**Study Design**

- Phase 2, open-label, single treatment arm

**Patient Population**

- Adults with imatinib-resistant or -intolerant CML-CP
- Standard eligibility criteria: Q57 > 1000 /ul most recent
- Nilotinib was given 400 mg orally bid 24 hours following meal

**RESULTS**

- **Cytogenetic Response is Characterized by:**
  - Complete hematological response (CHR)
  - Major cytogenetic response (MCyR)
  - Complete cytogenetic response (CCyR)
- **Definition of Imatinib Resistance and Intolerance (Intention-to-Treat analysis):**
  - Either treatment with imatinib ≥ 400 mg/day with disease progression ≥ 50% increase in WBC, blasts, basophils, eosinophils, or no hematologic response in bone marrow after 4 weeks, or patients receiving ≥ 400 mg/day of any other following mutations: L246E, Q252R, Q253D, Q253E, T315I, F359I, H377F.
  - **Primary resistance:**
    - No complete hematologic response (CHR) at or after 6 months
  - **Secondary resistance:**
    - Loss of CHR
    - Loss of minimal CyR, loss of MCyR, loss of complete cytogenetic response (CCyR)
    - Development of clonal evolution

**Intolerability**

- **Patients with AbL Kinase Domain:**
  - Most imatinib-resistant or -intolerant CML-CP patients treated with nilotinib remain free of progression ≥ 24 months.

**CONCLUSIONS**

- Nilotinib therapy results in rapid and durable hematological and cytogenetic responses in patients with imatinib-resistant or -intolerant CML-CP.
- **Nilotinib therapy achieved MCyR in a majority of imatinib-resistant and -intolerant CML-CP patients.**
- Nearly 60% of patients have MCyR and of these responding patients, 74% have CCyR.
- Most imatinib-resistant or -intolerant CML-CP patients treated with nilotinib remain free of progression ≥ 24 months following initiation of therapy.
- Nilotinib therapy results in 88% overall survival rate at 24 months following initiation of therapy in patients with imatinib-resistant or -intolerant CML-CP.
- Nilotinib exhibits excellent tolerability with myelosuppression events being low, predictable, and easily managed.
- Grade 3-4 nonhematologic AEs are uncommon and biochemical laboratory abnormalities are generally mild, transient, and manageable.