

Stifel Healthcare Conference 2014

geron



John Scarlett, M.D.
Chief Executive Officer
November 19, 2014

forward-looking statements

Except for statements of historical fact, the statements during this presentation and the question and answer session are forward-looking statements under the Safe Harbor provisions of the Private Securities Litigation Reform Act of 1995. These include, without limitation, statements regarding: the closing of and potential payments under the Janssen Collaboration Agreement; the timelines, prospects and plans for imetelstat, including clinical study initiation; the therapeutic potential and safety of imetelstat; and financial or operating projections or requirements. These statements involve risks and uncertainties that can cause actual results to differ materially from those in such forward-looking statements. These risks and uncertainties, include, without limitation: that the Collaboration Agreement will close and Geron will receive \$35 million from Janssen; that imetelstat is safe and efficacious enabling Geron to receive continuation, milestone and royalty payments from Janssen; and operational spending will occur as expected. Additional information and factors that could cause actual results to differ materially from those in the forward-looking statements are contained in Geron's periodic reports filed with the Securities and Exchange Commission under the heading "Risk Factors," including Exhibit 99.1 of Geron's current report on Form 8-K filed on November 13, 2014. Undue reliance should not be placed on forward-looking statements, which speak only as of the date they are made, and the facts and assumptions underlying the forward-looking statements may change. Except as required by law, Geron disclaims any obligation to update these forward-looking statements to reflect future information, events or circumstances.

Geron: well-positioned for future growth

- **Imetelstat: a novel and transformative oncology product candidate**
 - first telomerase inhibitor in clinical development
 - demonstrated disease-modifying activity in ET (ASH 2012) and MF (ASH 2013)
 - mechanism of action suggests broad activity across hematologic malignancies
- **Partnership with Janssen for worldwide imetelstat development and commercialization**
 - Janssen – extensive portfolio of products; deep expertise in hematologic malignancies
 - broad development plan for MF, MDS and AML as primary indications
 - Phase 2 MF study (expected start mid-2015)
 - Phase 2 MDS study (expected start end 2015)
 - joint governance committee oversight
- **Solid financial position**
 - potential collaboration cash flows: development, regulatory and sales milestones up to \$900 million; royalty tier percentage up to low twenties
 - collaboration expected to be self-funding
 - \$35M initial payment expected to cover Geron's 50% share of Phase 2 MF and MDS study costs
 - success-based continuation, milestone and royalty payments expected to cover future cost-sharing obligations
 - cash and investments as of September 30, 2014 is ~\$142.5M; with initial payment projected cash and investments as of December 31, 2014 is ~\$165M
- **Foundation for future growth**
 - imetelstat primed to maximize value creation
 - financial ability to diversify through acquisitions of new products, programs or companies

imetelstat

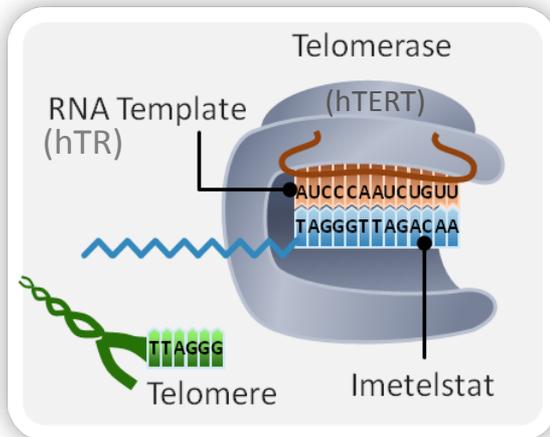
a telomerase inhibitor

Telomerase enzyme:

- adds nucleotide repeats to offset the loss of telomeric DNA occurring with each replication cycle
- not active in somatic cells; transiently upregulated in normal hematopoietic progenitor cells to maintain telomeres and support controlled proliferation
- highly upregulated in malignant progenitor cells, enabling continued and uncontrolled proliferation

Inhibiting telomerase activity as an approach to treating cancer:

- limits proliferative capacity of malignant cells, particularly progenitor cells



imetelstat binds to RNA template
preventing maintenance of telomeres

Imetelstat: a novel first-in-class telomerase inhibitor

- **Proprietary:** 13-mer thio-phosphoramidate oligonucleotide complementary to telomerase RNA template, with covalently-bound lipid tail to increase cell permeability/tissue distribution
- **Long half-life** in bone marrow, spleen, liver: estimated human $t_{1/2}$ = 41 hrs with doses 7.5 – 11.7 mg/kg
- **Potent competitive inhibitor** of telomerase enzyme activity: IC50 = 0.5-10 nM (cell-free)
- **Studied by Geron in 374 patients** as a single agent or in combination with standard therapies (primarily solid tumors)

hematologic myeloid malignancies with high unmet medical need

- arise from malignant progenitor cell clones in bone marrow
- express higher telomerase activity compared to normal cells
- limiting proliferation of malignant progenitors with imetelstat suggests disease-modifying activity

Myelofibrosis (MF)¹

- ~3,000 cases diagnosed per year in the US
- ~13,000 people in the US living with MF
- drug therapies include: ruxolitinib, hydroxyurea
- median survival: ~1-3 years for intermediate-2 or high risk disease (60-70%)

myelodysplastic syndromes (MDS)²

- ~12,000 cases diagnosed per year in the US
- up to ~60,000 people in the US living with MDS
- drug therapies include: hypomethylating agents, IMiDs, ATG, cyclosporine
- median survival: ~1-3 years for intermediate or high risk disease

acute myelogenous leukemia (AML)³

- ~13,000 cases diagnosed per year in the US
- ~37,000 people in the US living with or in remission from AML
- drug therapies include: cytotoxic agents
- poor prognosis following relapse from initial remission
- ~25% of patients diagnosed are alive after 5 years

¹Mehta et al, Leuk Lymphoma 2013, Jul (epub); Gangat et al, J Clin Oncol 2011, 29:392-397

² Sekeres, J Natl Compr Canc Netw 2011; 9:57-63
Malcovati et al, J Clin Oncol 2007; 25:3503-3510

³ NCI SEER database: www.seer.cancer.gov

preliminary efficacy data suggest transformative potential

- **Proof-of-concept study in essential thrombocythemia**

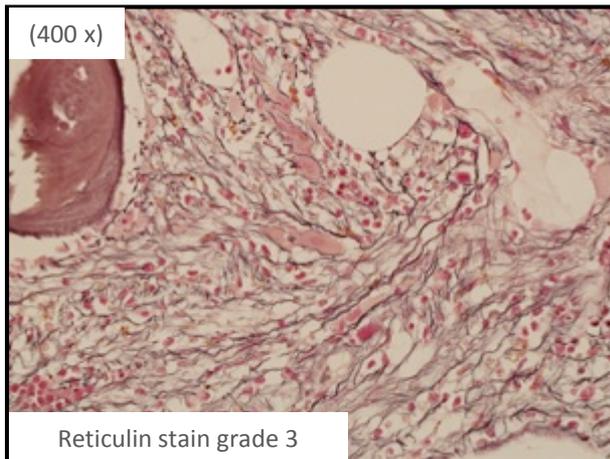
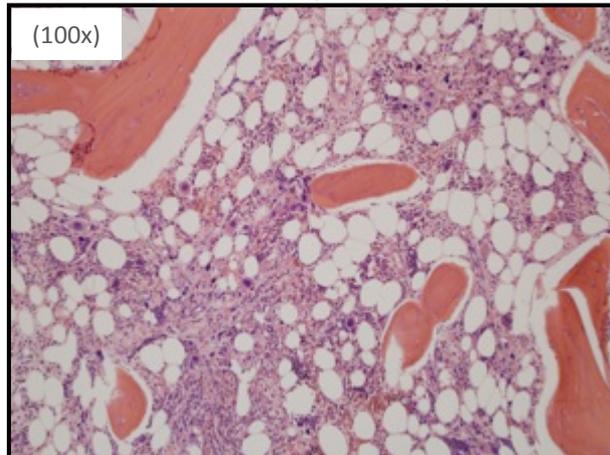
- compelling and durable hematologic responses
- significant and exceptional molecular responses suggested inhibition of malignant progenitor cell clones in relatively selective manner
- mechanism of action suggested broad activity across hematologic malignancies

- **Pilot study in myelofibrosis (MF)**

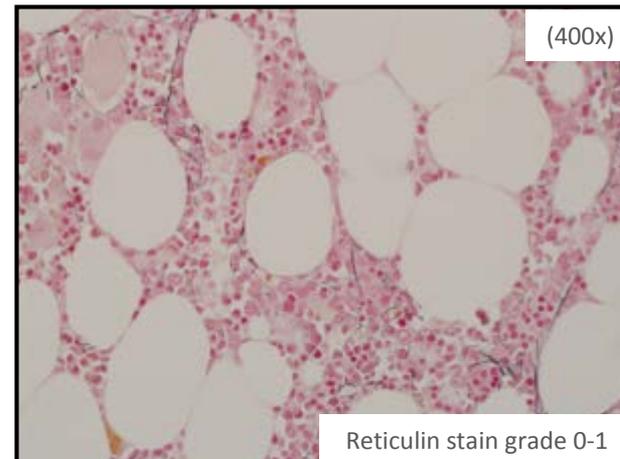
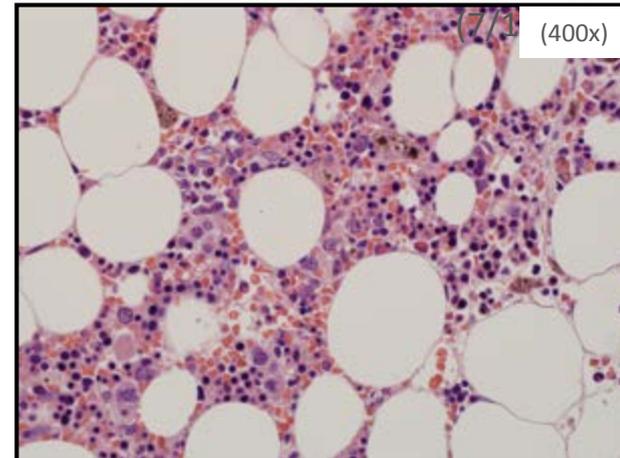
- intermediate-2 and high-risk MF patients (n=33)
- dosing intensity and schedule explored
- primary efficacy endpoints as defined by 2013 IWG criteria
- unprecedented complete and partial remission responses

patient 2 (prior Jak inhibitor): bone marrow complete remission on imetelstat

Baseline (1/28/13)



5.5-months post imetelstat therapy



efficacy data suggest differentiated activity in MF

	Arm A (n = 11)	Arm B (n = 11)	Total (n = 22)
Best Response by 2013 IWG criteria	N (%)	N (%)	N (%)
Overall Response (CR+PR+CI)	3 (27.3%)	6 (54.5%)	9 (40.9%)
Remission (CR+PR)	2 (18.2%)	3 (27.3%)	5 (22.7%)
Complete Remission (CR)	2 (18.2%) ¹	1 (9.1%)	3 (13.6%)
Partial Remission (PR)		2 (18.2%)	2 (9.1%)
Clinical Improvement (CI)	1 (9.1%)	3 (27.3%) ²	4 (18.2%)

Pending 12-week durability assessment (as of Oct 2013):

¹One patient who met the PR criteria on 4/30/2013 and converted to CR on 10/9/2013 (Arm A)

²One patient who met CI-by Liver Response on 10/14/2013 (Arm B)

- Median time to onset of CR or PR was 2.8 months (range 1.4 – 3.0)

Larger data set (n=33) to be updated at oral ASH presentation in December 2014

remissions observed in data suggest disease-modifying activity in MF

all manifestations of disease must be addressed in patients to achieve a remission

Patient number	1	2	3	4	5
Best response per IWG criteria	CR	CR	CR	PR	PR
Normal cellularity and reversal of bone marrow fibrosis	✓	✓	✓	✓	x
Normal peripheral blood counts and smears	✓	✓	✓	x	✓
Anemia response or transfusion independence	✓	✓	—	✓	—
Complete resolution of splenomegaly (by palpation)	✓	✓	✓	—	✓
Complete resolution of symptoms	—	✓	✓	—	✓

remission

clinical improvement

— = disease manifestation not present at baseline

preliminary safety data indicate myelosuppression is dose-limiting toxicity

- **Proof-of-concept study in essential thrombocythemia**

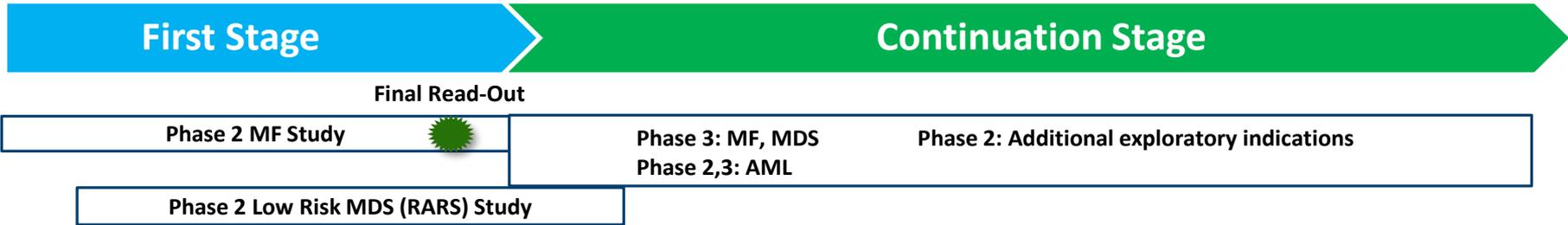
- cytopenias most frequently reported adverse event
- mild-to-moderate non-hematologic adverse events (gastrointestinal events and fatigue)
- persistent low-grade liver function test abnormalities with long-term administration
 - reversibility to normal or baseline after drug discontinuation

- **Pilot study in myelofibrosis**

- cytopenias most frequently reported adverse event
 - managed through dose hold rules and dose modifications
- non-hematologic adverse events (gastrointestinal events and fatigue) not dose-limiting

partnership with Janssen

exclusive worldwide collaboration for imetelstat



- Janssen to execute Phase 2 MF and Phase 2 MDS studies
- Janssen to provide Continuation Decision upon final read-out of Phase 2 MF study

- Geron has Opt-In right to share further US development and promotion costs
- Under Opt-In, Geron may co-promote by providing 20% of US sales force in lieu of paying 20% promotion costs

First Stage Economics	
Cost Share	50% Geron 50% Janssen
Upfront	\$35M

Continuation Stage Economics		
	Opt-In	Opt-Out
Cost Share	20% Geron 80% Janssen	100% Janssen
Continuation/US Rights Fee	\$65M	\$135M
Dev/Reg Milestones	up to \$470M	up to \$415M
Sales Milestones	up to \$350M	up to \$350M
Royalty % Tier *	Mid teens to low twenties	Double digit to mid-teens

* Calculated on worldwide net sales in any countries where regulatory exclusivity exists or there are valid claims under patent rights exclusively licensed to Janssen

upcoming events

- **ASH Annual Meeting (December 2014)**
 - oral presentation on updated Myelofibrosis Pilot Study safety and efficacy data
 - oral presentation on analysis of calreticulin (CALR) allelic burden in ET patients
 - poster presentations on imetelstat mechanism of action in MF and imetelstat efficacy in preclinical AML mouse models
- **MDS-RARS data from Mayo Clinic (presentation expected at 2015 medical conference)**
- **Initiation of Phase 2 MF study (expected start mid-2015)**
- **Initiation of Phase 2 MDS study (expected start end 2015)**

Geron: well-positioned for future growth

- **Imetelstat: a novel and transformative oncology product candidate**
 - first telomerase inhibitor in clinical development
 - demonstrated disease-modifying activity in ET (ASH 2012) and MF (ASH 2013)
 - mechanism of action suggests broad activity across hematologic malignancies
- **Partnership with Janssen for worldwide imetelstat development and commercialization**
 - Janssen – extensive portfolio of products; deep expertise in hematologic malignancies
 - broad development plan for MF, MDS and AML as primary indications
 - Phase 2 MF study (expected start mid-2015)
 - Phase 2 MDS study (expected start end 2015)
 - joint governance committee oversight
- **Solid financial position**
 - potential collaboration cash flows: development, regulatory and sales milestones up to \$900 million; royalty tier percentage up to low twenties
 - collaboration expected to be self-funding
 - \$35M initial payment expected to cover Geron's 50% share of Phase 2 MF and MDS study costs
 - success-based continuation, milestone and royalty payments expected to cover future cost-sharing obligations
 - cash and investments as of September 30, 2014 is ~\$142.5M; with initial payment projected cash and investments as of December 31, 2014 is ~\$165M
- **Foundation for future growth**
 - imetelstat primed to maximize value creation
 - financial ability to diversify through acquisitions of new products, programs or companies

thank you

geron



For further information, please contact:
Kevin Eng, Ph.D., Investor Relations
+1.650.473.7765 or investor@geron.com