**ABSTRACT 7031**

**Objective:** The authors conducted a phase II study of orally administered rigosertib (ON 01910. Na) in transfusion-dependent lower risk myelodysplastic syndrome (MDS) patients.

**Methods:**

**Patient Selection:** Eligible patients had previously treated lower risk MDS and a history of at least 3 transfusion-dependent (TDT) episodes within the prior 6 months.

**Treatment:** Patients received rigosertib 200 mg orally twice daily continuously for 4 weeks, followed by an 8-week treatment-free period. A minimum of 7 evaluable patients were enrolled in each of the 3-4 treatments.

**Endpoints:** The primary endpoint was the achievement of transfusion independence (TNI) for ≥12 weeks according to IPSS-R criteria and patient report.

**Results:**

- **Transfusion Independence:**
  - **Continuous dosing:** 50% of evaluable patients (≥12 weeks).
  - **Intermittent dosing:** 50% of evaluable patients (≥12 weeks).

- **Adverse Events:**
  - Most common events included:
    - Urinary tract infections
    - Dysuria
    - Cystitis
  - Two patients experienced serious adverse events:
    - Cystitis
    - Hypomethylating agents complete enrollment

- **Conclusions:**
  - Rigosertib is efficacious and generally well-tolerated in patients with lower risk MDS.
  - Transfusion independence is achieved in a subset of patients.

**Abbreviations:**
- IPSS-R: International Prognostic Scoring System Revised
- TNI: Transfusion Independence
- MDS: Myelodysplastic Syndrome
- ON 01910. Na: Rigosertib

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**Pharmacokinetics:**

**Dosing Schedule:**
- **Continuous dosing:** 200 mg BID
- **Intermittent dosing:** 1 week on/1 week off

**Adverse Events Leading to Treatment Discontinuations:**

- Urinary tract infections
- Dysuria
- Cystitis

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**Overall Transfusion Response:**

- **Continuous:** 2 8 25% 22%
- **Intermittent:** 13 26 50% 38%

**Total:** 15 34 44% 35%

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**CONCLUSIONS:**

- **Strong signal for transfusion independence**
- 50% of evaluable patients in non-del5q patients
- Possible synergy with ESA
- Interim dosing better tolerated
- Strategy for managing Urinary AEs

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**Acknowledgments:** The authors acknowledge the contributions of Dr. A. S. K. (Consortium of Research into Myeloid Disorders, NIH) for her invaluable advice and support.

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**References:**