MPN Clinical Trial Pipeline

MYELOFIBROSIS (MF)

Phase	Drug (Sponsor)	Trial Name (if available) and Notes
3	Fedratinib (BMS) NCT03755518	FREEDOM - High or intermediate risk patients previously treated with Ruxolitinib. Sub-study is combination with Luspatercept for anemia
Fedratinib was approved for use by the FDA in August 2019 in MF. Current trials of Fedratinib are for further exploration of its efficacy, dosing and long-term impact		
3	Pacritinib (CTI Biopharma) NCT03165734	PACIFICA - For patients with very low platelets (<50,000) who have had no or limited exposure to a JAK2 inhibitor
3	Parsaclisib plus Ruxolitinib (Incyte) NCT04551066	LIMBER-313 - New PI3 Kinase inhibitor investigational drug for patients who have not previously used JAK inhibitor or PI3 Kinase Inhibitor
3	Parsaclisib plus Ruxolitinib (Incyte) NCT04551053	LIMBER-304 - PI3 Kinase inhibitor for patients on a stable dose of Ruxolitinib needing improvement of response
3	Navitoclax (AbbVie) NCT04472598	TRANSFORM-1 - Investigational drug that focuses on cell death pathway to be used in combination with Ruxolitinib vs Ruxolitinib, alone, for JAK2 inhibitor and BH3 mimetic naïve patients, only
3	Navitoclax (AbbVie) NCT04468984	TRANSFORM-2 - Investigational drug that focuses on cell death pathway in combination with Ruxolitinib vs best available therapy for patients who failed Ruxolitinib previously or it stopped working for them
3	Pelabresib (Constellation Pharmaceuticals) NCT04603495	MANIFEST-2 - Investigational BET-inhibitor trial enrolling patients without prior JAK inhibitor experience ("JAK naïve")
3	Imetelstat (Geron) NCT04576156	IMpactMF - Investigational drug focused on overall survival in high-risk patients who have failed previous therapy
3	Luspatercept (Acceleron/BMS) NCT04717414	INDEPENDENCE - Study for patients on a JAK2 inhibitor therapy who require transfusions

3	Luspatercept (Acceleron/BMS) NCT04064060	Continuation/rollover study to evaluate the long-term safety of Luspatercept in patients previously on it
3	KRT-232 (Kartos) NCT03662126	BOREAS - New MDM2 inhibitor drug for higher risk patients who have failed a JAK inhibitor
2	Pelabresib (Constellation Pharmaceuticals) NCT02158858	MANIFEST-1 - New investigational drug based on BET-inhibition - trial enrolling patients without prior JAK inhibitor experience ("JAK naïve")
2	Thalidomide plus Ruxolitinib (MSKCC) NCT03069326	Addition of Thalidomide to patients responding sub optimally or progressing on Ruxolitinib
2	Bomedemstat (Imago BioSciences) NCT03136185	New investigational drug for intermediate or high-risk patients focused on inhibiting LSD1 for patients that have failed one standard therapy. Trial focuses on safety and lowering platelets
2	Tagraxofusp (Stemline Therapeutics) NCT02268253	Previously approved therapy for a rare blood cancer, BPDCN. This trial is for higher risk MF patients for whom a previous therapy was unsuccessful
2	Luspatercept (BMS/Acceleron) NCT03194542	Drug approved for MDS; this trial is for MF patients with anemia with or without transfusion dependence.
2	Navitoclax (AbbVie) NCT03222609	REFINE - Investigational drug for intermediate- or high-risk patients given alone or with Ruxolitinib
2	Ruxolitinib Phosphate (MD Anderson) NCT01787487	Combination with azacytidine for high-risk patients who have been previously treated or newly diagnosed
2	Ruxolitinib plus Enasidenib (Mt. Sinai) NCT04281498	For chronic MF or blast phase MPN patients with IDH2 mutation
2	Selinexor (Karyopharm Therapeutics) NCT04562870	Previously approved for lymphoma. This trial is for higher risk patients who have failed Ruxolitinib

2	Elotuzumab (MD Anderson) NCT04517851	Trial in patients with JAK2-mutated MF to see if Elotuzumab (previously approved for myeloma) may help to control disease and/or help to improve blood cell count and bone marrow function
2	9-ING-41 (Actuate) NCT04218071	Anti-cancer and anti-fibrotic investigational drug given alone or combined with Ruxolitinib in advanced and poor prognosis patients
2	GB2064 (Galecto) NCT04679870	Study of investigational orally administered inhibitor of LOXL2 for intermediate 2 or high-risk MF patients not currently on a JAK inhibitor
2	TL-895 (Telios) NCT04655118	An orally-administered investigational tyrosine kinase inhibitor for participants who have failed prior therapy, who are intolerant or ineligible to receive JAK inhibitor treatment
2	Itacitinib (Incyte) NCT04629508	LIMBER-213 – Investigational JAK1 inhibitor for patients who have previously received Ruxolitinib or Fedratinib
2	Fostamatinib (Wash U) NCT04543279	Previously approved therapy for patients with ITP to be tested in MF patients with severe thrombocytopenia at single site.
2	KRT-232 or TL-895 (Kartos) NCT04878003	Investigational MDM2 inhibitor or a new investigational tyrosine kinase inhibitor, TL-895, for patients who have had no prior JAK2 inhibitor treatment
1 / 2	KRT-232 plus TL-895 (Kartos) NCT04640532	Investigational MDM2 inhibitor in combination with a new investigational tyrosine kinase inhibitor, TL-895, for JAK inhibitor intolerant patients
1 / 2	KRT-232 (Kartos) NCT04485260	Investigational MDM2 inhibitor in combination with Ruxolitinib for patients with suboptimal response to Ruxolitinib alone after at least 18 weeks of treatment

1 / 2	CPX-351 plus Ruxolitinib (OHSU) NCT03878199	For advanced phase MPN patients using Ruxolitinib plus a previously approved secondary AML therapy
1 / 2	INCB000928 (Incyte) NCT04455841	Investigational ALK2 (ACVR1) inhibitor INCB000928 administered alone or in combination with Ruxolitinib in transfusion-dependent MF patients or those with symptomatic anemia
1 / 2	APG-1252 (Ascentage) NCT04354727	New investigational drug that focuses on cell death pathway for patients who have progressed after their initial therapy
1 / 2	Selinexor (Karyopharm) NCT04562389	Previously approved for lymphoma. Selinexor in combination with Ruxolitinib for MF patients who have not previously received a JAK inhibitor
1	ABBV-744 (AbbVie) NCT04454658	New BET inhibitor investigational drug – several study arms including ABBV-744 alone, or in combination with Ruxolitinib, or with Navitoclax
1	INCB057643 (Incyte) NCT04279847	New investigational BET inhibitor for MF patients who did not respond to prior treatment or relapsed
1	PU-H71 (Samus) NCT03935555	New investigational drug that focuses on tumor cell death pathway. This trial is evaluating safety and preliminary efficacy in combination with Ruxolitinib
1	TP3654 (SDP Oncology) NCT04176198	New investigational drug focused on fibrosis reduction when combined with Ruxolitinib in higher risk patients
1	PXS-5505 (PharmAxis) NCT04676529	Investigational anti-fibrotic drug taken orally that inhibits the lysyl oxidase family of enzymes
1	Pevonedistat (Wash U) NCT03386214	New targeted anti-inflammatory and pro-tumor cell death pathway investigational drug combined in this study with Ruxolitinib
1	LNK01002 (Lynk Pharmaceuticals) NCT04896112	Safety and tolerability of an orally active investigational triple kinase inhibitor LNK01002 in MF patients

POLYCYTHEMIA VERA (PV)

Phase	Drug (Sponsor)	Trial Focus
2	Rusfertide (Protagonist Therapeutics) NCT04767802	New iron regulatory investigational drug for PV patients who are newly diagnosed or for whom current therapy is not sufficient to control their hematocrit and have hematocrit >48% prior to dosing
2	Rusfertide (Protagonist Therapeutics) NCT04057040	New iron regulatory investigational drug focused on phlebotomy eligible PV patients
2	Ruxolitinib (Mass General/Incyte) NCT04644211	Study to determine if Ruxolitinib is effective in reducing the symptoms caused by low-risk PV
2	Bomedemstat (U of Miami) NCT04262141	New investigational drug IMG-7289 (Bomedemstat) focused on inhibiting LSD1 for patients that have failed one standard therapy. Trial focuses on improving blood counts

ESSENTIAL THROMBOCYTHEMIA (ET)

Phase	Drug (Sponsor)	Trial Focus
3	Ropeginterferon (PharmaEssentia) NCT04285086	SURPASS ET - New interferon investigational drug compared to Anagrelide in ET patients who have had a suboptimal or failed response to Hydroxyurea
2	Bomedemstat (Imago BioSciences) NCT04254978	New investigational drug focused on inhibiting LSD1 for ET patients who have high platelets and have failed at least one standard therapy
2	Bomedemstat (University of Texas) NCT04081220	Single center, investigator-initiated study of Bomedemstat for patients who are intolerant or resistant to Hydroxyurea
2	Bomedemstat (U of Miami) NCT04262141	New investigational drug IMG-7289 (Bomedemstat) focused on inhibiting LSD1 for patients that have failed one standard therapy. Trial focuses on improving blood counts
2	Ruxolitinib (Mass General/Incyte) NCT04644211	Study to determine if Ruxolitinib is effective in reducing the symptoms caused by low-risk essential thrombocythemia (ET)