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## Real-life observational cohort “TOPASE” subgroup analysis highlights efficacy of ponatinib to maintain or induce a deep molecular response in CP-CML patients

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**INTRODUCTION**

Ponatinib is a 3<sup>rd</sup> generation tyrosine kinase inhibitor (TKI) indicated in chronic phase (CP), accelerated phase (AP) and blast phase (BP) chronic myeloid leukemia (CML) as well as in patients with the gatekeeper T315I mutation. TOPASE is an observational cohort, currently carried out in 40 CML French centers, designed to evaluate, over a two-year follow-up period, the efficacy and safety of ponatinib in routine clinical practice. The present interim analysis focused on a subgroup of 24 patients (out of 110 included) at least in major molecular response (MMR) for whom ponatinib was prescribed for intolerance to previous TKIs or for increasing the depth of MR. Data cut-off for analysis was December 2020.

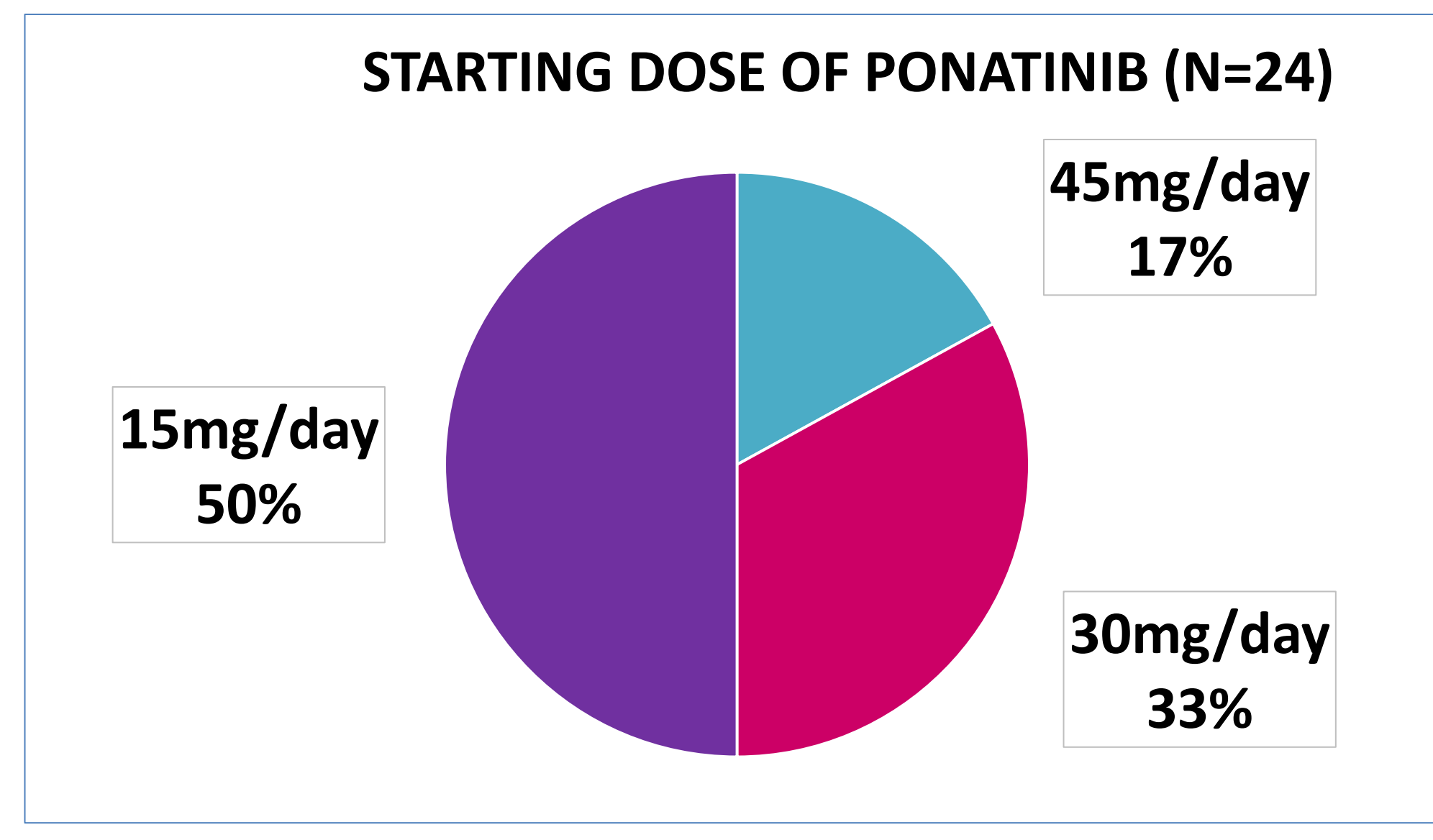
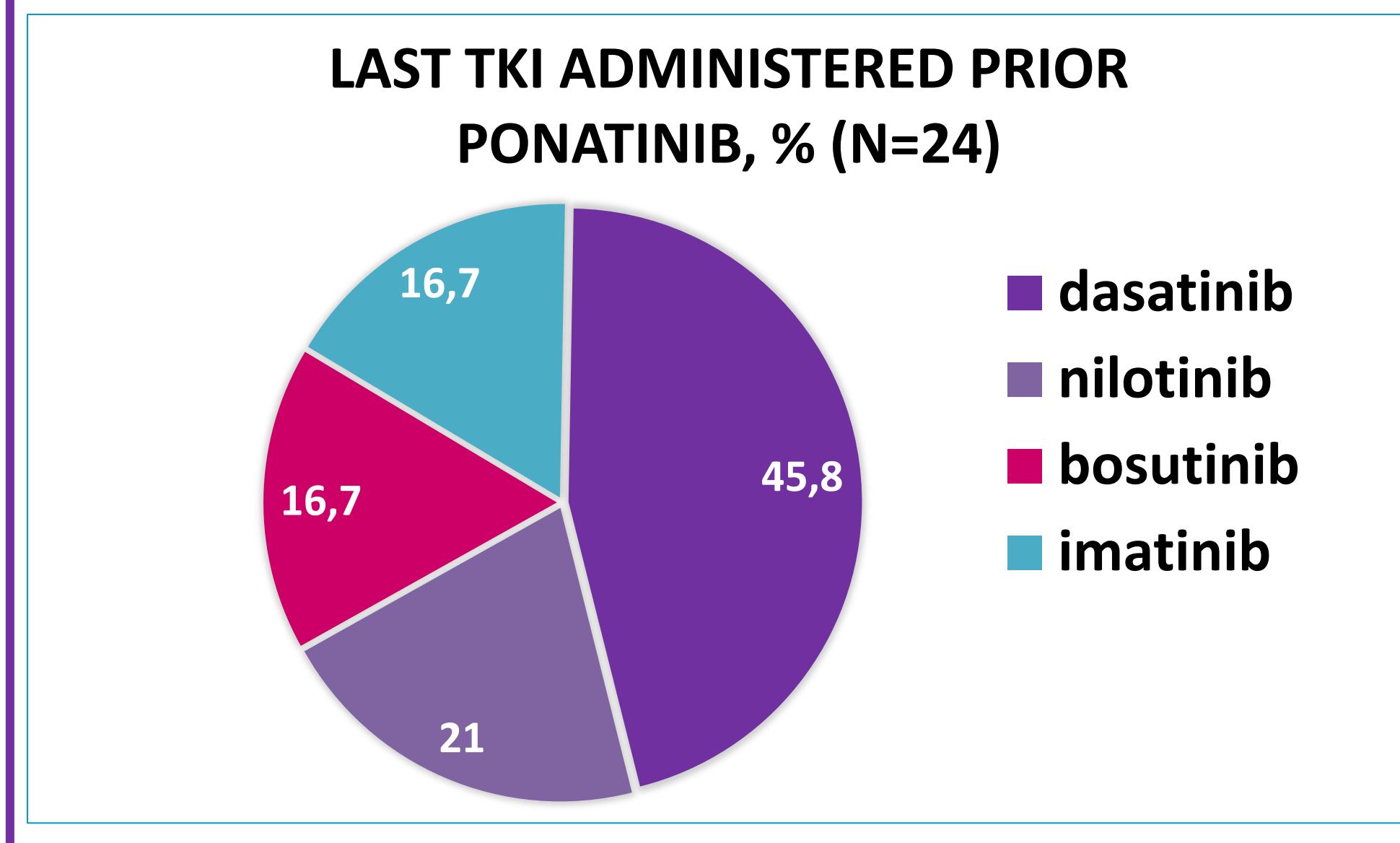
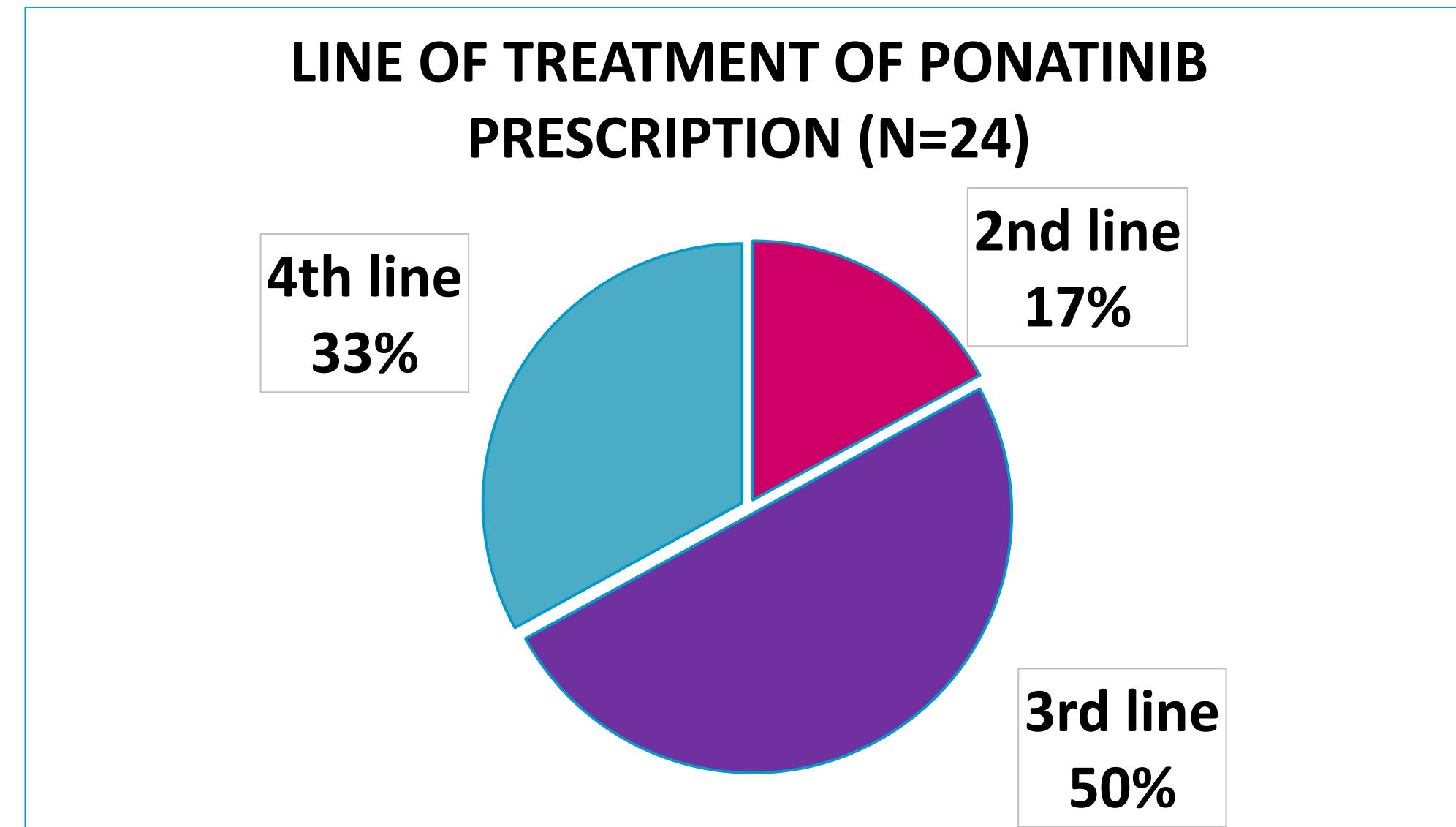
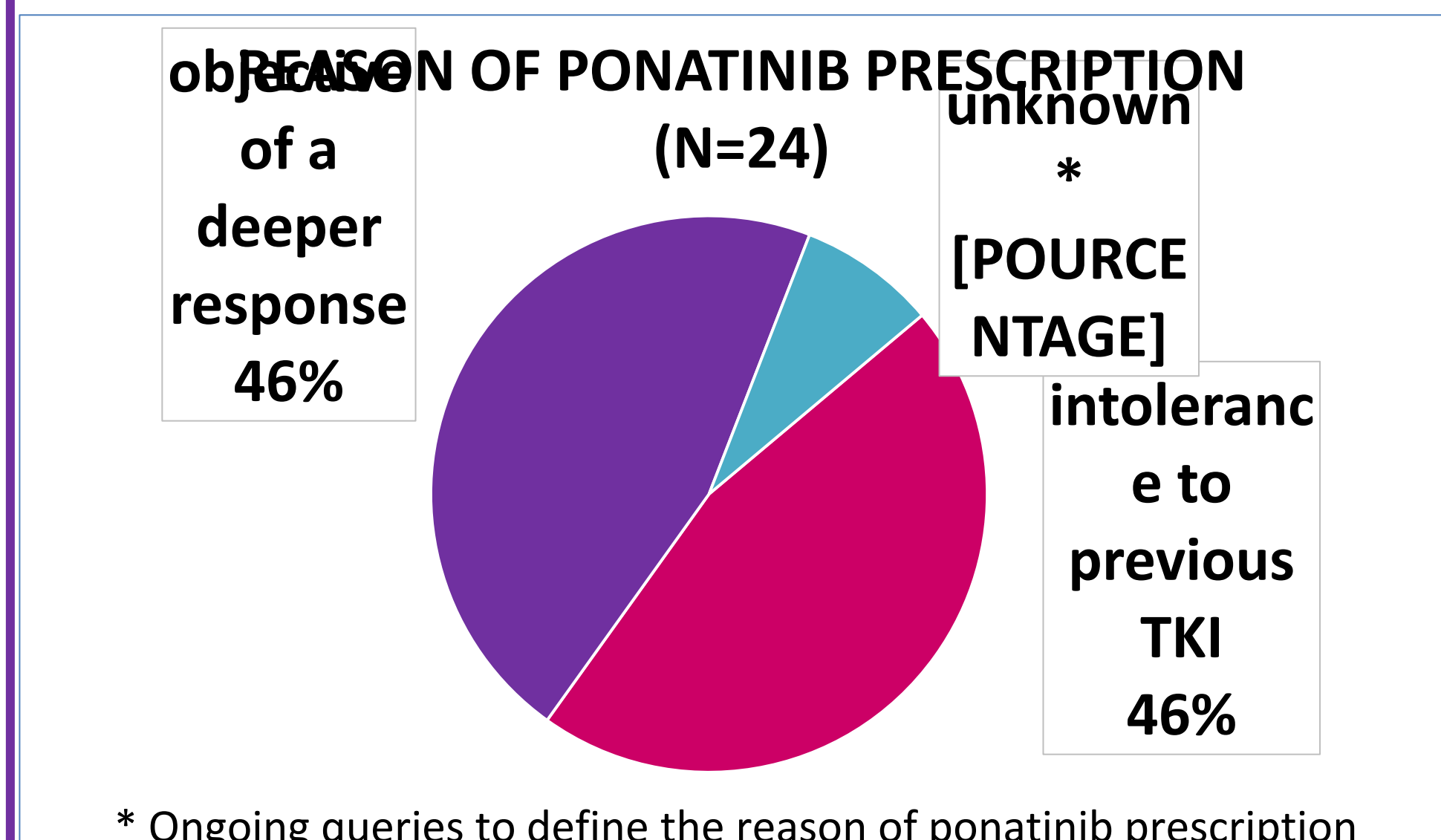
**RESULTS**

**CHARACTERISTICS OF PATIENTS**

Patients characteristics	Male/Female, %	Median age, year	Median age at diagnosis of CML, year
n = 24	46% / 54%	45.6 [38.0 - 60.4]	39.7 [23.4 - 53.5]

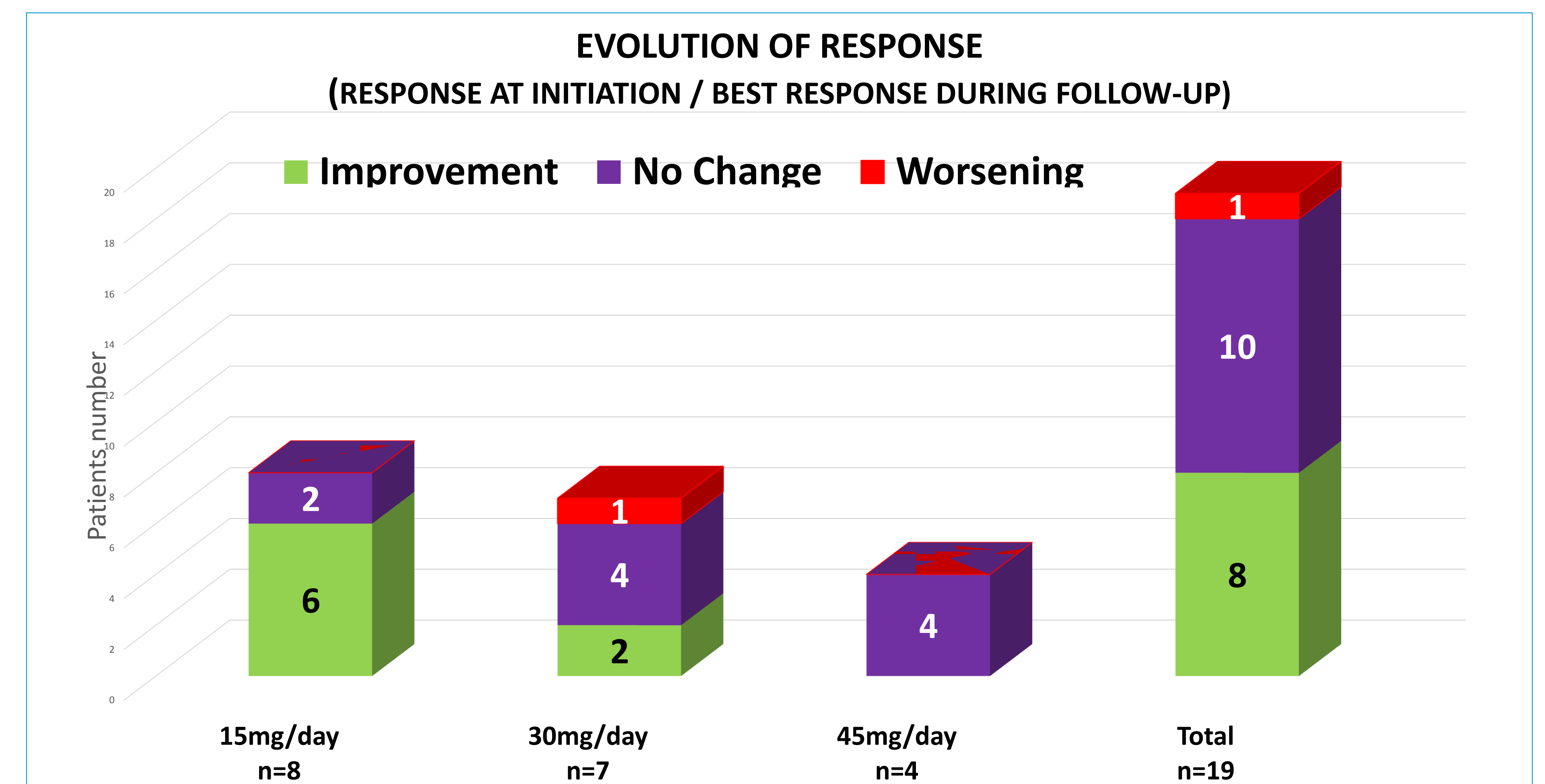
Among comorbidities at baseline, hypertension was present in 17% of patients (pt).  
One pt harbored a M244V mutation.

Sokal score distribution, %	Low risk	Intermediate risk	High risk
(not evaluable = 17,4%)	43.5 %	26.1 %	13 %



**EFFICACY ANALYSIS (performed on 19 CP-CML patients with at least one evaluation under treatment)**

- Out of 24 patients, 2 pts withdrew from the study (treatment discontinuation) before the first evaluation (1 for adverse event reason and 1 because of patient decision) and 3 pts were included before data cut-off without follow-up evaluation.
- Median treatment duration was of 16.5 months (range 3.48; 24.30).
- All pts maintained or increased the depth of MR except for 1 pt (worsening to MR2).
- Before ponatinib, only 3 pts were in MR4,5 or above. After ponatinib treatment, there were 11 pts in MR4,5 or above.

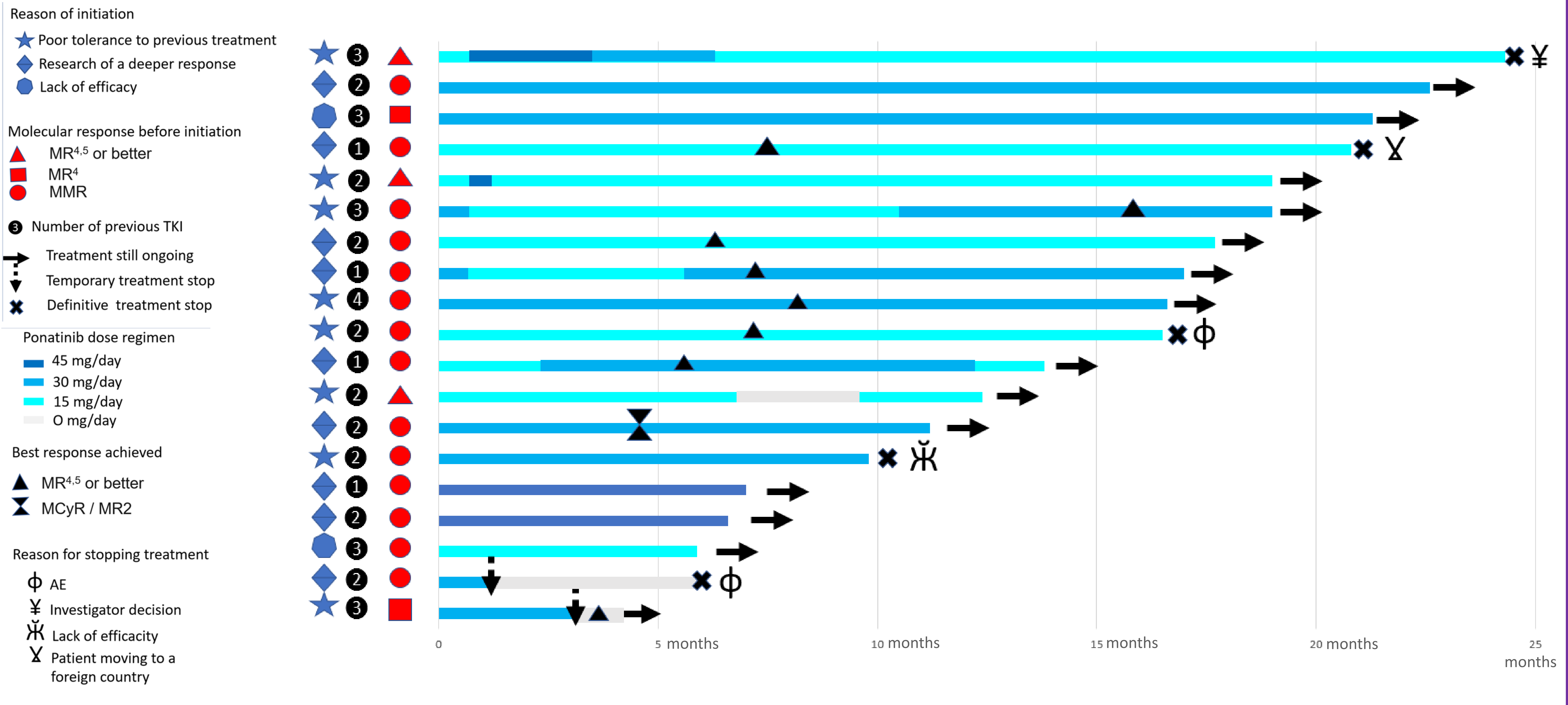


**SAFETY PROFILE**

**Non SAE related to ponatinib for 11 patients**

Skin and subcutaneous tissue disorders	5
Hypertension	3
Gastro-intestinal disorder	3
Musculoskeletal pain	2
Hepatobiliary disorder	1
Eye disorder	1
Increased lipase	1
Decreased appetite	1
Hot flush	1
Dizziness	1
Cough	1

Two pts (9.5%) encountered a serious adverse event (SAE) : 1 febrile neutropenia, 1 cerebrovascular accident; this last event led to treatment discontinuation.



**CONCLUSION**

Results from this subgroup analysis highlight the high efficiency of ponatinib to maintain or induce a deep MR in CP-CML patients, with an acceptable safety profile. Further analysis on a larger population would be necessary to confirm this result.

**Keywords:** BCR-ABL, Chronic myeloid leukemia, Targeted therapy, Tyrosine kinase inhibitor