Practical Considerations in the Treatment Myeloproliferative Neoplasms: Prognostication and Current Treatment

Indy Hematology

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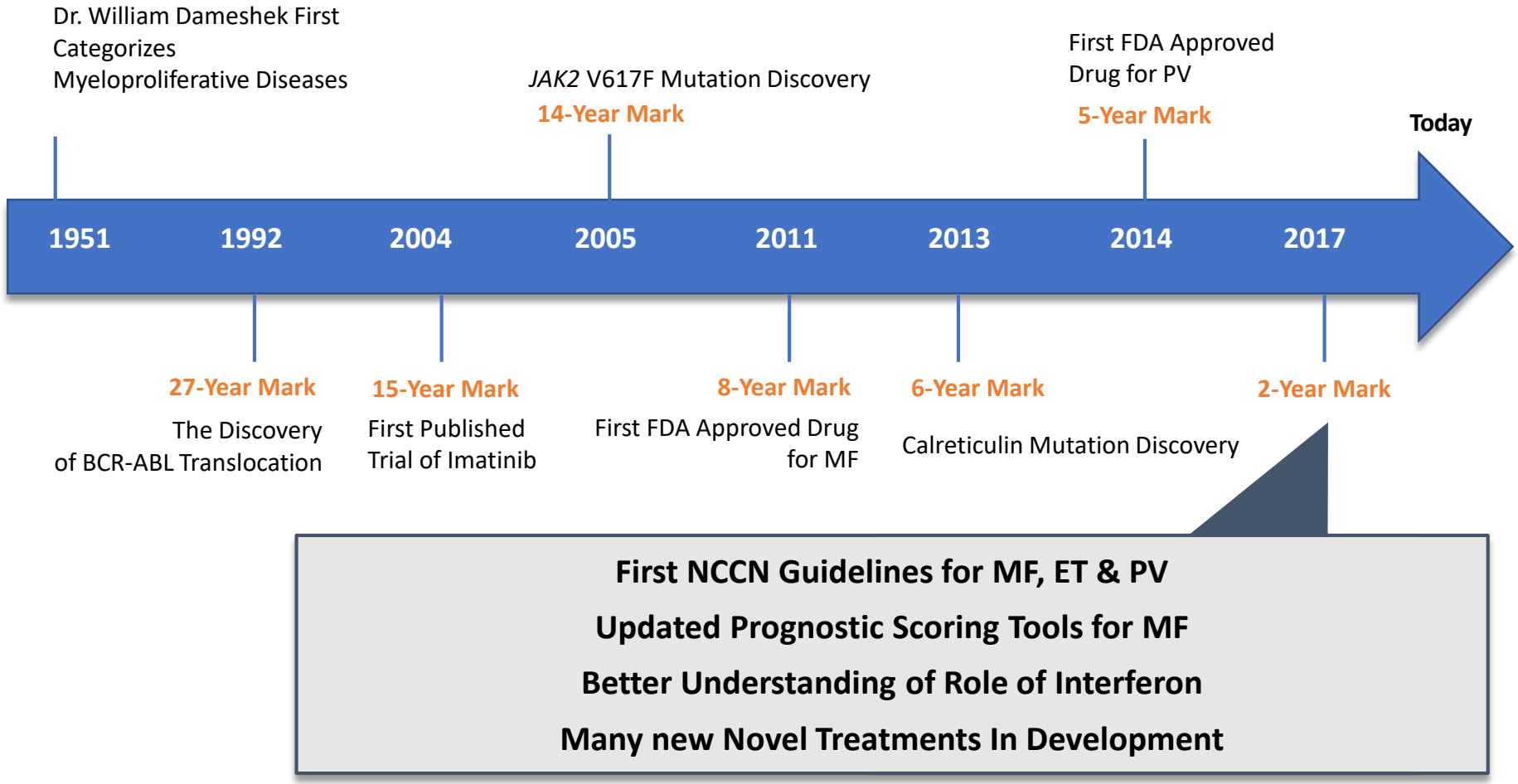
UC Irvine

March 9, 2019

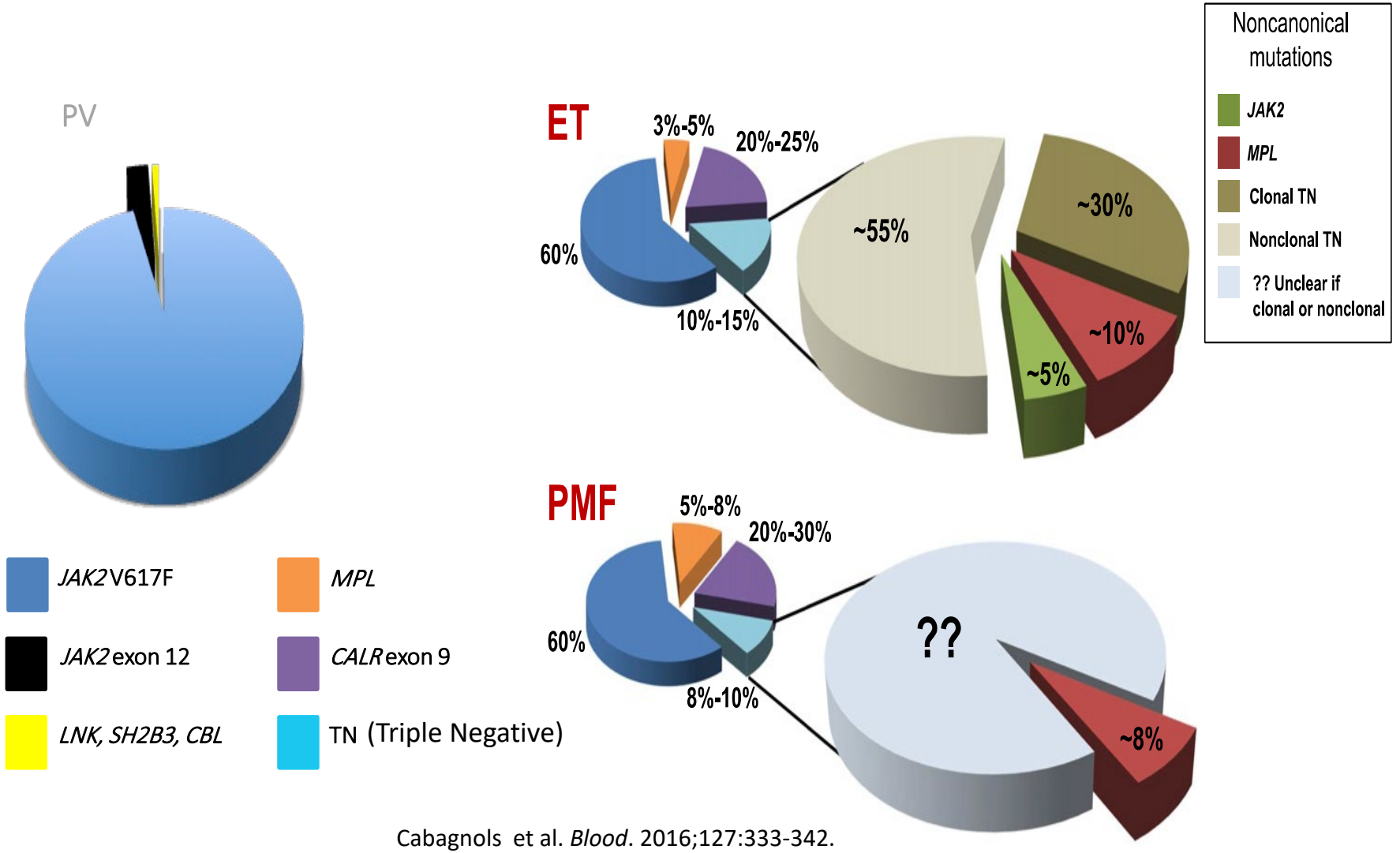
Disclosures: Angela Fleischman

- Incyte – speaker’s bureau

2019 In Perspective for Myeloproliferative Neoplasms (MPNs)



The Molecular Anatomy of MPNs: Few Patients Without an Evident Clone



Cabagnols et al. *Blood*. 2016;127:333-342.
Feenstra JD et al. *Blood*. 2016;127:325-332.
Harrison CN, Vannucchi AM. *Blood*. 2016;127:276-278.

MPN Clinical Oral Abstracts: American Society of Hematology (ASH) 2018

Sunday

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Prognostication in MPN

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IPSS and DIPSS Scoring System for MF

International Prognostic Scoring System (IPSS)

At diagnosis

IPSS risk and survival[1]		
Risk category	**Number of risk factors**	**Median survival, years**
Low	0	11.3
Intermediate-1	1	7.9
Intermediate-2	2	4.0
High	≥3	2.3

- Age >65 years
- Constitutional symptoms
- Hemoglobin <10 g/dL
- WBC count >25 × 10^9/L
- Blood blasts ≥1%

Dynamic International Prognostic Scoring System (DIPSS)

During treatment

DIPSS risk and survival[6]		
Risk category	**Number of risk factors**	**Median survival, years**
Low	0	Not Reached
Intermediate-1	1–2	14.2
Intermediate-2	3–4	4
High	5–6	1.5

1 point each
2 points for Hgb <10g/dL in DIPSS

Multiple Prognostic Scoring Systems in MF Utilizing Karyotype and Genetic Data

Leukemia

Article | OPEN | Published: 23 March 2018

Chronic myeloproliferative neoplasms

GIPSS: genetically inspired prognostic scoring system for primary myelofibrosis

Ayalew Tefferi, Paola Guglielmelli, Maura Nicolosi, Francesco Mannelli, Mythri Mudireddy, Niccolo Bartalucci, Christy M. Finke, Terra L. Lasho, Curtis A. Hanson, Rhett P. Ketterling, Kebede H. Begna, Naseema Gangat, Animesh Pardanani & Alessandro M. Vannucchi

Leukemia **32**, 1631–1642 (2018) | Download Citation

GIPSS is based exclusively on mutations and karyotype.

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CORRESPONDENCE

MIPSS70+ Version 2.0: Mutation and Karyotype-Enhanced International Prognostic Scoring System for Primary Myelofibrosis

Ayalew Tefferi, Paola Guglielmelli, Terra L. Lasho, Naseema Gangat, Rhett P. Ketterling, Animesh Pardanani, and ...

MIPSS70+ *version 2.0* utilizes both genetic and clinical risk factors.
MIPSS70+ *version 2.0* requires an online score calculator (http://www.mipss70score.it)

http://www.mipss70score.it/

#	Question	Answer
1	Anemia (hemoglobin <100g/L)	Yes No
2	Leucocytosis >25x10⁹/L	Yes No
3	Thrombocytopenia (platelet count <100x10⁹/L)	Yes No
4	Peripheral blood blast count ≥2%	Yes No
5	Bone marrow fibrosis grade ≥2	Yes No
6	Constitutional symptoms	Yes No
7	Absence of CALR type 1/like mutation	Yes No
8	HMR* category	Yes No
9	≥2 HMR mutated genes	Yes No
10	Unfavorable karyotype**	Yes No Not available

Score	Result
MIPSS70	
MIPSS70-plus	

* presence of at least one mutated gene among ASXL1, EZH2, SRSF2, IDH1/2
— *Vannucchi AM, Lasho TL, Guglielmelli P, et al: Mutations and prognosis in primary myelofibrosis. Leukemia 27:1861-9, 2013*

** indicates any abnormal karyotype other than normal karyotype or sole abnormalities of 20q-, 13q-, +9, chromosome 1 translocation/duplication, -Y or sex chromosome abnormality other than –Y
— *Tefferi A et al. Revised cytogenetic risk stratification in primary myelofibrosis. 2017; under submission.*

GIPSS features four and
MIPSS70+ *version 2.0* five risk categories.

What about prognostic scoring in PV/ET?

The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

Classification and Personalized Prognosis in Myeloproliferative Neoplasms

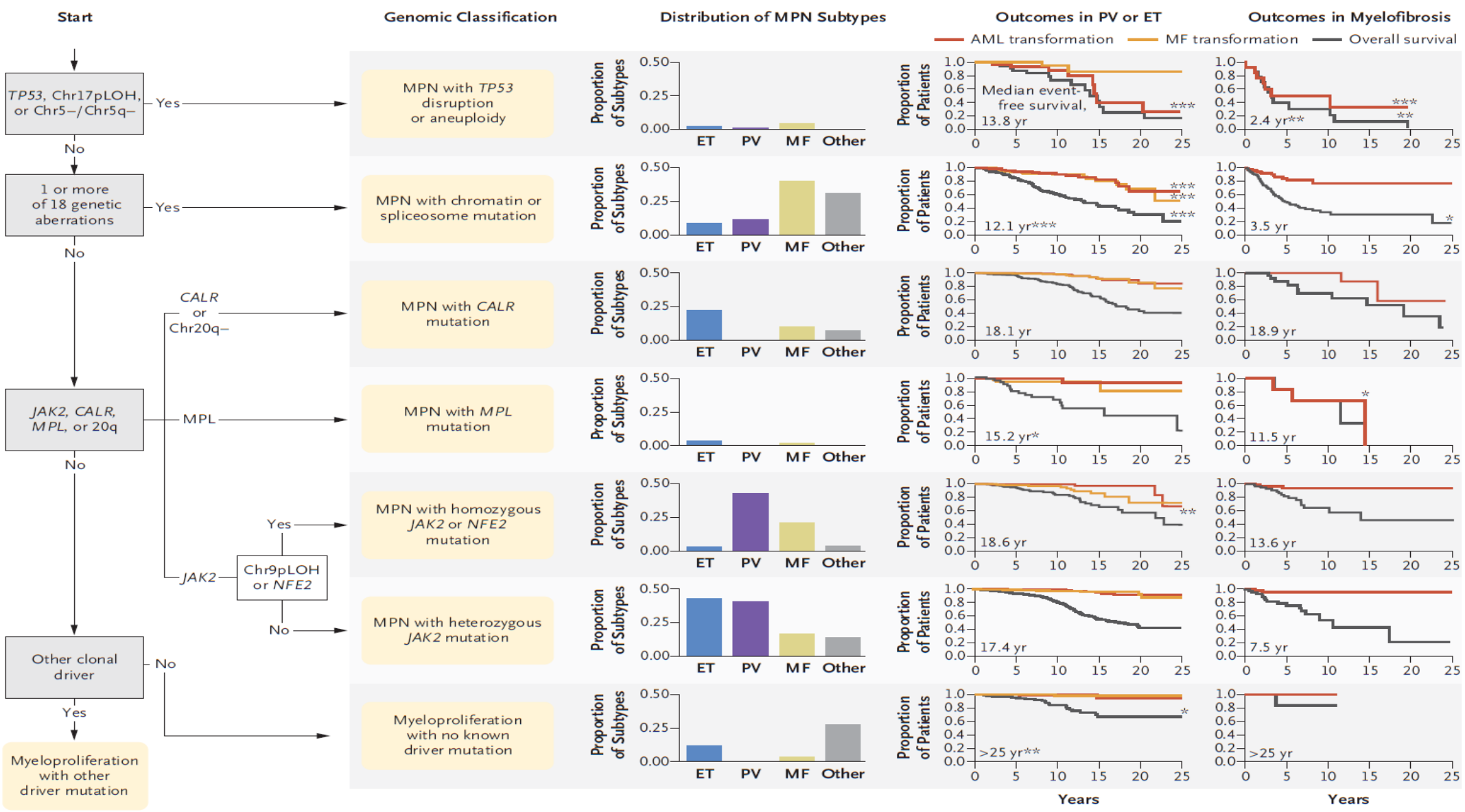
J. Grinfeld, J. Nangalia, E.J. Baxter, D.C. Wedge, N. Angelopoulos, R. Cantrill, A.L. Godfrey, E. Papaemmanuil, G. Gundem, C. MacLean, J. Cook, L. O'Neil, S. O'Meara, J.W. Teague, A.P. Butler, C.E. Massie, N. Williams, F.L. Nice, C.L. Andersen, H.C. Hasselbalch, P. Guglielmelli, M.F. McMullin, A.M. Vannucchi, C.N. Harrison, M. Gerstung, A.R. Green, and P.J. Campbell

N ENGL J MED 379;15 NEJM.ORG OCTOBER 11, 2018

Cohort of 2035 patients
- 1321 patients with ET
- 356 with PV
- 309 with MF
- 49 with other MPN

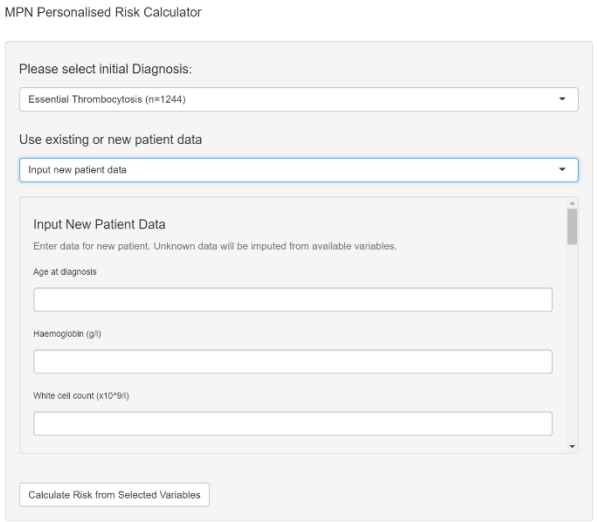
- Sequenced coding exons from 69 myeloid cancer genes in patients with myeloproliferative neoplasms, comprehensively annotating driver mutations and copy-number changes.
- Developed a genomic classification for myeloproliferative neoplasms and multistage prognostic models for predicting outcomes in individual patients.

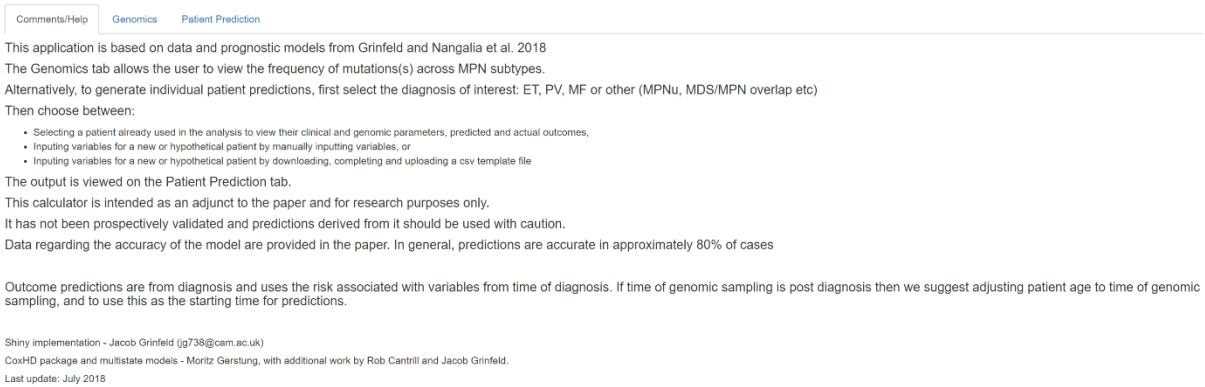
Genomic Subgroups in MPN and Phenotypic Characteristics

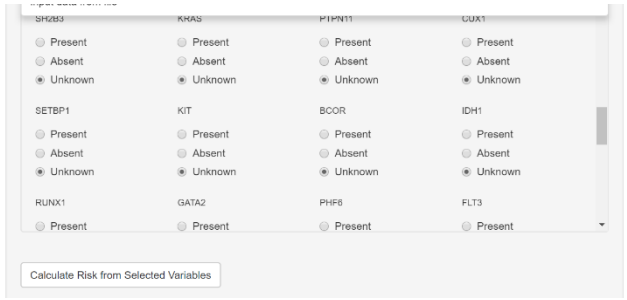


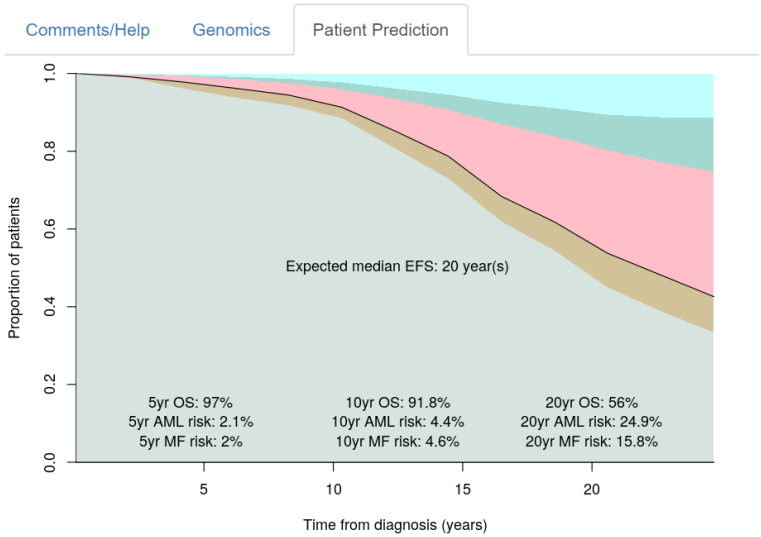
Grinfeld et al, NEJM 2018

https://cancer.sanger.ac.uk/mpn-multistage/









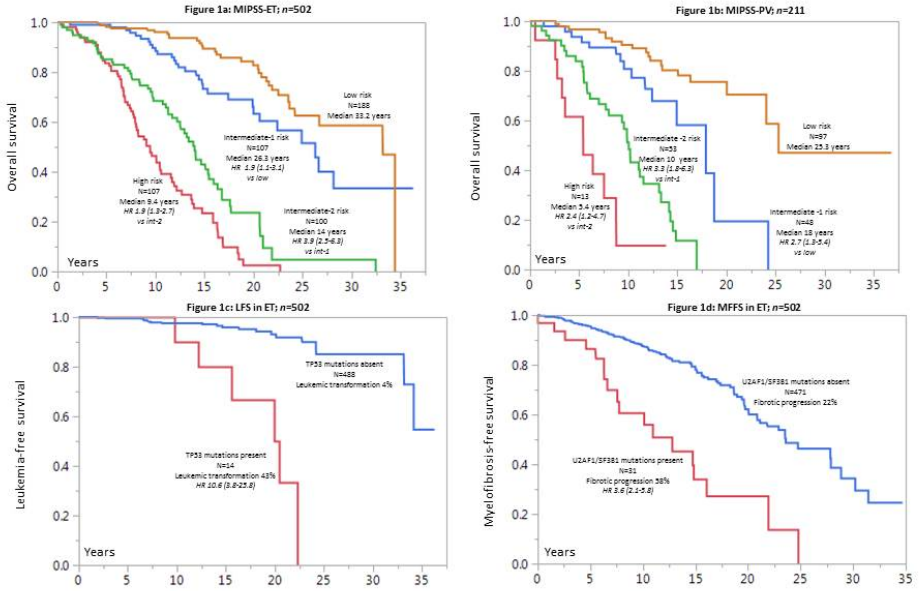
Mutation-Enhanced International Prognostic Systems for Essential Thrombocythemia (MIPSS-ET) and Polycythemia Vera (MIPSS-PV)

Ayalew Tefferi et al

- Spliceosome mutation information enhances survival prediction in ET and PV and identifies those at risk for fibrotic progression
- *TP53* mutations predict leukemic transformation in ET

Figure 1: Mutation-enhanced international prognostic scoring systems in essential thrombocythemia (MIPSS-ET; figure 1a) and polycythemia vera (MIPSS-PV; figure 1b). Analysis was based on a combined dataset of 713 cases, informative for all listed risk factors, from the Mayo Clinic , USA and University of Florence, Italy. Impact of mutations on leukemia-free (figure 1c) and myelofibrosis-free (figure 1d) survival in ET is also outlined.

ET survival risk factors: *SRSF2/SF3B1 mutations (2 points), age >60 years (4 points) and male sex (1 point) – low "0" points; intermediate-1 "1-2 points"; intermediate-2 "3-4 points"; high "≥5" points*
PV survival risk factors: *SRSF2 mutations (2 points), age >60 years (2 points), leukocyte count ≥11 x 10⁹/l (1 point) and abnormal karyotype (1 point) – low "0-1" points; intermediate-1 "2" points; intermediate-2 "3" points; high "≥4" points*



Abstract 578, ASH 2018

MPN Clinical Oral Abstracts: American Society of Hematology (ASH) 2018

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Interferon-alpha in MPN

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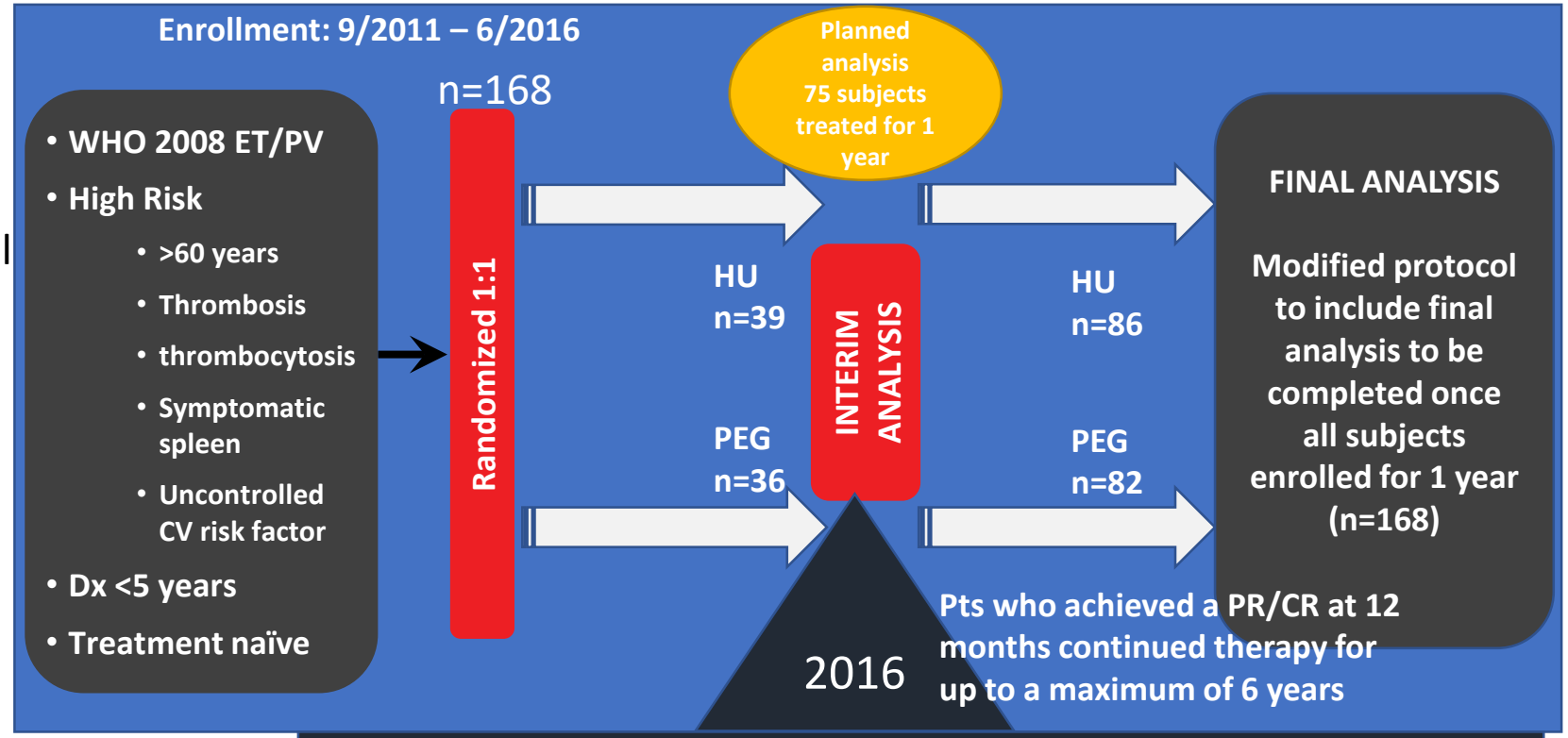
Results of the Myeloproliferative Neoplasms - Research Consortium (MPN-RC) 112 Randomized Trial of Pegylated Interferon Alfa-2a (PEG) Versus Hydroxyurea (HU) Therapy for the Treatment of High Risk PV and ET

John Mascarenhas et al

Randomized, open label, phase 3 clinical trial



- CR rate at 12 months was 33% (HU) and 28% (PEG); p=0.6
  - did not cross stopping boundary
- ORR was 69% (HU) and 81% (PEG)

Primary objective

- Compare the CR rate following HU vs. PEG at 12 months with 3 month confirmation.

Secondary objectives

- Toxicity and tolerability PR rates
- Incidence of specific pre-defined Toxicities and tolerance to therapy
- Impact of therapy on key biomarkers
- Survival and incidence of myelodysplastic syndrome, myelofibrosis, or leukemic transformation
- Incidence of major cardiovascular events.

Abstract 577, ASH 2018

WHO= World Health Organization
CR = Complete Response PR= Partical Response

CRITERIA FOR RESPONSE IN by Modified ELN criteria

ET

Complete response (CR)

- Platelet count ≤ 400 x 10^9/L AND
- No disease-related symptoms* AND
- Normal spleen size on imaging AND
- WBC ≤10 x 10^9/L

Partial response (PR)

In patients who do not meet criteria for complete response

Platelet count ≤600 x 10^9/L OR >50% reduction from baseline

No response (NR)

Any response that does not satisfy partial criteria

PV

Complete response (CR)

- Hematocrit ≤ 0.45 without phlebotomy AND
- Platelet count ≤ 400 x 10^9/L AND
- WBC ≤ 10 x 10^9/L AND
- Normal spleen size on imaging AND
- No disease related symptoms*

Partial response (PR)

In patients who do not meet criteria for complete response,

Hematocrit ≤45% without phlebotomy OR

response in any 3 of the remaining 4 criteria

No response (NR)

Any response that does not satisfy partial response

*Disease-related symptoms: microvascular disturbances, pruritus, headache

Barosi et al. Blood. 2009 May 14;113(20):4829-33

Abstract 577, ASH 2018

MPN-RC 112 Response:
12 MONTHS

Arm A (HU): 86 pts
PV: 44, ET: 42

168 pts randomized
Polycythemia Vera: 87
Essential Thrombocytopenia: 81

Arm B (PEG): 82 pts
PV: 43, ET: 39

Response at 12 months

	ET	PV	Total
CR	19 (45.2%)	13 (29.5%)	32 (37.2%)
PR	11 (26.2%)	17 (38.6%)	28 (32.6%)
NR	1	2	3
UE*	11 (3 **)	12 (3 **)	23 (6 **)
TOTAL	42	44	86

ORR= 69.8%

75.0% accounting for withdrawals

	ET	PV	Total
CR	17 (43.6%)	12 (27.9%)	29 (35.4%)
PR	10 (25.6%)	25 (58.1%)	35 (42.7%)
NR	3	2	5
UE*	9	4	13
TOTAL	39	43	82

ORR= 78.0%

Abstract 577, ASH 2018

24 Month Response Data

- When considering all 106 patients who were eligible to receive treatment for 24 months (due to study closure)

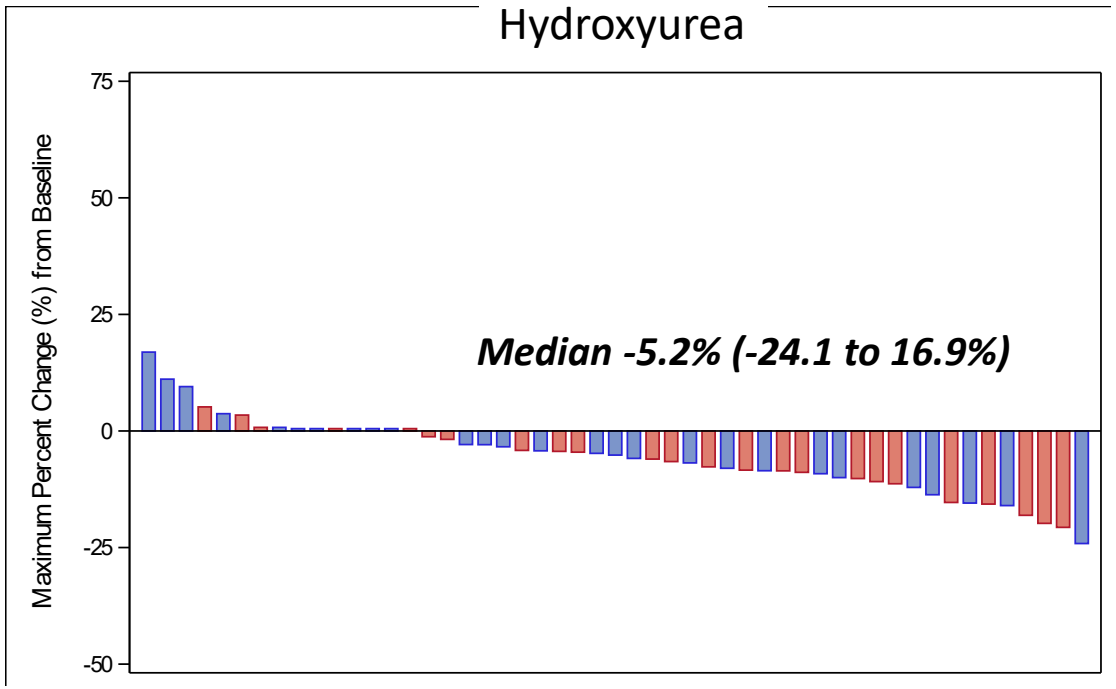
	HU (n=54)			PEG (n=52)		
	ET	**PV**	**Total**	**ET**	**PV**	**Total**
CR	6 (25%)	5 (16.7%)	11 (20.4%)	9 (37.5%)	7 (25%)	15 (28.8%)
PR	2 (8%)	9 (30%)	11 (20.4%)	5 (20.8%)	10 (35.7%)	16 (30.8%)
ORR	**8/24 (33.3%)**	**14/30 (46.7%)**	**22 /54 (40.7%)**	**14/24 (58.3%)**	**17/28 (60.7%)**	**31/52 (59.6%)**

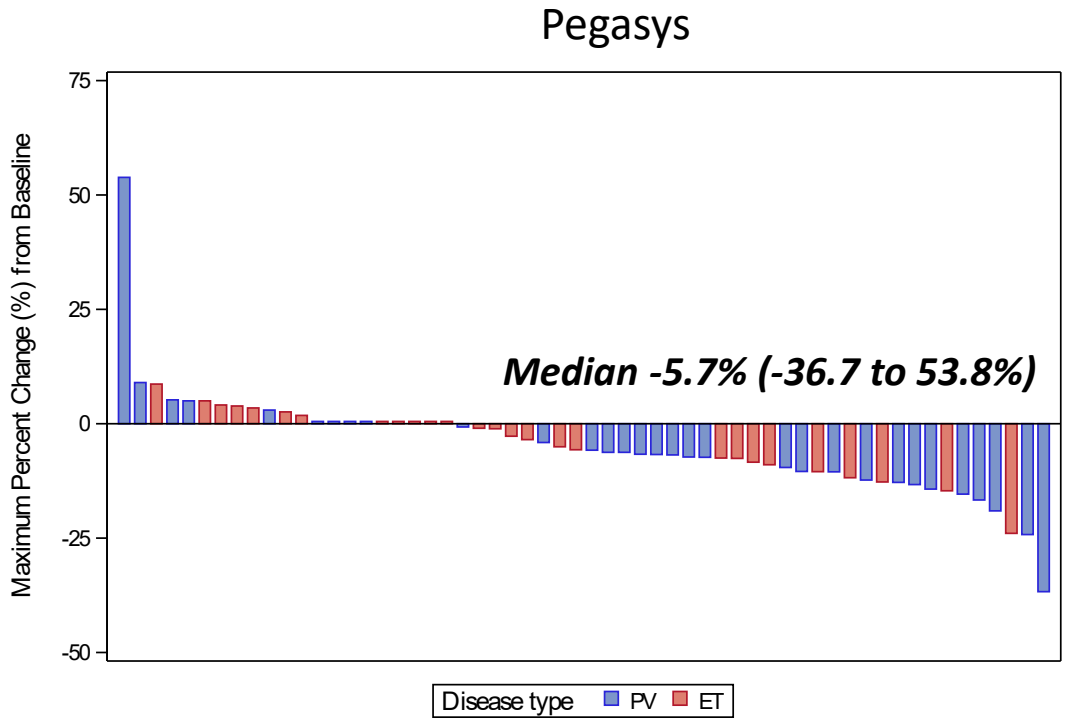
p=0.22

p=0.04

Abstract 577, ASH 2018

Spleen reduction by ultrasound





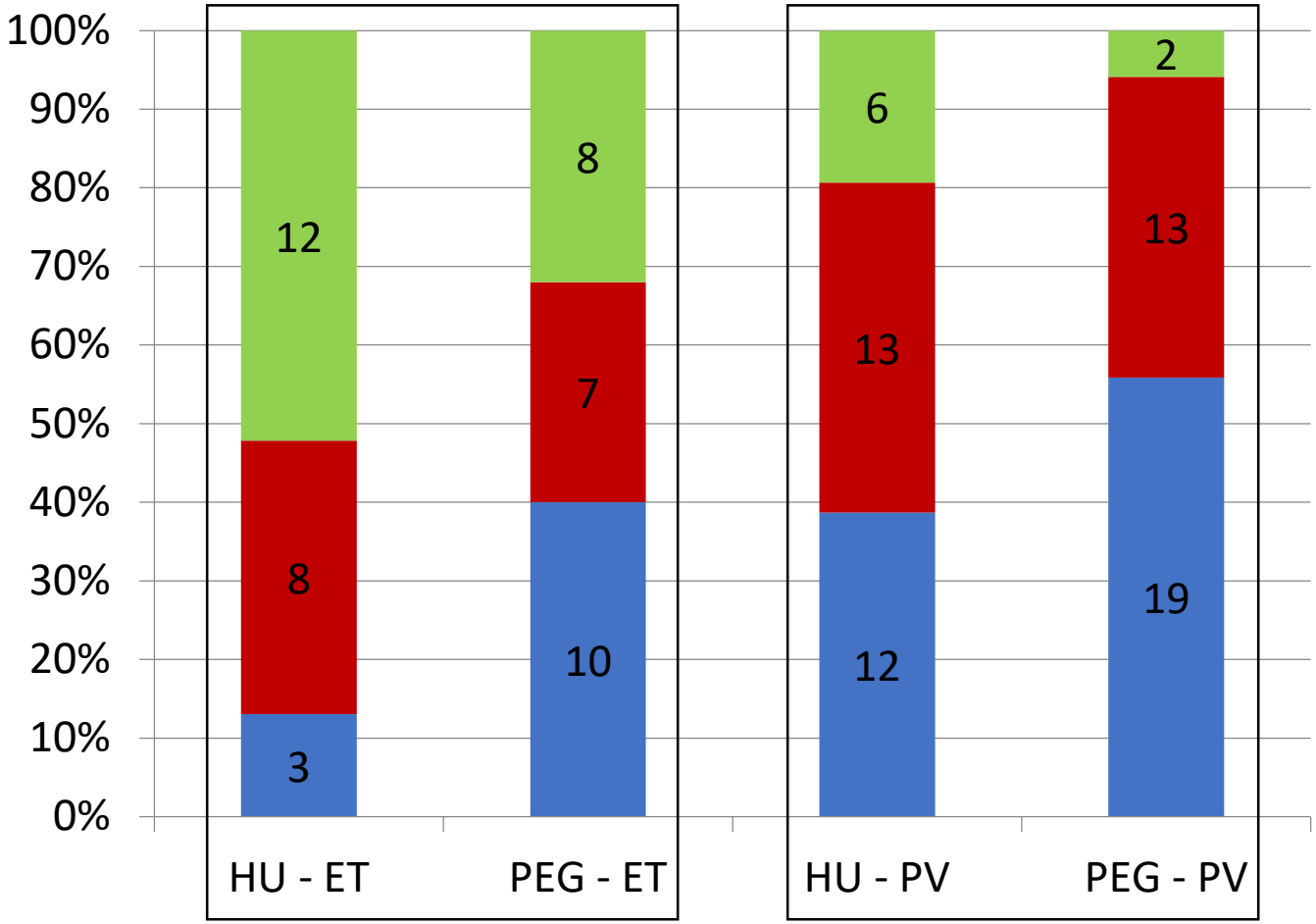
4/37 (10.8%) HU normalized spleen

6/36 (16.7%) PEG normalized spleen

In patients with spleen ≥ 13 cm at baseline:

Abstract 577, ASH 2018

Bone marrow response by Treatment Arm and Disease type Best Response (n=113)



CR

ET- disappearance of megakaryocyte hyperplasia
PV- presence of age-adjusted normocellularity and disappearance of trilinear hyperplasia

PR

ET- megakaryocyte hyperplasia reduced
PV- marrow cellularity and trilineage hyperplasia reduced
Reduction of MK and marrow cellularity by 30% but doesn't meet CR

NR

ET / PV- Does not satisfy partial histo-pathology remission

Adapted from ELN-IWG [Barosi et al. Blood. 2013 6;121(23):4778-81]

HU 18/54 (33.3%) vs. 10/59 (16.9%) for PEG, p=0.052

Abstract 577, ASH 2018

Summary of Grade 3/4 Adverse Events by Disease Strata

- Adverse event of Grade 3 or higher (any attribution): 60 pts (37.0%)
  - HU: 22 (27.5%)
  - PEG: 38 (46.3%)

	HU	PEG	Total
ET	12 (30.8%)	20 (51.3%)	32 (41.0%)
PV	10 (24.4%)	18 (41.9%)	28 (33.3%)
	22 (27.5%)	**38 (46.3%)**	**60 (37.0%)**

- Adverse event of Grade 4 or higher: 6 pts (3.7%)
  HU: n=4 (hyperuricemia, lung cancer, thrombocytopenia, sepsis)
  PEG: n=2 (hyperuricemia, dyspnea)

Abstract 577, ASH 2018

Conclusions

- Conducting independent randomized studies in MPN is challenging but necessary to establish optimal therapy
- No difference in hematologic CR between the two treatment arms at 12 and 24 months
- Toxicity is not a major reason for discontinuation in either arm
- BM pathologic responses appear more frequent in ET versus PV and no difference between treatment arms
- Meaningful differences in response and toxicity between these two agents over time were not observed and both agents appear to be effective therapies for treatment naïve ET/PV patients

Abstract 577, ASH 2018

Impact on MPN Symptoms and Quality of Life of Front Line Pegylated Interferon Alpha-2a Vs. Hydroxyurea in High Risk Polycythemia Vera and Essential Thrombocythemia

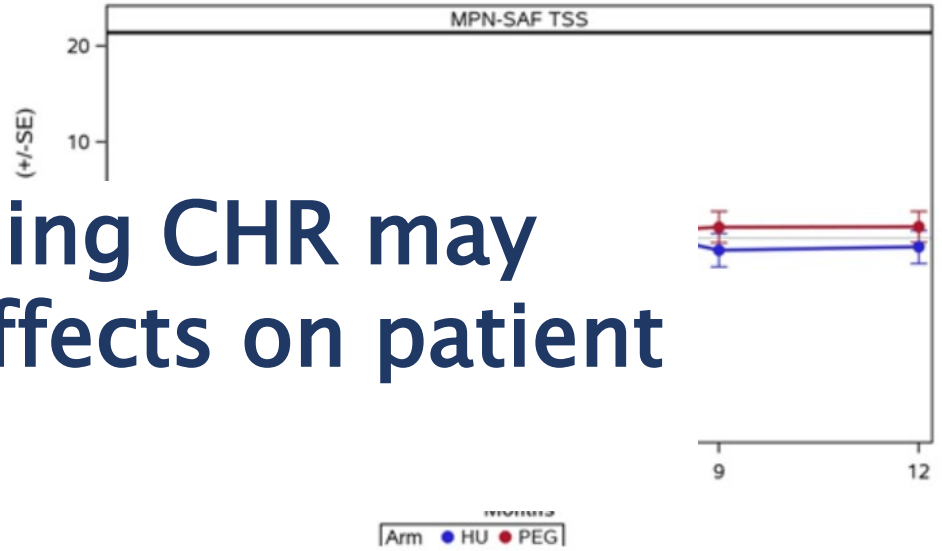
Ruben Mesa et al.

- On HU, pts experienced worsening QoL (physical, cognitive functioning. HRQoL) and some pe transient worsened s (inactivity, concentra

- On PEG, pts experien fever, dyspnea, appetite loss and PEG-related symptoms including flu-like symptoms, injection site irritation, blurry vision, and visual changes (all $p<0.05$), but not sad mood (not corrected for antidepressants).

Suggests obtaining CHR may have negative effects on patient symptoms

MPN-SAF TSS mean changes from baseline during treatment



Change in TSS significantly differed ($p=0.01$) between arms:

- Increasing symptoms on HU vs PEG at 3 and 6 mo
- Lower symptom burden on HU vs PEG at 9 and 12 mos

Abstract 3032, ASH 2018

Long-Term Efficacy and Safety of Recombinant Interferon Alpha-2 Vs. Hydroxyurea in Polycythemia Vera: Preliminary Results from the Three-Year Analysis of the Daliah Trial - a Randomized Controlled Phase III Clinical Trial

Trine Knudsen et al

- Examined the difference in efficacy and safety of low-dose r-IFNα in PV patients ≤ 60 or > 60 years of age compared to HU > 60 years of age.
- 90 newly diagnosed or previously phlebotomized PV patients only
- Patients ≤ 60 years were randomized (I:I) to r-IFNα-2a (Pegasys®) or to r-IFNα-2b (PegIntron®)
- Patients > 60 years were randomized (I:I:I) to either r-IFNα-2a, r-IFNα-2b or to HU
- Starting dose of r-IFNα-2a and r-IFNα-2b was 45 or 35 µg/week
- HU dose was 500 to 2000 mg/day.

Abstract 580, ASH 2018

Response Rates

- Overall Response Rate (ORR)
  - 68% (13/19) for HU
  - 42% (14/33) for r-IFNα ≤ 60 years
  - 39% (13/33) for r-IFNα > 60 years
- Partial Hematologic Remission (PHR)
  - 53% (10/19) for HU
  - 9% (3/33) for r-IFNα ≤ 60 years
  - 9% (3/33) for r-IFNα > 60 years
- Complete Hematologic Remission (CHR)
  - 16% (3/19) for HU
  - 33% (11/33) for r-IFNα ≤ 60 years
  - 30% (10/33) for r-IFNα > 60 years
- Maintenance of CHR
  - 11% (2/19) for HU
  - 21% (7/33) for r-IFNα ≤ 60 years
  - 18% (6/33) for r-IFNα > 60 years

Abstract 580, ASH 2018

Molecular Responses

47 JAK2^{V617F} positive patients were available for molecular response analysis after 36 months of therapy

- Partial Molecular Remission (PMR)
  - 21% (4/19) of HU treated patients
  - 24% (7/29) of r-IFNα treated patients ≤ 60 years
  - 18% (6/33) of r-IFNα > 60 years
- Complete Molecular Remission (CMR)
  - 7% (2/29) of the r-IFNα treated patients ≤ 60 years
- Median JAK2V617F reduction from baseline
  - 38% (31-63%) for HU
  - 79% (59-92%) for r-IFNα ≤ 60 years
  - 73% (49-97%) for r-IFNα > 60 years

Comparable Adverse Events (AE's) HU vs PEG

Discontinuation of treatment for any reason after 36 months of therapy

- 21% (4/19) for HU
- 52% (17/33) for r-IFNα ≤ 60 years
- 45% (15/33) for r-IFNα > 60 years

Toxicity related discontinuation

- 5% (1/19) for HU
- 30% (10/33) for both r-IFNα ≤ 60 and > 60 years

Grade 3-4 AEs

32% (6/19) of HU treated patients

27% (9/33) in r-IFNα treated patients ≤ 60 years

42% (14/33) r-IFNα treated patients > 60 years

Serious Adverse Events (SAEs)

21% (4/19) for HU

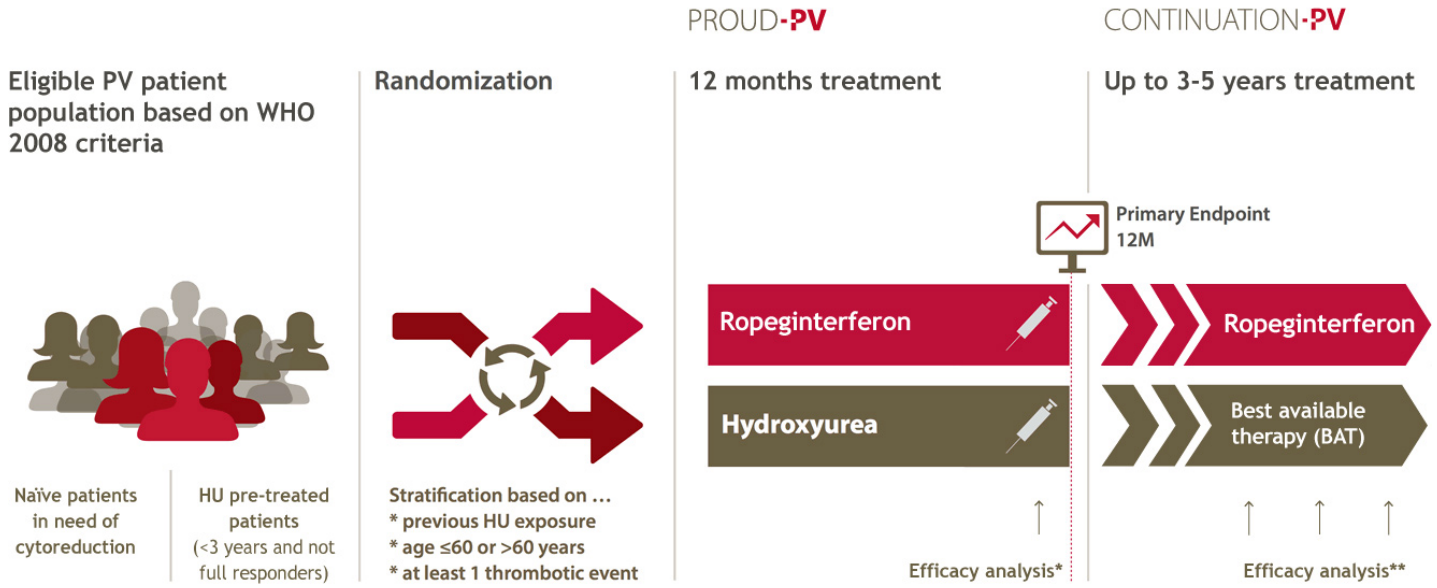
9% (3/33) for r-IFNα ≤ 60 years

24% (8/33) for r-IFNα > 60 years.

Abstract 580, ASH 2018

Evidence for Superior Efficacy and Disease Modification after Three Years of Prospective Randomized Controlled Treatment of Polycythemia Vera Patients with Ropeginterferon Alfa-2b Vs. HU/Best Available Therapy (BAT)

Heinz Gisslinger et al



*) non-inferiority: Hematologic Response

**) benefit: durable Hematologic Response, Progression Free Survival (PFS), PV symptom relief

Primary Outcome Measures / Primary Endpoints

- Disease response rate* at 12 months

** defined as hematocrit <45% without phlebotomy (at least 3 months since last phlebotomy), platelets <400 G/L, leukocytes <10 G/L , and normal spleen size*

Secondary Outcome Measures / Secondary Endpoints

- Disease response rate at 3, 6 and 9 months
- JAK2 allelic burden changes
- Time to response
- Duration of response
- Number of phlebotomies
- Blood parameters
- Spleen size
- Disease related symptoms
- Adverse events
- Protocol-specific adverse events of special interest

Abstract 579, ASH 2018

PROUD-PV Results

83 (Ropeg) and 70 (HU/BAT) patients completed the 36-month efficacy analysis time point

CHR

- Ropeg 70.5% vs HU/BAT 51.4%
  p=0.0122; RR [95% CI]: 1.38 [1.07-1.79]

CHR plus symptom improvement

- Ropeg 52.6% vs. HU/BAT 37.8%;
  p=0.0437; RR [95% CI]: 1.42 [1.01-2.00]

Abstract 579, ASH 2018

PROUD-PV Safety

Comparable numbers of patients experienced adverse events

- 89.8% for Ropeg
- 90.6% for HU

Treatment-related adverse events

- 74.8% for Ropeg
- 78.7% for HU

The most common (>10%) treatment-related adverse events anemia, thrombocytopenia and leukopenia occurred more frequently under HU, whereas GGT increase was mainly observed under Ropeg. No new safety signals appeared in the third year of treatment.

PROUD-PV molecular responses

*JAK2*V617F molecular response:

- 66.0% on Ropeg vs 27.0% on HU/BAT ($p<0.0001$; RR [95% CI]: 2.31 [1.56-3.42])
- MR strongly correlated with CHR

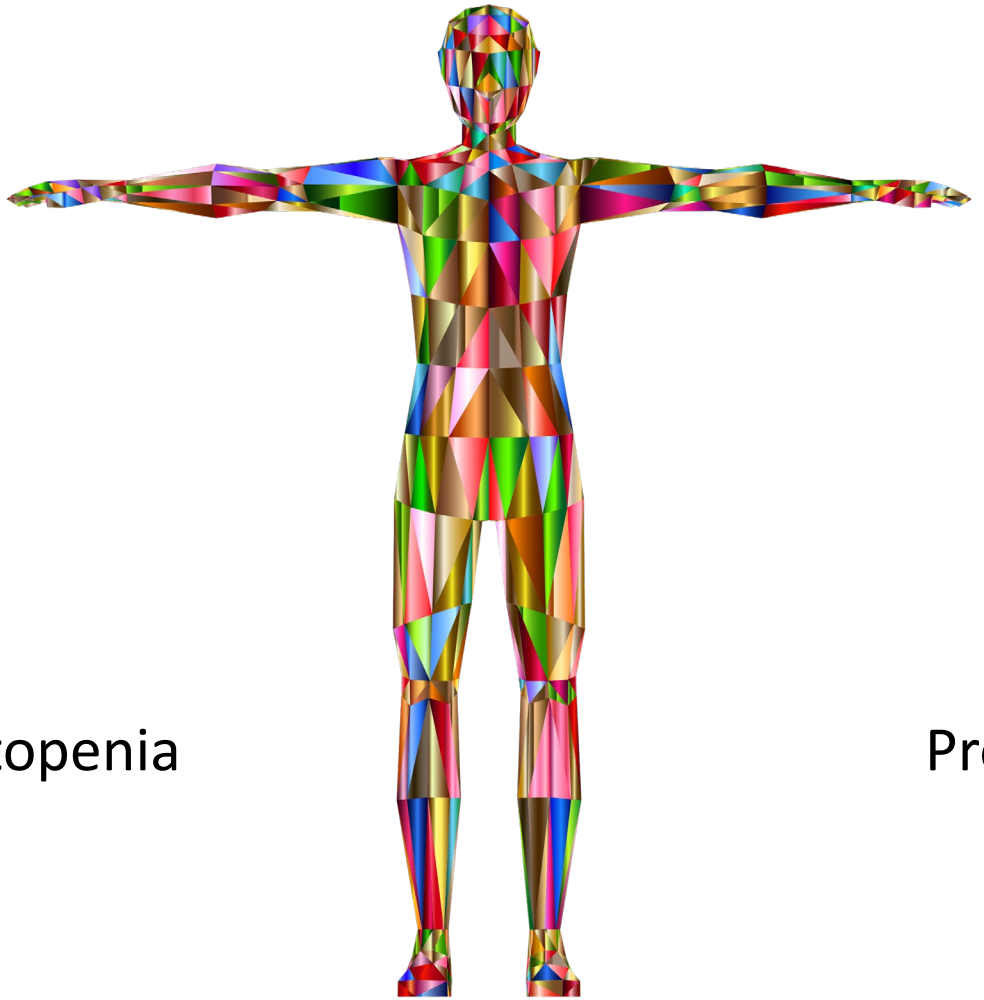
Ropeg was found to reduce non-JAK2V617F mutations such as TET2 burden in some patients, HU was not

Abstract 579, ASH 2018

Approach to Treating Myelofibrosis

Fatigue

ruxolitinib



Splenomegaly

ruxolitinib

Anemia, thrombocytopenia

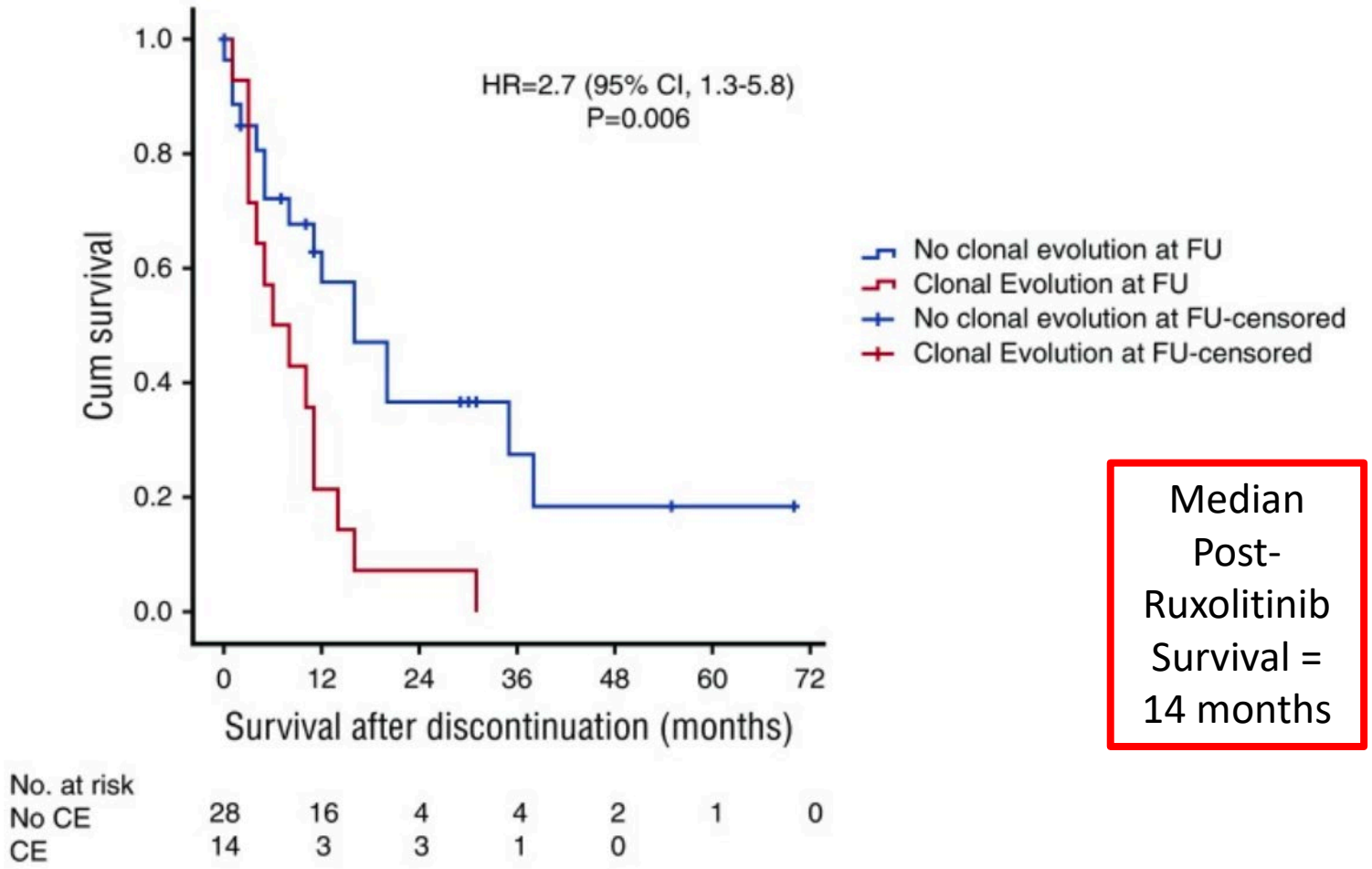
Supportive care

Danazol

Progression to AML

transplant

Survival After Ruxolitinib Discontinuation



Median Post-Ruxolitinib Survival = 14 months

Blood. 2017 Aug 31; 130(9): 1125–1131.

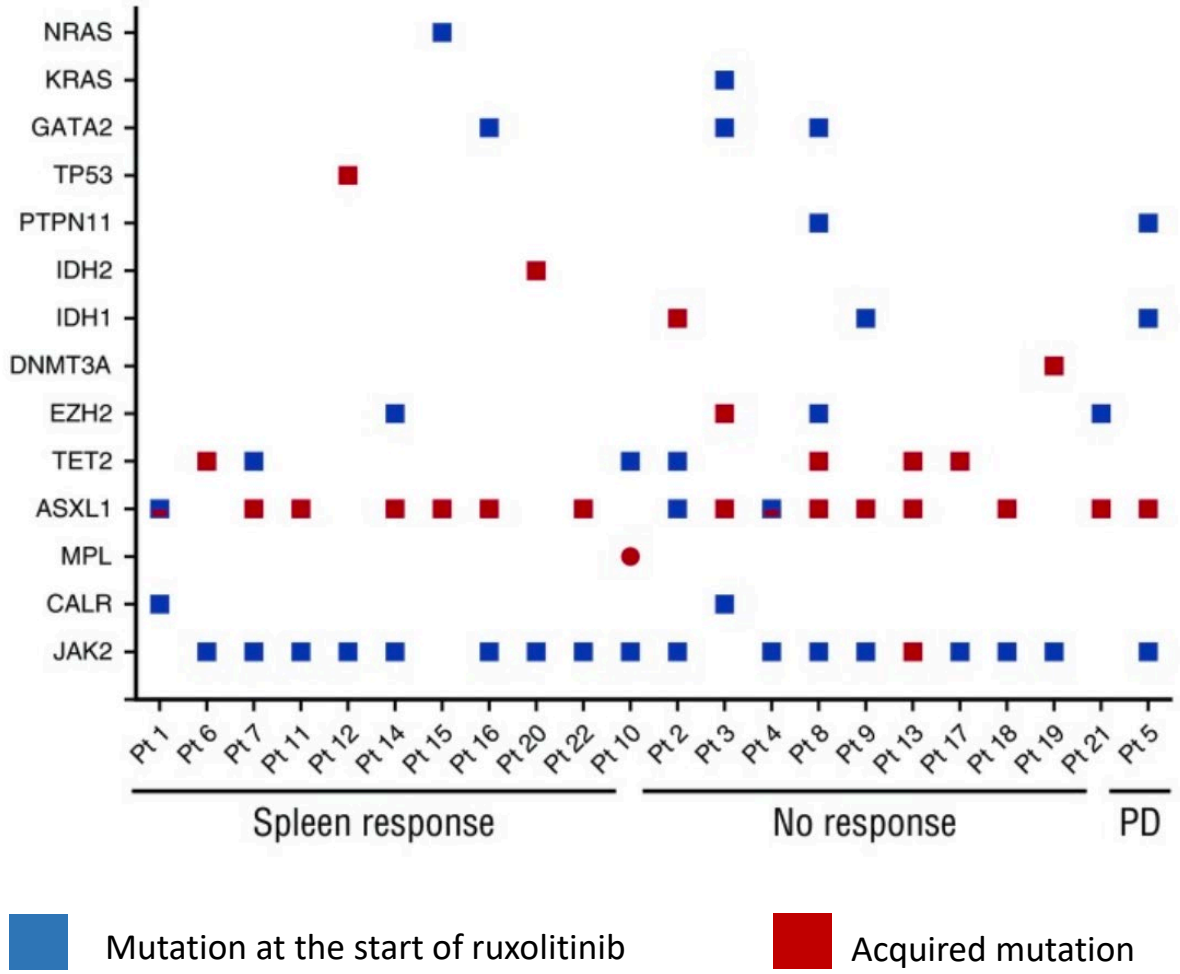
Mutations During Ruxolitinib Treatment

62 paired samples

-beginning of ruxolitinib

-ruxolitinib discontination

Average time on rux:
13.1 mo



22 out of 62 acquired new mutations while on rux

-most frequent in ASXL1, TET2, EZH2, TP53

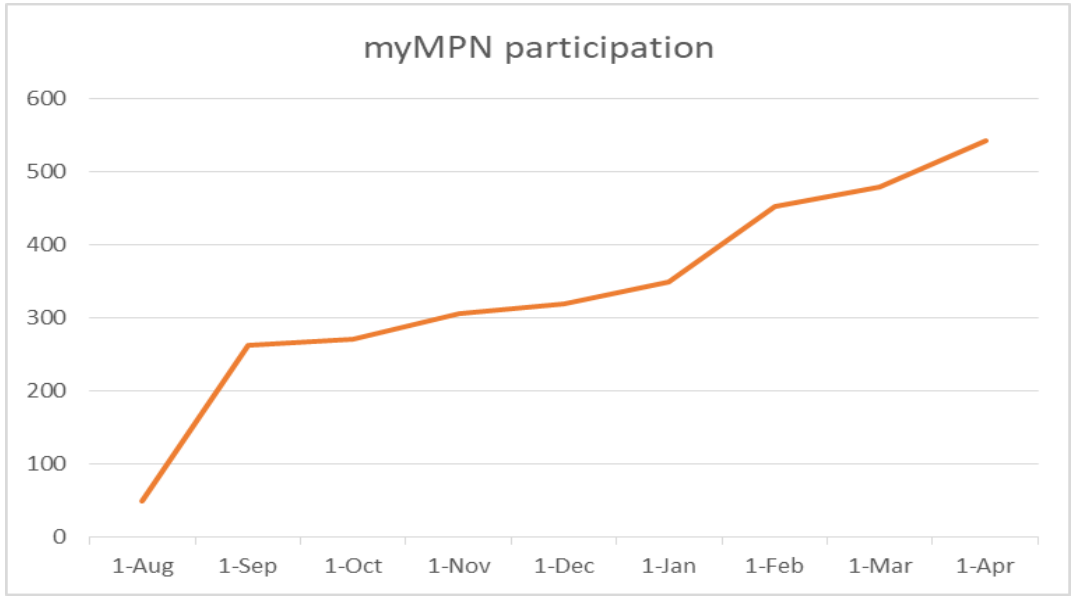
-also found 1 new MPL mutation

Blood. 2017 Aug 31; 130(9): 1125–1131.

My MPN Registry (Mobile MPN Monitoring): A Tool for People With PV, ET, or MF

www.mympn.org

- A digital hub for patients to record and anonymously share their unique MPN journey with the research community
- Registrants can access a secure online portal with a personalized dashboard; as they complete surveys listed on the dashboard, the registry will provide insights into how the user's MPN experience compares to other registry users
- All patient data is protected and only shared according to individual user privacy settings
- A place for eligible patients to connect with upcoming drug trials and research that will help increase our knowledge about PV, ET, and MF



ASH 2018. Abstract ID: #119033