Pipeline for ET and MF

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Conflict of Interest Disclosures

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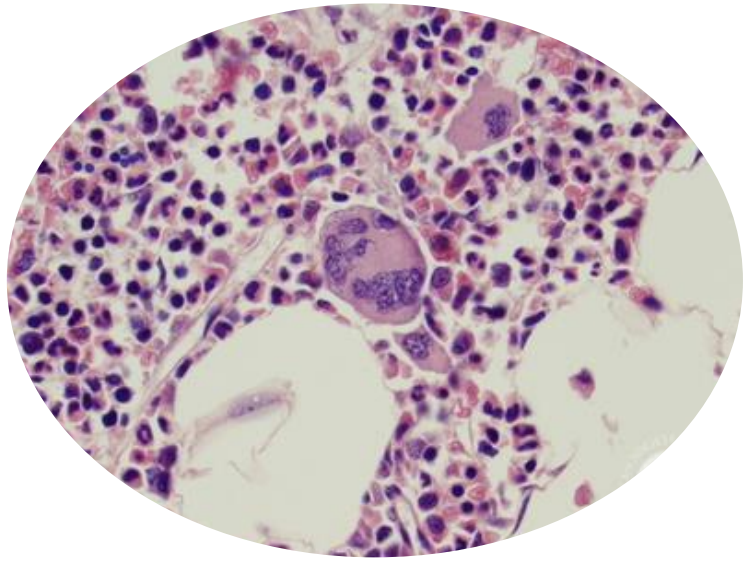
Advisory role:

AbbVie, AOP, BMS, Celgene, CTI, IMAGO, Novartis, Galacteo, Geron, Gilead, GSK, Janssen, Keros, Promedior, Roche, Shire.

ET: simple yet also complex and confusing:



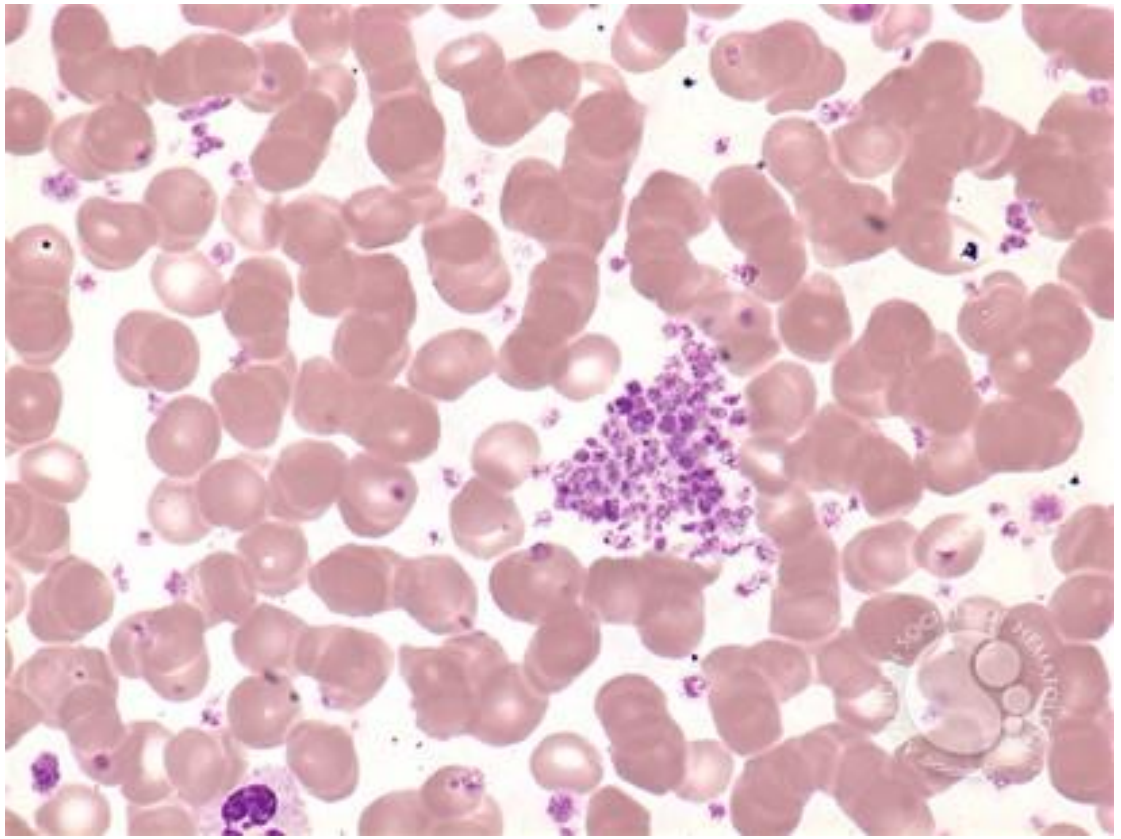
Message even re simple therapy (aspirin) is confused !

Should *CALR*-ET, triple negative ET be treated the same as *JAK2*- or *cMPL*-ET?

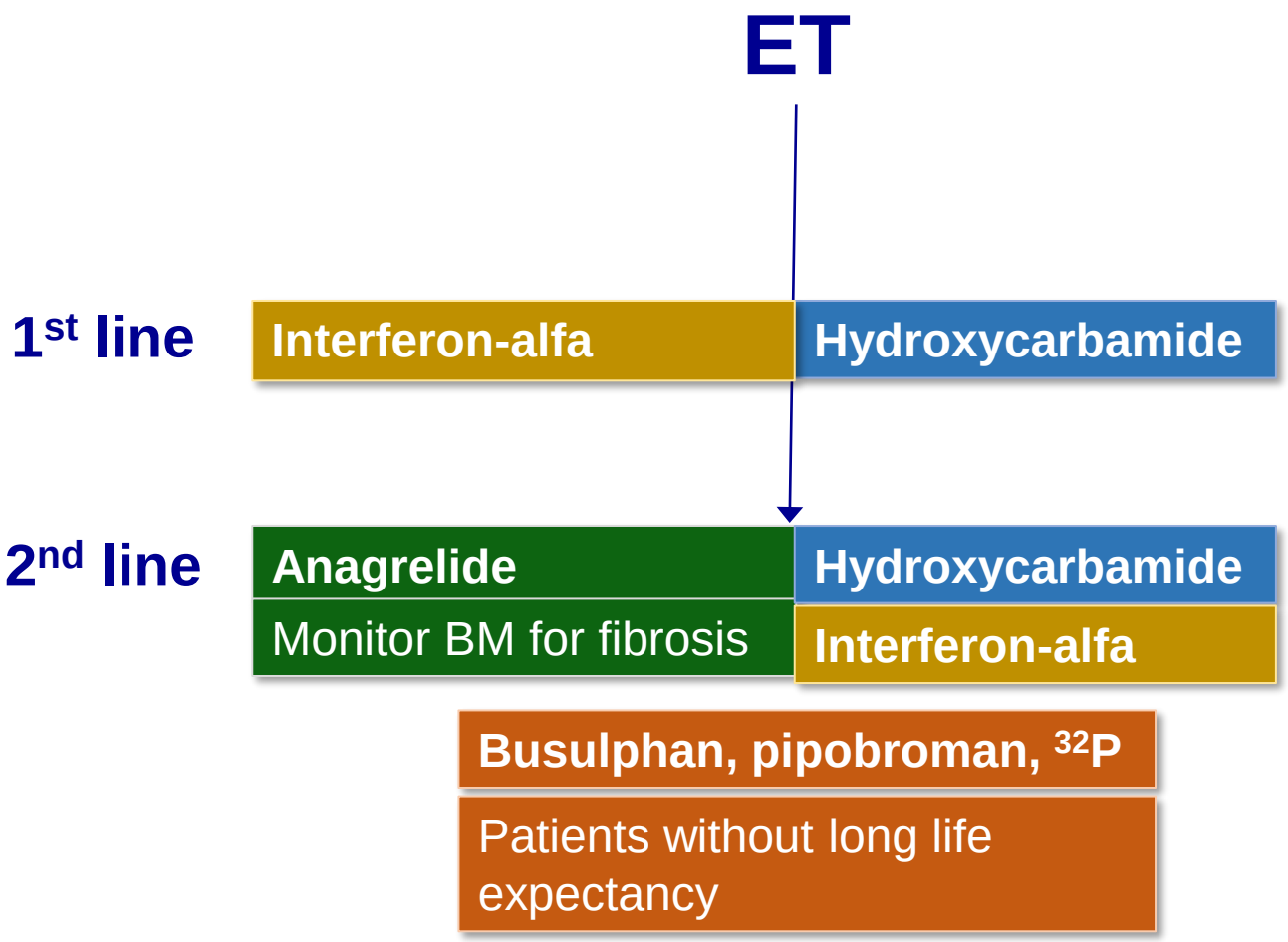
Should *JAK2*-ET be managed as PV?

Should we treat triple negative ET at all?

Essential thrombocythaemia:



Cytoreduction in high-risk ET simple but confusing:



In addition:

Currently unclear if we should really treat CALR-ET and triple negative ET in the same way as JAK2- or cMPL-ET

IPSET score defines most JAK negative ET as intermediate risk despite count and age

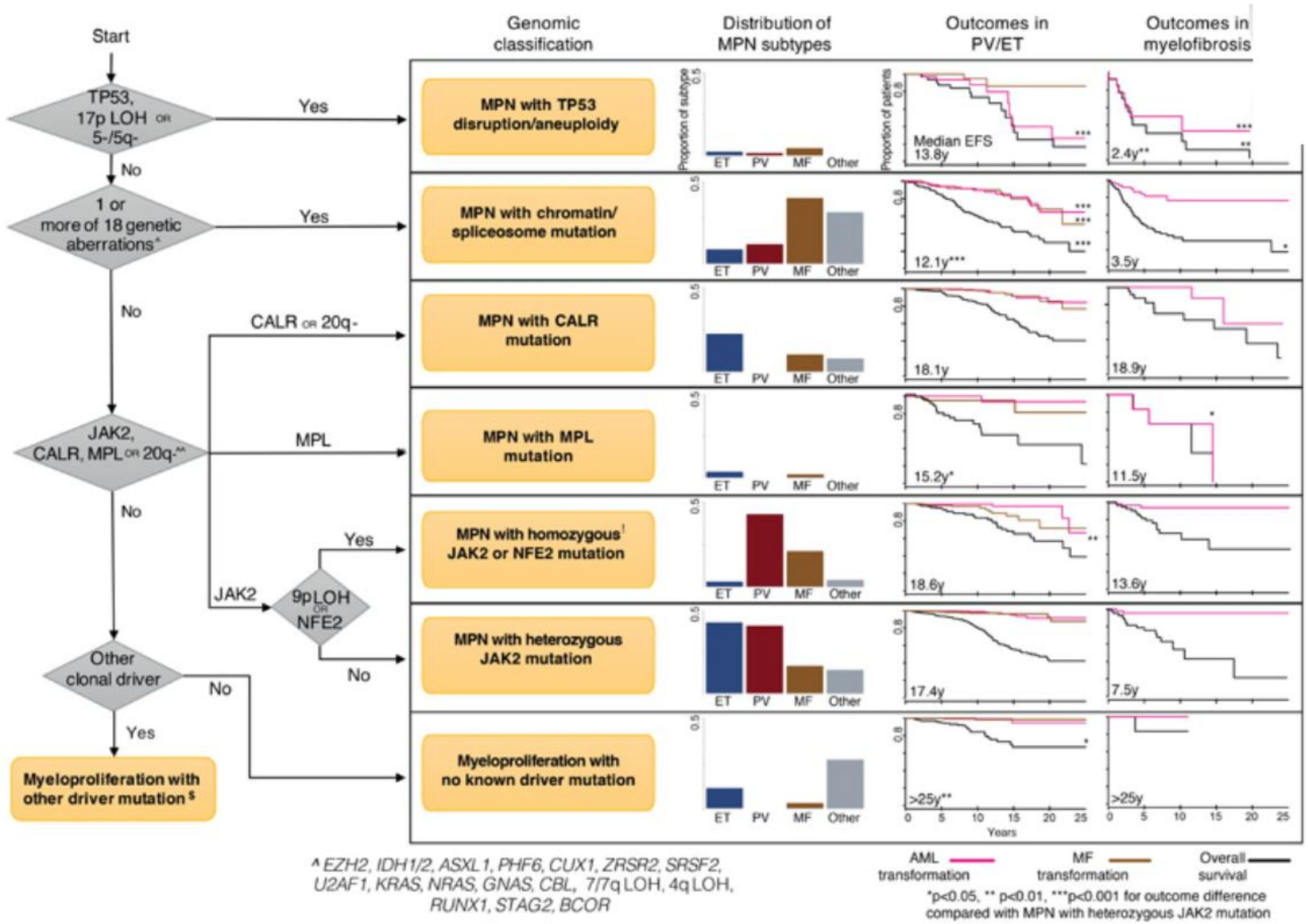
Should JAK2-ET be managed as PV?

The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

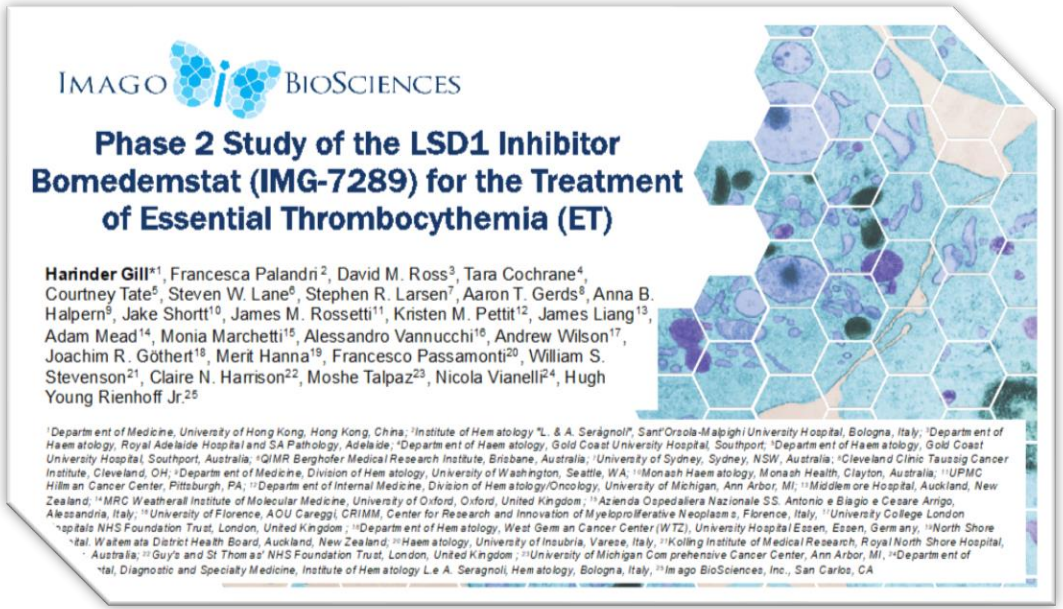
Classification and Personalized Prognosis in Myeloproliferative Neoplasms

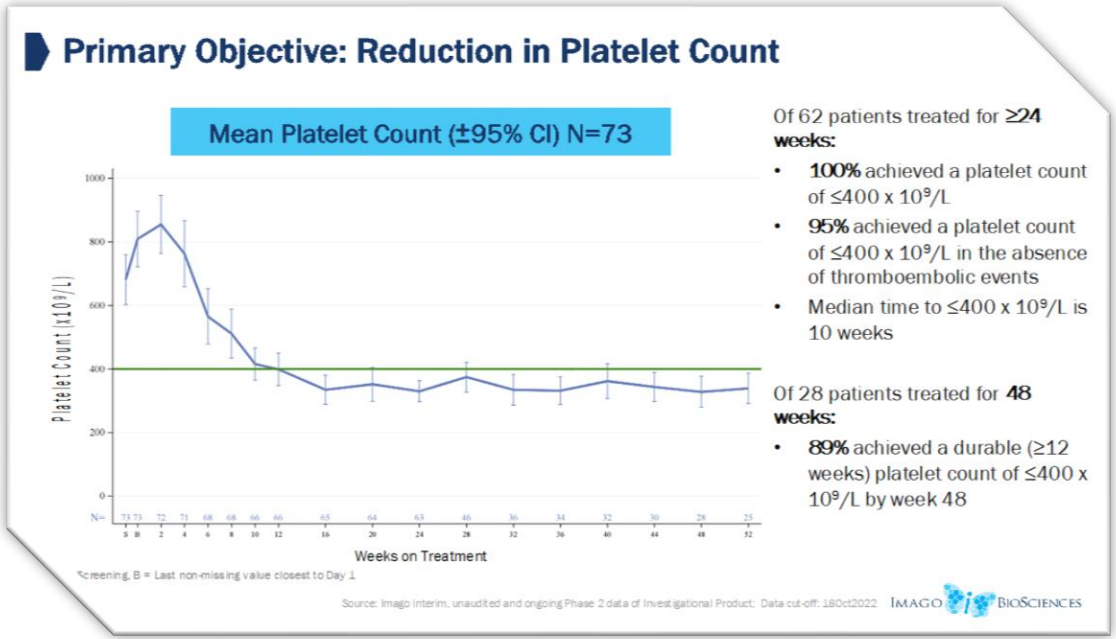
J. Grinfeld, J. Nangalia, E.J. Baxter, D.C. Wedge, N. Angelopoulos, R. Cantrill, A.L. Godfrey, E. Papaemmanuil, G. Gundem, C. MacLean, J. Cook, L. O'Neil, S. O'Meara, J.W. Teague, A.P. Butler, C.E. Massie, N. Williams, F.L. Nice, C.L. Andersen, H.C. Hasselbalch, P. Guglielmelli, M.F. McMullin, A.M. Vannucchi,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



New options in ET?

- Clinical trials with the **LSD1 inhibitor bomedemstat** – ALSO BEING EVALUATED IN PV, upfront and second line ET trials have begun





- Effective regardless of driver mutation

Response in the Initial 24 Week Treatment Period

	Bomedemstat N = 72[a]
Week 4	
Patients with assessment results at visit, n	71
Responders, n (%) [95% CI]	8 (11) [5.0-21.0]
Week 8	
Patients with assessment results at visit, n	68
Responders, n (%) [95% CI]	28 (41) [29.4-53.8]
Week 12	
Patients with assessment results at visit, n	68
Responders, n (%) [95% CI]	40 (59) [46.2-70.6]
Week 16	
Patients with assessment results at visit, n	65
Responders, n (%) [95% CI]	49 (75) [63.1-85.2]
Week 20	
Patients with assessment results at visit, n	63
Responders, n (%) [95% CI]	41 (65) [52.0-76.7]
Week 24	
Patients with assessment results at visit, n	64
Responders, n (%) [95% CI]	49 (77) [64.3-86.2]
P value	<0.0001

At week 24, **77% of patients had a response**, defined as a reduction in platelet count to ≤400 × 10^9/L with no new thromboembolic events

Data cutoff date: May 03, 2023.

[a]72 patients who received ≥1 dose of bomedemstat and had a baseline and ≥1 postbaseline assessment available were included in the efficacy analysis population.

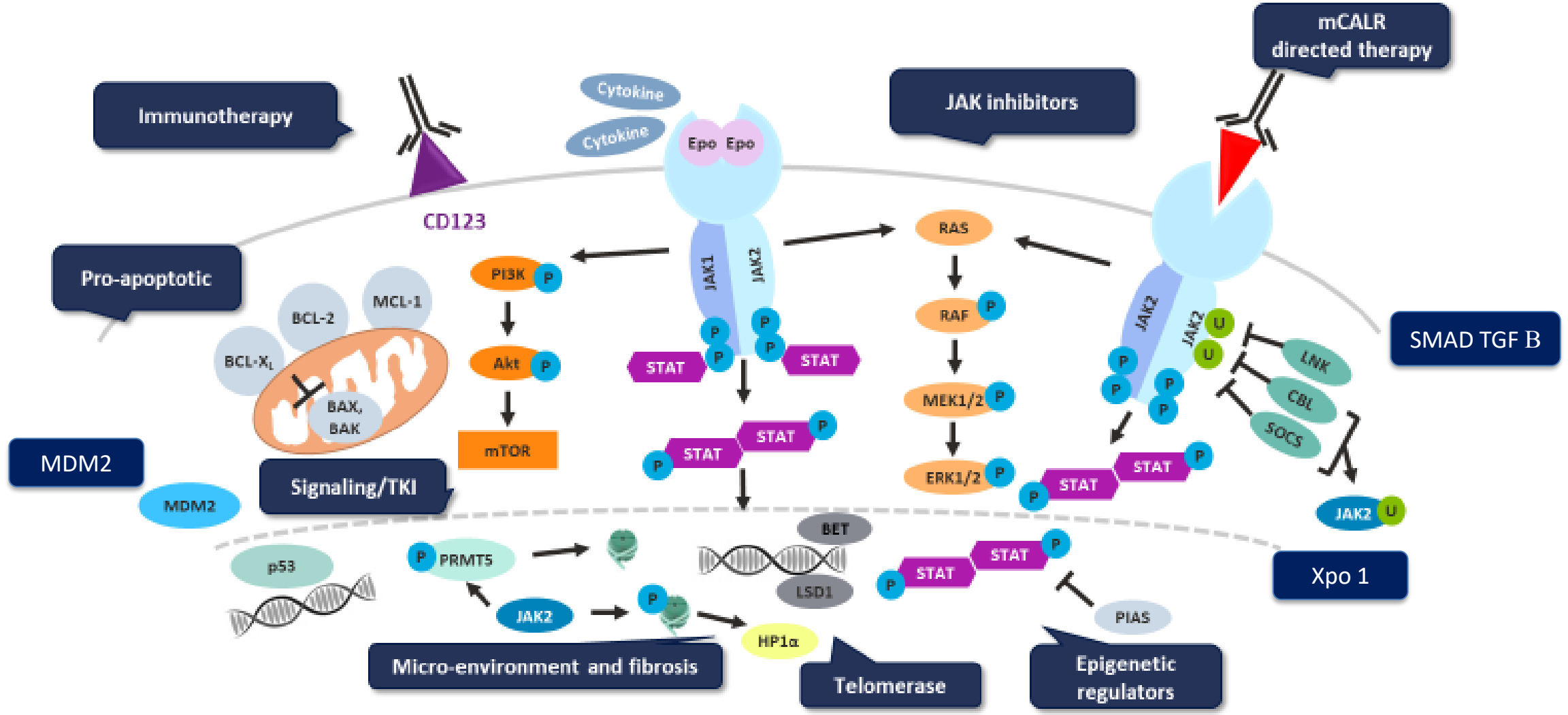
Pelabresib (CPI-0610) Monotherapy in Patients With High-Risk Essential Thrombocythemia Refractory or Intolerant to Hydroxyurea: Preliminary Results From the MANIFEST Study

Francesco Passamonti,[1] Andrea Patriarca,[2] Steven Knapper,[3] Candido Rivera,[4] Joseph M Scandura,[5] Timothy Devos,[6] Nikki Granacher,[7] Adam Mead,[8] Stephen Oh,[9] Jeanne Palmer,[10] Raajit K Rampal,[11] Lino Teichmann,[12] Qing Li,[13] Jean-Pierre Eliane,[13] Tzuu-Wang Chang,[13] Sandra Klein,[13] Gozde Colak,[13] Claire Harrison[14], *on behalf of the MANIFEST study investigators*

[1]Università degli Studi di Milano; Fondazione I.R.C.C.S. Ca' Granda Ospedale Maggiore Policlinico, Milano, Italy; [2]Hematology Unit, Department of Translational Medicine, University of Eastern Piedmont and AOU Maggiore della Carità, Novara, Italy; [3]Department of Haematology, Division of Cancer and Genetics, School of Medicine, Cardiff University, Cardiff, Wales, UK; [4]Mayo Clinic Florida, Jacksonville, FL, USA; [5]The Richard T. Silver, M.D. Myeloproliferative Neoplasms Center, Division of Hematology and Oncology, Cornell Medicine, New York, NY, USA; [6]Department of Hematology, University Hospitals Leuven and Laboratory of Molecular Immunology (Rega Institute), KU Leuven, Leuven, Belgium; [7]Ziekenhuis Netwerk Antwerpen, Antwerp, Belgium; [8]MRC Molecular Haematology Unit, MRC Weatherall Institute of Molecular Medicine, University of Oxford, John Radcliffe Hospital, Oxford, UK; [9]Washington University School of Medicine in St. Louis, St. Louis, MO, USA; [10]Mayo Clinic Arizona, Phoenix, AZ, USA; [11]Memorial Sloan Kettering Cancer Center, New York, NY, USA; [12]Universitätsklinikum Bonn, Bonn, Germany; [13]Constellation Pharmaceuticals, Inc., a MorphoSys Company, Boston, MA, USA; [14]Guy's and St Thomas' NHS Foundation Trust, London, UK.

Presentation Number S168

Future perspectives in the treatment of MF..



TKI, tyrosine kinase inhibitor.

Adapted from: Daver N & Assi R. *Oncol Hematol Rev* 2016; **12**:71–74; McLornan DP & Harrison CN. *Br J Haematol* 2020; **191**:21–36; Schieber M, *et al. Blood Cancer J* 2019; **9**:74; Tremblay D & Mascarenhas J. *Cells* 2021; **10**:1034.