophils <10 |
| Neutrophils <10 | Neutrophils <10  Phlebotomies  Total symptom score ≥20 | Neutrophils <10 |
| Neutrophils <10  Total symptom score ≥20  2 Individual symptom score >5 | Neutrophils <10 | Neutrophils <10 |
| Neutrophils <10  Phlebotomies  Total symptom score ≥20 | Phlebotomies  Individual symptom score >5 | Neutrophils <10  Phlebotomies  Platelet count >400×109/L and leukocyte count >10×109/L |
| Total symptom score ≥20  Individual symptom score >5 | Phlebotomies | Phlebotomies |
| Neutrophils <10  Total symptom score ≥20  2 Individual symptom score >5  Platelet count >400×109/L and leukocyte count >10×109/L | 2 Individual symptom score >5  Platelet count >400×109/L and leukocyte count >10×109/L | Platelet count >400×109/L and leukocyte count >10×109/L |
| Phlebotomies | Neutrophils <10  Phlebotomies  2 Individual symptom score >5 | Phlebotomies |
| Neutrophils <10 | Platelet count >400×109/L and leukocyte count >10×109/L | Platelet count >400×109/L and leukocyte count >10×109/L |

**Table S6. Treatment profile of patients with hematocrit level <45%.**

|  |  |  |
| --- | --- | --- |
| **Hematocrit <45%** | Baseline (n=46) | 24 months  (n=20) |
| Phlebotomy alone | 3 (6.5%) | 0 (0.0%) |
| HU alone | 25 (54.3%) | 14 (70%) |
| Phlebotomy + HU | 12 (26.1%) | 6 (30%) |
| No phebotomies or HU | 6 (13.0%) | 0 (0.0%) |