



ROCKstar: Pivotal Trial of Belumosudil (KD025) in cGVHD

KD025-213 Primary Analysis Topline Results

May 21st, 2020

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CEO Opening Remarks

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

ROCKstar (KD025-213): Primary Analysis Outcomes

- **Belumosudil (KD025) met the primary endpoint of Overall Response Rate (ORR) in the pivotal trial in cGVHD patients who have received 2 or more prior lines of systemic therapy:**
 - **73% ORR** with KD025 200 mg QD (95% CI: 60%, 83%)
 - **74% ORR** with KD025 200 mg BID (95% CI: 62%, 84%)
- **Responses observed across all key patient subgroups**
- **Durable responses**
 - 49% of responders have maintained responses for ≥ 20 weeks
 - Median duration of response (DOR) has not yet been reached
- **Well tolerated**
 - AEs have been consistent with those expected in this patient population

CI: Confidence Interval

ROCKstar Primary Analysis Results

KD025-213: Pivotal Clinical Trial of Belumosudil (KD025) in cGVHD

Sanjay Aggarwal, M.D., Senior Vice President, Clinical Development

ROCKstar: Pivotal Trial of Belumosudil (KD025) in cGVHD

ROCKstar (KD025-213): 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

- Ages ≥ 12
- 2-5 prior lines of systemic therapy for cGVHD
- Systemic therapy for cGVHD is indicated

R

**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 (DOR)
- Lee Symptom Score (QoL measurement)
- Changes in corticosteroid and calcineurin inhibitor dose
- Failure-free Survival (FFS)
- Overall Survival (OS)

ROCKstar: 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	<i>Primary endpoint met at interim analysis</i> Data presented at TCT, February 2020
Primary Analysis	6 months after completion of enrollment	Topline data announced May 2020
1-year Follow Up	12 months after completion of enrollment	Available Q4 2020

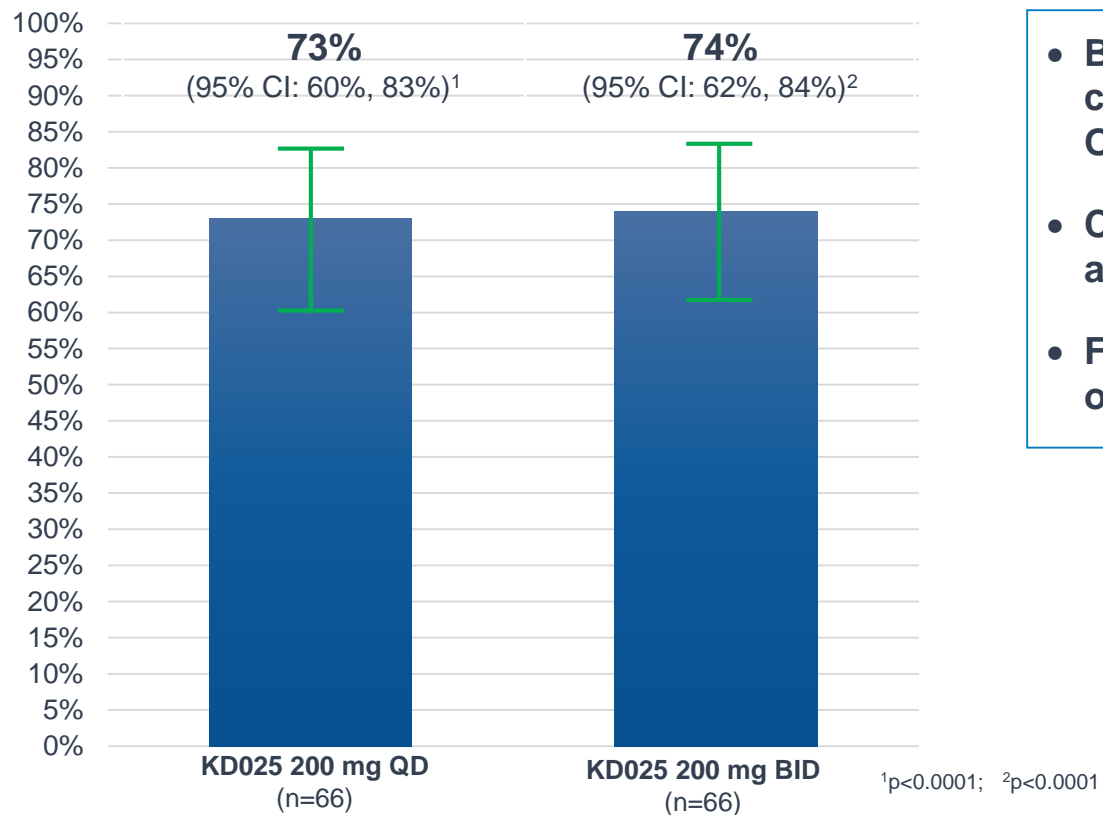
ROCKstar: Advanced Patient Population

Demographics and Baseline Characteristics

Demographics	KD025 QD (n=66)	KD025 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
NIH Severe cGVHD ¹ [n (%)]	45 (68%)	42 (64%)
Median prednisone dose (mg/kg/day)	0.2	0.2
≥4 organs involved [n (%)]	33 (50%)	35 (53%)
Prior ibrutinib treatment ¹	22 (33%)	24 (36%)
Prior ruxolitinib treatment	20 (30%)	18 (27%)
Refractory to line prior to enrollment, excluding unknown / missing	80% (45/56)	64% (34/53)

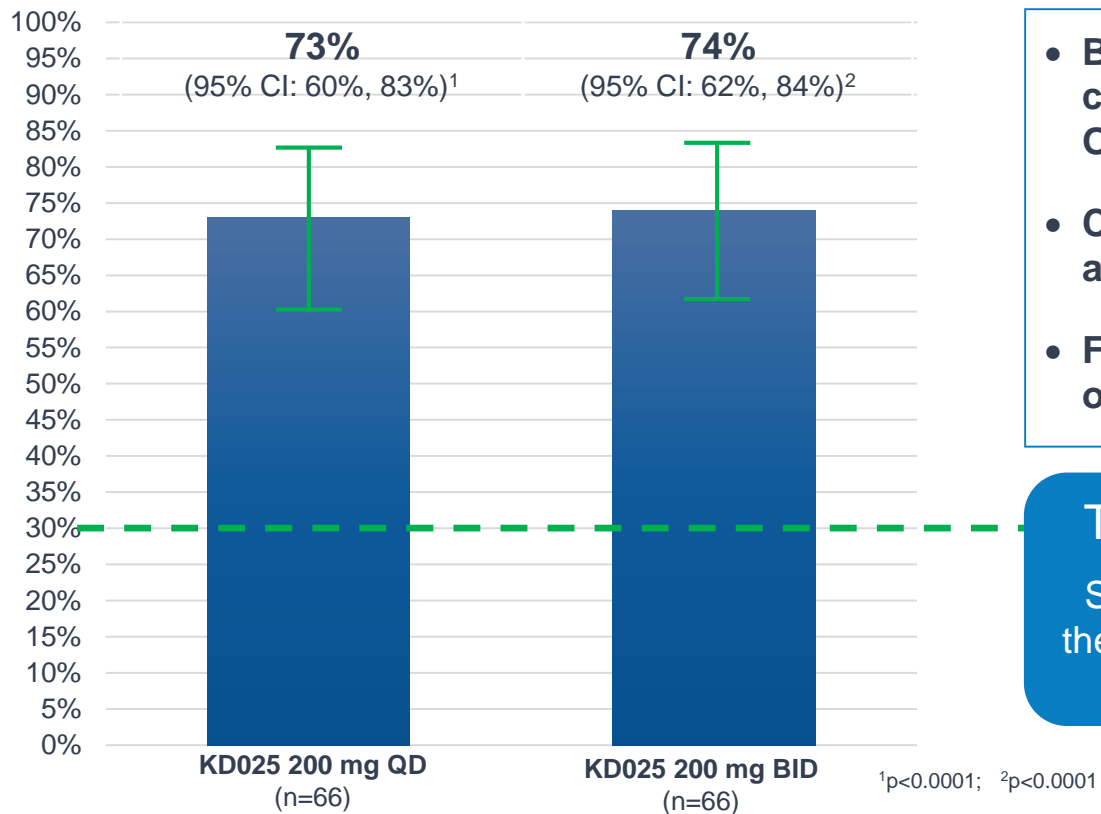
¹Stratification factor

ROCKstar Met Primary Endpoint



- Belumosudil (KD025) achieved clinically and statistically significant ORRs in both arms
- Complete responses have observed in all affected organ systems
- Four patients have achieved an overall complete response

ROCKstar Met Primary Endpoint

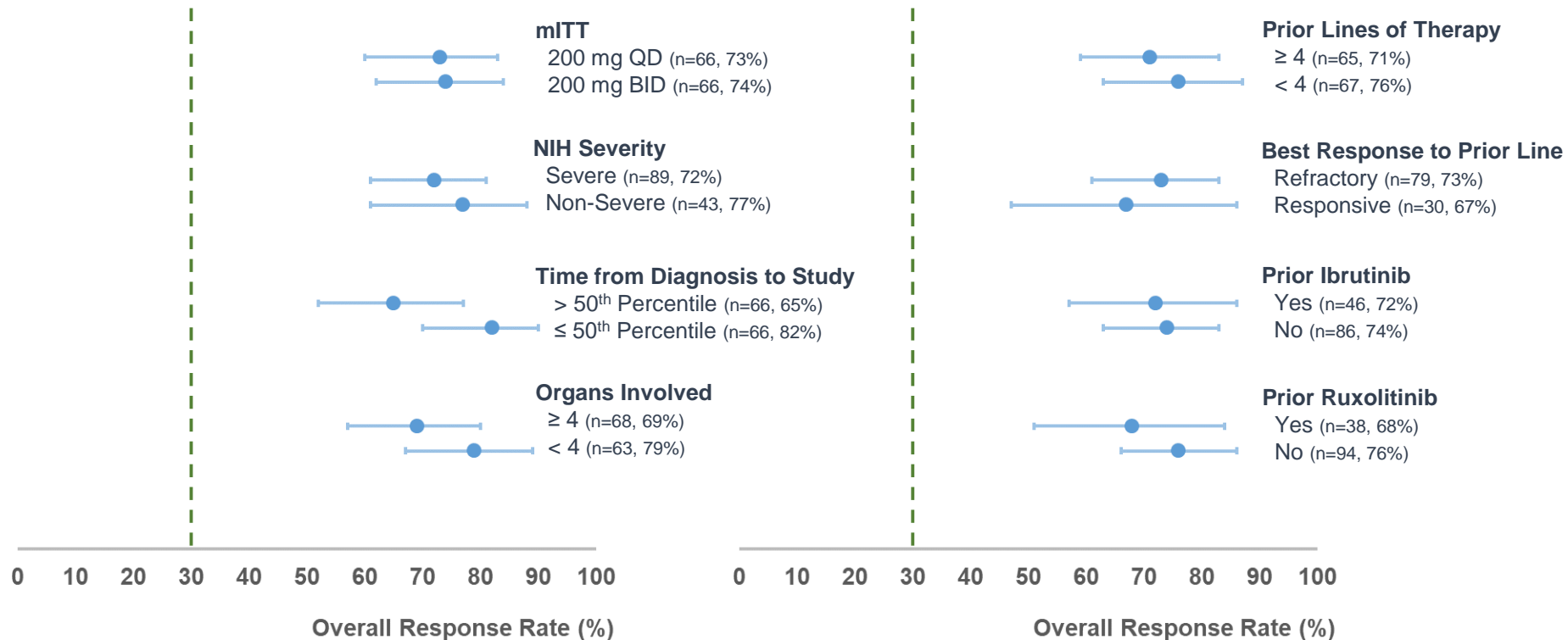


- Belumosudil (KD025) achieved clinically and statistically significant ORRs in both arms
- Complete responses have observed in all affected organ systems
- Four patients have achieved an overall complete response

Threshold for success: 30%

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

ROCKstar: Responses Observed Across All Key Subgroups



Pooled responses across arms, unless arms are stated

ROCKstar: Durability of Response

- **Durable responses achieved with belumosudil (KD025):**

- 49% of responders have maintained responses for ≥ 20 weeks
- Median duration of response (DOR) has not yet been reached
- ROCKstar durability data continue to mature

- **In earlier Phase 2a trial of belumosudil (KD025) in cGVHD (KD025-208) with a median follow-up of 2 years¹:**

- Median DOR of 35 weeks
- 43% of patients have remained on belumosudil for >1 year
- 28% of patients have remained on belumosudil for >1.5 years

**ROCKstar Primary Analysis
Data Cutoff (six months after
completion of enrollment):**

- Median duration of treatment: 29 weeks
- Median follow-up: 34 weeks

¹ Data as of February 19, 2020.

ROCKstar: Safety and Tolerability

Commonly Reported AEs, n (%)	KD025 QD (n=66)	KD025 BID (n=66)
All Grade, in ≥20%		
Fatigue	26 (39)	16 (24)
Diarrhea	20 (30)	18 (27)
Nausea	17 (26)	17 (26)
Cough	19 (29)	13 (20)
Dyspnea	20 (30)	11 (17)
URTI	14 (21)	16 (24)
Liver-related investigations (SMQB)	12 (18)	16 (24)
Peripheral edema	17 (26)	11 (17)

- AEs were overall consistent with those expected in cGVHD patients receiving corticosteroids and other immunosuppressants
- No CMV infection or reactivation observed
- No significant drug-related cytopenias reported

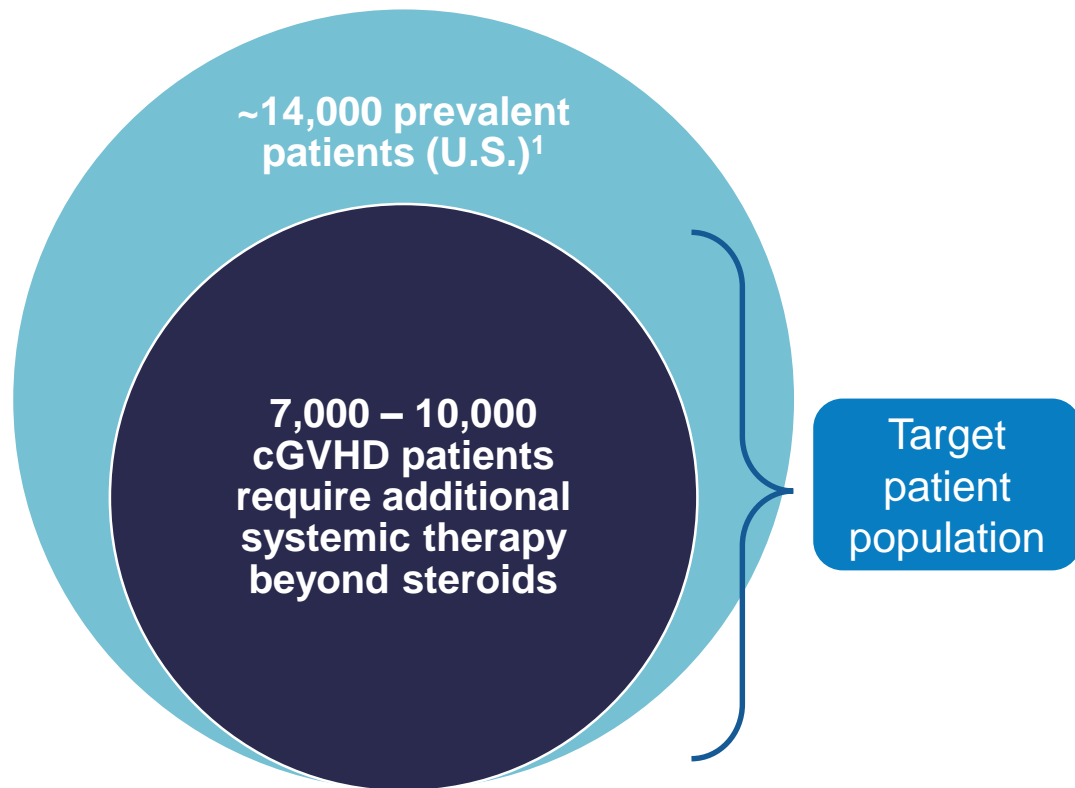
ROCKstar Pivotal Trial: Primary Analysis Summary

- **Belumosudil (KD025) met the primary endpoint in pivotal trial in cGVHD:**
 - 73% and 74% ORR with belumosudil 200 mg QD and 200 mg BID, respectively
- **49% of responders have maintained responses for ≥ 20 weeks**
 - Median DOR has not yet been reached; durability data continue to mature
- **Well tolerated**
 - No CMV infection or reactivation observed
 - No significant drug-related cytopenias reported
- **Data to be submitted for presentation at an upcoming medical meeting**

Commercial Overview

Haya Taitel, RPh, Senior Vice President, Chief Commercial Officer

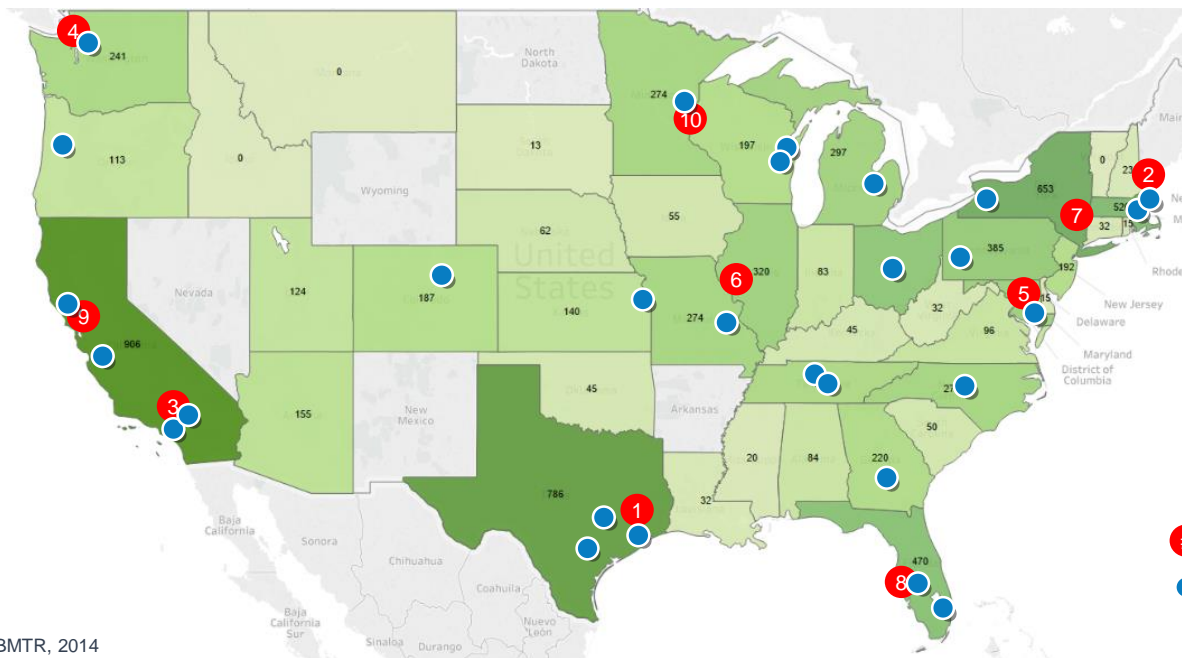
Significant cGVHD Market with Unmet Medical Needs



- **Significant cGVHD market**
 - 5,000 new cGVHD patients/year¹
 - 5-year overall survival of 55%²
- **Unmet therapeutic needs**
 - Steroids are standard of care in frontline treatment¹
 - 80% of cGVHD patients require additional treatment beyond initial therapy³
 - Patients cycle through lines of therapy every 2-4 months¹

Patients are Concentrated at Academic Transplant Centers

80% of cGVHD Patients are Treated at 70 Transplant Centers



Efficient Field Footprint
<15 total field associates to address cGVHD market

- # Top Allo-HCT center by volume
- KD025-213 trial site

Dark green indicates higher allo-HCT volume

Commercial Infrastructure in Place

Our efficient commercial model is optimized for profitable growth

Marketing & Sales
Market Access
Customer Service

Medical Affairs
Pharmacovigilance

Business Analytics
Commercial
Finance

CMC
Supply Chain

Legal / Compliance

Regulatory Affairs
Quality Assurance



Kadmon Pharmaceuticals: Commercial operation based in Warrendale, PA

Components in Place to Launch and Commercialize KD025



**Concentrated
Market**



**Experienced
Team**



**Existing
Commercial
Infrastructure**



**Strategic
Launch Plan**

Belumosudil in cGVHD: Path Forward

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

Belumosudil: A Novel cGVHD Treatment

Observed in KD025-208 and ROCKstar (KD025-213) Clinical Trials



ROCK2 inhibitor – novel dual modulator of inflammatory and fibrotic processes



Efficacy – high response rates in advanced cGVHD patients



Safety – well tolerated; 43% of patients have remained on treatment for 1 year¹



Improvement in QOL

¹ KD025-208 follow-up data

Belumosudil (KD025) in cGVHD: Path Forward

- **NDA submission for belumosudil (KD025) in cGVHD planned Q4 2020**
 - Pre-NDA meeting with FDA took place in March 2020
 - Belumosudil is being reviewed under Real-Time Oncology Review (RTOR) Pilot Program
 - FDA Breakthrough Therapy Designation and Orphan Drug Designation
- **Belumosudil (KD025) has broad therapeutic potential beyond cGVHD, including:**
 - Systemic sclerosis (Phase 2 clinical trial ongoing)
 - Bronchiolitis obliterans (BOS)
 - Other immune disorders

Q&A