This presentation includes forward-looking statements within the meaning of the Safe Harbor provisions of the Private Securities Litigation Reform Act of 1995. Such statements are subject to a number of risks and uncertainties, the outcome of which could materially and/or adversely affect actual future results and the trading price of our common stock. These forward-looking statements are based on current assumptions that involve risks, uncertainties and other factors that may cause the actual results, events or developments to be materially different from those expressed or implied by such forward-looking statements. These risks and uncertainties include, but are not limited to:

- our ability to conduct and complete clinical trials in our currently anticipated timeframes, including risks relating to potential delays in clinical trials caused by the COVID-19 pandemic, as well as our ability to submit an NDA for pacritinib in currently anticipated timelines;
- potential increases in patient morbidity and/or mortality in clinical trials due to the COVID-19 pandemic;
- clinical trials may not demonstrate safety and efficacy of pacritinib;
- the risk that even if a clinical trial meets one or more primary endpoints, the FDA may nevertheless determine that the benefit/risk profile of pacritinib at the selected doses does not support approval or that additional data is required for approval;
- our assumptions regarding planned expenditures and the sufficiency of cash to fund operations may be incorrect, including due to the impact of expanded product development and clinical activities on operating expenses, as well as delayed timelines due to the pandemic;
- adverse conditions in the general domestic and global economic markets, including instability due to the pandemic and/or social unrest;
- our ability to receive regulatory approval for pacritinib pursuant to the accelerated approval pathway or at all;
- the risk that pacritinib may be delayed to a point where it is not commercially viable, whether due to competition or loss of patent rights; and
- those risks more fully discussed in the section entitled "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2019 and subsequent quarterly reports on Form 10-Q.

These forward-looking statements speak only as of the date hereof and we assume no obligation to update these forward-looking statements. Readers are cautioned not to place undue reliance on such forward-looking statements.
Developing Pacritinib for Myelofibrosis

**Multi-kinase Activity**

PACRITINIB

- JAK2
- CSF-1R
- IRAK1

**Rolling NDA Submission Underway**

*Rolling NDA submission commenced in October 2020*

>1,200 patients dosed with pacritinib
# Pacritinib Development Program

<table>
<thead>
<tr>
<th>Program</th>
<th>Indication</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>Approved</th>
</tr>
</thead>
<tbody>
<tr>
<td>PACIFICA</td>
<td>Myelofibrosis, severe thrombocytopenia (enrolling)</td>
<td>✔️</td>
<td>✔️</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>PRE-VENT</td>
<td>Severe COVID-19 (enrolling)</td>
<td>✔️</td>
<td>✔️</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>PAC203</td>
<td>High risk myelofibrosis, second-line therapy</td>
<td>✔️</td>
<td>✔️</td>
<td></td>
<td></td>
</tr>
<tr>
<td>PERSIST-2</td>
<td>Myelofibrosis (platelets ≤100,000/µL)</td>
<td>✔️</td>
<td>✔️</td>
<td>✔️</td>
<td>✔️</td>
</tr>
<tr>
<td>PERSIST-1</td>
<td>Myelofibrosis (all platelet counts)</td>
<td>✔️</td>
<td>✔️</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
Pacritinib – A New Treatment Option for Myelofibrosis
Myelofibrosis and Unmet Medical Need

- A malignant bone marrow cancer
- Median survival 6 years after diagnosis
- Standard of care is ruxolitinib
- **Severe thrombocytopenia** is the most important unmet medical need
- One-third of MF patients seeking treatment are severely thrombocytopenic

**Debilitating Symptoms**

- **Impaired Blood Cell Production**
  - Thrombocytopenia, Anemia
- **Constitutional Symptoms**
  - Fatigue, Night Sweats, Itching, Bone Pain, Weight Loss
- **Symptomatic Splenomegaly**
  - Abdominal Pain, Early Satiety, Bloating
Severe Thrombocytopenia

Short overall survival when platelet count $<50 \times 10^9/\mu L$

Overall Survival 15 months

Source: Masarova L. et al., Eur J Haematol. 2017
Thrombocytopenia and Ruxolitinib

Rapid decline in platelet counts with ruxolitinib at doses of 15-20mg BID\(^1\)

….associated with dose reductions

….which can reduce clinical benefit\(^2\)

---

Severe Thrombocytopenia in MF

35% of MF patients have Severe Thrombocytopenia

Masarova & Mesa, Leukemia Research 2020
Two Paths to Severe Thrombocytopenia

Nearly all MF patients eventually develop thrombocytopenia

**Disease-related**
- At diagnosis or with disease progression
- Typically worsens over time

**Treatment-related**
- Typically treated with ruxolitinib
- Eventually discontinue ruxolitinib due to cytopenias

Severely thrombocytopenic patients typically have myelodepletive disease with low blood count and frequently require transfusion support.

Severe Thrombocytopenia in MF

Both Front-line and Second-line Patients have Severe Thrombocytopenia

- Front-line, treatment naive
- Discontinued ruxolitinib
- Low-dose ruxolitinib

~17,000 patients

### PERSIST-2 SVR Efficacy Data

**Significant improvement in SVR with First & Second Line Severely Thrombocytopenic Patients**

<table>
<thead>
<tr>
<th>PERSIST-2</th>
<th>Pacritinib 200mg BID (N=31)</th>
<th>BAT (N=32)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Patients with platelet counts &lt;50 x 10^9/L</td>
<td></td>
<td></td>
</tr>
<tr>
<td>≥35% SVR</td>
<td>29.0%</td>
<td>3.1%</td>
</tr>
<tr>
<td>P-value vs. BAT</td>
<td>0.0059</td>
<td></td>
</tr>
</tbody>
</table>

PAC203 Phase 2 SVR Efficacy Data

Significant improvement in SVR in Heavily Pretreated Severely Thrombocytopenic Patients

<table>
<thead>
<tr>
<th>PAC203 Phase 2</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Patients with platelet counts &lt;50 x 10^9/L</td>
<td>Pacritinib 200mg BID (N=24)</td>
</tr>
<tr>
<td>≥35% SVR</td>
<td>17.0%</td>
</tr>
</tbody>
</table>

Baseline platelet count
- <50,000/μL
- ≥50,000/μL

Gerds, et al., ASH 2019 Oral Presentation
# Pacritinib Safety Profile

**Predictable and manageable safety profile**

<table>
<thead>
<tr>
<th>Adverse Event (%) (all patients)</th>
<th>200mg BID (N=54)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Diarrhea</td>
<td>30%</td>
</tr>
<tr>
<td>Thrombocytopenia</td>
<td>41%</td>
</tr>
<tr>
<td>Nausea</td>
<td>28%</td>
</tr>
<tr>
<td>Fatigue</td>
<td>24%</td>
</tr>
<tr>
<td>Abdominal pain</td>
<td>24%</td>
</tr>
<tr>
<td>Pyrexia</td>
<td>13%</td>
</tr>
<tr>
<td>Anemia</td>
<td>24%</td>
</tr>
<tr>
<td>Peripheral edema</td>
<td>17%</td>
</tr>
<tr>
<td>Decreased appetite</td>
<td>19%</td>
</tr>
</tbody>
</table>

Gerds A. et al., ASH 2019 Oral Presentation.
Pacritinib Stabilizes Platelet Counts

Platelet counts remain stable over 36 weeks

Baseline platelet count <50,000/µL

Percent Change in platelets (median, 95% CI)

Time on Study (weeks)

Gerds A. et al., ASH 2019 Oral Presentation.
PACIFICA Phase 3 Clinical Trial

Primary or Secondary Myelofibrosis
Severe Thrombocytopenia (platelets <50 x 10^9/L)

Pacritinib 200 mg BID
Randomized 2:1
Physician’s Choice

Primary Analysis
N=168
SVR at 24 weeks

Secondary Analyses
N=348
TSS at 24 weeks
Overall Survival

• Now actively enrolling; targeting 130+ clinical sites worldwide
• 1st and 2nd-line MF patients with severe thrombocytopenia
• Powered for SVR (85%) and TSS (80%)
• Top-line primary analysis data expected in 2022*
• Potential post-marketing commitment following accelerated approval

SVR, ≥35% spleen volume reduction; TSS, ≥50% reduction in total symptom score. *Enrollment projections may change in light of the COVID-19 pandemic.
Pathway to Approval

• Pre-NDA meeting with the FDA in September 2020
  – Agreement reached on submission based on available data
  – Myelofibrosis and Severe Thrombocytopenia
    – Platelet count <50 x 10⁹/L

• No approved dosing for available therapies

• Submission intended to address unmet medical need
  – Limited therapeutic options for these patients
  – No approved dosing for available JAK2 inhibitor therapies

• Rolling NDA submission commenced October 2020
• Complete NDA submission anticipated in 1Q 2021
• Potential accelerated approval in 2021*

*Approval timeline dependent on priority review
Pacritinib and GVHD
Effective Therapies are Needed to Prevent GVHD After Allogeneic HCT

- Graft versus host disease (GVHD) frequently complicates allogeneic hematopoietic cell transplantation (alloHCT)
- The transplanted “graft” recognizes its new “host” as foreign, leading to organ dysfunction and death
- GVHD occurs despite standard immunosuppressive prophylaxis

Allogenic HSCT
(~9K annually in the US)

Prophylaxis Therapy

Grade II-IV acute GVHD (~40%)\(^1\)

Chronic GVHD (~40%)\(^2\)

---

\(^1\) The Glucksberg (I-IV) and the International Bone Marrow Transplant Registry (A-D) grading systems are used for aGvHD severity and are based on organ involvements and / or specific organ staging criteria.

\(^2\) NIH consensus criteria are used for cGvHD severity and are based on organ involvement and/or functional impairment. GlobalData; UpToDate; CIBMTR; Clearview Analysis.
Pacritinib + Sirolimus Promotes Immune Tolerance while Preserving Anti-Leukemic Effect

- Co-stimulation of IL-6 and CD28 drives GVHD directed immune response
- Immune response blocked by JAK2 + mTOR inhibition by shifting T cell populations away from alloreactivity (Th1/Th17 cells) and towards immune tolerance (Tregs/Th2 cells)

Selectivity JAK2 inhibition by pacritinib promotes immune tolerance while preserving the graft-versus-leukemia (GVL) effect (mediated by STAT5-dependent CD8/NK cells), unlike JAK1 inhibitors

1. Betts BC et al., PNAS 2018:115(7);1582-7
Phase I AlloHCT Study with PAC/SIR/TAC

- Single-arm Phase I/II alloHCT study presented at ASH2020
  - GVHD prophylaxis with pacritinib (PAC) added to standard sirolimus (SIR) and low-dose tacrolimus (TAC) therapy
  - 3+3 pacritinib dose escalation design
  - Assessments including safety and acute/chronic GVHD
  - Phase I completed, Phase II underway
GVHD Prophylaxis with PAC/SIR/TAC

**Efficacy**
- PAC 100 mg BID* → Phase II
- Acute GVHD (grade 2-4)
  - 25% - all cohorts
  - 17% - 100 mg BID cohort
  - 43% expected with SIR/TAC\(^1,2\)
- Moderate-to-severe chronic GVHD
  - No cases reported
  - 24% expected with SIR/TAC alone\(^2\)

**Safety**
- No engraftment delay or cytopenias
- No CMV reactivation at 100 mg BID
- 1 relapse (>12 mo. post transplant)

<table>
<thead>
<tr>
<th>Pacritinib Dose</th>
<th>aGVHD Onset (Day)</th>
<th>Grade of aGVHD</th>
</tr>
</thead>
<tbody>
<tr>
<td>100 mg daily</td>
<td>41</td>
<td>2 (GI)</td>
</tr>
<tr>
<td>100 mg daily</td>
<td>N/A</td>
<td>0</td>
</tr>
<tr>
<td>100 mg daily</td>
<td>N/A</td>
<td>0</td>
</tr>
<tr>
<td>100 mg daily</td>
<td>75</td>
<td>2 (GI)</td>
</tr>
<tr>
<td>100 mg daily</td>
<td>72</td>
<td>1</td>
</tr>
<tr>
<td>100 mg BID</td>
<td>54</td>
<td>1</td>
</tr>
<tr>
<td>100 mg BID</td>
<td>32</td>
<td>1</td>
</tr>
<tr>
<td>100 mg BID</td>
<td>29</td>
<td>1</td>
</tr>
<tr>
<td>100 mg BID</td>
<td>N/A</td>
<td>0</td>
</tr>
<tr>
<td>100 mg BID</td>
<td>20</td>
<td>4 (skin)**</td>
</tr>
<tr>
<td>100 mg BID</td>
<td>N/A</td>
<td>0</td>
</tr>
</tbody>
</table>

**Subject prematurely discontinued tacrolimus prior to GVHD onset due to a tacrolimus toxicity**

Pacritinib and COVID-19
COVID-19 Immune Dysregulation

• Patients with COVID-19 have high levels of cytokines involved in multiple signaling pathways
  – IL-1, IL-6, IL-7, TNF-α, ferritin, GM-CSF
• Elevations in IL-6 and IL-1 contribute to $T_H^{17}$ differentiation, implicated in pathogenesis of ARDS\(^1\)
• Elevations in ferritin and GM-CSF suggest macrophage activation syndrome, implicated in cytokine storm
• High cytokine levels associated with high mortality\(^2\)

Advantages of Pacritinib in COVID-19

- Targets multiple pathways rather than single cytokines
- May block key inflammatory pathways, including those involved in Th17 differentiation and macrophage stimulation
- No JAK1 inhibition – may preserve anti-viral response
Pacritinib Immunomodulatory Effects

Phase 3 PRE-VENT - Severe COVID-19

- Hospitalized patient with Severe COVID-19
- Age ≥ 18 years
- No other active infections
- History of bleeding (grade 2 or higher)

Primary Endpoint
• Mechanical Ventilation/ECMO, or death by 28 days

Key Secondary Endpoints
• 15-day and 28-day mortality
• Ventilator-free days

1:1 Randomization (N = 358)

SOC + Pacritinib 200 mg BID
SOC + Placebo

Interim analysis planned at 200 patients; data anticipated early 2021

Severe COVID-19 is defined as confirmed disease in patients who are hospitalized with hypoxia (blood oxygen saturation [SpO2] ≤93% on room air at sea level), respiratory rate >30, arterial oxygen partial pressure [PaO2]/fraction of inspired oxygen [FiO2] <300, or lung infiltrates >50% but do not require Invasive Mechanical Ventilation.
Financials
## Financial Overview

<table>
<thead>
<tr>
<th>Balance Sheet</th>
<th>9/30/20</th>
<th>12/31/19</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash, Cash Equivalents and Short-term investments</td>
<td>$57.4 M</td>
<td>$33.7 M</td>
</tr>
<tr>
<td>Debt</td>
<td>$6.2 M</td>
<td>$10.2 M</td>
</tr>
<tr>
<td>Common Shares outstanding</td>
<td>73.7 M shares*</td>
<td>58.0 M shares</td>
</tr>
</tbody>
</table>

*Does not include 44.8 M shares issuable underlying the outstanding preferred stock.
The Pacritinib Value Proposition

- Potential to serve a critical **unmet medical need** in Myelofibrosis
  - **Severe Thrombocytopenia** (platelet count <50 x 10⁹/L)
    - 35% of MF patients
    - No approved dosing with current therapies
- Demonstrated clinical benefit in Phase 2 and 3 trials
- **Rolling NDA submission** underway
- Potential **accelerated approval** in 2021*
- Launch planning underway
- Significant market opportunity
- Additional optionality from COVID and GVHD programs

*Approval timeline dependent on priority review
Contact: Maeve Conneighton
maeve@argotpartners.com