# One Mission, Inspired by Patients: Defeat Cancer.™

September 17, 2021





# OPENING REMARKS



## **Steve L. Hoerter**

President and Chief Executive Officer



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# INNOVATIVE PROGRAMS LEADING TO TRANSFORMATIVE GROWTH

Executing on our mission to discover, develop, and deliver important new medicines to patients for the **treatment of cancer** 



- Phase 3 data in 2<sup>nd</sup> line GIST (INTRIGUE) expected in 4Q 2021
- EU approval in 4<sup>th</sup> line GIST expected in 4Q 2021

### Positioned to Rapidly Advance Clinical Pipeline<sup>1</sup>

**Vimseltinib**, our potential best-in-class CSF1R inhibitor, Phase 3 MOTION study initiation expected in 4Q 2021

**Rebastinib**, our first-in-class TIE2 inhibitor, in combination with paclitaxel Phase 3 study initiation expected in 2022 DCC-3116, our first-in-class ULK program, initial data from the Phase 1 dose escalation expected in 2022



Notes: CSF1R=colony-stimulating factor 1 receptor; EU=European Union; GIST=gastrointestinal stromal tumor; TIE2=TEK tyrosine kinase; ULK=unc-51-like autophagy-activating kinase; (1) Represent planned 2021 and 2022 milestones.

## TODAY'S AGENDA



**Steve L. Hoerter** *President and Chief Executive Officer* 

### REBASTINIB DATA UPDATE FROM PHASE 1B/2 STUDY IN COMBINATION WITH PACLITAXEL IN PLATINUM RESISTANT OVARIAN CANCER (PROC)

**Robert L. Coleman, M.D., FACOG, FACS** *Gynecologic Oncologist and Chief Scientific Officer for US Oncology Research* 

## UNMET MEDICAL NEED AND EXPECTED MILESTONES

Matthew L. Sherman, M.D. Executive Vice President and Chief Medical Officer

### **REBASTINIB Q&A**

#### VIMSELTINIB DATA UPDATE FROM THE PHASE 1/2 STUDY IN TENOSYNOVIAL GIANT CELL TUMOR (TGCT)

**William D. Tap, M.D.** Chief of the Sarcoma Medical Oncology Service at Memorial Sloan Kettering Cancer Center

### **VIMSELTINIB PHASE 3 MOTION STUDY**

Matthew L. Sherman, M.D. Executive Vice President and Chief Medical Officer

### TGCT MARKET DYNAMICS AND VIMSELTINIB OPPORTUNITIES

**Daniel C. Martin** Senior Vice President and Chief Commercial Officer

### VIMSELTINIB Q&A

### **CLOSING REMARKS**

**Steve L. Hoerter** *President and Chief Executive Officer* 



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# REBASTINIB DATA UPDATE FROM PHASE 1B/2 STUDY IN COMBINATION WITH PACLITAXEL IN PROC



## Robert L. Coleman, M.D., FACOG, FACS

*Gynecologic Oncologist and Chief Scientific Officer for US Oncology Research* 



## OVARIAN CANCER: NATURAL HISTORY



# MOVING BEYOND THE PLATINUM-SENSITIVE /-RESISTANT PARADIGM

An emerging classification system for recurrent disease based on an increased understanding of the biology of ovarian cancer

### **Emerging new multiplex classification system**



## TREATMENT FREE INTERVAL AND ORR, PFS, AND OS



## UNMET MEDICAL NEED: TREATMENT EXPECTATIONS

International Journal of Gynecological Cancer • Volume 21, Number 1, January 2011 Multiple Lines of Chemotherapy: Outcomes

TABLE 2. Efficacy of all lines of therapy in the platinum-resistant/refractory setting (total lines therap	oy = 689)
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	Line of Therapy After Platinum Resistance				
	First	Second	Third	Fourth	Fifth+
n	274	196	127	62	30
Radiological response rate (CR + PR), %	15.7 <b>12-21%</b>	8.1 <b>5-13</b>	<b>%</b> <sup>1</sup> 3.1	<b>1-8%</b> <sup>1</sup> 1.6 <b>0-8%</b> <sup>1</sup>	0
Clinical benefit rate (CR, PR + SD), %	36.9	30.6	18.1	17.7	3.3
Serological response rate, %	49.3	37.1	32.2	23.7	13.3
PFI, median (95% CI), wk	18 (15–21)	16 (14–18)	13 (10–16)	13 (8–17)	8 (7–9)
OS, median (95% CI), wk	61 (53–69)	48 (40–56)	40 (33–47)	38 (22–53)	26 (21–31)

# A HIGHLY POTENT AND SELECTIVE TIE2 INHIBITOR

### TIE2 SIGNALING ACTS AS A REGULATOR OF TUMOR ANGIOGENESIS, INVASIVENESS, AND METASTASIS



- Rebastinib is a first-in-class investigational, orally administered, potent, and selective switch-control inhibitor of the TIE2 kinase
- TIE2 is the receptor for angiopoietins ANG-1 and ANG-2, an important family of vascular growth factors
- TIE2 receptors are expressed on endothelial cells and angiogenic macrophages, promoting the survival, maturation, and functional integrity of the vasculature; they play an important role in regulating tumor angiogenesis, invasiveness, and metastasis
- Rebastinib binds potently into the switch pocket of TIE2, stabilizing the inhibitory switch and displacing the activation switch to block TIE2 signaling<sup>1</sup>

# REBASTINIB IN COMBINATION WITH PACLITAXEL | PHASE 1B/2 STUDY STUDY DESIGN





Notes: BID=twice daily; (1) 80 mg/m2 IV infusion over 60 minutes weekly (day 1, day 8, and day 15 of repeated 28-day cycles); (2) Triple negative breast cancer, inflammatory breast cancer, and gynecological carcinosarcoma cohorts did not advance to Stage 2.

### REBASTINIB IN COMBINATION WITH PACLITAXEL | PHASE 1B/2 STUDY | PLATINUM-RESISTANT OVARIAN CANCER COHORT PATIENT DEMOGRAPHICS AND DISPOSITION



Seromucinous

**Endometrioid** 

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Notes: AE=adverse event; BID=twice daily; BRCA=breast cancer gene; mITT=modified intent-to-treat; PARP=poly adenosine diphosphate-ribose polymerase; (1) Patients who discontinued due to withdrawal of consent or an unrelated AE were excluded because they did not have a post baseline assessment; (2) Of the 2 patients who did not meet eligibility criteria, 1 had non-measurable disease at baseline and the other did not have ovarian cancer; (3) Includes one patient whose histology was classified as "Other, high-grade serious".

8 (21%) BRCA+

**Anti-PARP** 

Other

### REBASTINIB IN COMBINATION WITH PACLITAXEL | PHASE 1B/2 STUDY | PLATINUM-RESISTANT OVARIAN CANCER COHORT ENCOURAGING ANTI-TUMOR ACTIVITY



### Best Percent Change from Baseline in Tumor Size<sup>4,5</sup>







Notes: Data presented at the ESMO Congress 2021; Results are reported for patients in the platinum-resistant ovarian cancer expansion cohort with a cutoff date of June 22, 2021; safety population n=38, modified intent-to-treat population n=34; CBR=clinical benefit rate; ORR=objective response rate; (1) Includes 13 patients with objective response (10 confirmed, 3 to be confirmed at future follow-up), 18 stable disease, and 1 progressive disease; (2) Overall, 13 patients (34%) discontinued due to radiological PD, 9 patients (24%) discontinued due to an AE, 7 patients (18%) discontinued due to clinical PD, 2 patients (5%) chose to withdraw, and 1 patient (3%) died due to causes unrelated to rebastinib; (3) Clinical benefit rate at 16 weeks was defined as overall response of CR, PR, or SD according to RECIST v1.1 at the 16-week response essesments, respectively; (4) Patients with ≥1 post baseline radiological assessment are shown (n = 32); plot includes confirmed and unconfirmed responses; (5) Dotted lines dence 30% decrease and 20% increase in tumor size cutoffs for PR and PD, respectively.

# REBASTINIB IN COMBINATION WITH PACLITAXEL | PHASE 1B/2 STUDY | PLATINUM-RESISTANT OVARIAN CANCER COHORT PROMISING PROGRESSION-FREE SURVIVAL



mPFS of 9.1 months, ORR of 38%<sup>1</sup> (confirmed and unconfirmed), and 29%<sup>1</sup> (confirmed) were promising when considering benchmark data of single agent paclitaxel in PROC setting

BENCHMARK DATA<sup>2-4</sup>

mPFSORR3-4 months15%–25%



Notes: Data presented at the ESMO Congress 2021; Results are reported for patients in the platinum-resistant ovarian cancer expansion cohort with a cutoff date of June 22, 2021; safety population n=38, modified intent-to-treat population n=34; Cl= confidence interval; mPFS=median progression-free survival; NE=non-estimable; ORR=overall response rate; PROC=platinum-resistant ovarian cancer; (1) Includes 13 patients with objective response (10 confirmed, 3 to be confirmed at future follow-up), 18 stable disease, and 1 progressive disease; (2) Poveda AM, et al. *J Clin Oncol.* 2015;33:3836–38; (3) Oza A, et al. *Gynecol Oncol.* 2018;149:275–82; (4) Matulonis UA, et al. *Gynecol Oncol.* 2019;152:548–53.

### REBASTINIB IN COMBINATION WITH PACLITAXEL | PHASE 1B/2 STUDY | PLATINUM-RESISTANT OVARIAN CANCER COHORT ENCOURAGING TOLERABILITY PROFILE

### Table 5. Summary of treatment-emergent AEs ≥15% regardless of relatedness (n = 38)

Preferred term	Any grade	Grade 3	Preferred term	Any grade	Grade 3
Fatigue	22 (58%)	3(8%)	Hypomagnesemia	8 (21%)	0
Alopecia	16 (42%)	1 (3%) <sup>1</sup>	Urinary tract infection	8 (21%)	1 (3%)
Edema peripheral	15 (39%)	2(5%)	Abdominal distension	7 (18%)	0
Dry mouth	14 (37%)	0	Anemia	7 (18%)	1 (3%)
Nausea	14 (37%)	1 (3%)	Decreased appetite	7 (18%)	0
Peripheral sensory neuropathy	14 (37%)	0	Hypokalemia	7 (18%)	1 (3%)
Constipation	12 (32%)	0	Vomiting	7 (18%)	1 (3%)
Diarrhea	12 (32%)	2(5%)	Arthralgia	6 (16%)	0
Hypertension	12 (32%)	3(8%)	Cough	6 (16%)	0
Abdominal pain	11 (29%)	2(5%)	Dry eye	6 (16%)	0
Muscular weakness	10 (26%)	3(8%) <sup>2</sup>	Headache	6 (16%)	0
Stomatitis	10 (26%)	0	Nail discoloration	6 (16%)	0
Dyspnea	9 (24%)	1 (3%)	Pain in extremity	6 (16%)	1 (3%)
Dizziness	8 (21%)	0			

### Most AEs reported were Grade ≤2

- Four patients (11%) experienced five serious AEs at least possibly related to rebastinib:
- Grade 3 reversible muscular weakness (n = 2; 5%, occurred at 50 mg and 75 mg BID)
- Grade 2 constipation (n = 1; 3%)
- Grade 3 fatigue (n = 1; 3%)
- Grade 3 urinary tract infection (n = 1; 3%)



Notes: Data presented at the ESMO Congress 2021; Results are reported for patients in the platinum-resistant ovarian cancer expansion cohort with a cutoff date of June 22, 2021; safety population n=38, modified intent-to-treat population n=34; AE=adverse event; BID=twice daily; CTCAE=common terminology criteria for adverse events; SAE=serious AE; (1) Grade 3 alopecia is not in CTCAE, site queried and updated to Grade 2; (2) One patient had Grade 3 muscular weakness that was considered related to rebastinib but was not entered as an SAE. This event occurred at 100 mg BID.

# REBASTINIB IN COMBINATION WITH PACLITAXEL | PHASE 1B/2 STUDY | PLATINUM-RESISTANT OVARIAN CANCER COHORT PROMISING RESULTS

The updated safety and efficacy of rebastinib at 50 mg BID in combination with paclitaxel shows encouraging results in heavily pretreated patients with PROC, with an acceptable safety profile, supporting further development.





**Notes:** Data presented at the ESMO Congress 2021; Results are reported for patients in the platinum-resistant ovarian cancer expansion cohort with a cutoff date of June 22, 2021; safety population n=38, modified intent-to-treat population n=34; AE=adverse event; BID= twice daily; PFS=progression-free survival; PROC=platinum resistant ovarian cancer; (1) Includes 13 patients with objective response (10 confirmed, 3 to be confirmed at future follow-up), 18 stable disease, and 1 progressive disease; (2) Clinical benefit rate at 16 weeks was defined as overall response of CR, PR, or SD according to RECIST v1.1 at the 16-week response assessments, respectively.

# UNMET NEED AND EXPECTED MILESTONES



## Matthew L. Sherman, M.D.

Executive Vice President and Chief Medical Officer



# LIMITED TREATMENT OPTIONS WITH POOR OUTCOMES

### **Disease Summary**

- 22,000 incident cases a year in women in the U.S.<sup>1</sup>
- In 2020, ~14,000 women died from ovarian cancer in the U.S.<sup>1</sup>

### **Unmet Medical Need**

- Vast majority of patients experience disease recurrence
- Patients that experience disease recurrence eventually develop platinumresistant ovarian cancer (PROC)
- Outcomes are particularly poor for patients with PROC, driving the need for more effective therapies



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tes: PARPi=poly-ADP ribose polymerase inhibitor; (1) ACS Cancer Facts & Figures 2020; (2) Lou E. Et al. AMA Oncol. 2019;5(8):1222-1224; (3) Tinker D. et al. Gynecol Oncol. 2014;133(2):624-631.

### REBASTINIB IN COMBINATION WITH PACLITAXEL | PHASE 1B/2 STUDY | PLATINUM-RESISTANT OVARIAN CANCER COHORT PROMISING RESULTS SUPPORT FURTHER DEVELOPMENT

The updated safety and efficacy of rebastinib at 50 mg BID in combination with paclitaxel shows encouraging results in heavily pretreated patients with PROC, with an acceptable safety profile, supporting further development.

Pivotal Phase 3 study in PROC is anticipated to start in 2022, subject to discussions with health authorities

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**Notes:** Data presented at the ESMO Congress 2021; results are reported for patients in the platinum-resistant ovarian cancer expansion cohort with a cutoff date of June 22, 2021; safety population n=38, modified intent-to-treat population n=34; AE=adverse event; BID= twice daily; PFS=progression-free survival; PROC=platinum resistant ovarian cancer; (1) Includes 13 patients with objective response (10 confirmed, 3 to be confirmed at future follow-up), 18 stable disease, and 1 progressive disease; (2) Clinical benefit rate at 16 weeks was defined as overall response of CR, PR, or SD according to RECIST v1.1 at the 16-week response assessments, respectively.

# REBASTINIB Q&A



# VIMSELTINIB DATA UPDATE FROM THE PHASE 1/2 STUDY IN TGCT



## William D. Tap, M.D.

*Chief of the Sarcoma Medical Oncology Service at Memorial Sloan Kettering Cancer Center* 



### TENOSYNOVIAL GIANT CELL TUMOR (TGCT) A LOCALLY AGGRESSIVE TUMOR ASSOCIATED WITH SUBSTANTIAL MORBIDITY





	Disease Burden and Unmet Need for TGCT Patients
Disease characteristics	<ul> <li>Typically occurs in people 30-50 years old<sup>1</sup></li> <li>Genetic translocation causes overproduction of CSF1, triggering migration of inflammatory cells including CSF1R-expressing tumor-associated macrophages (TAMs) to tumor sites<sup>2</sup></li> </ul>
Diagnosis	Due to the slow-growing nature of TGCT and similar symptoms to other conditions like sports injuries and arthritis, patients may have a longer path to diagnosis
Common locations <sup>3</sup>	Knees   Hips   Ankles   Elbows   Shoulders
Patient burden	In the TOPP registry <sup>4</sup> , patients at baseline commonly reported multiple symptoms, including pain (78%), limited function (64%), swelling (61%), stiffness (55%), and use of analgesics (15%) <sup>5</sup>
Unmet need	<ul> <li>Surgical resection is standard treatment</li> <li>High rate of recurrence in diffuse TGCT</li> <li>CSF1R inhibition has demonstrated clinical benefit in diffuse TGCT</li> <li>Pexidartinib is only approved agent for TGCT patients no longer amenable to surgery (FDA approval in August 2019)         <ul> <li>FDA label includes boxed warning, REMS, and intensive liver monitoring due to off-target hepatotoxicity risks</li> <li>The EMA adopted the decision of refusal of the Turalio MAA in November 2020</li> </ul> </li> <li>Unmet medical need for effective CSF1R inhibitor with favorable safety/tolerability profile for TGCT patients</li> </ul>



Ankle image courtesy of Tap WD, et al. ASCO 2018, abstract 11502; Knee image courtesy of Tap WD, et al. N Engl J Med. 2015;373:428-437. **Notes:** CSF1=colony-stimulating factor 1; CSF1R=colony-stimulating factor 1 receptor; EMA=European Medicines Agency; FDA=U.S. Food and Drug Administration; MAA=Marketing Authorisation Application; REMS=Risk Evaluation and Mitigation Strategy; TGCT=tenosynovial giant cell tumor; (1) Mastboom et al. Acta Orthopaedica. 2017;88:688-694; (2) West et al. Proc Natl Sci USA. 2006; 103:690-695; (3) Common locations are specific to diffuse TGCT; (4) The TOPP registry was a multinational, multicenter, prospective observational study involving 12 tertiary sarcoma centers in 7 European countries, and 2 US sites; (5) Patients experienced more than or equal to 3 symptoms (52%).

### TENOSYNOVIAL GIANT CELL TUMOR (TGCT) PATIENT JOURNEY OF TGCT PATIENT NOT AMENABLE TO SURGERY





Source: Spierenburg, G. The Diffuse-Type TGCT Patient Journey: A Prospective Multicenter Study. Poster presented at: 2020 Connective Tissue Oncology Society (CTOS) Virtual Annual Meeting; November 18 – 21, 2020. Notes: Ortho=orthopedic; PCP=primary care physician; Rheum=rheumatologist; TGCT=tenosynovial giant cell tumor.

### VIMSELTINIB | TENOSYNOVIAL GIANT CELL TUMOR (TGCT) POTENTIAL BEST-IN-CLASS CSF1R INHIBITOR IN DEVELOPMENT



- Vimseltinib is an oral, switch-control TKI specifically designed to selectively and potently inhibit CSF1R
- Phase 1/2 study is ongoing in patients with solid tumors and TGCT
  - Enrollment complete for Cohort A (patients with no prior anti-CSF1/CSF1R therapy)
  - Enrollment ongoing for Cohort B (patients with prior anti-CSF1/CSF1R therapy)
- The recommended Phase 2 dose for vimseltinib in TGCT patients was determined to be 30 mg twice weekly



Notes: CSF1R=colony-stimulating factor 1 receptor; TGCT=tenosynovial giant cell tumor; TKI=tyrosine kinase inhibitor.

# VIMSELTINIB | ONGOING PHASE 1/2 STUDY OF PATIENTS WITH TGCT STUDY DESIGN

Enrollment in Phase 1 dose escalation is complete and ongoing in Phase 2 at the RP2D (30 mg twice weekly with no loading dose).

	Loading doses	Dose	
Cohort 5	30 mg QD x 5 days	30 mg twice weekly	
Cohort 8	30 mg QD x 3 days	10 mg QD	
Cohort 9	20 mg QD x 3 days	6 mg QD	
Expansion	NA	30 mg twice weekly	

Data presented at the ESMO Congress 2021 is from the Phase 1 dose escalation portion of the study and from Cohort A in the Phase 2 expansion portion of the study.





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Notes: Data presented at the ESMO Congress 2021; Results are reported for patients with TGCT with a data cutoff of June 7, 2021; EDC=electronic data capture; QD=once daily; RP2D=recommended Phase 2 dose; TGCT=tenosynovial giant cell tumor; (1) At least one post-baseline efficacy assessment; (2) 1 patient withdrew prior to evaluation; (3) 1 patient pending dosing data in the database.

### VIMSELTINIB | ONGOING PHASE 1/2 STUDY OF PATIENTS WITH TGCT BASELINE CHARACTERISTICS

		Phase 1 TGCT patients (n = 32)	Phase 2 Cohort A patients (n = 36)
Median A	age, years (range)	51 (23–73)	44 (21–71)
Sex			
	Female	17 (53%)	26 (72%)
	Male	15 (47%)	10 (28%)
Race			
	White	31 (97%)	28 (78%)
	Asian	1 (3%)	2 (6%)
	Not Reported or Missing	0	6 (17%)
Disease location			
	Knee	20 (63%)	20 (56%)
	Ankle	5 (16%)	5 (14%)
	Нір	4 (13%)	2 (6%)
	Foot	1 (3%)	6 (17%)
	Other <sup>1</sup>	2 (6%)	3 (8%)
Patients with at least one prior surgery		12 (38%)	32 (89%)
Patients with at least one prior systemic therapy		5 (16%)	2 (6%)
	Imatinib or nilotinib	4 (13%)	2 (6%)
	Lacnotuzumab (MCS-110)	1 (3%)	0





Notes: Data presented at the ESMO Congress 2021; Results are reported for patients with TGCT with a data cutoff of June 7, 2021; TGCT=tenosynovial giant cell tumor; Data are presented as n (%) unless otherwise noted; Percentages might not add up to 100% due to rounding; (1) Other locations include wrist, shoulder, and jaw.

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### VIMSELTINIB | ONGOING PHASE 1/2 STUDY OF PATIENTS WITH TGCT ENCOURAGING ANTI-TUMOR ACTIVITY



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Notes: Data presented at the ESMO Congress 2021; results are reported for patients with TGCT with a data cutoff of June 7, 2021; BIW=twice weekly; CR=complete response; ORR=objective response rate; PR=partial response; QD=once daily; SD=stable disease; TGCT=tenosynovial giant cell tumor; (1) After 3-day 20 mg QD loading dose; (2) After 3-day 30 mg QD loading dose; (3) After 5-day 30 mg QD loading dose; (4) No loading dose.

### VIMSELTINIB | ONGOING PHASE 1/2 STUDY OF PATIENTS WITH TGCT DURABLE RESPONSES TO TREATMENT OBSERVED





Notes: Data presented at the ESMO Congress 2021; results are reported for patients with TGCT with a data cutoff of June 7, 2021; BIW=twice weekly; ORR=objective response rate; QD=once daily; TGCT=tenosynovial giant cell tumor; #=1 patient had a local assessment for efficacy, but no central assessment was performed; (1) Median duration of treatment of 10.1 months across all phase 1 dose cohorts; (2) After 5-day 30 mg QD loading dose; (3) After 3-day 30 mg QD loading dose; (4) After 3-day 20 mg QD loading dose; (5) Active patients as of data cutoff of June 7, 2021; (6) No loading dose; (7) Median duration of treatment of 1.9 months in phase 2 cohort A.

### WINSELTINIB | ONGOING PHASE 1/2 STUDY OF PATIENTS WITH TGCT WELL-TOLERATED IN TGCT PATIENTS

### **TEAEs in ≥15% of Patients Receiving Vimseltinib**

	Phase 1				Phase 2	
Preferred term	Cohort 5 (n = 8)		All Patients <sup>1</sup> (n = 32)		Cohort A <sup>1</sup> (n = 36)	
	All grades	Grade 3/4	All grades	Grade 3/4	All grades	Grade 3/4
Blood CPK increased	7 (88%)	4 (50%)	20 (63%)	10 (31%)	19 (53%)	9 (25%)
Periorbital edema	3 (38%)	0	17 (53%)	0	8 (22%)	0
Fatigue	3 (38%)	0	15 (47%)	0	6 (17%)	0
AST increased	5 (63%)	1 (13%)	14 (44%)	4 (13%)	12 (33%)	0
ALT increased	2 (25%)	0	10 (31%)	1 (3%)	4 (11%)	0
Myalgia	0	0	9 (28%)	1 (3%)	5 (14%)	0
Arthralgia	2 (25%)	0	8 (25%)	1 (3%)	2 (6%)	0
Face edema	0	0	8 (25%)	0	0	0
Headache	3 (38%)	0	8 (25%)	0	10 (28%)	0
Lipase increased	1 (13%)	0	8 (25%)	3 (9%)	4 (11%)	0
Peripheral edema	1 (13%)	0	8 (25%)	0	5 (14%)	0
Pruritus	1 (13%)	0	8 (25%)	0	3 (8%)	0
Amylase increased	1 (13%)	1 (13%)	7 (22%)	2 (6%)	5 (14%)	0
Diarrhea	1 (13%)	1 (13%)	6 (19%)	1 (3%)	2 (6%)	0
Generalized edema	2 (25%)	0	6 (19%)	0	0	0
Hypertension	0	0	6 (19%)	2 (6%)	1 (3%)	0
Nausea	2 (25%)	0	6 (19%)	0	8 (22%)	0
Constipation	1 (13%)	0	5 (16%)	0	2 (6%)	0
Parasthesia	0	0	5 (16%)	0	1 (3%)	0
Rash macular	0	0	5 (16%)	0	0	0
Rash maculopapular	0	0	5 (16%)	0	5 (14%)	0
Asthenia	1 (13%)	0	3 (9%)	0	6 (17%)	0



Majority of the common
 (≥15%) TEAEs were
 ≤Grade 2

 Observed transaminase, pancreatic, and CPK enzyme elevations were mostly low grade and not associated with symptoms

No abnormalities in bilirubin levels reported



Notes: Data presented at the ESMO Congress 2021; Results are reported for patients with TGCT with a data cutoff of June 7, 2021; Data are presented as n (%) unless otherwise noted; ALT=alanine aminotransferase; AST=aspartate aminotransferase; CPK=creatine phosphokinase; TEAE=treatment-emergent adverse event; (1) TEAE cutoff of ≥15% based on all grades for total Phase 1 and Phase 2 Cohort A.

### VIMSELTINIB | ONGOING PHASE 1/2 STUDY OF PATIENTS WITH TGCT ENCOURAGING RESULTS SUPPORT FURTHER EVALUATION

- In patients with TGCT not amenable to surgical resection, vimseltinib demonstrated encouraging preliminary efficacy
- Vimseltinib was generally well-tolerated, and the safety profile remains manageable with longerterm follow-up across all Phase 1 dose cohorts and at the recommended Phase 2 dose in Cohort A
- These results support further evaluation of vimseltinib in the MOTION study, a randomized, placebo-controlled, Phase 3 trial in patients with TGCT not amenable to surgical resection





Notes: Data presented at the ESMO Congress 2021; results are reported for patients with TGCT with a data cutoff of June 7, 2021; TGCT= tenosynovial giant cell tumor.

# VIMSELTINIB PHASE 3 MOTION STUDY



## Matthew L. Sherman, M.D.

Executive Vice President and Chief Medical Officer



### VIMSELTINIB MOTION STUDY | PHASE 3 STUDY OF PATIENTS WITH TGCT A MULTICENTER, RANDOMIZED, PLACEBO-CONTROLED, DOUBLE-BLIND STUDY





Notes: BIW=twice weekly; TGCT=tenosynovial giant cell tumor.

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# TGCT MARKET DYNAMICS AND VIMSELTINIB OPPORTUNITIES



## **Daniel C. Martin**

Senior Vice President and Chief Commercial Officer



### TENOSYNOVIAL GIANT CELL TUMOR (TGCT) A SIGNIFICANT OPPORTUNITY EXISTS TO IMPROVE THE LIVES OF TGCT PATIENTS





Notes: PCP=primary care physician; TGCT=tenosynovial giant cell tumor; (1) Mastboom et al. Acta Orthopaedica. 2017;88(6):688-694; (2) Internal Deciphera estimates based on market research and secondary data; estimates are inherently uncertain; (3) Ehrenstein. J Rheumatol. 2017;44(10):1476-1483.

### VIMSELTINIB | TENOSYNOVIAL GIANT CELL TUMOR (TGCT) POTENTIAL BEST-IN-CLASS PROFILE

Products Used In TGCT<sup>1</sup>

# pexidartinib nilotinib sunitinib

## **Existing Product Profiles**

### Imatinib

- Not FDA or EMA approved for TGCT
- Weak CSF1R inhibitor
- Guideline listed based on retrospective data showing 19% ORR<sup>2,3</sup>

### Pexidartinib

- FDA approved for TGCT, not approved in EU
- Inhibitor of CSF1R, KIT, FLT3, and PDGFRA/B
- Boxed warning for hepatoxicity, REMS, intensive liver monitoring

# Vimseltinib Opportunity

### High unmet need

- Lifelong condition with significant morbidity
- HCPs and patients cite desire for highly effective therapy without having to sacrifice safety and tolerability<sup>1</sup>
- No approved therapies ex-US

### **Potential Best-In-Class Profile<sup>4</sup>**

- Highly potent & selective CSF1R inhibitor
- Deep and durable responses
- Limited off-target toxicities with no observed cholestatic hepatoxicity

### Strong strategic fit

- TGCT and GIST are sarcomas with overlapping KOLs and call-points
- Significant operational synergies



Notes: CSF1R=colony-stimulating factor 1 receptor; EMA=European Medicines Agency; FDA=Food and Drug Administration; FLT3=FMS-like tyrosine kinase 3; GIST=Gastrointestinal stromal tumor; HCP=healthcare provider; KIT=KIT prot-oncogene receptor tyrosine kinase; KOL=key opinion leader; PDGFRA/B=platelet derived growth factor A/B; REMS=risk evaluation and mitigation strategy; TGCT=tenosynovial giant cell tumor; (1) Internal Deciphera market research; (2) NCCN Guidelines Version 2.2021 Soft Tissue Sarcoma; (3) Cassier et al Cancer 2012:119:1649-1655; (4) Based on data from phase 1/2 study presented at ESMO Congress 2021 (cut-off date June 7, 2021).

# VIMSELTINIB Q&A

decīphera 3

# CLOSING REMARKS



## **Steve L. Hoerter**

President and Chief Executive Officer



# INNOVATIVE PROGRAMS LEADING TO TRANSFORMATIVE GROWTH

Executing on our mission to discover, develop, and deliver important new medicines to patients for the **treatment of cancer** 



- Phase 3 data in 2<sup>nd</sup> line GIST (INTRIGUE) expected in 4Q 2021
- EU approval in 4<sup>th</sup> line GIST expected in 4Q 2021

### Positioned to Rapidly Advance Clinical Pipeline<sup>1</sup>

**Vimseltinib**, our potential best-in-class CSF1R inhibitor, Phase 3 MOTION study initiation expected in 4Q 2021

**Rebastinib**, our first-in-class TIE2 inhibitor, in combination with paclitaxel Phase 3 study initiation expected in 2022 DCC-3116, our first-in-class ULK program, initial data from the Phase 1 dose escalation expected in 2022



Notes: CSF1R=colony-stimulating factor 1 receptor; EU=European Union; GIST=gastrointestinal stromal tumor; TIE2=TEK tyrosine kinase; ULK=unc-51-like autophagy-activating kinase; (1) Represent planned 2021 and 2022 milestones.

# THANK YOU

