Oral Pacritinib Versus Best Available Therapy to Treat Myelofibrosis With Thrombocytopenia (PAC326)

This study is currently recruiting participants. (see Contacts and Locations)

Verified December 2014 by *CTI BioPharma*

Sponsor:
CTI BioPharma

Information provided by (Responsible Party):
CTI BioPharma

ClinicalTrials.gov Identifier:
NCT02055781

First received: February 3, 2014
Last updated: December 4, 2014
Last verified: December 2014
History of Changes

Purpose

The primary hypothesis of the study is that treatment with either once-daily or twice-daily pacritinib results in a greater proportion of patients with thrombocytopenia and myelofibrosis achieving ≥ 35% reduction in spleen volume from baseline to Week 24 than treatment with Best Available Therapy, and a greater proportion of patients achieving a ≥ 50% reduction in total symptom score from baseline to Week 24 as measured by the Myeloproliferative Neoplasm Symptom Assessment Form **2.0**.

Condition	Intervention	Phase
Primary Myelofibrosis Post-polycythemia Vera Myelofibrosis Post-essential Thrombocythemia Myelofibrosis	Drug: Pacritinib Drug: Best Available Therapy	Phase 3

Study Type: Interventional
Study Design: Allocation: Randomized
Endpoint Classification: Safety/Efficacy Study
Intervention Model: Parallel Assignment
Masking: Open Label
Primary Purpose: Treatment

Official Title: A Randomized Controlled Phase 3 Study of Oral Pacritinib Versus Best Available Therapy in Patients With Thrombocytopenia and Primary Myelofibrosis, Post-Polycythemia Vera Myelofibrosis, or Post-Essential Thrombocythemia Myelofibrosis

Resource links provided by NLM:

Genetics Home Reference related topics: essential thrombocythemia polycythemia vera primary myelofibrosis

Genetic and Rare Diseases Information Center resources: Chronic Myeloproliferative Disorders Essential Thrombocythemia Myelofibrosis Polycythemia Vera Splenomegaly

U.S. FDA Resources

Further study details as provided by CTI BioPharma:

Primary Outcome Measures:

- Efficacy [Time Frame: Baseline to Week 24] [Designated as safety issue: No]

To compare the efficacy of two dose-schedule arms of pacritinib (pooled once-daily and twice-daily dosing arms) with that of Best Available Therapy in patients with thrombocytopenia and primary myelofibrosis, post-polycythemia vera myelofibrosis, or post-essential thrombocythemia myelofibrosis; the efficacy measure for this analysis is the proportion of patients achieving a ≥ 35% reduction in spleen volume from baseline to week 24 by magnetic resonance imaging (MRI) or computed tomography (CT) and the proportion of patients achieving a ≥ 50% reduction in total symptom score from baseline to Week 24 as measured by the Myeloproliferative Neoplasm Symptom Assessment Form 2.0.

Secondary Outcome Measures:

- Efficacy [Time Frame: Baseline to Week 24] [Designated as safety issue: No]

  To compare the efficacy of once-daily pacritinib with that of Best Available Therapy, as assessed by the proportion of patients achieving a ≥ 35% reduction in spleen volume from baseline to Week 24 by magnetic resonance imaging (MRI) or computed tomography (CT) and the proportion of patients achieving a ≥ 50% reduction in the total symptom score from baseline to Week 24 on the Myeloproliferative Neoplasm Symptom Assessment Form 2.0.

- Efficacy [Time Frame: Baseline to Week 24] [Designated as safety issue: No]

  To compare the efficacy of twice-daily pacritinib with that of Best Available Therapy, as assessed by the proportion of patients achieving a ≥ 35% reduction in spleen volume from baseline to Week 24 by magnetic resonance imaging (MRI) or computed tomography (CT) and the proportion of patients achieving a ≥ 50% reduction in the total symptom score from baseline to Week 24 on the myeloproliferate Neoplasm Symptom Assessment Form 2.0.

Estimated Enrollment:	300
Study Start Date:	December 2013
Estimated Study Completion Date:	April 2018
Estimated Primary Completion Date:	April 2015 (Final data collection date for primary outcome measure)

Arms	Assigned Interventions
Experimental: Pacritinib, Once Daily Pacritinib 400 mg taken orally, once daily	Drug: Pacritinib
Experimental: Pacritinib, Twice Daily Pacritinib 200 mg taken orally, twice daily	Drug: Pacritinib
Active Comparator: Best Available Therapy Best Available Therapy includes any physician-selected treatment for primary myelofibrosis, post-polycythemia vera myelofibrosis, or post-essential thrombocythemia myelofibrosis, such as approved JAK2 inhibitors, and may include any treatment received before study entry. Best Available Therapy may include ruxolitinib, other approved JAK2 inhibitors, hydroxyurea, glucocorticoids, erythropoietic agents, immunomodulatory agents, mercaptopurine, danazol, interferons, cytarabine, melphalan, or other agents and may also include no treatment and symptom-directed treatment without myelofibrosis-specific treatment.	Drug: Best Available Therapy

Eligibility

Ages Eligible for Study:	18 Years and older
Genders Eligible for Study:	Both
Accepts Healthy Volunteers:	No

Criteria

Inclusion Criteria:

- Intermediate -1 or -2 or high-risk Myelofibrosis (per Passamonti et al 2010)
- Thrombocytopenia (platelet count ≤ 100,000/μL) at any time after signing informed consent
- Palpable splenomegaly ≥ 5 cm on physical examination
- Total Symptom Score ≥ 13 on the MPN-SAF TSS 2.0, not including the inactivity question
- Patients who are platelet or red blood cell transfusion-dependent are eligible
- Adequate white blood cell counts (with low blast counts), liver function, and renal function
- At least 6 months from prior splenic irradiation

- At least 1-4 weeks since prior myelofibrosis therapy, including any erythropoietic or thrombopoietic agent
- Not pregnant, not lactating, and agree to use effective birth control
- Able and willing to undergo frequent MRI or CT assessments and complete symptom assessments using a patient-reported outcome instrument

Exclusion Criteria:

- Prior treatment with more than 2 JAK2 inhibitors or with pacritinib
- There is no maximum cumulative prior JAK2 inhibitor treatment
- History of (or plans to undergo) spleen removal surgery or allogeneic stem cell transplant
- Ongoing gastrointestinal medical condition such as Crohn's disease, Inflammatory bowel disease, chronic diarrhea, or constipation
- Active bleeding that requires hospitalization during the screening period
- Cardiovascular disease, including recent history or currently clinically symptomatic and uncontrolled: congestive heart failure, arrhythmia, angina, QTc prolongation or other QTc risk factors, myocardial infarction
- Other malignancy within last 3 years other than certain limited skin, cervical, prostate, breast, or bladder cancers
- Other ongoing, uncontrolled illnesses (including HIV infection and active hepatitis A, B, or C), psychiatric disorder, or social situation that would prevent good care on this study
- Life expectancy < 6 months

Contacts and Locations

Choosing to participate in a study is an important personal decision. Talk with your doctor and family members or friends about deciding to join a study. To learn more about this study, you or your doctor may contact the study research staff using the Contacts provided below. For general information, see Learn About Clinical Studies.

Please refer to this study by its ClinicalTrials.gov identifier: NCT02055781

Contacts

Contact: Valentina Zhukova-Harrill, MD +44 (0) 131-200-6320

Contact: Gordon Thomson, PM +44 (0) 131-440-6441

Show 76 Study Locations

Sponsors and Collaborators

CTI BioPharma

Investigators

Study Director: Mary Campbell, MD CTI BioPharma

More Information

No publications provided

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ClinicalTrials.gov Identifier:	NCT02055781 History of Changes
Other Study ID Numbers:	**PERSIST-2** (PAC326)
Study First Received:	February 3, 2014
Last Updated:	December 4, 2014
Health Authority:	United States: Food and Drug Administration

Keywords provided by CTI BioPharma:

Myelofibrosis
Post-Polycythemia Vera Myelofibrosis
Post-Essential Thrombocythemia Myelofibrosis
Primary Myelofibrosis
Polycythemia
Polycythemia Vera
Thrombocythemia, Essential
Thrombocythemia
Myeloproliferative Disorders
Bone Marrow Disease
Hemorrhagic Disorders
Splenomegaly
Pacritinib
MPN-SAF
MPN-SAF TSS
Anemia
Myeloproliferative
Myeloproliferative Neoplasm
Spleen
Spleen volume

Hematologic Diseases
Blood Platelet Disorders

Thrombocytopenia
SB1518

Additional relevant MeSH terms:
Polycythemia
Polycythemia Vera
Primary Myelofibrosis
Thrombocythemia, Essential
Thrombocytopenia
Thrombocytosis

Blood Coagulation Disorders
Blood Platelet Disorders
Bone Marrow Diseases
Hematologic Diseases
Hemorrhagic Disorders
Myeloproliferative Disorders

ClinicalTrials.gov processed this record on December 07, 2014