MPN Clinical Trial Pipeline

With the help of patients who choose to participate, clinical trials determine whether a therapy is safe and effective in treating a particular disease. In some cases (as noted in the list), a treatment may have been previously approved by the Food and Drug Administration (FDA) to treat a different disease but, in many cases, the therapy is investigational. Data collected in clinical trials is necessary for the FDA to review prior to approving any new therapy or a new use of a therapy previously approved to treat a different disease.

MYELOFIBROSIS (MF)

Phase	Drug (Sponsor)	Trial Name (if available) and Notes
3	Fedratinib (BMS) NCT03755518	FREEDOM – For high or intermediate risk patients previously treated with ruxolitinib. A sub-study is in combination with luspatercept for anemia. Fedratinib was approved by the FDA for 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 - PI3 kinase inhibitor for patients who have not previously used a 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 – For patients without prior experience with a JAK2 inhibitor and BH3 mimetic, navitoclax focuses on cell death pathway and is used in combination with ruxolitinib versus ruxolitinib alone
3	Navitoclax (AbbVie) NCT04468984	TRANSFORM-2 - For patients who did not benefit from ruxolitinib previously or for whom it stopped working, navitoclax focuses on cell death pathway in combination with ruxolitinib versus best available therapy
3	Pelabresib (Constellation Pharmaceuticals) NCT04603495	MANIFEST-2 – BET inhibitor for patients without prior experience with a JAK inhibitor
3	Imetelstat (Geron) NCT04576156	IMpactMF - For high risk patients who did not benefit from previous therapy this trial focuses on improving overall survival

Phase	Drug (Sponsor)	Trial Name (if available) and Notes
3	Luspatercept (Acceleron/BMS) NCT04717414	INDEPENDENCE - For patients on a JAK2 inhibitor who require transfusions. Luspatercept was previously approved by the FDA for another blood cancer.
3	Luspatercept (Acceleron/BMS) NCT04064060	Continuation/rollover study to evaluate the long-term safety of luspatercept in patients previously on it
3	Navtemadlin (Kartos) NCT03662126	BOREAS - MDM2 inhibitor for higher risk patients who did not see improvement on a JAK inhibitor
2	Pelabresib (Constellation Pharmaceuticals) NCT02158858	MANIFEST-1 – BET inhibitor for patients with and without prior experience on a JAK inhibitor
2	Tagraxofusp (Stemline Therapeutics) NCT02268253	For higher risk patients for whom a previous therapy was unsuccessful. Tagraxofusp was previously approved by the FDA for a different rare blood cancer.
2	Ruxolitinib Phosphate (MD Anderson) NCT01787487	Combination with azacytidine for high risk patients who have been previously treated or are newly diagnosed and are able to travel to the trial site in Houston, TX
2	Ruxolitinib plus Enasidenib (Mt. Sinai) NCT04281498	For chronic or blast phase MPN patients with IDH2 mutation
2	Selinexor (Karyopharm Therapeutics) NCT04562870	For higher risk patients who did not benefit from ruxolitinib. Selinexor was previously approved by the FDA for another blood cancer.
2	Elotuzumab (MD Anderson) NCT04517851	For patients with the JAK2 mutation who are able to travel to the trial site in Houston, TX. Elotuzumab was previously approved by the FDA for another blood cancer.
2	9-ING-41 (Actuate) NCT04218071	For patients with advanced disease. 9-ING-41 is an anti-cancer and anti-fibrotic given alone or in combination with ruxolitinib.

Phase	Drug (Sponsor)	Trial Name (if available) and Notes
2	GB2064 (Galecto) NCT04679870	Inhibitor of LOXL2 for intermediate 2 or high risk patients not currently on a JAK inhibitor
2	TL-895 (Telios) NCT04655118	Tyrosine kinase inhibitor for patients who did not benefit from prior therapy and who are intolerant or ineligible to receive a JAK inhibitor
2	Itacitinib (Incyte) NCT04629508	LIMBER-213 – JAK1 inhibitor for patients who previously received ruxolitinib or fedratinib
2	Fostamatinib (Washington University) NCT04543279	Tyrosine kinase inhibitor for patients with severe thrombocytopenia able to travel to the trial site in St. Louis, MO. Fostamatinib was previously approved by the FDA for another indication.
2	Decitabine (University of Washington) NCT04282187	Decitabine with ruxolitinib or fedratinib for patients with accelerated or blast phase MPNs prior to hematopoietic stem cell transplant who are able to travel to the trial site in Seattle, WA
2	Ropeginterferon (Mayo Clinic) NCT02370329	PEG-proline-interferon alpha-2b aimed at improving the body's immune response and slowing the growth of disease for patients who are able to travel to the trial site in Scottsdale, AZ
2	Navtemadlin or TL-895 (Kartos) NCT04878003	MDM2 inhibitor or tyrosine kinase inhibitor for patients who have had no prior experience on a JAK2 inhibitor
2	Selinexor (University of Utah) NCT03627403	For patients who could not tolerate ruxolitinib or any other JAK 1/2 inhibitors previously or for whom it stopped working who are able to travel to the trial site in Salt Lake City, UT. Selinexor was previously approved by the FDA for another blood cancer.
1 / 2	Navtemadlin plus TL-895 (Kartos) NCT04640532	MDM2 inhibitor in combination with tyrosine kinase inhibitor for patients who cannot tolerate a JAK inhibitor
1 / 2	Navtemadlin (Kartos) NCT04485260	MDM2 inhibitor in combination with ruxolitinib for patients with suboptimal response to ruxolitinib alone after at least 18 weeks of treatment

Phase	Drug (Sponsor)	Trial Name (if available) and Notes
1 / 2	CPX-351 plus Ruxolitinib (Oregon Health & Science University) NCT03878199	For advanced phase patients ruxolitinib is administered in addition to CPX-351 which was previously approved by the FDA for another blood cancer
1 / 2	INCB000928 (Incyte) NCT04455841	ALK2 inhibitor for transfusion-dependent patients or those with symptomatic anemia administered alone or in combination with ruxolitinib
1 / 2	APG-1252 (Ascentage) NCT04354727	For patients who have progressed after their initial therapy, APG-1252 focuses on cell death pathway
1 / 2	Selinexor (Karyopharm) NCT04562389	For patients who have not previously received a JAK inhibitor, Selinexor is combined with ruxolitinib. Selinexor was previously approved by the FDA for another blood cancer.
1	ABBV-744 (AbbVie) NCT04454658	BET inhibitor for patients currently or formerly on a JAK inhibitor with several study arms including ABBV-744 alone, in combination with ruxolitinib, or in combination with navitoclax
1	INCB057643 (Incyte) NCT04279847	Study of BET inhibitor for patients who did not respond to prior treatment or relapsed
1	Selumetinib/Azacitidine (University of Chicago) NCT03326310	Study of azacitidine and MEK inhibitor selumetinib for intermediate or high risk patients able to travel to trial site in Chicago, IL. Study drugs were previously approved by the FDA for other indications.
1	Fedratinib/Enasidenib/Ivosidenib (University of Chicago) NCT04955938	For MPN patients with an IDH mutation this study has two arms. Enasidenib and ivosidenib were previously approved by the FDA for other indications. Ivosidenib will be given initially to IDH1 participants followed by a combination of ivosidenib with fedratinib. For IDH2 patients enasidenib monotherapy will be followed by combination with fedratinib.
1	PU-H71 (Samus) NCT03935555	For patients already on a stable dose of ruxolitinib, PU-H71 focuses on tumor cell death pathway
1	INCB057643 (Incyte) NCT04279847	Study of BET inhibitor alone and in combination with ruxolitinib

Phase	Drug (Sponsor)	Trial Name (if available) and Notes
1	TP-3654 (SDP Oncology) NCT04176198	For higher risk patients who were ineligible for or did not previously benefit from a JAK inhibitor. TP-3654 focuses on fibrosis reduction
1	LNK01002 (Lynk Pharmaceuticals) NCT04896112	Triple kinase inhibitor focused on anti-tumor activity for patients able to travel to trial sites in Michigan
1	TAS1553 (Astex Pharmaceuticals) NCT04637009	Ribonuclease reductase inhibitor for blast phase AML or MPN patients for whom prior approved therapy was not successful

POLYCYTHEMIA VERA (PV)

Phase	Drug (Sponsor)	Trial Name (if available) and Notes
2	Ruxolitinib (Massachusetts General Hospital/Incyte) NCT04644211	For low risk patients able to travel to trial sites in Massachusetts to determine if ruxolitinib is effective in symptom reduction
2	Bomedemstat (University of Miami) NCT04262141	For patients able to travel to the trial site in Miami, FL who did not benefit from one standard therapy, bomedemstat focuses on inhibiting LSD1 and improving blood counts.
1	Fedratinib/Enasidenib/Ivosidenib (University of Chicago) NCT04955938	For MPN patients with an IDH mutation this study has two arms. All three study drugs were previously approved by the FDA for other indications. Ivosidenib will be given initially to IDH1 participants followed by a combination of ivosidenib with fedratinib. For IDH2 patients enasidenib monotherapy will be followed by combination with fedratinib.
1	Navitoclax (AbbVie) NCT04041050	Multipart trial for MPN patients who require treatment and who refuse standard therapy, who did not benefit from prior therapy or for whom it stopped working. Parts 3 and 4 are recruiting in US and Europe. Part 3 is navitoclax alone and part 4 is navitoclax with celecoxib which was previously approved by the FDA for another indication.

ESSENTIAL THROMBOCYTHEMIA (ET)

Phase	Drug (Sponsor)	Trial Name (if available) and Notes
3	Ropeginterferon (PharmaEssentia) NCT04285086	SURPASS ET – For patients who have had a suboptimal response or did not benefit from hydroxyurea, response of patients is compared to response of patients on anagrelide. Note that this interferon formulation (ropeg interferon alfa-2b-njft) was approved by the FDA for use in PV in November 2021.
2	Bomedemstat (Imago BioSciences) NCT04254978	For patients who have high platelets and did not benefit from at least one standard therapy, bomedemstat focuses on inhibiting LSD1
2	Bomedemstat (University of Texas) NCT04081220	For patients who are intolerant or resistant to hydroxyurea and are able to travel to the trial site in San Antonio, TX
2	Bomedemstat (University of Miami) NCT04262141	For patients who did not benefit from one standard therapy and are able to travel to the trial site in Miami, FL, bomedemstat focuses on inhibiting LSD1 and improving blood counts
2	Ruxolitinib (Massachusetts General Hospital/Incyte) NCT04644211	For low risk patients able to travel to trial sites in Massachusetts to determine if ruxolitinib is effective in symptom reduction
1	Fedratinib/Enasidenib/Ivosidenib (University of Chicago) NCT04955938	For MPN patients with an IDH mutation this study has two arms. All three study drugs were previously approved by the FDA for other indications. Ivosidenib will be given initially to IDH1 participants followed by a combination of ivosidenib with fedratinib. For IDH2 patients enasidenib monotherapy will be followed by combination with fedratinib.
1	Navitoclax (AbbVie) NCT04041050	Multipart trial for MPN patients who require treatment and who refuse standard therapy, who did not benefit from prior therapy or for whom it stopped working. Parts 3 and 4 are recruiting in US and Europe. Part 3 is navitoclax alone and part 4 is navitoclax with celecoxib which was previously approved by the FDA for another indication.