



KD025-213: Pivotal Trial of KD025 in cGVHD

Interim Analysis Topline Results

November 11, 2019

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CEO Opening Remarks

Harlan W. Waksal, M.D., President and CEO

KD025-213: Interim Analysis Outcomes

- **KD025 met the primary endpoint at the interim analysis of the pivotal trial in cGVHD (KD025-213)**
- **KD025 achieved statistically significant and clinically meaningful Overall Response Rates (ORRs):**
 - 64% ORR with KD025 200 mg QD (95% CI: 51%, 75%)
 - 67% ORR with KD025 200 mg BID (95% CI: 54%, 78%)
- **KD025 has been well tolerated**
 - AEs have been consistent with those expected in this patient population
- **Results from primary analysis expected Q1 2020**
- **Data will be submitted for presentation at an upcoming scientific meeting**

KD025-208: Updated Results

Phase 2 Clinical Trial of KD025 in cGVHD

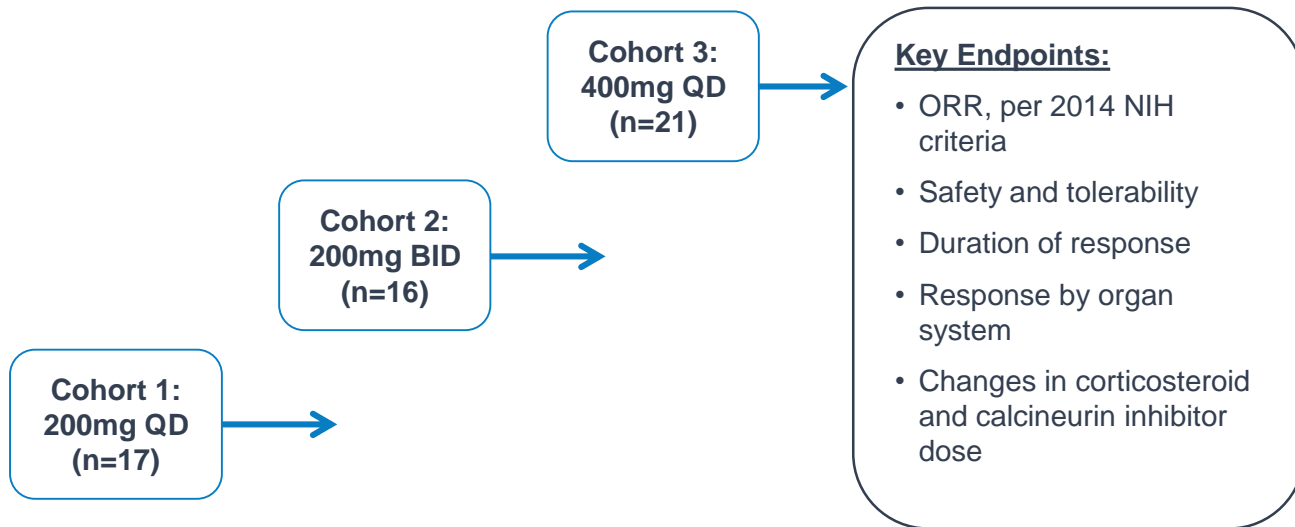
Sanjay Aggarwal, M.D., SVP, Clinical Development

KD025-208: Phase 2a Study of KD025 for Patients with cGVHD

Study initiated September 2016; Conducted at 7 U.S. Sites

Key Eligibility Criteria:

- Adults who have had allogeneic hematopoietic cell transplantation (HCT) with steroid-dependent or steroid-refractory cGVHD
- Have persistent active cGVHD after at least 2 months of steroid therapy
- Receiving glucocorticoid therapy +/- calcineurin inhibitor therapy for cGVHD
- 1-3 prior lines of treatment for cGVHD



Key Endpoints:

- ORR, per 2014 NIH criteria
- Safety and tolerability
- Duration of response
- Response by organ system
- Changes in corticosteroid and calcineurin inhibitor dose

KD025-208: Updated Data With Additional 6 Months of Follow-up

| KD025-208 ORR | | | | |
|---------------|------------------------------|-------------------------------|------------------------------|------------------------|
| | KD025 200 mg QD (n=17) | KD025 200 mg BID (n=16) | KD025 400 mg QD (n=21) | mITT (n=54) |
| ORR 95% CI | 65% (38, 85) | 69% (41, 89) | 62% (38, 82) | 65% (51, 77) |

Data as of March 8, 2019

Clinically Meaningful and Durable Responses

- **ORR of 65% across all 3 cohorts**
 - Responses observed in all organ systems, including in organs with fibrotic disease
 - Median of duration of response: 34 weeks
- **Well tolerated**
 - No apparent increased risk of infection observed
 - 24% of patients have remained on KD025 therapy for >1.5 years as of June 30, 2019

KD025-213: Interim Analysis Results

Pivotal Clinical Trial of KD025 in cGVHD

KD025-213: Ongoing Pivotal Trial of KD025 in cGVHD

KD025-213 (ROCKstar): A Phase 2, Open-Label, Randomized, Multicenter Study to Evaluate the Efficacy and Safety of KD025 in Subjects With cGVHD After At Least 2 Prior Lines of Systemic Therapy

Key Eligibility Criteria:

- Adults and adolescents who have had allogeneic HCT
- Active cGVHD
- Received 2-5 prior lines of systemic therapy for cGVHD

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**KD025 200 mg QD
(n=63)**

**KD025 200 mg BID
(n=63)**

Treat to clinically significant progression

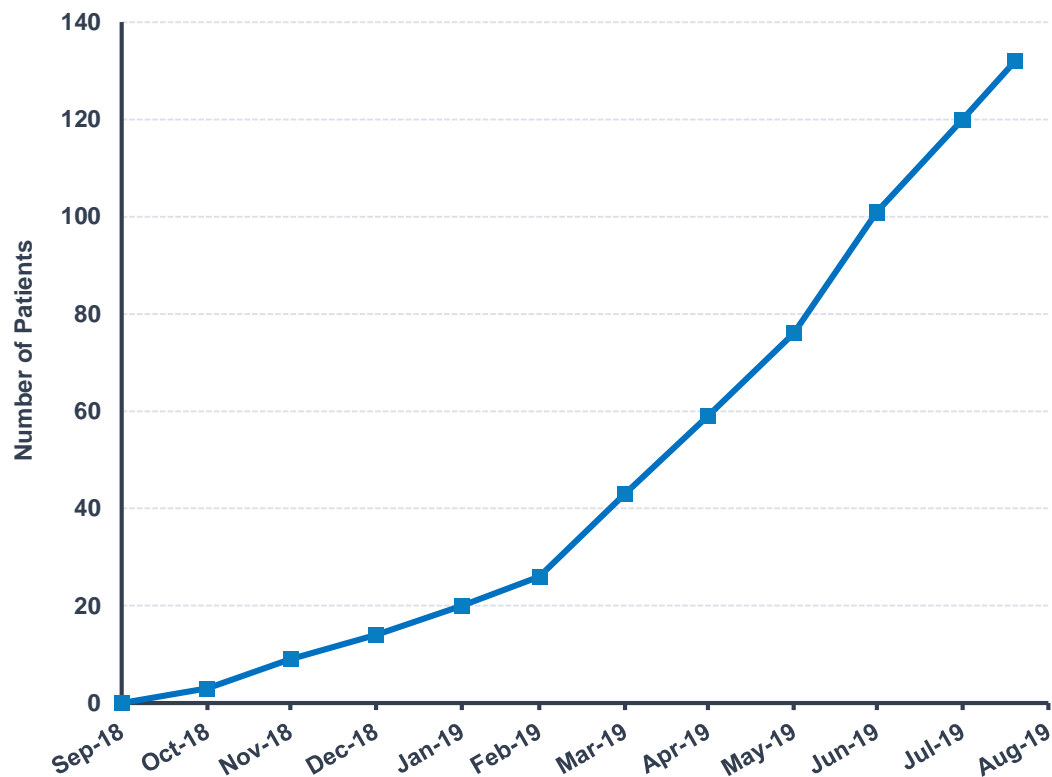
Primary Endpoint:

- ORR, per 2014 NIH criteria

Key Secondary Endpoints:

- Safety
- Duration of response
- Response by organ system
- Lee Symptom Score (QoL measurement)
- Changes in corticosteroid and calcineurin inhibitor dose

KD025-213: Fully Enrolled in Less Than 10 Months



- **Conducted at 32 U.S. sites**
- **First Patient In: Oct 2018**
- **Last Patient In: Aug 2019**
- **Final mITT / safety: n=132**
 - 66 patients per arm

KD025-213: Real-World Patient Population

Demographics and Baseline Characteristics

| Demographics | KD025 200 mg QD (n=66) | KD025 200 mg BID (n=66) |
|---|------------------------|-------------------------|
| Median age [years (range)] | 53 (21-77) | 57 (21-77) |
| Male (%) | 64 | 50 |
| Median prior lines of therapy | 3 | 4 |
| Median time from cGVHD diagnosis to enrollment (months) | 25 | 30 |
| ≥4 Organs Involved [n (%)] | 34 (52%) | 35 (53%) |
| Median prednisone dose (mg/kg/day) | 0.2 | 0.2 |
| Stratification Factors: | | |
| Severe cGVHD [n (%)] | 45 (68%) | 42 (64%) |
| Prior Ibrutinib Treatment | 23 (35%) | 22 (33%) |

KD025-213: Statistical Analysis Plan

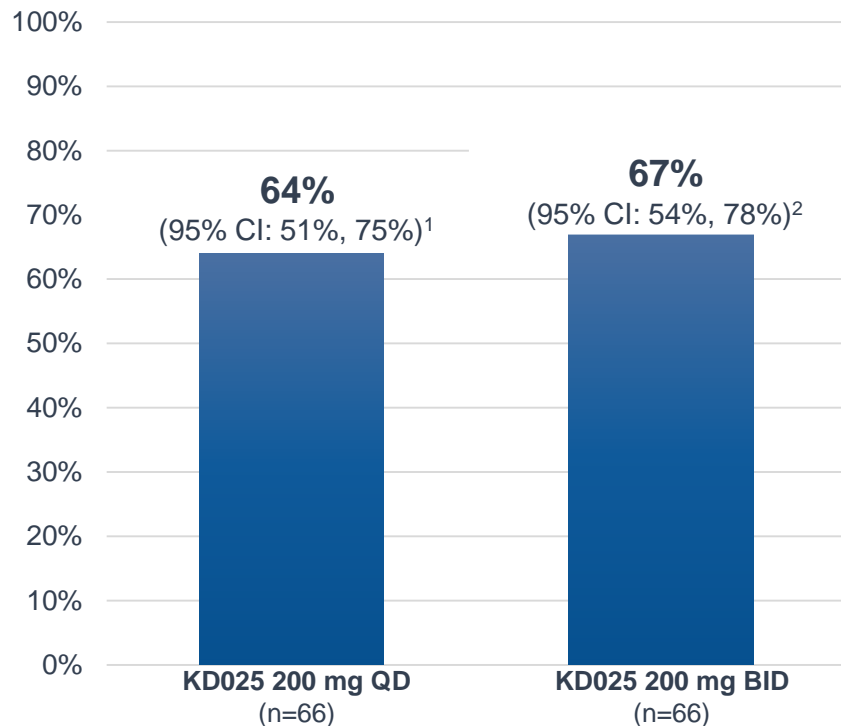
Primary Endpoint: ORR

Statistical significance is achieved if the lower bound of the 95% CI of ORR exceeds 30%

| | Timepoint | Status |
|-------------------------|--|-------------------------------|
| Interim Analysis | 2 months after completion of enrollment (Oct 2019) | Reported November 2019 |
| Primary Analysis | 6 months after completion of enrollment (Feb 2020) | Planned Q1 2020 |

KD025-213 Met Primary Endpoint at Interim Analysis

ORR Results Have Exceeded Threshold For Success



- **KD025 achieved clinically and statistically significant ORRs in both arms**
- **KD025 has been well tolerated**
 - AEs have been consistent with those expected in this patient population

¹p<0.0001; ²p<0.0001

Closing Remarks

Harlan W. Waksal, M.D., President and CEO

KD025 in cGVHD: Path Forward

- **KD025 met the primary endpoint at interim analysis of pivotal trial in cGVHD (KD025-213)**
 - 64% ORR with KD025 200 mg QD (95% CI: 51%, 75%)
 - 67% ORR with KD025 200 mg BID (95% CI: 54%, 78%)
- **FDA granted Breakthrough Therapy Designation to KD025 in cGVHD after at least 2 prior lines of systemic therapy (Oct 2018)**
- **Pre-NDA meeting with FDA for KD025 in cGVHD anticipated Q1 2020**
- **Results from primary analysis expected Q1 2020**
- **Data will be submitted for presentation at an upcoming scientific meeting**
- **Kadmon plans to file an NDA for KD025 in cGVHD in 2020, subject to FDA feedback**

Q&A