

geron

Stifel 2015 Healthcare Conference

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Forward-Looking Statements

Except for the historical information contained herein, this presentation contains forward-looking statements made pursuant to the “safe harbor” provisions of the Private Securities Litigation Reform Act of 1995. Investors are cautioned that statements in this press release regarding: (i) timing of data presentations, including data from the Mayo Clinic MDS-RARS patient cohort; (ii) timing and management of planned and potential clinical trials of imetelstat to be conducted under the collaboration agreement with Janssen, including the current Phase 2 clinical trial in MF and the planned Phase 2/3 clinical trial in MDS, and other potential activities under the collaboration agreement with Janssen; (iii) the safety and efficacy of imetelstat; (iv) the current designs of the Phase 2 clinical trial in MF and planned Phase 2/3 clinical trial in MDS, including planned reviews or analyses of clinical data; (v) the potential receipt by Geron of additional payments up to a potential total of \$900 million for the achievement of development, regulatory and commercial milestones, and royalties from sales of imetelstat; (vi) Geron’s desire to diversify; and (vii) other statements that are not historical facts, constitute forward-looking statements. 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, risks and uncertainties related to: (i) the uncertain, time-consuming and expensive product development and regulatory process, including whether Geron and Janssen will succeed in overcoming all of the clinical safety and efficacy, technical, scientific, manufacturing and regulatory challenges in the development and commercialization of imetelstat; (ii) regulatory authorities permitting the clinical trials to begin or continue to proceed; (iii) Janssen’s ability to enroll patients in any of the planned or potential clinical trials of imetelstat; (iv) the fact that Janssen may terminate the collaboration agreement for any reason; (v) whether imetelstat is safe and efficacious, and whether any future efficacy or safety results may cause the benefit-risk profile of imetelstat to become unacceptable; (vi) the fact that Geron may not receive any milestone, royalty or other payments from Janssen; (vii) the ability of Geron and Janssen to protect and maintain intellectual property rights for imetelstat; (viii) Geron’s dependence on Janssen, including the risks that if Janssen were to breach or terminate the collaboration agreement or otherwise fail to successfully develop and commercialize imetelstat and in a timely manner, Geron would not obtain the anticipated financial and other benefits of the collaboration agreement and the clinical development or commercialization of imetelstat could be delayed or terminated; (ix) whether imetelstat can be applied to any or to multiple hematologic malignancies; and (x) whether Geron is able to acquire any new product candidates, programs or companies to enable it to diversify. Additional information on the above risks and uncertainties and other 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 Geron’s quarterly report on Form 10-Q for the quarter ended September 30, 2015. 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.

Imetelstat: a novel and transformative oncology product candidate

- First telomerase inhibitor in clinical development
- Disease-modifying activity suggested by clinical data in essential thrombocythemia (ET) and myelofibrosis (MF)
- Recent publication of data from both trials in the New England Journal of Medicine (September 2015)
- Initial data in MDS expected at the American Society of Hematology Annual Meeting in December

Strategic partnership with Janssen for worldwide imetelstat development and commercialization

- Potential collaboration cash flows: development, regulatory and sales milestones up to \$900M and royalty tier % up to low twenties
- Broad development plan for imetelstat in MF, MDS and AML as primary indications

Clinical development of imetelstat led by Janssen

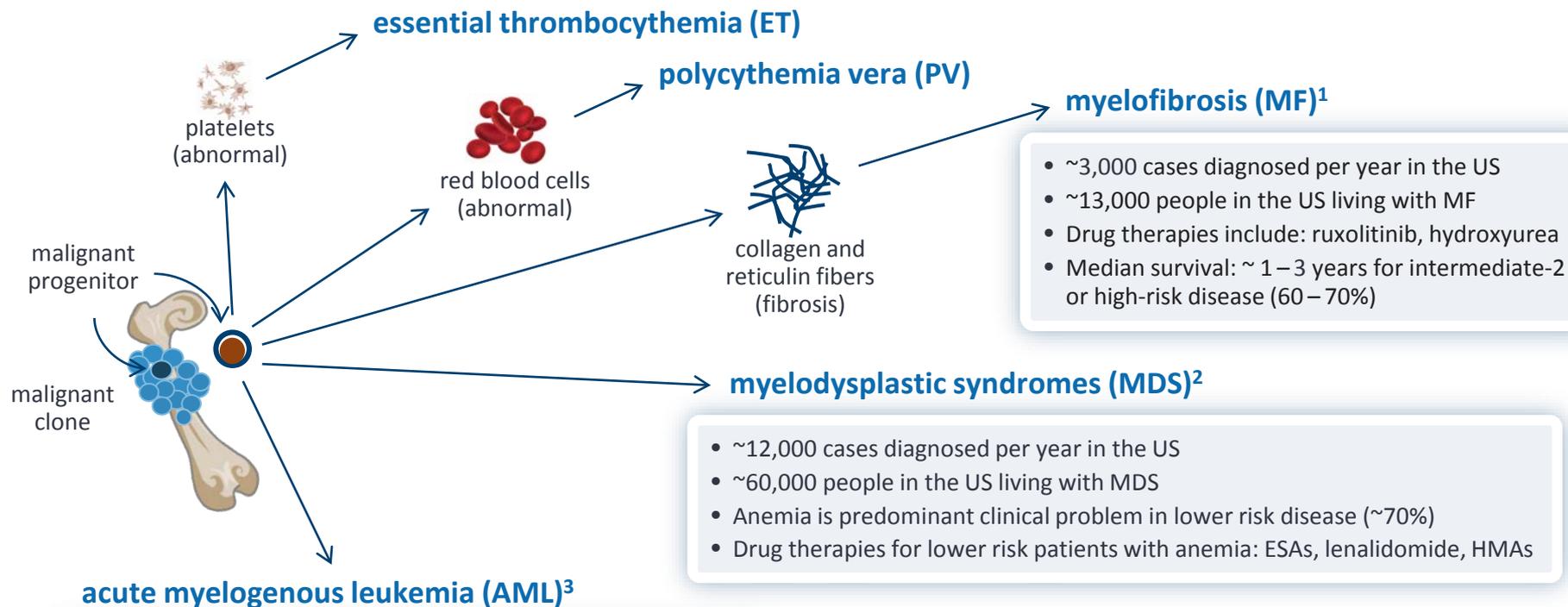
- Phase 2 MF study (first patient dosed in September 2015)
 - ~200 patients (2 dosing regimens) with Intermediate-2 and High risk MF patients who are relapsed after or refractory to JAK inhibitor treatment
- Planned Phase 2/3 MDS study in two parts (expected to open to patient enrollment by the end of 2015)
 - ~200 transfusion dependent patients (Part 1 ~30; Part 2 ~170) with Low and Intermediate-1 risk MDS that have relapsed after or refractory to ESA treatment

Foundation for future growth

- Strong financial position: ~\$151 million in cash and investments as of September 30, 2015
- Imetelstat primed to maximize value creation with collaboration expected to be self-funding
- Seeking to diversify through future acquisitions of new oncology products, programs or companies

Hematologic Malignancies

Arise from Malignant Progenitor Cell Clones in the Bone Marrow



- ~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%)

- ~12,000 cases diagnosed per year in the US
- ~60,000 people in the US living with MDS
- Anemia is predominant clinical problem in lower risk disease (~70%)
- Drug therapies for lower risk patients with anemia: ESAs, lenalidomide, HMAs

- ~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, HMAs
- Poor prognosis following relapse from initial remission
- ~25% of patients diagnosed are alive after 5 years

¹ Mehta 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 Clinical Data Suggest Transformative Potential of Imetelstat

Proof-of-concept study in essential thrombocythemia

- Compelling and durable hematologic and molecular responses
- Molecular responses suggest effect on underlying malignant progenitor cell clones

Pilot study in myelofibrosis

- Unprecedented complete and partial remission responses suggest disease-modifying activity
 - CR or PR: 7/33 (21%) patients
- Durable responses recently reported: median for CR 18 months (range 7 to 20+ as of Dec 5, 2014)
- Spleen responses (by palpation lasting greater than 12 weeks): 34.8% (8/23)
 - Spleen response in JAK inhibitor experienced subgroup: 27.3% (3/11)

Myelosuppression is the dose limiting toxicity observed

- Cytopenias most frequently reported adverse event
 - Managed through dose hold rules and dose modifications
- 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 observed for majority of patients

Baerlocher GM, et al. Telomerase Inhibitor Imetelstat in Patients with Essential Thrombocythemia. N Engl J Med 2015;373:920-928

Tefferi A, et al. A Pilot Study of the Telomerase Inhibitor Imetelstat for Myelofibrosis. N Engl J Med 2015;373:908-919

Tefferi A, et al. ASH 2014 Abstract #634: Imetelstat, a Telomerase Inhibitor, Therapy for Myelofibrosis: A Pilot Study

Partnership with Janssen

Exclusive Worldwide Collaboration for Imetelstat

First Stage

Phase 2 MF study (IMbark™)

Primary analysis

Phase 2/3 MDS study (IMerge™)

- Janssen to execute Phase 2 MF and Phase 2/3 MDS studies
- Janssen to provide Continuation Decision following primary analysis of Phase 2 MF study

First Stage Economics

Cost Share	50% Geron 50% Janssen
Upfront	\$35M

Continuation Stage

Phase 3: MF, MDS
Phase 2,3: AML

Phase 2: Additional exploratory indications

- Geron has Opt-In right to share further US development and promotion costs
- Under Opt-In, Geron may provide 20% of US selling effort with sales force personnel, in lieu of funding 20% of US promotion costs

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

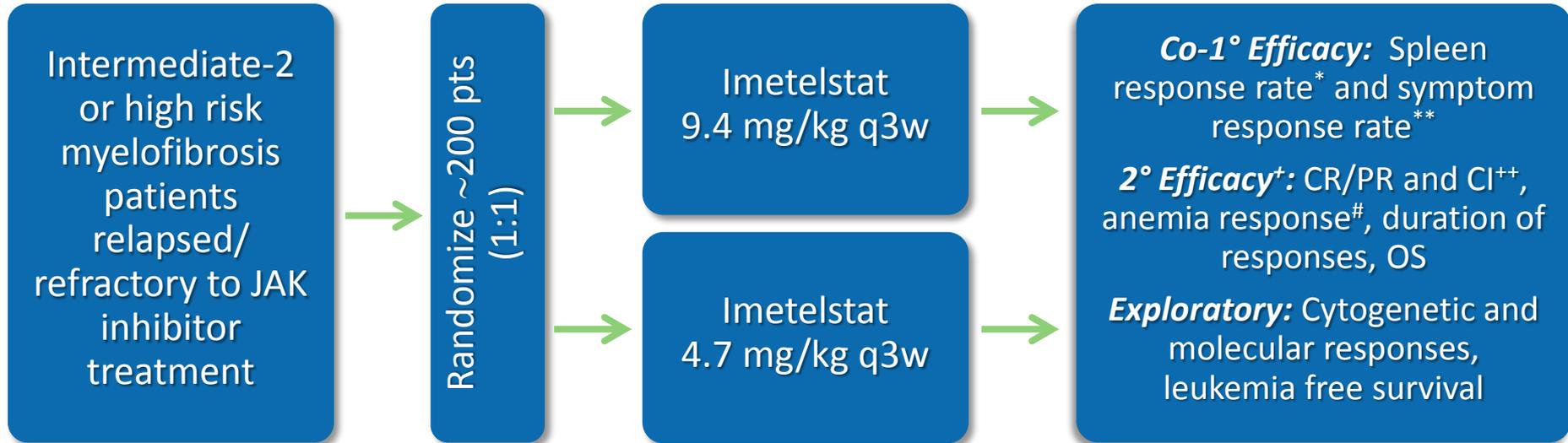
**Calculated on worldwide net sales in any countries where regulatory exclusivity exists or there are valid claims under patent rights exclusively licensed to Janssen

Phase 2 Trial in Myelofibrosis (IMbark™)

An open label, single-blind study being conducted by Janssen Biotech, Inc.



- Multi-center across North America, Europe, and Asia
- Objectives: Define proper dosing and confirm efficacy using current validated regulatory endpoints
- Opened for enrollment in July 2015; first patient dosed in September 2015



* Spleen response rate defined as the percentage of participants who achieve a $\geq 35\%$ reduction in spleen volume at Week 24 from baseline measured by imaging scans.

** Symptom response rate defined as the percentage of participants who achieve $\geq 50\%$ reduction in Total Symptom Score (TSS) at Week 24 from baseline as measured by the modified Myelofibrosis Symptom Assessment Form (MFSAF) version 2.0 diary.

+ Complete list of secondary endpoints can be found on clinicaltrials.gov.

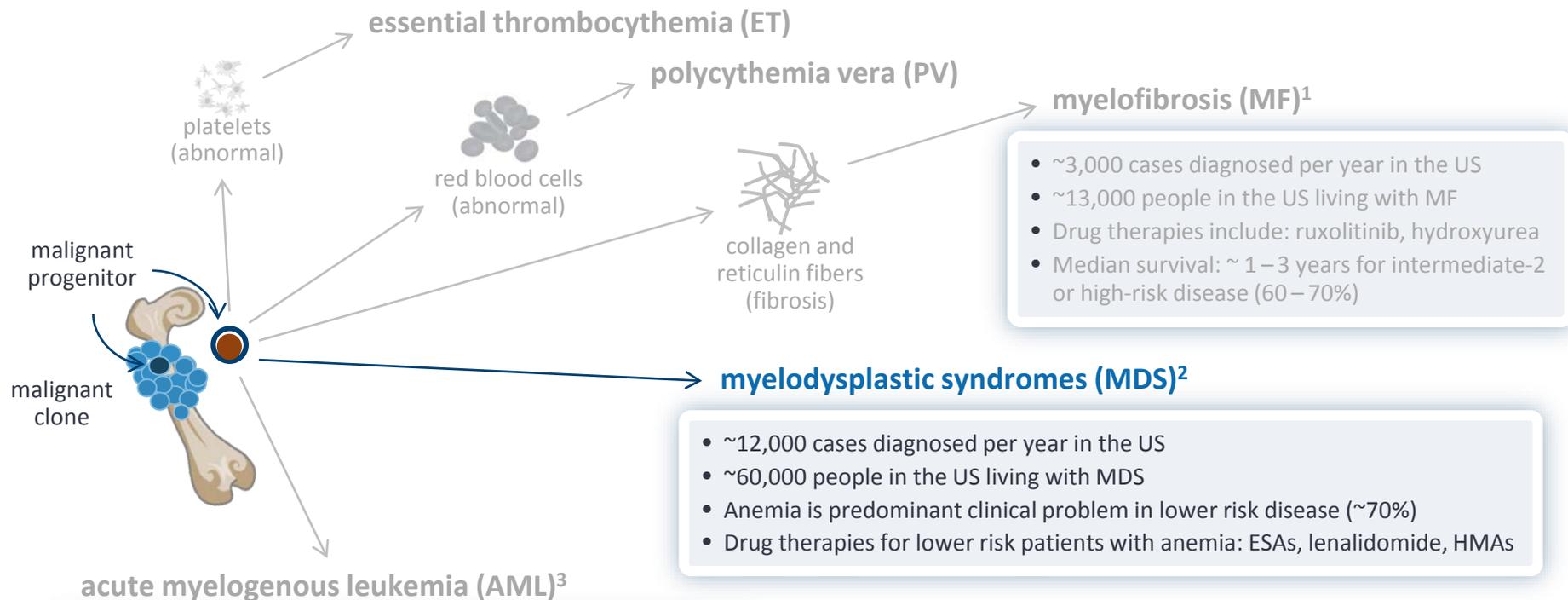
++ Complete remission (CR) or partial remission (PR), and clinical improvement (CI) per modified 2013 IWG-MRT criteria.

Anemia response per 2013 IWG-MRT criteria.

q3w = every 3 weeks; OS = overall survival

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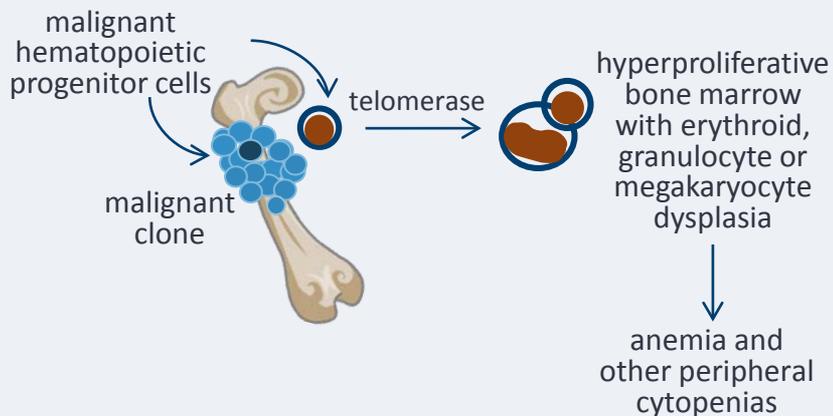
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Myelodysplastic Syndromes (MDS)

Disease Characteristics

Diverse group of clonal hematologic malignancies with disordered and ineffective production of the myeloid lineage in the bone marrow characterized by abnormal cell morphology and counts



Sekeres J, Natl Compr Canc Netw 2011; 9:57-63
Malcovati et al, J Clin Oncol 2007; 25:3503-3510
Greenberg P et al, Blood 1997; 89:2079-2028
Bejar R & Steensma DP, Blood 2014; 124:2793-2803

- Most common myeloid malignancy
 - ~12,000 cases diagnosed per year in the US
 - ~60,000 people in the US living with MDS
- Median age at diagnosis is ~70 years
- Up to 30% of patients progress to acute leukemia (AML)
- Median survival by International Prognostic Scoring System (IPSS) :
 - 3.5 - 5.7 years for lower risk MDS (IPSS low and intermediate-1 risk; ~70%)
 - 0.4 - 1.2 years for higher risk MDS (IPSS intermediate-2 and high risk; ~30%)
- Chronic anemia is predominant clinical problem in lower risk MDS and many patients become dependent on transfusions
- Transfusion dependency may lead to iron overload and is associated with shorter survival (2 units RBC* per month may reduce life expectancy by 50%) and increased risk of transformation to AML

*RBC = red blood cell

Treatments for lower risk MDS patients with anemia are inadequate

- Erythropoiesis stimulating agent (ESA):
 - Patients may experience a transient (median duration of ~2 years) improvement in anemia
 - Patients who do not respond or who relapse within 6 months are predicted to have poor survival (median of ~3 years)
- Lenalidomide:
 - Approved for **del 5q** patients with transfusion-dependent anemia (represents 10-20% of MDS patients)
 - **Non-del 5q** patients resistant to ESA: in a clinical trial ~27% patients achieved RBC transfusion independence (>8 weeks) with a median duration of ~8 months (sNDA filed with PDUFA scheduled for April 2016)

No new drug approved for MDS in the US in almost a decade

- As in MF, MDS is a disease driven by highly proliferative malignant progenitor cells in the bone marrow with high telomerase activity

Initial Testing of Imetelstat Provides Clinical Rationale in MDS

Preliminary data from Mayo Clinic Pilot Study to be presented at the ASH Annual Meeting in December 2015

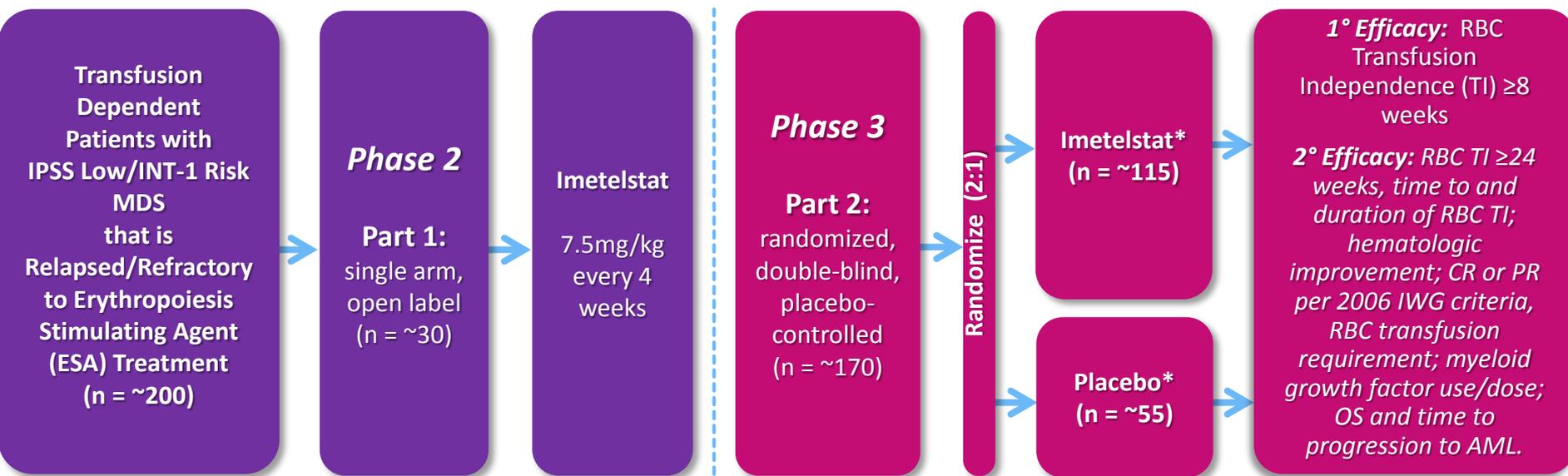
- **November 2015 ASH abstract presents data from a May 10, 2015 cut-off**
- **9 patients with MDS-RARS (refractory anemia with ring sideroblasts)**
 - IPSS risk classification: intermediate-1 (n=7); intermediate-2 (n=2)
 - all patients were anemic (hemoglobin <10 g/dL)
 - 8 of 9 patients were RBC transfusion dependent per 2006 IWG criteria for MDS
 - 7 of 9 patients had received prior treatments, including 6 with ESAs
- **Imetelstat administered at starting dose of 7.5 mg/kg every 4 weeks**
 - median duration of treatment with imetelstat was 13.7 months (range 6.6 - 17.9 months)
- **3 of the 8 (38%) transfusion dependent patients became transfusion independent**
 - defined as not requiring transfusions for at least 8 weeks
 - median duration was 28 weeks (range of 9 – 37 weeks)
- **Safety data are consistent with prior imetelstat studies**
- **Additional and updated data expected during the ASH presentation**

Phase 2/3 Trial in MDS (IMerge™)

A two part, global, multi-center study to be conducted by Janssen Biotech, Inc.



- Objectives: Part 1 to evaluate safety and efficacy of imetelstat to advance to Part 2; Part 2 to compare imetelstat to placebo using a regulatory validated endpoint
- Part 2 enabled based on Janssen's assessment of a satisfactory benefit-risk profile
- Expected to open for enrollment in 2015



INT-1 = intermediate-1 risk

*dosing as in Part 1

Also endpoints as in Part 1

Supportive care permitted in both arms: RBC transfusions, myeloid growth factors per investigator discretion as clinically needed and local guidelines

Rationale for Study Design



Patient Population

Targets significant unmet medical need population

- Chronic anemia remains clinical problem in lower risk MDS
- No approved alternative therapies upon resistance or relapse to ESAs*

Endpoints

Primary endpoint reflects current validated regulatory pathway

- Transfusion independence can reduce potential for iron overload

Secondary endpoints capture depth of responses

- For potential differentiation of imetelstat efficacy compared to current therapies

Dosing Regimen

Same regimen as used in Mayo Clinic Pilot Study MDS-RARS cohort

- Dosing adjustments allowed in the study

*Except lenalidomide in the del 5q patients with transfusion-dependent anemia

Upcoming Events

ASH Annual Meeting in December 2015

- **Oral presentations:**

- Telomerase Inhibitor Imetelstat Therapy in Refractory Anemia with Ring Sideroblasts with or without Thrombocytosis (Abstract #55)
- Dynamics of Mutations in Patients with ET Treated with Imetelstat (Abstract #57)
- Session: Myeloproliferative Syndromes: Clinical: Early and Late Stage Trials in MPN, on Saturday, December 5, 2015 between 9:30 and 11 am Eastern Time

- **Poster presentation:**

- Activity of the Telomerase Inhibitor GRN163L (Imetelstat) on Acute Myeloblastic Leukemia Blasts Is Enhanced by DNA Methyltransferase Inhibitors Irrespective of TERT Promoter Methylation Status (Abstract #1267)

- **Webcast of analyst and investor event:**

- December 5, 7:30 pm ET

Initiation of IMerge™ Phase 2/3 MDS study to be conducted by Janssen

- Anticipated to be open to patient enrollment by the end of 2015

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Thank you