Oral Pacritinib Versus Best Available Therapy to Treat Myelofibrosis

**Purpose**

The primary hypothesis of the study is that treatment with pacritinib results in a greater proportion of patients achieving ≥ 35% reduction in spleen volume from baseline to Week 24 than treatment with BAT.

### Condition

Primary Myelofibrosis  
Post-polycythemia Vera Myelofibrosis  
Post-essential Thrombocythemia Myelofibrosis

### Intervention

Drug: Pacritinib  
Drug: Best Available Therapy

### Phase

Phase 3

**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 pacritinib with that of best available therapy (BAT) in patients with primary myelofibrosis (PMF), post-polycythemia vera myelofibrosis (PPV-MF), or post-essential thrombocythemia myelofibrosis (PET-MF); 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)
Secondary Outcome Measures:
- Symptomatic Efficacy [Time Frame: Baseline to week 24] [Designated as safety issue: No]
  To compare pacritinib with best available therapy with respect to the proportion of patients with >= 50% reduction in total score from baseline to week 24 on the Myeloproliferative Neoplasm Symptom Assessment Form (MPN-SAF TSS)

Estimated Enrollment: 322
Study Start Date: December 2012
Estimated Study Completion Date: January 2018
Estimated Primary Completion Date: December 2014 (Final data collection date for primary outcome measure)

<table>
<thead>
<tr>
<th>Arms</th>
<th>Assigned Interventions</th>
</tr>
</thead>
<tbody>
<tr>
<td>Experimental: Pacritinib</td>
<td>Drug: Pacritinib</td>
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<tr>
<td>Pacritinib 400 mg taken orally, once daily</td>
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<tr>
<td>Active Comparator: Best Available Therapy</td>
<td>Drug: Best Available Therapy</td>
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<td>BAT includes any physician-selected treatment for PMF, PPV-MF, or PET-MF with the exclusion of JAK inhibitors (inhibitors of Janus kinases). For example, BAT may include hydroxyurea, glucocorticoids, erythropoietic agents, immunomodulatory agents, mercaptopurine, danazol, interferons, cytarabine, melphalan, or other agents.</td>
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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)
- 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
- No spleen radiation therapy for 6-12 months
- Last therapy for myelofibrosis was 2-4 weeks ago, including any erythropoietic or thrombopoietic agent
- Not pregnant, not lactating, and agree to use effective birth control

Exclusion Criteria:
- Prior treatment with a JAK2 inhibitor
- 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
- 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 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: NCT01773187

Show 81 Study Locations
Sponsors and Collaborators
CTI BioPharma

Investigators
Study Director: James Dean, MD, PhD CTI BioPharma

More Information
No publications provided

Responsible Party: CTI BioPharma
ClinicalTrials.gov Identifier: NCT01773187
Other Study ID Numbers: PERSIST-1 (PAC325)
Study First Received: January 18, 2013
Last Updated: June 30, 2014
Health Authority: United States: Food and Drug Administration

Key words provided by CTI BioPharma:
Myelofibrosis
Post-Polycythemia Vera Myelofibrosis
Post-Essential Thrombocytethemia Myelofibrosis
Primary Myelofibrosis
Polycythemia
Polycythemia Vera
Thrombocytethemia, Essential
Thrombocytethemia
Myeloproliferative Disorders
Bone Marrow Disease
Hematologic Diseases
Blood Platelet Disorders

Additional relevant MeSH terms:
Polycythemia
Polycythemia Vera
Primary Myelofibrosis
Thrombocytethemia, Essential
Thrombocytosis
Blood Coagulation Disorders

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